UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the quarterly period ended September 30, 2023

or

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the transition period from___ to
Commission File Number: 001-40315

Reneo Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**47-2309515**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
18575 Jamboree Road, Suite 275-S, Irvine, CA	**92612**
(Address of Principal Executive Offices)	**(Zip Code)**

(Registrant's telephone number, including area code): (858) 283-0280

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading symbol**	**Name of each exchange on which registered**
Common Stock, par value $0.0001 per share	**RPHM**	**The Nasdaq Stock Market LLC**

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
		Smaller reporting company	☒
Non-accelerated filer	☒	Emerging growth company	☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of November 9, 2023, there were 33,311,787 shares of the registrant's common stock, $0.0001 par value per share, outstanding.

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PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

RENEO PHARMACEUTICALS, INC.

Consolidated Balance Sheets
(In thousands, except share and par value data)

	September 30, 2023	December 31, 2022
	(Unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	$ 11,737	$ 19,927
Short-term investments	113,877	81,246
Prepaid expenses and other current assets	3,158	5,180
Total current assets	128,772	106,353
Property and equipment, net	529	453
Right-of-use assets	1,006	1,292
Other non-current assets	81	84
Total assets	$ 130,388	$ 108,182
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 2,473	$ 1,893
Accrued expenses	10,765	4,827
Operating lease liabilities, current portion	325	404
Total current liabilities	13,563	7,124
Operating lease liabilities, less current portion	812	1,059
Performance award	1,069	29
Total liabilities	15,444	8,212
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.0001 par value; 200,000,000 shares authorized at September 30, 2023 and December 31, 2022; 33,872,166 and 24,699,553 shares issued and outstanding at September 30, 2023 and December 31, 2022, respectively	3	3
Additional paid-in capital	305,479	236,693
Accumulated deficit	(190,517)	(136,683)
Accumulated other comprehensive loss	(21)	(43)
Total stockholders' equity	114,944	99,970
Total liabilities and stockholders' equity	$ 130,388	$ 108,182

The accompanying notes are an integral part of these consolidated financial statements.

RENEO PHARMACEUTICALS, INC.

Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except share and per share data)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Operating expenses:				
Research and development	$ 13,622	$ 9,938	$ 39,009	$ 27,348
General and administrative	7,266	3,902	19,038	11,938
Total operating expenses	20,888	13,840	58,047	39,286
Loss from operations	(20,888)	(13,840)	(58,047)	(39,286)
Other income	1,692	833	4,213	931
Net loss	(19,196)	(13,007)	(53,834)	(38,355)
Unrealized gain (loss) on short-term investments	10	(194)	22	(60)
Comprehensive loss	$ (19,186)	$ (13,201)	$ (53,812)	$ (38,415)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.57)	$ (0.53)	$ (1.81)	$ (1.57)
Weighted-average shares used in computing net loss per share, basic and diluted	33,807,945	24,496,313	29,718,689	24,472,974

The accompanying notes are an integral part of these consolidated financial statements.

RENEO PHARMACEUTICALS, INC.

Consolidated Statements of Changes in Stockholders' Equity
(In thousands, except share data)
(Unaudited)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances, December 31, 2022	**24,699,553**	**$ 3**	**$ 236,693**	**$ (43)**	**$ (136,683)**	**$ 99,970**
Stock based compensation	—	—	1,157	—	—	1,157
Issuance of common stock in connection with at-the-market facility, net of issuance costs	407,877	—	1,009	—	—	1,009
Other comprehensive income	—	—	—	55	—	55
Net loss	—	—	—	—	(15,107)	(15,107)
Balances, March 31, 2023	**25,107,430**	**$ 3**	**$ 238,859**	**$ 12**	**$ (151,790)**	**$ 87,084**
Issuance of common stock in public offering, net of offering costs	7,906,250	—	58,862	—	—	58,862
Issuance of common stock in private placement, net of offering costs	625,000	—	4,667	—	—	4,667
Stock based compensation	—	—	1,207	—	—	1,207
Issuance of common stock in connection with equity plans	162,108	—	282	—	—	282
Other comprehensive loss	—	—	—	(43)		(43)
Net loss	—	—	—	—	(19,531)	(19,531)
Balances, June 30, 2023	**33,800,788**	**$ 3**	**$ 303,877**	**$ (31)**	**$ (171,321)**	**$ 132,528**
Stock based compensation	—	—	1,429	—	—	1,429
Issuance of common stock in connection with equity plans	71,378	—	173	—	—	173
Other comprehensive income	—	—	—	10	—	10
Net loss	—	—	—	—	(19,196)	(19,196)
Balances, September 30, 2023	**33,872,166**	**$ 3**	**$ 305,479**	**$ (21)**	**$ (190,517)**	**$ 114,944**

RENEO PHARMACEUTICALS, INC.

Consolidated Statements of Changes in Stockholders' Equity (Continued)
(In thousands, except share data)
(Unaudited)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances, December 31, 2021	**24,455,390**	**$ 3**	**$ 231,902**	**$ 34**	**$ (84,728)**	**$ 147,211**
Stock based compensation	—	—	1,107	—	—	1,107
Issuance of common stock in connection with equity plans	3,160	—	6	—	—	6
Other comprehensive income	—	—	—	30	—	30
Net loss	—	—	—	—	(13,036)	(13,036)
Balances, March 31, 2022	**24,458,550**	**$ 3**	**$ 233,015**	**$ 64**	**$ (97,764)**	**$ 135,318**
Stock based compensation	—	—	1,006	—	—	1,006
Issuance of common stock in connection with equity plans	21,096	—	41	—	—	41
Other comprehensive income	—	—	—	104	—	104
Net loss	—	—	—	—	(12,312)	(12,312)
Balances, June 30, 2022	**24,479,646**	**$ 3**	**$ 234,062**	**$ 168**	**$ (110,076)**	**$ 124,157**
Stock based compensation	—	—	990	—	—	990
Issuance of common stock in connection with equity plans	50,000	—	99	—	—	99
Other comprehensive loss	—	—	—	(194)	—	(194)
Net loss	—	—	—	—	(13,007)	(13,007)
Balances, September 30, 2022	**24,529,646**	**$ 3**	**$ 235,151**	**$ (26)**	**$ (123,083)**	**$ 112,045**

The accompanying notes are an integral part of these consolidated financial statements.

RENEO PHARMACEUTICALS, INC.

Consolidated Statements of Cash Flows
(In thousands)
(Unaudited)

	Nine Months Ended September 30,	
	2023	**2022**
Cash flows from operating activities		
Net loss	$ (53,834)	$ (38,355)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	3,793	3,103
Depreciation and amortization	126	63
Amortization/accretion on short-term investments	(3,551)	(169)
Changes in the fair value of performance award	1,040	(378)
Non-cash lease expense	354	338
Loss on disposal of fixed asset	5	3
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	2,025	1,146
Accounts payable and accrued expenses	6,507	2,838
Operating lease liabilities	(394)	(338)
Net cash used in operating activities	(43,929)	(31,749)
Cash flows from investing activities		
Purchases of property and equipment	(196)	(96)
Purchase of available-for-sale short-term investments	(190,058)	(67,329)
Proceeds from maturities of available-for-sale short-term investments	161,000	36,500
Net cash used in by investing activities	(29,254)	(30,925)
Cash flows from financing activities		
Proceeds from public offering of common stock, net of offering costs	58,862	—
Proceeds from private placement of common stock, net of offering costs	4,667	—
Proceeds from issuance of common stock under the at-the-market facility, net of offering costs	1,009	—
Proceeds from issuance of common stock in connection with equity plans	455	146
Net cash provided by financing activities	64,993	146
Net decrease in cash and cash equivalents	(8,190)	(62,528)
Cash and cash equivalents, beginning of period	19,927	124,660
Cash and cash equivalents, end of period	$ 11,737	$ 62,132
Noncash operating activities:		
Right-of-use assets obtained in exchange for lease obligations	$ —	$ 1,524
Noncash investing and financing activities:		
Property and equipment in accounts payable	$ 11	$ —

The accompanying notes are an integral part of these consolidated financial statements.

1. Organization and Business

Organization

Reneo Pharmaceuticals, Inc. (Reneo or the Company) commenced operations on September 22, 2014 as a clinical-stage pharmaceutical company focused on the development of therapies for patients with rare genetic mitochondrial diseases. In December 2017, the Company in-licensed mavodelpar (REN001), a novel oral peroxisome proliferator-activated receptor delta (PPARδ) agonist.

Liquidity

From its inception in 2014, the Company has incurred significant losses and negative cash flows from operations. For the three and nine months ended September 30, 2023, the Company had a net loss of $19.2 million and $53.8 million, respectively, and used $43.9 million of cash in operating activities during the nine months ended September 30, 2023. As of September 30, 2023, the Company had an accumulated deficit of $190.5 million.

The Company follows Accounting Standards Codification (ASC) Topic 205-40, *Presentation of Financial Statements—Going Concern*, which requires that management perform a two-step analysis over its ability to continue as a going concern. Management must first evaluate whether there are conditions and events that raise substantial doubt about the Company's ability to continue as a going concern and to meet its obligations as they become due within one year after the date that the consolidated financial statements are issued (step 1). If management concludes that substantial doubt is raised, management is also required to consider whether its plans alleviate that doubt (step 2).

As of September 30, 2023, the Company had cash, cash equivalents and short-term investments of $125.6 million, which the Company expects are sufficient to fund operations through the potential submission of a new drug application for mavodelpar for the treatment of primary mitochondrial myopathies in the United States anticipated in the first half of 2024, however, based on the anticipated increase in commercial development expenses for mavodelpar, not for more than one year from the date of the filing of this Quarterly Report on Form 10-Q. Accordingly, management has concluded that substantial doubt exists about the Company's ability to continue as a going concern. Management plans to alleviate this risk by raising additional capital through public or private equity offerings or debt financings, credit or loan facilities, collaborations, strategic alliances, licensing arrangements or a combination of one or more of these funding sources. In addition, the Company has the ability to defer certain commercial development activities until additional capital is received.

There can be no assurance that the Company will be successful in obtaining additional funding, that the Company's projections of its future working capital needs will prove accurate, or that any additional funding would be sufficient to continue operations in future years. If the Company is not able to secure adequate additional funding, the Company may be forced to make reductions in spending, extend payment terms with suppliers, liquidate assets where possible, and/or suspend or curtail planned programs. Any of these actions could negatively impact the Company's business, results of operations, and future prospects. The Company's ability to raise additional capital may be adversely impacted by potential worsening global economic conditions, disruptions to, and volatility in, financial markets in the United States and worldwide, including those resulting from armed conflicts, infectious diseases, bank failures, actual or perceived changes in interest rates and economic inflation. The Company may not be able to secure additional financing in a timely manner or on favorable terms, if at all. In addition, successful transition to attaining profitable operations is dependent upon achieving a level of revenues adequate to support the Company's cost structure.

2. Summary of Significant Accounting Policies

Basis of Presentation and Consolidation

The Company has prepared the accompanying unaudited consolidated financial statements pursuant to the rules and regulations of the Securities and Exchange Commission. Certain information and note disclosures normally included in annual financial statements prepared in accordance with accounting principles generally accepted in the United States of America (GAAP) have been condensed or omitted, although the Company believes that the disclosures made are adequate to make the information not misleading. The Company recommends that the unaudited consolidated financial statements be read in conjunction with the audited consolidated financial statements and the notes thereto included in the Company's Annual Report on Form 10-K for the year ended December 31, 2022.

In the opinion of management, all adjustments, including normal recurring adjustments, considered necessary for a fair statement of the financial statements, have been included in the accompanying unaudited financial statements. Interim results are not necessarily indicative of results that may be expected for any other interim period or for an entire year.

The accompanying unaudited consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries as of September 30, 2023; all intercompany transactions and balances have been eliminated.

Summary of Significant Accounting Policies

The significant accounting policies used in the preparation of these consolidated financial statements for the nine months ended September 30, 2023 are consistent with those discussed in Note 2 to the consolidated financial statements in the Company's Annual Report on Form 10-K for the year ended December 31, 2022.

New Accounting Pronouncements

Recently Adopted Accounting Pronouncements

In June 2016, the Financial Accounting Standard Board (FASB) issued Accounting Standard Update (ASU) 2016-13, *Financial Instruments - Credit Losses (ASC 326), Measurement of Credit Losses on Financial Instruments*. The standard amends the impairment model by requiring entities to use a forward-looking approach based on expected losses to estimate credit losses for most financial assets and certain other instruments that are not measured at fair value through net income. For available-for-sale debt securities, entities will be required to recognize an allowance for credit losses rather than a reduction in the carrying value of the asset. Entities will no longer be permitted to consider the length of time that fair value has been less than amortized cost when evaluating when credit losses should be recognized. This new guidance was effective for the Company as of January 1, 2023. The Company adopted the guidance as of January 1, 2023, with no material impact on its financial statements and related disclosures.

Recent Accounting Pronouncements Not Yet Adopted

In June 2022, the FASB issued ASU 2022-03, *Fair Value Measurement (ASC 820): Fair Value Measurement of Equity Securities Subject to Contractual Sale Restrictions*. The ASU clarifies that (1) a contractual restriction on the sale of an equity security is not considered part of the unit of account of the equity security and, therefore, is not considered in measuring fair value, (2) an entity cannot, as a separate unit of account, recognize and measure a contractual sale restriction, and (3) new disclosure requirements for equity securities subject to contractual sale restrictions that are measured at fair value in accordance with ASC 820. The new guidance is effective for the Company for fiscal years beginning after December 15, 2023. The Company is currently evaluating the impact of the adoption of this standard on its consolidated financial statements.

3. Net Loss Per Share

The Company computes basic loss per share by dividing the net loss attributable to common stockholders by the weighted average number of common shares outstanding for the period, without consideration for common stock equivalents. Diluted net loss per share assumes the conversion, exercise or issuance of all potential common stock equivalents, unless the effect of inclusion would be anti-dilutive. For purposes of this calculation, common stock shares to be issued upon exercise of all outstanding stock options and restricted stock units were excluded from the diluted net loss per share calculation for the three and nine months ended September 30, 2023 and 2022 because such shares are anti-dilutive.

Historical outstanding anti-dilutive securities not included in the diluted net loss per share calculation include the following:

	As of September 30,	
	2023	**2022**
Common stock options outstanding	6,095,807	4,470,120
Unvested restricted stock units	441,500	309,500
Total	6,537,307	4,779,620

4. Fair Value Measurements

ASC Topic 820, *Fair Value Measurement*, establishes a fair value hierarchy for instruments measured at fair value that distinguishes between assumptions based on market data (observable inputs) and the Company's own assumptions (unobservable inputs). Observable inputs are inputs that market participants would use in pricing an asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability and are developed based on the best information available in the circumstances.

ASC Topic 820 identifies fair value as the exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As a basis for considering market participant assumptions in fair value measurements, ASC Topic 820 establishes a three-tier fair value hierarchy that distinguishes between the following:

- Level 1 – Observable inputs such as quoted prices in active markets for identical assets or liabilities.
- Level 2 – Inputs, other than quoted prices in active markets, which are observable for the asset or liability, either directly or indirectly.
- Level 3 – Unobservable inputs in which there is little or no market data, which requires the Company to develop its own assumptions.

Assets and liabilities measured at fair value are classified in their entirety based on the lowest level of input that is significant to the fair value measurement. The Company's assessment of the significance of a particular input to the fair value measurement in its entirety requires management to make judgments and consider factors specific to the asset or liability. The Company's financial assets are subject to fair value measurements on a recurring basis.

The Company categorizes its money market funds as Level 1, using the quoted prices in active markets. Commercial paper and U.S. treasury securities are categorized as Level 2, using significant other observable inputs. The fair value of the Company's investments in certain money market funds is their face value and such instruments are classified as Level 1 and are included in cash and cash equivalents on the consolidated balance sheets.

In connection with the Company's chief executive officer's (CEO) employment agreement, he is entitled to receive a special performance bonus in the amount of $7.5 million (Performance Award), payable in cash, common stock or a combination of cash and common stock, at the election of the Company, based on achievement of certain conditions as described in more detail in Note 9. The Company estimated the fair value of the Performance Award using a Monte Carlo simulation, which incorporates the stock price at the date of the valuation and utilizes Level 3 inputs such as volatility, probabilities of success, and other inputs that are not observable in active markets. The Performance Award is required to be measured at fair value on a recurring basis each reporting period, with changes in the fair value recognized in general and administrative expense in the consolidated statements of operations and comprehensive loss over the derived service period of the award.

No assets or liabilities were transferred into or out of their classifications during the nine months ended September 30, 2023.

The recurring fair value measurement of the Company's assets and liabilities measured at fair value at September 30, 2023 consisted of the following (in thousands):

	Quoted Prices in Active Markets For Identical Items (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets				
Cash and cash equivalents:				
Money market investments	$ 4,946	$ —	$ —	$ 4,946
Short-term investments:				
U.S. treasury securities	—	113,877	—	113,877
Total	$ 4,946	$ 113,877	$ —	$ 118,823
Liabilities				
Performance award	$ —	$ —	$ 1,069	$ 1,069
Total	$ —	$ —	$ 1,069	$ 1,069

The recurring fair value measurement of the Company's assets and liabilities measured at fair value at December 31, 2022 consisted of the following (in thousands):

	Quoted Prices in Active Markets For Identical Items (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets				
Cash and cash equivalents:				
Money market investments	$ 9,365	$ —	$ —	$ 9,365
Commercial paper	—	4,978	—	4,978
Short-term investments:				
U.S. treasury securities	—	76,253	—	76,253
Commercial paper	—	4,993	—	4,993
Total	$ 9,365	$ 86,224	$ —	$ 95,589
Liabilities				
Performance award	$ —	$ —	$ 29	$ 29
Total	$ —	$ —	$ 29	$ 29

The following table summarizes changes in fair value measurements of the Performance Award during the nine months ended September 30, 2023 (in thousands):

	Performance Award
Balance as of January 1, 2023	$ 29
Change in fair value	1,040
Balance as of September 30, 2023	$ 1,069

5. Marketable Debt Securities

The Company's investments in debt securities are carried at fair value and classified as current assets available-for-sale as they mature within 12 months and represent the investment of funds available for current operations. Unrealized gains and losses on available-for-sale debt securities are included in other comprehensive income or loss, and charged to income or expense in the period when realized. The following tables summarize the gross unrealized gains and losses of the Company's available-for-sale securities (in thousands):

	As of September 30, 2023			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
Available-for-sale securities:				
U.S. treasury securities	$ 113,898	$ 1	$ (22)	$ 113,877
Total	$ 113,898	$ 1	$ (22)	$ 113,877

	As of December 31, 2022			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
Available-for-sale securities:				
U.S. treasury securities	$ 76,297	$ 2	$ (46)	$ 76,253
Commercial paper	4,993	—	—	4,993
Total	$ 81,290	$ 2	$ (46)	$ 81,246

6. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	As of September 30, 2023	As of December 31, 2022
Accrued clinical and regulatory	$ 5,176	$ 1,872
Accrued contract manufacturing cost	1,005	1,583
Accrued compensation	3,830	807
Accrued other	754	565
Total accrued expenses	$ 10,765	$ 4,827

7. Leases

The Company's headquarters are located in Irvine, California, where it leases office space. The Company leases additional office space located in Sandwich, United Kingdom. The lease terms for the Irvine and Sandwich offices extend through November 30, 2026 and October 23, 2027, respectively.

Other information related to the Company's operating leases as of the balance sheet dates presented are as follows:

	As of September 30,	
	2023	2022
Weighted incremental borrowing rate	5%	5%
Weighted average remaining lease term (in years)	3.4	3.8
Cash paid for amounts included in the measurement of lease liabilities (in thousands)	$ 430	$ 397
Lease expense (in thousands)	$ 354	$ 338

Maturities of lease liabilities by fiscal year for the Company's operating leases are as follows (in thousands):

	As of September 30, 2023
2023 (remaining three months)	$ 94
2024	381
2025	381
2026	343
2027	34
Total lease payments	1,233
Less: Imputed interest	(96)
Present value of lease liabilities	$ 1,137

8. Stockholders' Equity

On May 8, 2023, the Company completed a public offering in which it sold an aggregate of 7,906,250 shares of common stock, which included the full exercise of the underwriters' option to purchase an additional 1,031,250 shares of common stock, at a price of $8.00 per share. The Company received total net proceeds from the offering of approximately $58.9 million, after deducting underwriting discounts and commissions and offering expenses.

On May 9, 2023, the Company completed a concurrent private placement in which it sold an aggregate of 625,000 shares of common stock to Abingworth Bioventures 8 L.P., a holder of more than 5% of the Company's common stock, at a price of $8.00 per share. The Company received total net proceeds of approximately $4.7 million, after deducting advisor fees and other estimated fees and expenses.

9. Stock-Based Compensation

In March 2021, the Company's board of directors adopted the Company's 2021 Equity Incentive Plan (2021 Plan), which is the successor to the Company's 2014 Equity Incentive Plan (2014 Plan). As of the effective date of the 2021 Plan, awards granted under the 2014 Plan that are forfeited or otherwise become available under the 2014 Plan will be included and available for issuance under the 2021 Plan. Under the 2021 Plan, the Company may grant stock options, stock appreciation rights, restricted stock awards, restricted stock units, and other awards to individuals who are employees, officers, directors or consultants of the Company and its affiliates.

Under the 2014 Plan, certain employees were granted the ability to early exercise their options. The shares of common stock issued pursuant to the early exercise of unvested stock options are restricted and continue to vest over the requisite service period after issuance. The Company has the option to repurchase any unvested shares at the original purchase price upon any voluntary or involuntary termination. The shares purchased by the employees pursuant to the early exercise of stock options are not deemed, for accounting purposes, to be outstanding until those shares vest. As of September 30, 2023, there were no unvested shares of common stock outstanding that were issued pursuant to the early exercise of stock options.

Shares Reserved for Future Issuance

As of September 30, 2023, the Company had reserved shares of its common stock for future issuance as follows:

	Shares Reserved
Common stock options outstanding	6,095,807
Unvested restricted stock units	441,500
Available for future grants under the 2021 Equity Incentive Plan	1,300,869
Available for future grants under the 2021 Employee Stock Purchase Plan	546,341
Total shares of common stock reserved	8,384,517

Stock Options

A summary of the Company's stock option activity and related information during the nine months ended September 30, 2023 is as follows:

	Options Outstanding	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2022	5,877,745	$ 4.47	8.2	$ 907
Granted	548,915	$ 6.88		
Exercised	(149,190)	$ 2.15		
Forfeited/Expired	(181,663)	$ 5.60		
Outstanding at September 30, 2023	6,095,807	$ 4.71	7.8	$ 19,172
Vested at September 30, 2023	2,946,079	$ 4.94	7.0	$ 8,770
Exercisable at September 30, 2023	3,416,089	$ 4.52	6.8	$ 10,045

Options exercisable at September 30, 2023 include vested options and options eligible for early exercise. All outstanding options as of September 30, 2023 are expected to vest.

Unrecognized stock-based compensation expense at September 30, 2023 was $9.5 million, which is expected to be recognized over a weighted-average vesting term of 2.4 years.

The weighted-average assumptions used in the Black-Scholes option pricing model to determine the fair value of the employee stock option grants were as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Risk-free interest rate	4.3%	3.1%	4.0%	2.5%
Expected volatility	91.2%	93.6%	88.4%	87.2%
Expected term (in years)	6.1	6.0	5.9	5.9
Expected dividend yield	— %	— %	— %	— %

Risk-free interest rate. The Company bases the risk-free interest rate assumption on the U.S. Treasury's rates for U.S. Treasury zero-coupon bonds with maturities similar to those of the expected term of the award being valued.

Expected volatility. Since the Company does not have sufficient trading history for its common stock the expected volatility assumption is based on a blend of volatilities of the Company's share price and a peer group of similar companies whose share prices are publicly available. For awards granted prior to April 2023, the expected volatility assumption was based on the volatilities of a peer group of similar companies whose share prices are publicly available. The peer group was developed based on companies in the biotechnology industry.

Expected term. The expected term represents the period of time that options are expected to be outstanding. Because the Company does not have historical exercise behavior, it determines the expected life assumption using the simplified method, which is an average of the contractual term of the option and its vesting period.

Expected dividend yield. The Company bases the expected dividend yield assumption on the fact that it has never paid cash dividends and has no present intention to pay cash dividends.

Restricted Stock Units (RSUs)

RSUs consist of time-based units (TSUs), performance-based units (PSUs) and market-based units (MSUs). The following table summarizes RSU activity during the nine months ended September 30, 2023:

	Number of RSUs	Weighted-Average Grant Date Fair Value
Unvested at December 31, 2022	329,500	$ 5.70
Granted	117,000	$ 5.72
Cancelled	(5,000)	$ 2.14
Unvested at September 30, 2023	441,500	$ 5.75

Time-Based Units

TSUs typically vest over four years, with 25% vesting on the one-year anniversary of the employee's hire date and the remainder vesting monthly or quarterly over the following three years subject to the employee's continued employment with the Company through the vesting dates. The fair value of the awards was based on the value of the Company's common stock at the grant date of the award and expense is recognized on a

straight-line basis. The Company had 57,000 unvested shares underlying TSUs as of September 30, 2023. Unrecognized stock-based compensation expense at September 30, 2023 was $0.4 million, which is expected to be recognized over a weighted-average vesting term of 2.1 years. Stock-based compensation expense related to the TSUs during the three and nine months ended September 30, 2023 was immaterial.

Performance-Based Units

The vesting of the PSUs is based on the Company achieving certain milestones and are subject to the employee's continued employment with the Company through the achievement date. The fair value of the awards was based on the value of the Company's common stock at the grant date of the award and expense recognition is based on the probability of achieving the performance conditions. Stock-based compensation expense is adjusted in future periods for subsequent changes in the expected outcome of the performance conditions. The Company had 264,500 unvested shares underlying PSUs as of September 30, 2023. The Company concluded that achievement of the performance conditions was not probable as of September 30, 2023, and therefore no stock-based compensation expense was recognized for the three and nine months ended September 30, 2023. As of September 30, 2023, there was $1.7 million of unrecognized stock-based compensation expense related to the PSUs that were deemed not probable of vesting.

Market-Based Units

The vesting of the MSUs is based on the Company's closing stock price trading above $20 per share for 30 consecutive trading days subject to the employee's continued employment with the Company through the date of achievement. The fair value was based on Monte Carlo simulation model on the grant date. Stock-based compensation expense is recognized over the derived service period of approximately 3 years. The Company had 120,000 unvested shares underlying MSUs as of September 30, 2023. Stock-based compensation expense related to the MSUs during the three and nine months ended September 30, 2023 was immaterial. As of September 30, 2023, there was $0.2 million of unrecognized stock-based compensation expense related to MSUs.

Performance Award

In connection with the CEO's employment agreement, he is entitled to receive a Performance Award in the amount of $7.5 million, payable in cash, common stock or a combination of cash and common stock, at the election of the Company, in the event that (i) the Company's market value exceeds $750.0 million utilizing the volume-weighted average of the closing sale price of its common stock on the Nasdaq Stock Market or other principal exchange for each of the 30 trading days immediately prior to the measurement date, or (ii) the fair market value of the net proceeds available for distribution to the Company's stockholders in connection with a change in control as defined in the Company's severance benefit plan, as determined in good faith by its board of directors, exceeds $750.0 million. The Company has determined that the Performance Award is subject to ASC 718, *Compensation – Stock Compensation* and includes both market and performance conditions. Since the Company's initial public offering (IPO), neither of the events have yet been satisfied. The Company estimated the fair value of the Performance Award at each reporting period using the Monte Carlo simulation (Note 4), which is recognized as stock-based compensation expense over the derived service period.

The Company recognized approximately $0.2 million and $1.0 million in compensation expense related to the change in the fair value of the Performance Award for the three and nine months ended September 30, 2023, respectively. The expense is reflected in consolidated statements of operations and comprehensive loss in the general and administrative expense.

2021 Employee Stock Purchase Plan (ESPP)

In March 2021, the Company's board of directors adopted the ESPP, which became effective immediately prior to the execution of the underwriting agreement in connection with the Company's IPO. During the nine months ended September 30, 2023, 188,015 shares of common stock were purchased under the ESPP.

In September 2021, the Company's board of directors adopted the Company's 2021 United Kingdom Sharesave Sub-plan (SAYE). An allocation of 25,875 shares of common stock from the ESPP reserve pool was approved and reserved for issuance under the SAYE. No shares have been issued under the SAYE through September 30, 2023.

Stock-Based Compensation Expense

The following table summarizes stock-based compensation expense, including expense associated with options, TSUs, MSUs and award modifications for unvested options, reflected in the consolidated statements of operations and comprehensive loss (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Research and development	$ 537	$ 411	$ 1,504	$ 1,184
General and administrative	892	579	2,289	1,919
Total	$ 1,429	$ 990	$ 3,793	$ 3,103

10. License Agreement

In December 2017, the Company entered into a license agreement with vTv Therapeutics LLC (vTv Therapeutics) (the vTv License Agreement), under which the Company obtained an exclusive, worldwide, sublicensable license under certain vTv Therapeutics intellectual property to develop, manufacture and commercialize PPARδ agonists and products containing such PPARδ agonists, including mavodelpar, for any therapeutic, prophylactic or diagnostic application in humans. Under the terms of the vTv License Agreement, the Company paid vTv Therapeutics an initial upfront license fee of $3.0 million and issued an aggregate of 576,443 shares of its common stock to vTv Therapeutics.

Upon the achievement of certain pre-specified development and regulatory milestones, the Company is also required to pay vTv Therapeutics milestone payments totaling up to $64.5 million. The Company is also required to pay vTv Therapeutics up to $30.0 million in total sales-based milestones upon achievement of certain sales thresholds of the licensed product. As of September 30, 2023, the Company has paid an aggregate of $2.0 million in development and regulatory milestone payments. In addition, the Company is obligated to make royalty payments to vTv Therapeutics at mid-single digit to low teen percentage royalty rates, based on tiers of annual net sales of licensed products, subject to certain customary reductions. There were no milestone payments achieved or recorded for the three and nine months ended September 30, 2023 and 2022.

11. Subsequent Events

On October 30, 2023, pursuant to a Common Stock Repurchase Agreement with vTv Therapeutics, the Company repurchased 576,443 shares of the Company's common stock from vTv Therapeutics at a price of $7.64 per share for an aggregate purchase price of approximately $4.4 million. The repurchase was made directly in a private, non-underwritten transaction. Subsequently, the Company retired the repurchased shares.

On November 13, 2023, the Company entered into an at-the-market equity offering sales agreement (the 2023 ATM Facility) with Leerink Partners LLC (Leerink) under which the Company may offer and sell, from time to time, at its sole discretion, up to $100.0 million in shares of its common stock. The Company has not yet sold any shares of its common stock under the 2023 ATM Facility. Concurrently with entering into the 2023 ATM Facility, the Company and Leerink agreed to terminate the Company's at-the-market equity offering sales agreement entered into on May 2, 2022, with Leerink (previously SVB Securities LLC) under which the Company could offer

and sell, from time to time, at its sole discretion, up to $20.0 million in shares of its common stock (the 2022 ATM Facility). The Company had sold an aggregate of $1.2 million under the 2022 ATM Facility as of the termination date.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes included elsewhere in this Quarterly Report on Form 10-Q and our audited consolidated financial statements and notes thereto and the related Management's Discussion and Analysis of Financial Condition and Results of Operations included in our Annual Report on Form 10-K for the year ended December 31, 2022. Unless otherwise indicated, all references in this Quarterly Report on Form 10-Q to "Reneo," the "company," "we," "our," "us" or similar terms refer to Reneo Pharmaceuticals, Inc. and its subsidiary.

Forward-Looking Statements

In addition to historical financial information, this discussion contains forward-looking statements based upon current expectations that involve risks and uncertainties. Our actual results could differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth in the section titled "Risk Factors" under Part II, Item 1A below. In some cases, you can identify forward-looking statements by terminology such as "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potentially," "predict," "should," "will" or the negative of these terms or other similar expressions.

In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this Quarterly Report on Form 10-Q, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain, and investors are cautioned not to unduly rely upon these statements.

Overview

We are a clinical-stage pharmaceutical company focused on the development and commercialization of therapies for patients with rare genetic mitochondrial diseases, which are often associated with the inability of mitochondria to produce adenosine triphosphate (ATP). Our lead product candidate, mavodelpar, is a potent and selective agonist of the peroxisome proliferator-activated receptor delta (PPARδ). Mavodelpar has been shown to increase transcription of genes involved in mitochondrial function and increase fatty acid oxidation (FAO), and may increase production of new mitochondria.

The PPAR family of nuclear hormone receptors, PPARα, PPARγ, and PPARδ, control the transcription of genes critical for regulating energy metabolism and homeostasis. PPARδ is highly expressed in muscle, kidney, brain, and liver tissue. Activation of PPARδ results in changes in the expression of genes involved with multiple aspects of energy metabolism including uptake of fatty acids, utilization of fatty acids as an energy source, and mitochondrial biogenesis.

Increases in PPARδ activity also correlate with a shift in muscle tissue towards oxidative, fat-consuming type I fibers that are associated with endurance as opposed to glycolytic, type II fibers. In preclinical and clinical studies, increased PPARδ activity through transgenic overexpression or pharmacological activation increases muscular strength and endurance across a variety of functional measures. Mavodelpar was studied in healthy male volunteers with one leg immobilized to produce muscle atrophy. Compared to placebo, administration of mavodelpar resulted in statistically significant increases in expression of genes involved in mitochondrial oxidative phosphorylation, and statistically significant improvements in muscle strength. Mavodelpar was studied in an open-label trial in patients with primary mitochondrial myopathies (PMM) with confirmed mitochondrial gene defects. Patients with PMM in this trial exhibited improved function, reduced symptoms, and increased expression of genes involved in mitochondrial function. Mavodelpar was also studied in an open-label trial in patients with long-chain fatty acid oxidation disorder (LC-FAOD). In this trial, patients with LC-FAOD due to certain gene defects exhibited improved function and reduced symptoms.

As a PPARδ agonist, mavodelpar may benefit patients with genetic mitochondrial myopathies who experience weakness, fatigue, or deterioration in muscle due to impaired mitochondrial energy production. Patients with these diseases are unable to perform many everyday activities, can experience cardiomyopathy and other organ dysfunction, and typically have a reduced life expectancy. We are currently developing mavodelpar in rare genetic diseases that typically present with myopathy, including PMM and LC-FAOD.

There are currently no approved therapies for the treatment of PMM, representing a high unmet medical need.

We have received orphan drug designations for mavodelpar in the United States for PMM and LC-FAOD. Additionally, we have received orphan drug designations for mavodelpar for mitochondrial encephalomyopathy, lactic acidosis, and neurological stroke-like episodes (MELAS), a form of PMM, and long-chain 3-hydroxy acyl-CoA dehydrogenase (LCHAD), a form of LC-FAOD in Europe. We have received Fast Track designation for mavodelpar for the treatment of patients with PMM and LC-FAOD due to LCHAD deficiency, one of the predominant LC-FAOD genotypes.

Mavodelpar for the Treatment of PMM

We completed an open-label Phase 1b study of mavodelpar in patients with PMM due to mitochondrial DNA (mtDNA) defects to assess the safety and tolerability of mavodelpar, and evaluated changes in patient function using a 12-minute walk test (12MWT). Mavodelpar was well-tolerated and had an adequate safety profile in this trial. Compared to baseline, patients receiving mavodelpar once-daily for 12 weeks experienced an average increase in distance of 104.4 meters in the 12MWT, an average increase in weight-adjusted peak VO2 of 1.7 mL/min/kg, a reduction in fatigue and pain, and increased expression of genes involved with transport and metabolism of nutrients in the mitochondria including Pyruvate dehydrogenase lipoamide kinase isozyme 4 (PDK4), Angiopoietin-like 4 (ANGPTL4), and Solute carrier family 25 member 34 (SLC25A34).

Based on these results, we initiated the STRIDE study, a global, randomized, double-blind, placebo-controlled pivotal Phase 2b trial of mavodelpar in adult patients with PMM due to mtDNA defects. We completed enrollment of 213 patients in March 2023, exceeding target enrollment of 200 patients, and expect to announce topline data in December 2023. If successful, we anticipate potential submissions of a new drug application (NDA) in the first half of 2024 and a Marketing Authorization Application (MAA) in late 2024.

The STRIDE study is designed to investigate the efficacy and safety of 100 mg mavodelpar administered once-daily over a 24-week period. The primary efficacy endpoint of the trial is the change from baseline in the distance walked during the 12MWT at week 24. Secondary and exploratory endpoints include changes from baseline in PROMIS® Short Form Fatigue 13a, Modified Fatigue Impact Scale (MFIS), Patient Global Impression of Change (PGIC), Patient Global Impression of Severity (PGIS), 30 Second Sit-To-Stand (30STS) Test, Brief Pain Inventory (BPI), 36-Item Health Survey (SF-36), Work Productivity and Activity Impairment Questionnaire: Specific Health Problem (WPAI:SHP), and Pedometer Step Count.

We are also conducting the STRIDE AHEAD study, a 24-month, open-label extension (OLE) trial outside of the United States in patients with PMM due to mtDNA defects who participated in STRIDE or the mavodelpar Phase 1b study. STRIDE AHEAD is designed to evaluate the long-term safety and tolerability of 100 mg mavodelpar administered once-daily over a 24-month period. Over 65 patients have been treated beyond 52-weeks of dosing in this study. The STRIDE AHEAD study has been amended to allow enrollment of patients with PMM due to nuclear DNA (nDNA) defects, and the first patient was dosed in November 2023.

Based on interactions with the U.S. Food and Drug Administration (FDA), European Medicines Agency (EMA), and several other national regulatory agencies in Europe, we believe that positive results from the ongoing pivotal STRIDE and STRIDE AHEAD studies could potentially support registration of mavodelpar for adult patients with PMM in the United States and Europe. We intend to submit the data from STRIDE, together with the data from STRIDE AHEAD, to the FDA in the first half of 2024 and the EMA in late 2024.

Mavodelpar for the Treatment of LC-FAOD

We completed an open-label Phase 1b study in LC-FAOD adult patients with nDNA defects to assess the safety and tolerability of mavodelpar, and measure changes in functional test such as walk distance, exercise capacity and patient-reported symptoms that could serve as potential endpoints in future clinical studies. The study included patients with defective LCHAD, carnitine palmitoyltransferase 2 (CPT2), very long-chain acyl-CoA dehydrogenase (VLCAD), or trifunctional protein (TFP).

A total of 24 patients were enrolled, including patients with defective LCHAD (n=5), CPT2 (n=8), VLCAD (n=9), or TFP (n=2). We initiated the trial with a dose of 50 mg once-daily in the first three patients followed by 100 mg once-daily in all subsequent patients. The LCHAD and CPT2 groups had the greatest improvement over baseline in 12MWT (73.7 and 51.9 meters, respectively).

In the LC-FAOD Phase 1b study, mavodelpar was well tolerated. The most common adverse events experienced by patients were rhabdomyolysis (4 patients) and myalgia (4 patients), the majority reported to be mild or moderate in severity.

We also completed the FORWARD study, a 16-week, observational, non-interventional study in patients with LC-FAOD with different nDNA mutations to better understand the natural history of LC-FAOD and changes in patient function and symptoms over time. A total of 58 patients participated in the FORWARD study, including patients with defective LCHAD (n=16), CPT2 (n=30), or VLCAD (n=12).

Based on the results of the LC-FAOD Phase 1b study, in conjunction with the results of the FORWARD study, we intend to continue the development of mavodelpar for certain genotypes of patients with LC-FAOD. Results of the studies were presented at the International Network of Fatty Acid Oxidation Research and Management Conference in August 2022.

License Agreement

In December 2017, we entered into a License Agreement with vTv Therapeutics LLC (vTv Therapeutics) (the vTv License Agreement), under which we obtained an exclusive, worldwide, sublicensable license under certain vTv Therapeutics intellectual property to develop, manufacture and commercialize PPARδ agonists and products containing such PPARδ agonists, including mavodelpar, for any therapeutic, prophylactic or diagnostic application in humans. Under the terms of the vTv License Agreement, we paid vTv Therapeutics an initial upfront license fee of $3.0 million and issued an aggregate of 576,443 shares of our common stock to vTv Therapeutics.

Upon the achievement of certain pre-specified development and regulatory milestones, we are also required to pay vTv Therapeutics milestone payments totaling up to $64.5 million. We are also required to pay vTv Therapeutics up to $30.0 million in total sales-based milestones upon achievement of certain sales thresholds of the licensed product. As of September 30, 2023, we have paid an aggregate of $2.0 million in development and regulatory milestone payments. In addition, we are obligated to make tiered royalty payments to vTv Therapeutics at mid-single digit to low teen percentage royalty rates, based on tiers of annual net sales of licensed products, subject to certain customary reductions. There were no milestone payments achieved or recorded for the three and nine months ended September 30, 2023 and 2022.

Components of Our Results of Operations

Operating Expenses

Research and Development

Research and development expenses primarily relate to preclinical and clinical development of mavodelpar and include:

- personnel expenses, including salaries, benefits, and stock-based compensation expense;

- external expenses incurred under agreements with contract research organizations (CROs), investigative sites and consultants to conduct and support our preclinical studies and clinical trials;
- raw materials related to manufacturing of our product candidate for clinical trials and preclinical studies, including fees paid to third-party manufacturers;
- expenses related to regulatory activities, including filing fees paid to regulatory agencies;
- expenses related to medical affairs activities, including field teams to initiate relevant disease education and publications;
- depreciation and maintenance expenses; and
- fees for maintaining licenses under our third-party licensing agreements.

Research and development expenses are recognized as incurred and payments made prior to the receipt of goods or services to be used in research and development are capitalized until the goods or services are received. Costs for certain activities, such as manufacturing and preclinical studies and clinical trials, are generally recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and collaborators. We expense amounts paid to acquire licenses associated with products under development when the ultimate recoverability of the amounts paid is uncertain and the technology has no alternative future use when acquired.

The following table summarizes our research and development expenses (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Clinical and regulatory	$ 7,373	$ 5,555	$ 20,408	$ 14,143
Contract manufacturing cost	2,609	2,469	7,761	6,802
Nonclinical	632	577	3,719	3,060
Medical affairs	2,217	193	4,883	409
Research and development-other expense	791	1,144	2,238	2,934
Total	$ 13,622	$ 9,938	$ 39,009	$ 27,348

We expect our research and development expenses to increase substantially for the foreseeable future as we advance our product candidate into and through clinical trials, continue to conduct preclinical studies and pursue regulatory approval of our product candidate. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming. The actual probability of success for our product candidate may be affected by a variety of factors including: the safety and efficacy of our product candidate, early clinical data, investment in our clinical program, competition, manufacturing capability and commercial viability. We may never succeed in achieving regulatory approval for our product candidate. As a result of the uncertainties discussed above, at this time we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development of and obtain regulatory approval for our product candidate. Our research and development costs may vary significantly based on factors such as:

- the scope, rate of progress, expense and results of clinical trials and preclinical studies;
- per patient trial costs;
- the number of trials required for approval;
- the number of sites included in the trials;
- the countries in which the trials are conducted;
- the number of patients that participate in the trials;

- uncertainties in patient enrollment or drop out or discontinuation rates;
- potential additional safety monitoring requested by regulatory agencies;
- the duration of patient participation in the trials and follow-up;
- the safety and efficacy of our product candidate;
- the cost and timing of manufacturing our product candidates; and
- the extent to which we establish strategic collaborations or other arrangements.

General and Administrative

General and administrative expenses consist primarily of salaries, benefits, and stock-based compensation expense for personnel in executive, finance and administrative functions. General and administrative expenses also include professional fees for accounting, legal, and commercial development, corporate facility costs not otherwise included in research and development expenses, and insurance.

We expect our general and administrative expenses to increase for the foreseeable future to support overall growth of our corporate infrastructure, as well as our commercial development activities.

Other Income

Other income consists of interest income on our cash, cash equivalents and short-term investments.

Results of Operations

Comparison of Three Months Ended September 30, 2023 and 2022 (Unaudited)

The following table summarizes our results of operations (in thousands):

	Three Months Ended September 30,		
	2023	2022	Change
Operating expenses:			
Research and development	$ 13,622	$ 9,938	$ 3,684
General and administrative	7,266	3,902	3,364
Total operating expenses	20,888	13,840	7,048
Loss from operations	(20,888)	(13,840)	(7,048)
Other income	1,692	833	859
Net loss	$ (19,196)	$ (13,007)	$ (6,189)

Research and Development

Research and development expenses for the three months ended September 30, 2023 were $13.6 million, compared to $9.9 million for the three months ended September 30, 2022. This increase of $3.7 million was primarily due to an increase of $2.2 million related to clinical development and contract manufacturing costs to support the marketing registration for mavodelpar, an increase of $1.0 million in medical affairs costs, and an increase of $0.9 million in personnel-related costs due to additional headcount offset by a decrease of $0.5 million of other research and development activities.

General and Administrative

General and administrative expenses for the three months ended September 30, 2023 were $7.3 million, compared to $3.9 million for the three months ended September 30, 2022. This increase of $3.4 million was primarily due to an increase of $2.1 million in commercial development activities and an increase of $1.2 million in facility and personnel-related costs due to additional headcount.

Other Income

The increase in other income for the three months ended September 30, 2023, compared to the same period in the prior year was due to a higher interest rate earned on our short-term investments and an increase in short-term investment balance.

Comparison of Nine Months Ended September 30, 2023 and 2022 (Unaudited)

The following table summarizes our results of operations (in thousands):

	Nine Months Ended September 30,		
	2023	2022	Change
Operating expenses:			
Research and development	$ 39,009	$ 27,348	$ 11,661
General and administrative	19,038	11,938	7,100
Total operating expenses	58,047	39,286	18,761
Loss from operations	(58,047)	(39,286)	(18,761)
Other income	4,213	931	3,282
Net loss	$ (53,834)	$ (38,355)	$ (15,479)

Research and Development

Research and development expenses for the nine months ended September 30, 2023 were $39.0 million, compared to $27.3 million for the nine months ended September 30, 2022. This increase of $11.7 million was primarily due to an increase of $7.6 million related to clinical development and contract manufacturing costs to support the marketing registration for mavodelpar, an increase of $2.7 million in personal-related costs due to additional headcount, and an increase of $1.8 million in medical affairs costs offset by a decrease of $0.4 million of other research and development activities.

General and Administrative

General and administrative expenses for the nine months ended September 30, 2023 were $19.0 million, compared to $11.9 million for the nine months ended September 30, 2022. This increase of $7.1 million was primarily due to an increase of $3.7 million in commercial development activities and an increase of $3.2 million in facility and personnel-related costs due to additional headcount.

Other Income

The increase in other income for the nine months ended September 30, 2023, compared to the same period in the prior year was due to a higher interest rate earned on our short-term investments and an increase in short-term investment balance.

Liquidity and Capital Resources

Since inception, we have financed our operations primarily through the sale of equity securities. We have not generated any revenue from the sale of any products. As of September 30, 2023, we had available cash, cash equivalents and short-term investments of approximately $125.6 million.

In May 2022, we entered into an at-the-market equity offering sales agreement (2022 ATM Facility) with Leerink Partners LLC (previously SVB Securities LLC) (Leerink) under which we could offer and sell, from time to time, at our sole discretion, up to $20.0 million in shares of our common stock. As of September 30, 2023, we had sold and issued approximately 500,000 shares of our common stock pursuant to the 2022 ATM Facility at a weighted-average price of $2.48 per share, resulting in aggregate gross proceeds to us of $1.2 million. Sales commissions to Leerink and other issuance expenses were immaterial. The remaining capacity under the 2022 ATM Facility was approximately $18.8 million in shares of common stock as of September 30, 2023. In November 2023, we terminated the 2022 ATM Facility.

In May 2023, we completed a public offering in which we sold an aggregate of 7,906,250 shares of common stock which included the full exercise of the underwriters' option to purchase an additional 1,031,250 shares of common stock, at a price of $8.00 per share. Total net proceeds from the public offering, after deducting underwriting discounts and commissions and offering expenses, were approximately $58.9 million.

Also, in May 2023, we completed a concurrent private placement in which we sold an aggregate of 625,000 shares of common stock to Abingworth Bioventures 8 L.P. (Abingworth), a holder of more than 5% of our common stock, at a price of $8.00 per share (the Private Placement). Total net proceeds from the Private Placement, after deducting advisor fees and other estimated fees and expenses, were approximately $4.7 million.

On October 30, 2023, pursuant to a Common Stock Repurchase Agreement with vTv Therapeutics, we repurchased 576,443 shares of our common stock from vTv Therapeutics at price of $7.64 per share for an aggregate purchase price of approximately $4.4 million. The repurchase was made directly in a private, non-underwritten transaction. Subsequently, we retired the repurchased shares.

In November 2023, we entered into an at-the-market equity offering sales agreement (2023 ATM Facility) with Leerink under which we may offer and sell, from time to time, at our sole discretion, up to $100.0 million in shares of our common stock. We have not yet sold any shares of our common stock under the 2023 ATM Facility.

Operating Capital Requirements

From our inception in 2014, we have incurred significant losses and negative cash flows from operations. For the three and nine months ended September 30, 2023, we had a net loss of $19.2 million and $53.8 million, respectively, and used $43.9 million of cash in operating activities during the nine months ended September 30, 2023. As of September 30, 2023, we have an accumulated deficit of $190.5 million.

We have incurred operating losses since inception and we expect to continue to incur substantial expenses related to our development activities for the foreseeable future as we continue product development for mavodelpar. Since product development is time-consuming and expensive, we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. We are subject to all the risks related to the development and commercialization of a product candidate, including those discussed in the section titled "Risk Factors" under Part II, Item 1A below.

As of September 30, 2023, we have cash, cash equivalents and short-term investments of $125.6 million, which we expect will be sufficient to fund operations through the potential submission of a new drug application for mavodelpar for the treatment of PMM in the United States anticipated in first half of 2024, however, based on

the anticipated increase in commercial development expenses for mavodelpar, not for more than one year from the date of the filing of this Quarterly Report on Form 10-Q. Accordingly, management has concluded that substantial doubt exists about our ability to continue as a going concern . Management plans to alleviate this risk by raising additional capital through public or private equity offerings or debt financings, credit or loan facilities, collaborations, strategic alliances, licensing arrangements or a combination of one or more of these funding sources. In addition, we have the ability to defer certain commercial development activities until additional capital is received.

There can be no assurance that we will be successful in obtaining additional funding, that our projections of our future working capital needs will prove accurate, or that any additional funding would be sufficient to continue operations in future years. If we are not able to secure adequate additional funding, we may be forced to make reductions in spending, extend payment terms with suppliers, liquidate assets where possible, and/or suspend or curtail planned programs. Any of these actions could negatively impact our business, results of operations, and future prospects. Our ability to raise additional capital may be adversely impacted by potential worsening global economic conditions, disruptions to, and volatility in, financial markets in the United States and worldwide, including those resulting from armed conflicts, infectious diseases, bank failures, actual or perceived changes in interest rates and economic inflation. We may not be able to secure additional financing in a timely manner or on favorable terms, if at all. In addition, successful transition to attaining profitable operations is dependent upon achieving a level of revenues adequate to support our cost structure.

Cash Flows

The following table summarizes our cash flows (unaudited and in thousands):

	Nine Months Ended September 30,	
	2023	**2022**
Net cash used in operating activities	$ (43,929)	$ (31,749)
Net cash used in by investing activities	(29,254)	(30,925)
Net cash provided by financing activities	64,993	146
Net decrease in cash and cash equivalents	$ (8,190)	$ (62,528)

Operating Activities

Net cash used in operating activities for the nine months ended September 30, 2023 was $43.9 million, consisting primarily of our net loss of $53.8 million adjusted for non-cash items of $1.8 million primarily due to $3.8 million of stock-based compensation expense and $1.0 million in fair value of a performance award, offset by $3.6 million of amortization/accretion on short term investments and a $8.1 million net change in operating assets and liabilities. The change in our net operating assets and liabilities was primarily due to a decrease in prepaid and other current assets of $2.1 million and an increase in accounts payable and accrued expenses of $6.4 million due to timing of receipt of invoices and payments.

Net cash used in operating activities for the nine months ended September 30, 2022 was $31.7 million, consisting primarily of our net loss of $38.4 million adjusted for non-cash items of $3.0 million primarily due to stock-based compensation expense and a $3.6 million net change in operating assets and liabilities. The change in our net operating assets and liabilities was primarily due to an increase in accounts payable and accrued expenses of $2.8 million due to timing of receipt of invoices and payments and a decrease in prepaid and other current assets of $1.1 million.

Investing Activities

Net cash used in investing activities for the nine months ended September 30, 2023 was $29.3 million, consisting primarily of the net purchase of available-for-sale short-term investments.

Net cash used in investing activities for the nine months ended September 30, 2022 was $30.9 million, consisting primarily of the net purchase of available-for-sale short-term investments.

Financing Activities

Net cash provided by financing activities for the nine months ended September 30, 2023 was $65.0 million, consisting primarily of the net proceeds of $58.9 million, $4.7 million, and $1.0 million from the sale of common stock in our May 2023 public offering, the Private Placement, and the 2022 ATM Facility, respectively.

Net cash provided by financing activities for the nine months ended September 30, 2022 was immaterial.

Material Cash Requirements

The discussion below summarizes our significant contractual obligations and commitments as of September 30, 2023.

Leases. See Note 7 of Notes to Consolidated Financial Statements included in this Quarterly Report on Form 10-Q for information regarding our leases, including the future operating lease minimum payments.

Performance Award. See Note 9 of Notes to Consolidated Financial Statements included in this Quarterly Report on Form 10-Q for information regarding a special performance award that our chief executive officer may be entitled to receive, including the maximum payout.

vTv License Agreement. See Note 10 of Notes to Consolidated Financial Statements included in this Quarterly Report on Form 10-Q for information regarding the vTv License Agreement, including potential milestone and royalty payments.

In addition to the contractual obligations above, we also expect to have future material cash requirements related to our contract manufacturing, preclinical and clinical programs, and personnel expenses.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States of America. The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, and expenses and the disclosure of contingent assets and liabilities at the date of the consolidated financial statements. We base our estimates on historical experience, known trends and events, and various other factors that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ materially from these estimates under different assumptions or conditions.

Our critical accounting policies are described in the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies and Estimates" in our Annual Report on Form 10-K for the year ended December 31, 2022, and the notes to our consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q. During the nine months ended September 30, 2023, there were no material changes to our critical accounting policies from those discussed in our Annual Report on Form 10-K for the year ended December 31, 2022.

Recent Accounting Pronouncements

See Note 2 of Notes to Consolidated Financial Statements included in this Quarterly Report on Form 10-Q for a description of recent accounting pronouncements applicable to our consolidated financial statements.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Not required for smaller reporting companies.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation and supervision of our principal executive officer and our principal financial officer, have evaluated our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the Exchange Act)), as of the end of the period covered by this Quarterly Report on Form 10-Q. Based on that evaluation, our principal executive officer and our principal financial officer have concluded that as of September 30, 2023, our disclosure controls and procedures were effective to provide reasonable assurance that information we are required to disclose in reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in Securities and Exchange Commission (SEC) rules and forms, and that such information is accumulated and communicated to our management, including our principal executive officer and our principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the three months ended September 30, 2023 that materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1. Legal Proceedings

From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. We are not currently a party to any material legal proceedings. Regardless of outcome, litigation can have an adverse impact on us due to defense and settlement costs, diversion of management resources, negative publicity, reputational harm and other factors, and there can be no assurances that favorable outcomes will be obtained.

Item 1A. Risk Factors

We face many risks and uncertainties, as more fully described in this section under the heading "Risk Factors." Some of these risks and uncertainties are summarized below. The summary below does not contain all of the information that may be important to you, and you should read this summary together with the more detailed discussion of these risks and uncertainties contained in "Risk Factors."

Risks Related to Our Business and Industry

- We have incurred significant net losses since our inception in 2014 and anticipate that we will continue to incur significant net losses for the foreseeable future. We have identified conditions and events that raise substantial doubt about our ability to continue as a going concern.
- We will need substantial additional capital to develop and commercialize mavodelpar and any future product candidates and implement our operating plan. If we fail to complete additional financings, we may be forced to delay, reduce or eliminate our product development programs or commercialization efforts.
- We currently depend entirely on the success of mavodelpar, which is our only product candidate. If we are unable to advance mavodelpar through clinical development, obtain regulatory approvals, and ultimately commercialize mavodelpar, or experience significant delays in doing so, our business will be materially harmed.
- Our clinical trials may fail to adequately demonstrate the safety and efficacy of mavodelpar, which could prevent or delay regulatory approval and commercialization.
- Preclinical and clinical drug development is a lengthy and expensive process with uncertain outcomes, and results of earlier studies and trials may not be predictive of future trial results.
- If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.
- Any delays in the commencement or completion, or termination or suspension, of our clinical trials could result in increased costs to us, delay or limit our ability to generate revenue, and adversely affect our commercial prospects.
- The regulatory approval process is lengthy, expensive and uncertain, and we may be unable to obtain regulatory approval for our product candidates under applicable regulatory requirements. The denial or delay of any such approval would delay commercialization of our product candidates and adversely impact our ability to generate revenue, our business and our results of operations.
- If the market opportunities for mavodelpar and any future product candidates are smaller than we believe they are, or we face substantial competition in our markets, our future revenue may be adversely affected, and our business may suffer.
- We may not be successful in our efforts to expand our pipeline by identifying additional indications for which to investigate mavodelpar in the future. We may expend our limited resources to pursue a

particular indication or formulation for mavodelpar and fail to capitalize on product candidates, indications or formulations that may be more profitable or for which there is a greater likelihood of success.

- We currently have no marketing and sales organization. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell mavodelpar and any future product candidates, we may not be able to generate product revenues.

Risks Related to Our Reliance on Third Parties

- We depend on the vTv License Agreement, and termination of this license could result in the loss of significant rights, which would harm our business.
- We rely on third parties to conduct, supervise, and monitor our clinical trials. If these third parties do not successfully carry out their contractual duties, meet rigorously enforced regulatory requirements, or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize mavodelpar.
- We rely completely on third parties to manufacture our preclinical and clinical drug supplies and we intend to rely on third parties to produce commercial supplies of mavodelpar and any future product candidates, if approved, and these third parties may fail to obtain and maintain regulatory approval for their facilities, fail to provide us with sufficient quantities of drug product or fail to do so at acceptable quality levels or prices.

Risks Related to Our Intellectual Property

- Our success depends on our ability to obtain and maintain sufficient intellectual property protection for mavodelpar, any future product candidates, and other proprietary technologies.
- We cannot ensure that patent rights relating to inventions described and claimed in our pending patent applications will issue or that patents based on our patent applications will not be challenged and rendered invalid and/or unenforceable.
- If the scope of any patent protection we obtain is not sufficiently broad, or if we lose any of our patent protection and/or other market exclusivity, our ability to prevent our competitors from commercializing similar or identical product candidates may be adversely affected.
- We may not be able to protect our intellectual property rights throughout the world.
- If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties, such as the vTv License Agreement, or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.
- We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming, and unsuccessful. Further, our issued patents could be found invalid or unenforceable if challenged in court, and we may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights.

Risks Related to Ownership of Our Common Stock

- Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

An investment in shares of our common stock involves a high degree of risk. You should carefully consider the risks described below, as well as the other information in this Quarterly Report on Form 10-Q, including our consolidated financial statements and the related notes and "Management's Discussion and Analysis of Financial Condition and Results of Operations" before deciding whether to purchase, hold or sell shares of our common stock. The occurrence of any of the risks described below could harm our business, financial condition, results of operations, growth prospects, and/or stock price or cause our actual results to differ materially from those contained in forward-looking statements we have made in this Quarterly Report on Form 10-Q and those we may make from time to time. You should consider all of the risk factors described when evaluating our business. We have marked with an asterisk (*) those risk factors that reflect changes from the similarly titled risk factors included in our Annual Report on Form 10-K for the year ended December 31, 2022.

Risks Related to Our Business and Industry

We have incurred significant net losses since our inception in 2014 and anticipate that we will continue to incur significant net losses for the foreseeable future. We have identified conditions and events that raise substantial doubt about our ability to continue as a going concern.*

We are a clinical-stage pharmaceutical company founded in 2014, and our operations to date have focused primarily on raising capital, establishing and protecting our intellectual property portfolio, organizing and staffing our company, business planning, and conducting preclinical and clinical development of, and manufacturing development for, our only product candidate, mavodelpar. Additionally, as an organization, we have not yet demonstrated an ability to successfully complete clinical development, obtain regulatory approvals, manufacture a commercial-scale product, or conduct sales and marketing activities necessary for successful commercialization. As we build our capabilities and expand our organization, we have not yet demonstrated an ability to overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the pharmaceutical area. Consequently, any predictions about our future performance may not be as accurate as they would be if we had a history of successfully developing and commercializing pharmaceutical products.

Investment in pharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effectiveness in the targeted indication or an acceptable safety profile, gain regulatory approval and become commercially viable. We have no products approved for commercial sale and have not generated any revenue to date, and we continue to incur significant research and development and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred significant net losses since our inception. If mavodelpar is not successfully developed and approved in the United States or Europe, we may never generate any revenue. For the nine months ended September 30, 2023 and 2022, we reported a net loss of $53.8 million and $38.4 million, respectively. As of September 30, 2023, we had an accumulated deficit of $190.5 million.

We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our clinical development of, and seek regulatory approvals for, mavodelpar and any future product candidates. We may encounter unforeseen expenses, difficulties, complications, delays, and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenues. Our prior net losses and expected future net losses have had and will continue to have an adverse effect on our stockholders' equity and working capital. Because of the numerous risks and uncertainties associated with drug development, we are unable to accurately predict the timing or amount of increased expenses, or when, if at all, we will be able to achieve profitability.

The uncertainties around our ability to obtain additional funding raise substantial doubt regarding our ability to continue as a going concern for a period of twelve months following the date that these consolidated financial statements were issued. See Note 1 of Notes to Consolidated Financial Statements included in this Quarterly Report on Form 10-Q for a detailed discussion.

We will need substantial additional capital to develop and commercialize mavodelpar and any future product candidates and implement our operating plan. If we fail to complete additional financings, we may be forced to delay, reduce or eliminate our product development programs or commercialization efforts.*

Our operations have consumed substantial amounts of cash since our inception. We expect to continue to spend substantial amounts of capital to continue the clinical development of, and seek regulatory approval for, mavodelpar and any future product candidates. We will require significant additional amounts of capital in order to prepare for commercialization, and, if approved, to launch and commercialize mavodelpar.

As of September 30, 2023, we had cash, cash equivalents and short-term investments of $125.6 million. Based on our current operating plan, we believe that our cash, cash equivalents and short-term investments as of September 30, 2023, will be sufficient to fund operations through the potential submission of a new drug application for mavodelpar for the treatment of PMM in the United States anticipated in the first half of 2024. However, changing circumstances may cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. Our future capital requirements will depend on many factors, including:

- the scope, progress, results and costs of clinical trials and preclinical studies for mavodelpar;
- the scope, prioritization and number of our research and indications we pursue;
- the costs and timing of manufacturing for our product candidate, mavodelpar;
- the costs, timing, and outcome of regulatory review of mavodelpar;
- the timing and amount of the milestone or other payments we must make to vTv Therapeutics and any future licensors;
- the terms and timing of establishing and maintaining collaborations, licenses and other similar arrangements;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other product candidates and technologies;
- the costs of securing manufacturing arrangements for commercial production;
- our ability to achieve sufficient market acceptance, coverage and adequate reimbursement from third-party payors and adequate market share and revenue for any approved products; and
- the costs of establishing or contracting for sales and marketing capabilities if we obtain regulatory approvals to market our product candidate.

In any event, we will require additional capital for the further development and commercialization of mavodelpar and any future product candidates and may need to raise additional funds sooner if we choose to expand more rapidly than we presently anticipate. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

Until such time as we can generate significant revenue from sales of our product candidate, if ever, we expect to finance our operations through public or private equity offerings or debt financings, credit or loan facilities, collaborations, strategic alliances, licensing arrangements or a combination of one or more of these funding sources. Additional funds may not be available to us on acceptable terms or at all.

In May 2022, we entered into the 2022 ATM Facility with Leerink under which we could offer and sell, from time to time, at our sole discretion, up to $20.0 million in shares of our common stock. As of September 30, 2023,

the remaining capacity under the 2022 ATM Facility was approximately $18.8 million in shares of common stock. In November 2023, we terminated the 2022 ATM Facility.

In May 2023, we completed a public offering in which we sold an aggregate of 7,906,250 shares of common stock, which included the full exercise of the underwriters' option to purchase an additional 1,031,250 shares of common stock, at a price of $8.00 per share. Total net proceeds from this offering, after deducting underwriting discounts and commissions and offering expenses, were approximately $58.9 million.

Also, in May 2023, we completed the Private Placement in which we sold an aggregate of 625,000 shares of common stock to Abingworth, a holder of more than 5% of our common stock, at a price of $8.00 per share. Total net proceeds from the Private Placement, after deducting advisor fees and other estimated fees and expenses, were approximately $4.7 million.

In November 2023, we entered into the 2023 ATM Facility with Leerink under which we may offer and sell, from time to time, at our sole discretion, up to $100.0 million in shares of our common stock. We have not yet sold any shares of our common stock under the 2023 ATM Facility.

Our ability to raise additional funds may be adversely impacted by potential worsening global economic conditions and disruptions to, and volatility in, the credit and financial markets in the United States and worldwide, including those resulting from armed conflicts, infectious diseases, bank failures, actual or perceived changes in interest rates and economic inflation. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back, or discontinue the development or commercialization of mavodelpar or other research and development initiatives. We also could be required to seek collaborators for mavodelpar and any future product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to mavodelpar and any future product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves.

We currently depend entirely on the success of mavodelpar, which is our only product candidate. If we are unable to advance mavodelpar through clinical development, obtain regulatory approvals, and ultimately commercialize mavodelpar, or experience significant delays in doing so, our business will be materially harmed.*

We currently only have one product candidate, mavodelpar, and our business and future success depends entirely on our ability to develop, obtain regulatory approval for, and then successfully commercialize, mavodelpar, which is currently in clinical development in patients with PMM and patients with LC-FAOD. This may make an investment in our company riskier than similar companies that have multiple product candidates in active development that may be able to better sustain failure of a lead product candidate.

The success of mavodelpar will depend on several factors, including the following:

- successful enrollment in our ongoing and planned clinical trials and completion of such clinical trials with favorable results and passing applicable good clinical practice (GCP) inspections;
- acceptance by the FDA and EMA of data from our STRIDE, STRIDE AHEAD, or future clinical trials in patients with PMM;
- demonstration of a positive risk/benefit profile for mavodelpar in the relevant patient population, to the satisfaction of applicable regulatory authorities;
- meeting chemistry, manufacturing and controls (CMC) requirements and passing applicable good manufacturing practice (GMP) inspections;
- the outcome, timing, and cost of meeting regulatory requirements established by the FDA, EMA, and other comparable foreign regulatory authorities;

- receipt of marketing approvals from applicable regulatory authorities, including one or more NDA from the FDA and marketing authorizations from the European Commission (based on the opinion of the Committee for Medicinal Products for Human Use (CHMP) of the EMA, and maintaining such approvals);
- establishing commercial manufacturing relationships and receiving/importing commercial supplies approved by the FDA and other regulatory authorities from any future third-party manufacturer;
- establishing sales, marketing, and distribution capabilities and commercializing mavodelpar, if approved, whether alone or in collaboration with others;
- acceptance, if and when approved, by patients, the medical community and third-party payors;
- obtaining and maintaining third-party coverage and adequate reimbursement;
- establishing and maintaining patent and trade secret protection and regulatory exclusivity for mavodelpar;
- maintaining an acceptable risk/benefit safety profile of mavodelpar following approval; and
- maintaining and growing an organization of people who can develop and commercialize mavodelpar.

If we do not achieve one or more of these factors, many of which are beyond our control, in a timely manner or at all, we could experience significant delays or an inability to develop, obtain regulatory approvals or commercialize mavodelpar.

Even if regulatory approvals are obtained, we may never be able to successfully commercialize mavodelpar. In addition, we will need to transition at some point from a company with a development focus to a company capable of supporting commercial activities. We may not be successful in such a transition. Accordingly, we may not be able to generate sufficient revenue through the sale of mavodelpar to continue our business.

Our clinical trials may fail to adequately demonstrate the safety and efficacy of mavodelpar, which could prevent or delay regulatory approval and commercialization.*

Before obtaining regulatory approvals for the commercial sale of a product candidate, we must demonstrate through lengthy, complex, and expensive preclinical testing and clinical trials that a product candidate is both safe and effective for use in each target indication. Clinical trials often fail to demonstrate safety and efficacy of the product candidate studied for the target indication. Most product candidates that commence clinical trials are never approved by regulatory authorities for commercialization. Further, we have used patient reported outcomes in our clinical trials, including our Phase 1b study of mavodelpar in PMM, such as the MFIS, the BPI, and the 36-Item Short Form Health Survey that assesses the general health of patients. Such patient reported outcomes are based on subjective patient feedback and can be inherently difficult to evaluate. Such patient reported outcomes can be influenced by factors outside of our control and can vary widely from day to day for a particular patient, and from patient-to-patient and site-to-site within a clinical trial. It is possible that the FDA or other regulatory agencies will not accept such patient reported outcomes, and any such non-acceptance may require changes to existing trial protocols or the conduct of additional clinical trials. Moreover, our ongoing pivotal STRIDE study and our completed Phase 1b studies in patients with PMM and LC-FAOD utilize a 12MWT as an assessment of endurance and exercise tolerance in patients rather than the six-minute walk test (6MWT) which is more commonly used.

Preclinical and clinical drug development is a lengthy and expensive process with uncertain outcomes, and results of earlier studies and trials may not be predictive of future trial results.*

Preclinical and clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. A failure of one or more preclinical or clinical trials can occur at any stage of testing. The results of preclinical studies and early clinical trials of mavodelpar may not be predictive of the results of

later-stage clinical trials. In addition, product candidates in later stages of clinical trials may fail to show a positive risk/benefit profile despite having progressed through preclinical studies and initial clinical trials. Also, because there are no approved drugs for PMM and only one approved product, a caloric supplement, for LC-FAOD, there are no regulatory precedents by which we can be guided with respect to regulatory endpoints.

As such, we cannot be certain that our ongoing and planned clinical trials will be successful. Many companies in the pharmaceutical industry have suffered significant setbacks in advanced clinical trials due to the inability to enroll patients in rare disease clinical trials and the lack of efficacy or safety profiles, notwithstanding promising results in earlier trials. Moreover, preclinical and clinical data is often susceptible to varying interpretations and analyses. Our completed clinical trials have involved a limited number of patients and clinical trial sites and have been open-label or uncontrolled trials. We may face significant setbacks as we expand the number of patients and clinical sites, potentially affecting the efficiency of trial execution and the consistency of trial data, which may delay or prevent regulatory approval of mavodelpar. Any safety concerns observed in any one of our clinical trials in our targeted indications could limit the prospects for regulatory approval of mavodelpar in those and other indications, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Further, if patients drop out of our clinical trials, miss scheduled doses or follow-up visits, or otherwise fail to follow clinical trial protocols, the integrity of data from our clinical trials may be compromised or not accepted by the FDA, EMA or other regulatory authorities, which would represent a significant setback for the applicable program.

If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.*

We may not be able to initiate or continue our clinical trials for mavodelpar and any future product candidates if we are unable to identify and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA, EMA and comparable foreign regulatory authorities. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the clinical trial, the design of the clinical trial, competing clinical trials, and clinicians' and patients' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating.

In particular, each clinical indication for which we are evaluating mavodelpar is a rare genetic disease with limited patient populations from which to draw participants in clinical trials. We will be required to identify and enroll a sufficient number of patients with the disease under investigation for our clinical trials of mavodelpar. Potential patients may not be adequately diagnosed or identified with the diseases which we are targeting or may not meet the entry criteria for our clinical trials. Additionally, other pharmaceutical companies with more resources and greater experience in drug development and commercialization are targeting similar genetic mitochondrial diseases we are targeting and may do so with respect to additional indications we target in the future. Any recruiting of clinical trial patients by competitors from the patient populations we are targeting in our ongoing or future clinical trials may delay or make it more difficult to fully enroll our clinical trials. Our inability to enroll a sufficient number of patients for any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether. In addition, we rely on CROs and clinical trial sites to ensure proper and timely conduct of our clinical trials and, while we have agreements governing their services, we will have limited influence over their actual performance.

We are unable to predict with confidence the likelihood or duration of such patient enrollment delays and difficulties. If patient enrollment is delayed for an extended period of time, our clinical trials could be delayed or otherwise adversely affected.

Any delays in the commencement or completion, or termination or suspension, of our clinical trials could result in increased costs to us, delay or limit our ability to generate revenue, and adversely affect our commercial prospects.*

Before we can initiate clinical trials for mavodelpar or any future product candidates, we must submit the results of preclinical studies to the FDA or comparable foreign regulatory authorities, along with other information, including information about CMC, and our proposed clinical trial protocol, as part of an investigational new drug application or similar regulatory filing under which we must receive authorization to proceed with clinical development.

Before obtaining marketing approval from regulatory authorities for the sale of mavodelpar or any future product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of mavodelpar and any future product candidates in humans. Clinical testing is expensive, time-consuming, and uncertain as to outcome. In addition, we may rely in part on preclinical, clinical and quality data generated by CROs and other third parties for regulatory submissions for mavodelpar and any future product candidates. While we have or will have agreements governing these third parties' services, we have limited influence over their actual performance. If these third parties do not make data available to us, or, if applicable, do not make regulatory submissions in a timely manner, in each case pursuant to our agreements with them, our development programs may be significantly delayed, and we may need to conduct additional clinical trials or collect additional data independently. In either case, our development costs would increase.

We do not know whether our current or any future clinical trials will begin on time, need to be redesigned, enroll an adequate number of patients, or be completed on schedule, if at all. The commencement and completion of clinical trials can be delayed for a number of reasons, including delays related to:

- obtaining regulatory authorizations to commence a clinical trial or reaching a consensus with regulatory authorities on clinical trial design or implementation;
- any failure or delay in reaching an agreement with CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- obtaining approval from one or more institutional review boards (IRBs) or Ethics Committees (ECs);
- IRBs or ECs refusing to approve, suspending or terminating the clinical trial at an investigational site, precluding enrollment of additional subjects, or withdrawing their approval of the clinical trial;
- changes to clinical trial protocols;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- sites deviating from clinical trial protocol or dropping out of a clinical trial;
- the FDA or comparable foreign regulatory authorities' failure to accept our proposed manufacturing processes and suppliers and/or requirement to provide additional information regarding our manufacturing processes before providing marketing authorization;
- manufacturing sufficient quantities of mavodelpar or any future product candidates or obtaining sufficient quantities of combination therapies for use in clinical trials;
- subjects failing to enroll or remain in our trials at the rate we expect, or failing to return for post-treatment follow-up;
- subjects choosing an alternative treatment for the indications for which we are developing mavodelpar and any future product candidates, or participating in competing clinical trials;
- lack of adequate funding to continue our clinical trials;

- subjects experiencing severe or unexpected drug-related adverse effects;
- occurrence of serious adverse events (SAEs) in clinical trials of the same class of agents conducted by other companies;
- inability to establish confirmatory evidence with the regulatory agencies where only a single arm trial is feasible to conduct in certain rare disease populations;
- a facility manufacturing mavodelpar or any of its components being ordered by the FDA or comparable foreign regulatory authorities to temporarily or permanently shut down due to violations of current good manufacturing practice (cGMP) regulations or other applicable requirements, or infections or cross-contaminations of mavodelpar in the manufacturing process;
- any changes to our manufacturing process, suppliers or formulation that may be necessary or desired;
- third-party vendors not performing manufacturing and distribution services in a timely manner or to sufficient quality standards;
- supply chain disruptions such as scarcity of raw materials used to manufacture mavodelpar;
- impact of possible trade disputes with countries where mavodelpar or its ingredients are manufactured;
- third-party clinical investigators losing the licenses or permits necessary to perform our clinical trials, not performing our clinical trials on our anticipated schedule or consistent with the clinical trial protocol, GCP or other regulatory requirements;
- third-party contractors not performing data collection or analysis in a timely or accurate manner; or
- third-party contractors becoming debarred or suspended or otherwise penalized by the FDA or other government or regulatory authorities for violations of regulatory requirements, in which case we may need to find a substitute contractor, and we may not be able to use some or all of the data produced by such contractors in support of our marketing applications.

We could encounter delays if a clinical trial is suspended or terminated by us, by the IRBs or ECs of the institutions in which such trials are being conducted or by the FDA or comparable foreign regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or comparable foreign regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. Currently, the FDA and other foreign regulatory agencies have placed a class-wide requirement on all PPAR agonists asking sponsors to complete the two-year rat and mouse carcinogenicity studies before conducting studies longer than six-months in duration. As a result, it may take longer to enroll patients in the long-term safety trial, which could adversely affect the timing of our regulatory submissions for marketing approval. In addition, changes in regulatory requirements and policies may occur, and we may need to amend clinical trial protocols to comply with these changes. Amendments may require us to resubmit our clinical trial protocols to IRBs for reexamination, which may impact the costs, timing, or successful completion of a clinical trial.

Further, conducting clinical trials in foreign countries, which we are doing for mavodelpar and expect to do for any future product candidates, presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

Moreover, principal investigators for our clinical trials may serve and have served as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain

circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the trial. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of mavodelpar.

If we experience delays in the completion of, or termination of, any clinical trial of mavodelpar or any future product candidates, the commercial prospect of mavodelpar or any future product candidates will be harmed, and our ability to generate product revenue will be delayed. Moreover, any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, termination or suspension of, or a delay in the commencement or completion of, clinical trials may also ultimately lead to the denial of regulatory approval of mavodelpar or any future product candidates. Further, delays to our clinical trials that occur as a result could shorten any period during which we may have the exclusive right to commercialize mavodelpar and our competitors may be able to bring products to market before we do, and the commercial viability of mavodelpar could be significantly reduced. Any of these occurrences may harm our business, financial condition, results of operations and prospects significantly.

Use of mavodelpar or any future product candidates could be associated with side effects, adverse events or other properties that could delay or prevent regulatory approval or result in significant negative consequences following marketing approval, if any.

As is the case with pharmaceuticals generally, it is likely that there may be side effects and adverse events associated with the use of mavodelpar and any future product candidates. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of side effects or unexpected characteristics. Undesirable side effects caused by mavodelpar and any future product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign authorities. If drug-related SAEs are observed, our trials could be suspended or terminated, and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval for mavodelpar for any or all targeted indications. The drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition, results of operations and prospects significantly.

Additionally, if mavodelpar and any future product candidates receive marketing approval, and we or others later identify undesirable side effects caused by such product candidate, a number of potentially significant negative consequences could result, including:

- we may be forced to suspend marketing of that product, or decide to remove the product from the marketplace;
- regulatory authorities may withdraw approvals or change their approvals of such product;
- regulatory authorities may require additional warnings on the label or limit access of that product to selective specialized centers with additional safety reporting and with requirements that patients be geographically close to these centers for all or part of their treatment;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we may be required to change the way the product is administered;

- we could be subject to fines, injunctions, or the imposition of criminal or civil penalties, or sued and held liable for harm caused to subjects or patients; and
- the product may become less competitive, and our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of mavodelpar and any future product candidates, if approved, and could significantly harm our business, results of operations, and prospects.

The regulatory approval process is lengthy, expensive and uncertain, and we may be unable to obtain regulatory approval for our product candidates under applicable regulatory requirements. The denial or delay of any such approval would delay commercialization of our product candidates and adversely impact our ability to generate revenue, our business and our results of operations.*

The clinical development, manufacturing, labeling, storage, record-keeping, advertising, promotion, import, export, marketing, and distribution of mavodelpar is subject to extensive regulation by the FDA in the United States and by comparable foreign regulatory authorities in foreign markets. In the United States, we are not permitted to market mavodelpar and any future product candidates until we receive regulatory approval from the FDA. The process of obtaining regulatory approval is expensive, often takes many years following the commencement of clinical trials and can vary substantially based upon the type, complexity, and novelty of the product candidates involved, as well as the target indications and patient population. Approval policies or regulations may change, and the FDA has substantial discretion in the drug approval process, including the ability to delay, limit, or deny approval of a product candidate for many reasons. Despite the time and expense invested in clinical development of product candidates, regulatory approval is never guaranteed. Neither we nor any future collaborator is permitted to market mavodelpar and any future product candidates in the United States until we receive approval of an NDA from the FDA. We have not previously submitted an NDA to the FDA, or similar drug approval filings to comparable foreign authorities.

Prior to obtaining approval to commercialize a product candidate in the United States or in foreign markets, we must demonstrate with substantial evidence from adequate and well-controlled clinical trials, and to the satisfaction of the FDA or comparable foreign regulatory authorities, that such product candidates are safe and effective for their intended uses. Results from nonclinical studies and clinical trials can be interpreted in different ways. Even if we believe the nonclinical or clinical data for mavodelpar are promising, such data may not be sufficient to support approval by the FDA and comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authorities, as the case may be, may also require us to conduct additional preclinical studies or clinical trials for mavodelpar and any future product candidates either prior to or post-approval, or may object to elements of our clinical development program.

Mavodelpar and any future product candidates could fail to receive regulatory approval for many reasons, including the following:

- serious and unexpected drug-related side effects may be experienced by participants in our clinical trials or by people using drugs similar to or in the same class as mavodelpar and any future product candidates;
- the population studied in the clinical trial may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- the FDA or comparable foreign regulatory authorities may not accept clinical data from trials which are conducted at clinical facilities or in countries where the standard of care is potentially different from that of the United States;
- we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that a product candidate is safe and effective for any of its proposed indications;

- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- the data collected from clinical trials of mavodelpar, and any future product candidates may not be sufficient to satisfy the FDA or comparable foreign regulatory authorities to support the submission of an NDA or other comparable submissions in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Any of the above events could prevent us from achieving market approval of mavodelpar or any future product candidates and could substantially increase the costs of commercializing mavodelpar or any future product candidates. The demand for mavodelpar or any future product candidates could also be negatively impacted by any adverse effects of a competitor's product or treatment.

Of the large number of drugs in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized. The lengthy approval process as well as the unpredictability of future clinical trial results may result in our failing to obtain regulatory approval to market mavodelpar and any future product candidates, which would significantly harm our business, financial condition, results of operations and prospects.

Even if we eventually complete clinical trials and receive approval of an NDA or foreign marketing application for mavodelpar and any future product candidates, the FDA or comparable foreign regulatory authority may grant approval contingent on the performance of costly additional clinical trials, including Phase 4 clinical trials, and/or the implementation of a risk evaluation and mitigation strategy (REMS) which may be required to ensure safe use of the drug after approval. The FDA or the comparable foreign regulatory authority also may approve a product candidate for a more limited indication or patient population than we originally requested, and the FDA or comparable foreign regulatory authority may not approve the labeling that we believe is necessary or desirable for the successful commercialization of a product. Any delay in obtaining, or inability to obtain, applicable regulatory approval would delay or prevent commercialization of that product candidate and would materially adversely impact our business and prospects.

Our business has been and could continue to be adversely affected by the COVID-19 pandemic in regions where we or third parties on which we rely have significant manufacturing facilities, clinical trial sites or other business operations. The COVID-19 pandemic could adversely affect our operations, as well as the business or operations of our manufacturers, CROs, or other third parties with whom we conduct business.*

Our business has been and could continue to be adversely affected by the COVID-19 pandemic and we may experience ongoing disruptions that could severely impact our business and clinical trials.

Our clinical trials have been, and may in the future be, affected by the COVID-19 pandemic. For example, as a result of the COVID-19 pandemic, our Phase 1b study of mavodelpar in patients with PMM was closed early and we temporarily paused enrollment in our other Phase 1b studies. In particular, we have and could continue to

experience slowdown of patient enrollment and patient scheduled visits in all of our ongoing and future clinical trials.

If patient enrollment is delayed for an extended period of time, our ongoing and planned clinical trials could be delayed or otherwise adversely affected. Similarly, our ability to recruit and retain principal investigators and site staff who, as healthcare providers, may have heightened exposure to COVID-19, may be adversely impacted.

In addition, we may encounter a shortage in supplies of, or in delays in shipping, our study drug or other components of the clinical trial vital for successful conduct of the trial. Further, the successful conduct of our clinical trials depends on retrieving laboratory data from patients. Any failure by the laboratories with which we work to send us such data could impair the progress of such clinical trials. These events could delay our clinical trials, increase the cost of completing our clinical trials, and negatively impact the integrity, reliability, or robustness of the data from our clinical trials.

We cannot predict future disruptions which may occur due to COVID-19. We continue to monitor the status of COVID-19 and will adjust our strategy accordingly in order to mitigate the impact on our business operations.

Preliminary, interim and topline data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose interim, topline, or preliminary data from our clinical trials, including from our studies of mavodelpar in patients with PMM and LC-FAOD, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations, and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the interim, topline, or preliminary results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Interim, topline, and preliminary data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, such data should be viewed with caution until the final data are available. Adverse differences between preliminary, interim, or topline data and final data could significantly harm our business prospects.

Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions, or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability, or commercialization of the particular product candidate or product and our company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is the material or otherwise appropriate information to include in our disclosure, and any information we determine not to disclose may ultimately be deemed significant with respect to future decisions, conclusions, views, activities or otherwise regarding a particular product, product candidate, or our business. If the interim, topline, or preliminary data that we report differ from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, mavodelpar and any future product candidates may be harmed, which could harm our business, operating results, prospects, or financial condition.

If the market opportunities for mavodelpar and any future product candidates are smaller than we believe they are, or we face substantial competition in our markets, our future revenue may be adversely affected, and our business may suffer.

If the size of the market opportunities in each of our target indications for mavodelpar and any future product candidates is smaller than we anticipate, we may not be able to achieve profitability and growth. We focus our clinical development of mavodelpar on therapies for adult patients with genetic mitochondrial diseases with relatively small patient populations. Given the relatively small number of patients who have the diseases that we

are targeting and intend to target with mavodelpar, it is critical to our ability to grow and become profitable that we continue to successfully identify patients with these rare genetic mitochondrial diseases. In addition, our estimates of the patient populations for our target indications have been derived from a variety of sources, including the scientific literature, payor claims data, surveys of clinics, patient foundations, and market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. The effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. In addition, the potentially addressable patient population for PMM and LC-FAOD may be limited or may not be amenable to treatment with mavodelpar, if approved. Further, even if we obtain significant market share for mavodelpar in PMM or LC-FAOD, we may never achieve profitability despite obtaining such significant market share, as other pharmaceutical companies with more resources and greater experience in drug development and commercialization are or may be targeting this same genetic mitochondrial disease.

We may not be successful in our efforts to expand our pipeline by identifying additional indications for which to investigate mavodelpar in the future. We may expend our limited resources to pursue a particular indication or formulation for mavodelpar and fail to capitalize on product candidates, indications or formulations that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we are focused on specific indications for mavodelpar. As a result, we may fail to generate additional clinical development opportunities for mavodelpar for a number of reasons, including, mavodelpar may in certain indications, on further study, be shown to have harmful side effects, limited to no efficacy, or other characteristics that suggest it is unlikely to receive marketing approval and achieve market acceptance in such additional indications.

While our initial focus is to advance mavodelpar for PMM to regulatory approval, we may plan to conduct several clinical trials for mavodelpar in parallel over the next several years, including multiple clinical trials in PMM and LC-FAOD, which may make our decision as to which additional indications to focus on more difficult. As a result, we may forego or delay pursuit of opportunities with other indications that could have had greater commercial potential or likelihood of success. However, we may focus on or pursue one or more of our target indications over other potential indications and such development efforts may not be successful, which would cause us to delay the clinical development and approval of mavodelpar. Furthermore, research programs to identify additional indications for mavodelpar require substantial technical, financial, and human resources. We are pursuing a tablet formulation for mavodelpar and may pursue additional formulations. However, we may not successfully develop these additional formulations for chemistry-related, stability-related, or other reasons. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for specific indications may not yield any commercially viable products.

Additionally, we may pursue additional in-licenses or acquisitions of development-stage assets or programs, which entails additional risk to us. Identifying, selecting and acquiring promising product candidates requires substantial technical, financial, and human resources expertise. Efforts to do so may not result in the actual acquisition or license of a particular product candidate, potentially resulting in a diversion of our management's time and the expenditure of our resources with no resulting benefit. For example, if we are unable to identify programs that ultimately result in approved products, we may spend material amounts of our capital and other resources evaluating, acquiring and developing products that ultimately do not provide a return on our investment.

Obtaining and maintaining regulatory approval for a product candidate in one jurisdiction does not mean that we will be successful in obtaining regulatory approval for that product candidate in other jurisdictions.

Obtaining and maintaining regulatory approval for a product candidate in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, while a failure or

delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. For example, even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the United States, including additional preclinical studies or clinical trials as clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for mavodelpar is also subject to approval.

We expect to submit a MAA to the EMA for approval of mavodelpar in the European Union (EU) for the treatment of PMM and other clinical indications if data support registration. As with the FDA, obtaining an MAA, issued by the European Commission, based on the opinion of the CHMP of the EMA, is a similarly lengthy and expensive process. Regulatory authorities in jurisdictions outside of the United States and the EU also have requirements for approval for product candidates with which we must comply prior to marketing in those jurisdictions. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of mavodelpar in certain countries. If we fail to comply with the regulatory requirements in international markets and/or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of mavodelpar will be harmed, which would adversely affect our business, prospects, financial condition, and results of operations.

We currently have no marketing and sales organization. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell mavodelpar and any future product candidates, we may not be able to generate product revenues.

We currently do not have a commercial organization for the marketing, sales, and distribution of pharmaceutical products. To commercialize mavodelpar and any future product candidates, we must build our marketing, sales, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. We intend to build a highly specialized commercial organization to support the commercialization of mavodelpar, if approved, in the United States and the EU.

The establishment and development of our own sales force or the establishment of a contract sales force to market mavodelpar and any future product candidates will be expensive and time-consuming and could delay any commercial launch. Moreover, we may not be able to successfully develop this capability. We will have to compete with other pharmaceutical and biotechnology companies to recruit, hire, train, and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of mavodelpar. To the extent we rely on third parties to commercialize mavodelpar, if approved, we may have little or no control over the marketing and sales efforts of such third parties and our revenues from product sales may be lower than if we had commercialized mavodelpar and any future product candidates ourselves. In the event we are unable to develop our own marketing and sales force or collaborate with a third-party marketing and sales organization, we would not be able to commercialize mavodelpar or any future product candidates.

If we receive regulatory approval for mavodelpar and any future product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense, and we may be subject to penalties if we fail to comply with regulatory requirements or experience unanticipated problems with any product.

Any regulatory approvals that we receive may be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including post-market studies or clinical trials, and surveillance to monitor safety and effectiveness. The FDA may also require us to adopt a REMS to ensure that the benefits of treatment with such product candidate outweigh the risks for each potential patient, which may include, among other things, a

communication plan to health care practitioners, patient education, extensive patient monitoring, or distribution systems and processes that are highly controlled, restrictive and more costly than what is typical for the industry. We or our collaborators may also be required to adopt a REMS or engage in similar actions, such as patient education, certification of health care professionals, or specific monitoring, if we or others later identify undesirable side effects caused by any product that we develop alone or with collaborators.

In addition, if the FDA or a comparable foreign regulatory authority approves a product candidate, the manufacturing, quality control, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export, and recordkeeping for the approved product will be subject to extensive and ongoing regulatory requirements. The FDA and comparable foreign regulatory authorities also require submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMP requirements and GCP for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product candidate, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- issue warning letters or untitled letters;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners, or require other restrictions on the labeling or marketing of such products;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend, withdraw or modify regulatory approval;
- suspend or modify any ongoing clinical trials;
- refuse to approve pending applications or supplements to applications filed by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products, refuse to permit the import or export of products, or require us to initiate a product recall.

The occurrence of any event or penalty described above may inhibit our ability to commercialize mavodelpar and any future product candidates and generate revenue and could require us to expend significant time and resources in response and could generate negative publicity.

Advertising and promotion of any product candidate that obtains approval in the United States will be heavily scrutinized by the FDA, the U.S. Federal Trade Commission, the Department of Justice (the DOJ), the Office of Inspector General of the U.S. Department of Health and Human Services (HHS), state attorneys general, members of the U.S. Congress, and the public. Additionally, advertising and promotion of any product candidate that obtains approval outside of the United States will be heavily scrutinized by comparable foreign entities and stakeholders. Violations, including actual or alleged promotion of our products for unapproved or off-label uses, are subject to enforcement letters, inquiries, and investigations, and civil and criminal sanctions by the FDA, DOJ, or comparable foreign bodies. Any actual or alleged failure to comply with labeling and promotion requirements may result in fines, warning letters, mandates to corrective information to healthcare practitioners, injunctions, or civil or criminal penalties.

The FDA's and other regulatory authorities' policies may change, and additional government regulations may be enacted that could prevent, limit or delay regulatory approval for mavodelpar and any future product candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing

approval that we may have obtained and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition, and results of operations.

Disruptions at FDA and other U.S. and foreign government agencies caused by funding shortages or global health concerns could hinder their ability to hire and retain key leadership and other personnel, or otherwise prevent new products and services from being developed or commercialized in a timely manner, which could negatively impact our business.

The ability of the FDA and comparable foreign regulatory authorities to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes. Average review times at the FDA have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other U.S. and foreign agencies such as the EMA, following its relocation to Amsterdam and resulting staff changes, may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, including for 35 days beginning on December 22, 2018, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

Even if we obtain regulatory approval for mavodelpar and any future product candidates, mavodelpar and any future product candidates may not gain market acceptance among physicians, patients, healthcare payors and others in the medical community.

Mavodelpar and any future product candidates may not be commercially successful. The commercial success of mavodelpar or any future product candidates, if approved, will depend significantly on the broad adoption and use of such product by physicians and patients for approved indications. The degree of market acceptance of mavodelpar or any future products, if approved, will depend on a number of factors, including:

- the clinical indications for which such product candidate is approved;
- physicians and patients considering the product as a safe and effective treatment;
- the potential and perceived advantages of the product over alternative treatments;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or other regulatory authorities;
- limitations or warnings contained in the labeling approved by the FDA or other regulatory authorities;
- the timing of market introduction of the product as well as competitive products;
- the cost of treatment in relation to alternative treatments;
- the availability of coverage and adequate reimbursement by third-party payors and government authorities;
- the willingness of patients to pay out-of-pocket in the absence of coverage and adequate reimbursement by third-party payors and government authorities;
- relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies; and

- the effectiveness of our sales and marketing efforts and those of any collaboration or distribution partner on whom we rely for sales in foreign jurisdictions.

If mavodelpar and any future product candidate is approved but fails to achieve market acceptance among physicians, patients, healthcare payors or others in the medical community, we will not be able to generate significant revenues, which would have a material adverse effect on our business, prospects, financial condition, and results of operations. In addition, even if mavodelpar and any future product candidate gains acceptance, the markets for the treatment of patients with our target indications may not be as significant as we estimate.

If mavodelpar and any future product candidate is approved for marketing, and we are found to have improperly promoted off-label uses, we may become subject to prohibitions on the sale or marketing of mavodelpar and any future product candidates, significant fines, penalties, sanctions, or product liability claims, and our image and reputation within the industry and marketplace could be harmed.

The FDA, DOJ, and comparable foreign authorities strictly regulate the marketing and promotional claims that are made about pharmaceutical products, such as mavodelpar, if approved. In particular, a product may not be promoted for uses or indications that are not approved by the FDA or comparable foreign authorities as reflected in the product's approved labeling. However, if we receive marketing approval for mavodelpar and any future product candidates, physicians can prescribe such product to their patients in a manner that is inconsistent with the approved label in their independent professional judgment. If we are found to have promoted such off-label uses, we may receive warning letters from the FDA and comparable foreign authorities and become subject to significant liability, which would materially harm our business. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. If we become the target of such an investigation or prosecution based on our marketing and promotional practices, we could face similar sanctions, which would materially harm our business. In addition, management's attention could be diverted from our business operations, significant legal expenses could be incurred, and our reputation could be damaged. The FDA and other U.S. and foreign governmental authorities have also required that companies enter into consent decrees or imposed permanent injunctions under which specified promotional conduct is changed or curtailed in order to resolve enforcement actions. If we are deemed by the FDA, DOJ, or other U.S. and foreign governmental authorities to have engaged in the promotion of mavodelpar or any future product candidate for off-label use, we could be subject to certain prohibitions or other restrictions on the sale or marketing and other operations or significant fines and penalties, and the imposition of these sanctions could also affect our reputation and position within the industry.

Coverage and reimbursement may be limited or unavailable in certain market segments for mavodelpar and any future product candidates, which could make it difficult for us to sell mavodelpar and any future product candidates profitably.

Successful sales of mavodelpar and any future product candidates, if approved, depend on the availability of coverage and adequate reimbursement from third-party payors. Patients who are prescribed medicine for the treatment of their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance, and we may not obtain such coverage or adequate reimbursement. Moreover, we focus our clinical development of mavodelpar on therapies for patients with genetic mitochondrial diseases with relatively small patient populations. As a result, we must rely on obtaining appropriate coverage and reimbursement for these populations.

Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which drugs they will cover and the amount of reimbursement they will provide. Reimbursement by a third-party payor may depend upon a number of factors, including, but not limited to, the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;

- safe, effective, and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining coverage and reimbursement approval for a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data for the use of our products. We may not be able to provide data sufficient to obtain coverage and adequate reimbursement. Assuming we obtain coverage for a given product, the resulting reimbursement payment rates might not be adequate or may require co-payments that patients find unacceptably high. Patients are unlikely to use mavodelpar or any future product candidate unless coverage is provided, and reimbursement is adequate to cover a significant portion of the cost. Additionally, the reimbursement rates and coverage amounts may be affected by the approved label for mavodelpar or any future product candidate. If coverage and reimbursement of our future products are unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, we may be unable to achieve or sustain profitability.

In addition, the market for mavodelpar and any future product candidates will depend significantly on access to third-party payors' drug formularies or lists of medications for which third-party payors provide coverage and reimbursement. The industry competition to be included in such formularies often leads to downward pricing pressures on pharmaceutical companies. Also, third-party payors may refuse to include a particular branded drug in their formularies or otherwise restrict patient access through formulary controls or otherwise to a branded drug when a less costly generic equivalent or another alternative is available.

In the United States, no uniform policy of coverage and reimbursement for drug products exists among third-party payors. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement rates, but also have their own methods and approval process apart from Medicare determinations. Therefore, coverage and reimbursement for drug products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of mavodelpar and any future product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be obtained. Coverage policies and third-party payor reimbursement rates may change at any time. Therefore, even if favorable coverage and reimbursement status is attained, less favorable coverage policies and reimbursement rates may be implemented in the future.

We intend to seek approval to market mavodelpar in the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for mavodelpar, we will be subject to rules and regulations in those jurisdictions. In some foreign countries, including those in the EU, the pricing of prescription pharmaceuticals and biologics is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after obtaining marketing approval for a drug candidate and in some countries, products cannot be marketed until after such a price has been agreed. In addition, market acceptance and sales of a product will depend significantly on the availability of coverage and adequate reimbursement from third-party payors for a product and may be affected by existing and future health care reform measures.

Recently enacted legislation, future legislation and healthcare reform measures may increase the difficulty and cost for us to obtain marketing approval for and commercialize mavodelpar and any future product candidates and may affect the prices we may set.*

In the United States and some foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the healthcare system, including cost-containment measures that may reduce or limit coverage and reimbursement for newly approved drugs and affect our ability to profitably

sell any product candidates for which we obtain marketing approval. In particular, there have been and continue to be a number of initiatives at the U.S. federal and state levels that seek to reduce healthcare costs and improve the quality of healthcare.

For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (collectively, the Affordable Care Act) was enacted in the United States. Among the provisions of the Affordable Care Act of importance to our potential product candidates, the Affordable Care Act: established an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents; expands eligibility criteria for Medicaid programs; increased the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program; created a new Medicare Part D coverage gap discount program; established a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in and conduct comparative clinical effectiveness research, along with funding for such research; and established a Center for Medicare and Medicaid Innovation at the Centers for Medicare & Medicaid Services (CMS) to test innovative payment and service delivery models to lower Medicare and Medicaid spending.

There have been judicial, executive and Congressional challenges to certain aspects of the Affordable Care Act. By way of example, on June 17, 2021, the U.S. Supreme Court dismissed a challenge on procedural grounds that argued the Affordable Care Act is unconstitutional in its entirety because the "individual mandate" was repealed by Congress. Further, on August 16, 2022, President Biden signed the Inflation Reduction Act of 2022 (the IRA) into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in Affordable Care Act marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and creating a newly established manufacturer discount program. It is possible that the Affordable Care Act will be subject to judicial or Congressional challenges in the future. It is unclear how any such challenges and the healthcare reform measures of the Biden administration will impact the Affordable Care Act and our business.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. On August 2, 2011, the Budget Control Act of 2011 was signed into law, which, among other things, included aggregate reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, including the Infrastructure Investments and Jobs Act and Consolidated Appropriations Act of 2023, will remain in effect until 2032. Additionally, on March 11, 2021, President Biden signed the American Rescue Plan Act of 2021 into law, which eliminates the statutory Medicaid drug rebate cap, currently set at 100% of a drug's average manufacturer price, for single source and innovator multiple source drugs, beginning January 1, 2024. In addition, on January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

Further, there has been heightened governmental scrutiny in the United States of pharmaceutical pricing practices in light of the rising cost of prescription drugs. Such scrutiny has resulted in several recent congressional inquiries, presidential executive orders and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products.

At the federal level, in July 2021, the Biden administration released an executive order, "Promoting Competition in the American Economy," with multiple provisions aimed at prescription drugs. In response to Biden's executive order, on September 9, 2021, HHS released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform and sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. In addition, the IRA, among other things, (1) directs HHS to negotiate the price of certain high-cost, single-source drugs and biologics covered under Medicare and (2) imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation. The IRA permits HHS to implement many of these provisions

through guidance, as opposed to regulation, for the initial years. These provisions will take effect progressively starting in fiscal year 2023. On August 29, 2023, HHS announced the list of the first ten drugs that will be subject to price negotiations, although the Medicare drug price negotiation program is currently subject to legal challenges. HHS has and will continue to issue and update guidance as these programs are implemented. It is currently unclear how the IRA will be implemented but is likely to have a significant impact on the pharmaceutical industry. Under the IRA, certain categories of drugs are excluded from price negotiations, including drugs that receive orphan drug designation as the only FDA-approved indication. While we have obtained orphan drug designation for mavodelpar, if we seek additional indications, or fail to maintain our orphan drug status, we may become subject to the price negotiation process. This could reduce the ultimate price that we receive for mavodelpar, which could negatively affect our business, results of operations, financial conditions, and prospects. Further, in response to the Biden administration's October 2022 executive order, on February 14, 2023, HHS released a report outlining three new models for testing by the Center for Medicare and Medicaid Innovation which will be evaluated on their ability to lower the cost of drugs, promote accessibility, and improve quality of care. It is unclear whether the models will be utilized in any health reform measures in the future.

At the state level, individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition, and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs.

We cannot predict the likelihood, nature, or extent of health reform initiatives that may arise from future legislation or administrative action. We expect that the Affordable Care Act and other healthcare reform measures, including those that may be adopted in the future may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from third-party payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize mavodelpar, if approved.

A variety of risks associated with marketing mavodelpar and any future product candidates internationally could materially adversely affect our business.

We plan to seek regulatory approval for mavodelpar and any future product candidates internationally and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- differing regulatory requirements in foreign countries, including differing reimbursement, pricing and insurance regimes, including as a result of Brexit;
- the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market (with low or lower prices) rather than buying them locally;
- unexpected changes in tariffs, trade barriers, price and exchange controls, and other regulatory requirements;
- economic weakness, including inflation, bank failures, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration, and labor laws for employees living or traveling internationally;

- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the U.S. Foreign Corrupt Practices Act of 1977 (FCPA) or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities internationally; and
- business interruptions resulting from geo-political actions, including war and terrorism.

These and other risks associated with our international operations may materially adversely affect our ability to attain or maintain profitable operations.

If we fail to develop and commercialize additional product candidates, we may be unable to grow our business.

We may seek to in-license or acquire late preclinical or development-stage product candidates that have the potential to complement our existing portfolio. If we decide to pursue the development and commercialization of any additional product candidates, we may be required to invest significant resources to acquire or in-license the rights to such product candidates or to conduct drug discovery activities. We do not currently have the necessary drug discovery personnel or expertise adequate to discover and develop an additional product candidate on our own. Any other product candidates will require additional, time-consuming development efforts, and significant financial resources, prior to commercial sale, including preclinical studies, extensive clinical trials, and approval by the FDA and applicable foreign regulatory authorities. All product candidates are prone to the risks of failure that are inherent in pharmaceutical product development, including the possibility that the product candidate will not be shown to be sufficiently safe and/or effective for approval by regulatory authorities. In addition, we may not be able to acquire, discover, or develop any additional product candidates, and any additional product candidates we may develop may not be approved, manufactured, or produced economically, successfully commercialized or widely accepted in the marketplace, or be more effective than other commercially available alternatives. Research programs to identify new product candidates require substantial technical, financial, and human resources whether or not we ultimately identify any candidates. If we are unable to develop or commercialize any other product candidates, our business and prospects will suffer.

We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.*

The pharmaceutical industry is characterized by intense competition and rapid innovation. Although we believe that we hold a leading position in our focus on rare genetic mitochondrial diseases, our competitors may be able to develop other compounds or drugs that are able to achieve similar or better results. Our potential competitors include major multinational pharmaceutical companies, established and start-up biotechnology companies, specialty pharmaceutical companies and universities and other research institutions. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations and well-established sales forces. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Competition

may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring or licensing on an exclusive basis drug products that are more effective or less costly than mavodelpar. We believe the key competitive factors that will affect the development and commercial success of mavodelpar are efficacy, safety and tolerability profile, reliability, convenience of dosing, price and reimbursement.

There are no approved therapies indicated for the treatment of PMM in any country. In September 2023, CymaBay Therapeutics, Inc. announced positive topline results from its Phase 3 pivotal RESPONSE study. The 52-weeks plus study evaluated the long-term safety and efficacy of seladelpar, a selective PPARδ agonist in patients with primary biliary cholangitis. Astellas Pharma Inc. is conducting a Phase 2/3 clinical trial of up to 52-weeks plus a long-term safety study with bocidelpar, a selective PPARδ agonist. In addition, Stealth BioTherapeutics Corp., Abliva AB, Tisento Therapeutics Inc., Khondrion B.V. and Minovia Therapeutics are also developing therapies for patients with mitochondrial disease.

There is one product approved in the United States for LC-FAOD. In June 2020, a new form of medium chain triglyceride (MCT) oil called DOJOLVI® (triheptanoin) was approved and indicated in the United States as a source of calories for patients with LC-FAOD. We are not aware of any drug interventional studies underway or currently announced for LC-FAOD.

Our commercial potential could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient, or are less expensive than products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market or make our development more complicated. We believe the key competitive factors affecting the success of mavodelpar are likely to be efficacy, safety, and convenience.

Even though we have obtained orphan drug designation for mavodelpar for the treatment of PMM and LC-FAOD in the United States and LCHAD and MELAS in the EU, we may not be able to obtain or maintain the benefits associated with orphan drug status, including market exclusivity.*

Regulatory authorities in some jurisdictions, including the United States and the EU, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare genetic mitochondrial disease or condition, which is generally defined as a patient population of fewer than 200,000 people in the United States, or a patient population of greater than 200,000 people in the United States, but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the EU, the criteria for designating an “orphan medicinal product” are similar in principle to those in the United States. A medicinal product may be designated as orphan if (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (a) such condition affects no more than five in 10,000 persons in the EU when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the EU to justify investment; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the EU, or if such a method exists, the product will be of significant benefit to those affected by the condition.

Generally, if a drug with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug may be entitled to a period of marketing exclusivity, which precludes the FDA or the European Commission from approving another marketing application for the same drug for the same indication for that time period. Another drug may receive marketing approval prior to mavodelpar. The applicable period is seven years in the United States and ten years in the EU, which may be extended by six months and two years, respectively, in the case of product candidates that have complied with the respective regulatory agency’s agreed upon pediatric investigation plan. The exclusivity period in the EU can be reduced to six years if, at the end of the fifth year, it is established that a drug no longer meets the criteria for

orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare genetic mitochondrial disease or condition. In addition, even after a drug is granted orphan exclusivity and approved, the FDA and the European Commission can subsequently approve another drug containing a similar active substance or substances, and which is intended to treat the same condition before the expiration of the seven-year (or ten-year in the EU) exclusivity period if the FDA or European Commission concludes that the later drug is safer, more effective or otherwise clinically superior. In addition, if an orphan designated product receives marketing approval for an indication broader than or different from what is designated, such product may not be entitled to orphan exclusivity. Even though the FDA has granted orphan drug designation to mavodelpar for the treatment of PMM and LC-FAOD in the United States and LCHAD and MELAS in the EU, if we receive approval for mavodelpar for a modified or different indication, our current orphan designations may not provide us with exclusivity. Moreover, if adopted in the form proposed, the recent European Commission proposals to revise the existing EU laws governing authorization of medicinal products may result in a decrease in data and market exclusivity for our product candidates in the EU.

Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process. Also, regulatory approval for any product candidate may be withdrawn, and other product candidates may obtain approval before us and receive orphan drug exclusivity, which could block us from entering the market.

Even if we obtain orphan drug exclusivity for mavodelpar, that exclusivity may not effectively protect us from competition because different drugs can be approved for the same condition before the expiration of the orphan drug exclusivity period.

A Fast Track designation by the FDA may not actually lead to a faster development or regulatory review or approval process for mavodelpar in any designated indication.

If a product candidate is intended for the treatment of a serious or life-threatening condition and the product candidate demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for Fast Track designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation for other indications, we cannot assure you that the FDA would decide to grant it. Even though we have received Fast Track designation for mavodelpar for the treatment of patients with PMM and LC-FAOD due to LCHAD deficiency, one of the predominant LC-FAOD genotypes, we may not experience a faster development process, review or approval. The FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data from our development program.

We may form or seek strategic alliances or enter into additional licensing arrangements in the future, and we may not realize the benefits of such alliances or licensing arrangements.

We may form or seek strategic alliances, create joint ventures or collaborations or enter into additional licensing arrangements with third parties that we believe will complement or augment our development and commercialization efforts with respect to mavodelpar and any future product candidates that we may develop. We intend to establish commercial partnerships outside of the United States and key European markets. Any of these relationships may require us to incur non-recurring and other charges, increase our near-and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business. In addition, we face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. If we license products or businesses, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture. Following a strategic transaction or license, we may not achieve the revenues or cash flows that justifies such transaction. Any delays in entering into new strategic partnership agreements related to mavodelpar could

delay the development and commercialization of mavodelpar in certain geographies for certain indications, which would harm our business prospects, financial condition, and results of operations.

We are highly dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.*

Our ability to compete in the highly competitive biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific, and medical personnel. We are highly dependent on our management, scientific, and medical personnel. The loss of the services of any of our executive officers or other key employees and our inability to find suitable replacements could potentially harm our business, prospects, financial condition or results of operations.

We conduct our operations in Irvine, California and Sandwich, United Kingdom (UK) as well as remotely as a hybrid office/virtual organization. These regions serve as the headquarters to many other biotechnology and pharmaceutical companies and academic and research institutions. Competition for skilled personnel in our market is intense and may limit our ability to hire and retain highly qualified personnel on acceptable terms or at all. The withdrawal of the UK from the EU may also negatively affect our ability to attract and retain employees, particularly those from the EU.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided stock options and restricted stock units that vest over time and performance-based restricted stock units that vest upon satisfaction of certain performance-based conditions. The value to employees such stock options, restricted stock units and performance-based restricted stock units may be significantly affected by movements in our stock price that are beyond our control and may at any time be insufficient to counteract more lucrative offers from other companies. Despite our efforts to retain valuable employees, members of our management, scientific, and development teams may terminate their employment with us on short notice. Although we have employment agreements and/or offer letters with our key employees, these arrangements provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. We do not maintain “key man” insurance policies on the lives of these individuals or the lives of any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled junior, mid-level, and senior managers as well as junior, mid-level, and senior scientific and medical personnel.

Many of the other biotechnology and pharmaceutical companies that we compete against for qualified personnel have greater financial and other resources, different risk profiles, and a longer history in the industry than we do. They may also provide more diverse opportunities and better chances for career advancement. Some of these characteristics are more appealing to high quality candidates than what we can offer. If we are unable to continue to attract and retain high quality personnel, the rate and success at which we can discover, develop and commercialize product candidates will be limited.

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.*

As of September 30, 2023, we had 55 employees. As our development and commercialization plans and strategies develop, we expect to need additional development, managerial, operational, financial, sales, marketing, and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- identifying, recruiting, integrating, maintaining, and motivating additional employees;
- managing our internal development efforts effectively, including the clinical and regulatory review process for mavodelpar and any future product candidates, while complying with our contractual obligations to contractors and other third parties; and
- improving our operational, financial and management controls, reporting systems and procedures.

Our future financial performance and our ability to commercialize mavodelpar will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time to managing these growth activities. To date, we have used the services of outside vendors to perform tasks including clinical trial management, manufacturing, statistics and analysis, regulatory affairs, formulation development, and other drug development functions. Our growth strategy may also entail expanding our group of contractors or consultants to implement these tasks going forward. Because we rely on numerous consultants, effectively outsourcing many key functions of our business, we will need to be able to effectively manage these consultants to ensure that they successfully carry out their contractual obligations and meet expected deadlines. However, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed, or terminated, and we may not be able to obtain regulatory approval for mavodelpar and any future product candidates or otherwise advance our business. We may not be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all. If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize mavodelpar and any future product candidates and, accordingly, may not achieve our research, development and commercialization goals.

Our employees, independent contractors, principal investigators, CROs, consultants, and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk that employees, independent contractors, principal investigators, CROs, consultants, and vendors may engage in fraudulent or other illegal activity. Misconduct by these parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates: (i) the rules of the FDA and other similar foreign regulatory bodies, including those rules that require the reporting of true, complete, and accurate information to the FDA and other similar foreign regulatory bodies; (ii) manufacturing standards; (iii) healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws or (iv) laws that require the true, complete, and accurate reporting of our financial information or data. These laws may impact, among other things, our current activities with principal investigators and research subjects, as well as proposed and future sales, marketing, and education programs. In particular, the promotion, sales, and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing, and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs, and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials.

If we obtain regulatory approval for mavodelpar and begin commercializing those products in the United States, the EU and other countries or jurisdictions, our potential exposure under the laws of such countries and jurisdictions will increase significantly, and our costs associated with compliance with such laws are also likely to increase. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, disgorgement, monetary fines, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs and equivalent foreign healthcare programs, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations.

Our relationships with customers, healthcare providers, and third-party payors may be subject, directly or indirectly, to federal, state and comparable foreign healthcare fraud and abuse laws, false claims laws, and

other healthcare laws and regulations. If we or our employees, independent contractors, consultants, commercial partners, or vendors violate these laws, we could face substantial penalties.

Our relationships with customers, healthcare providers, and third-party payors may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, and other healthcare laws and regulations. These laws may impact, among other things, our clinical research program, as well as our proposed and future sales, marketing, and education programs. In particular, the promotion, sales and marketing of healthcare items and services is subject to extensive laws and regulations designed to prevent fraud, kickbacks, self-dealing, and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive, and other business arrangements. The U.S. healthcare laws and regulations that may affect our ability to operate include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, any person or entity from knowingly and willfully, offering, paying, soliciting or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, the purchasing, leasing, ordering or arranging for the purchase, lease, or order of any item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. The term "remuneration" has been broadly interpreted to include anything of value. Although there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, the exceptions and safe harbors are drawn narrowly. Practices that may be alleged to be intended to induce prescribing, purchases or recommendations, include any payments of more than fair market value, and may be subject to scrutiny if they do not qualify for an exception or safe harbor. In addition, a person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation;
- federal civil and criminal false claims laws, including the federal civil False Claims Act, and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other federal government programs that are false or fraudulent or knowingly making a false statement to improperly avoid, decrease or conceal an obligation to pay money to the federal government, including federal healthcare programs. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act and the civil monetary penalties statute;
- the federal Health Insurance Portability and Accountability Act of 1996 (HIPAA) which created new federal civil and criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up by any trick, scheme or device, a material fact or making any materially false, fictitious or fraudulent statements in connection with the delivery of, or payment for, healthcare benefits, items or services. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and their respective implementing regulations, which impose requirements on certain healthcare providers, health plans, and healthcare clearinghouses, known as covered entities, and their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information as well as their covered subcontractors; and
- the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the

Children's Health Insurance Program (with certain exceptions) to report annually to CMS information related to payments or other transfers of value made to physicians, (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), other healthcare professionals (such as physician assistants and purse practitioners), and teaching hospitals, as well as ownership and investment interests held by such physicians and their immediate family members.

We may also be subject to state and foreign equivalents of each of the healthcare laws described above, among others, some of which may be broader in scope. For example, we may be subject to the following: anti-kickback and false claims laws and regulations that may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third party payors, including private insurers, or that apply regardless of payor; laws and regulations that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; laws and regulations that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, marketing expenditures, or drug pricing; and laws and regulations requiring the registration of pharmaceutical sales and medical representatives.

Additionally, we may be subject to consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Because of the breadth of these laws and regulations and the narrowness of the statutory exceptions and regulatory safe harbors available, it is possible that some of our business activities, or our arrangements with physicians, could be subject to challenge under one or more of such laws and regulations. It is not always possible to identify and deter employee misconduct or business noncompliance, and the precautions we take to detect and prevent inappropriate conduct may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. Efforts to ensure that our business arrangements will comply with applicable healthcare laws and regulations may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If we or our employees, independent contractors, consultants, commercial partners and vendors violate these laws and regulations, we may be subject to investigations, enforcement actions and/or significant penalties, including the imposition of significant civil, criminal and administrative penalties, damages, disgorgement, monetary fines, imprisonment, possible exclusion from participation in Medicare, Medicaid and other healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and regulations, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of mavodelpar outside the United States will also likely subject us to foreign equivalents of the healthcare laws and regulations mentioned above, among other foreign laws and regulations.

We are subject to stringent and evolving U.S. and foreign laws, regulations, rules, contractual obligations, policies and other obligations related to data privacy and security. Our actual or perceived failure to comply with such obligations could lead to regulatory investigations or actions; litigation (including class claims) and mass arbitration demands; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse business consequences.*

In the ordinary course of business, we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, processing) personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, data we collect about trial participants in connection with clinical trials, and sensitive third-party data (collectively, sensitive data). Our data processing activities subject us to numerous data privacy and security obligations, such as

various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements, and other obligations, relating to data privacy and security.

In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, state and federal health information privacy laws, personal data privacy laws, and consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), and other similar laws (e.g., wiretapping laws). In addition, we may obtain health data from third parties (including research institutions from which we obtain clinical trial data) that is subject to privacy and security requirements under HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 (HITECH), and their respective implementing regulations. Depending on the facts and circumstances, we could be subject to civil, criminal, and administrative penalties if we knowingly obtain, use, or disclose individually identifiable protected health information in a manner that is not authorized or permitted by HIPAA.

In addition, the California Consumer Privacy Act of 2018, as amended by the California Privacy Rights Act of 2020 (CPRA) (collectively, the CCPA), applies to personal information of consumers, business representatives, and employees who are California residents, and requires businesses to provide specific disclosures in privacy notices and honor requests of such individuals to exercise certain privacy rights, including those noted below. The CCPA provides for administrative fines of up to $7,500 per violation and allows private litigants affected by certain data breaches to recover significant statutory damages. Although the CCPA exempts some data processed in the context of clinical trials, the CCPA increases compliance costs and potential liability with respect to other personal data we maintain about California residents. Further, the CPRA expanded the CCPA's requirements, including by adding a new right for individuals to correct their personal information and establishing a new regulatory agency to implement and enforce the law. Other states, such as Virginia, Colorado, Utah, and Connecticut have also passed comprehensive privacy laws, and similar laws are being considered in several other states, as well as at the federal and local levels. These state laws and the CCPA provide individuals with certain rights concerning their personal information, including the right to access, correct, or delete certain personal information, and opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. While these states, like the CCPA, also exempt some data processed in the context of clinical trials, these developments may further complicate compliance efforts, and increase legal risk and compliance costs for us and the third parties upon whom we rely.

Outside the United States, an increasing number of laws, regulations, and industry standards govern data privacy and security. For example, the European Union's General Data Protection Regulation (EU GDPR), the United Kingdom's GDPR (UK GDPR), Canada's Personal Information Protection and Electronic Documents Act (PIPEDA), Australia's Privacy Act, and New Zealand's Privacy Act, impose strict requirements for processing personal data. For example, under the EU GDPR and UK GDPR, companies may face temporary or definitive bans on data processing and other corrective actions, fines of up to 20 million euros or 17.5 million pounds, respectively, or 4% of annual global revenue, in each case, whichever is greater, or private litigation related to processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests.

In addition, we may be unable to transfer personal data from Europe and other jurisdictions to the United States or other countries due to data localization requirements or limitations on cross-border data flows. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area (EEA) and the UK have significantly restricted the transfer of personal data to the United States and other countries whose privacy laws they generally believe are inadequate. Other jurisdictions may adopt similarly stringent interpretations of their data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the United States in compliance with law, such as the EEA and UK's standard contractual clauses, the UK's International Data Transfer Agreement / Addendum, and the EU-U.S. Data Privacy Framework and the UK extension thereto (which allows for transfers for relevant U.S.-based organizations who self-certify compliance and participate in the Framework), these mechanisms are subject to legal challenges, and

there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the United States. If there is no lawful manner for us to transfer personal data from the EEA and the UK or other jurisdictions to the United States, or if the requirements for a legally-compliant transfer are too onerous, we could face significant adverse consequences, including the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions (such as Europe) at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors and other third parties, and injunctions against our processing or transferring of personal data necessary to operate our business. These challenges and risks concerning cross-border transfers of personal data out of the EEA and UK to recipients in other jurisdictions, notably recipients in the United States, may be of particular significance to us and our operations as the majority of the trials we conduct take place in locations outside the United States, with a large number occurring in the EEA or UK. Furthermore, companies that transfer personal data out of the EEA and UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators, individual litigants, and activities groups. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers of personal data out of Europe for allegedly violating the GDPR's cross-border data transfer limitations.

In addition to data privacy and security laws, we are also bound by contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful.

Our employees and personnel may use generative artificial intelligence (AI) technologies to perform their work, and the disclosure and use of personal information in generative AI technologies is subject to various privacy laws and other privacy or legal obligations (such as copyright infringement). Governments have passed and are likely to pass additional laws regulating generative AI. Any use of this technology could result in additional compliance costs, regulatory investigations and actions, and consumer lawsuits. If we are unable to use generative AI, it could make our business less efficient and result in competitive disadvantages.

We publish privacy policies, marketing materials and other statements, such as compliance with certain certifications or self-regulatory principles, regarding data privacy and security. If these policies, marketing materials or statements are found to be deficient, lacking in transparency, deceptive, unfair, or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators or other adverse consequences.

Obligations related to data privacy and security (and consumers' data privacy expectations) are quickly changing, becoming increasingly stringent, and creating uncertainty. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires us to devote significant resources and may necessitate changes to our services, information technologies, systems, and practices and to those of any third parties that process sensitive data on our behalf.

We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties upon whom we rely may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties upon which we rely fail, or are perceived to have failed, to address or comply with applicable data privacy and security obligations, we could face significant consequences. These consequences may include, but are not limited to, government enforcement actions (e.g., investigations, fines, penalties, audits, inspections, and similar); litigation (including class-action claims) and mass arbitration demands; additional reporting requirements and/or oversight; bans on processing personal data; and orders to destroy or not use personal data.

In particular, plaintiffs have become increasingly more active in bringing privacy-related claims against companies, including class claims and mass arbitration demands. Some of these claims allow for the recovery of statutory damages on a per violation basis, and, if viable, carry the potential for monumental statutory damages, depending on the volume of data and the number of violations.

Any of these events could have a material adverse effect on our reputation, business, or financial condition, including but not limited to: loss of customers; interruptions or stoppages in our business operations (including clinical trials); inability to process sensitive data or to operate in certain jurisdictions; limited ability to develop or commercialize our products; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or revision or restructuring of our operations.

The withdrawal of the UK from the EU may adversely impact our ability to obtain regulatory approvals of our product candidates in the UK, result in restrictions or imposition of taxes and duties for importing our product candidates into the EU or UK, and may require us to incur additional expenses in order to develop, manufacture and commercialize our product candidates in the EU or UK.*

Following the result of a referendum in 2016, the UK left the EU on January 31, 2020, commonly referred to as Brexit. Pursuant to the formal withdrawal arrangements agreed between the UK and the EU, the UK was subject to a transition period until December 31, 2020 (the Transition Period) during which EU rules continued to apply. A trade and cooperation agreement (the Trade and Cooperation Agreement) that outlines the future trading relationship between the UK and the EU was agreed on in December 2020, provisionally applied from January 1, 2021 and became formally effective on May 1, 2021. Since the expiry of the Transition Period, the UK operates under a distinct regulatory regime. Since January 1, 2021, the EU laws which have been transposed into UK law through secondary legislation continue to be applicable as "retained EU law". As there is no general power to amend these regulations, the UK government passed a new Medicines and Medical Devices Act which seeks to address regulatory gaps through implementing regulations and delegated powers covering the fields of human medicines, clinical trials of human medicines, veterinary medicines and medical devices. The purpose of the Act is to enable the existing UK regulatory frameworks to be updated. Although regulatory authorities in the UK have indicated that new UK rules will be put in place, detailed proposals are yet to be published. Significant political and economic uncertainty therefore remains about how much the relationship between the UK and EU will differ as a result of the UK's withdrawal. EU pharmaceutical laws have continued to apply to Northern Ireland since the expiry of the Transition Period (as laid out in the Protocol on Ireland and Northern Ireland). On February 27, 2023, the European Commission and the UK government reached a political agreement in principle, commonly referred to as the "Windsor Framework". The purpose of the agreement is to establish a set of joint solutions that would allow goods to be traded between Great Britain and Northern Ireland and between Northern Ireland and Ireland whilst ensuring the integrity of the EU Single Market. New legislation must be passed by the UK and the EU in order to implement the provisions of the Windsor Framework, including those that relate to medicinal products. The Windsor Framework provides, however, that medicinal products to be placed on the market in Northern Ireland will be authorized solely in accordance with UK laws.

Since a significant proportion of the regulatory framework in the UK applicable to our business and our product candidates is derived from EU directives and regulations, Brexit, has had, and may continue to have, a material impact upon the regulatory regime with respect to the development, manufacture, importation, approval and commercialization of our product candidates in the UK or the EU. For example, Great Britain (GB) is no longer covered by the centralized procedures for obtaining EU-wide marketing authorization (MA) from the European Commission (based on the opinion of the CHMP of the EMA), and a separate MA will be required to market our product candidates in GB, including mavodelpar and any future product candidates. Any delay in obtaining, or an inability to obtain, any marketing approvals in GB, as a result of Brexit or otherwise, would prevent us from commercializing mavodelpar in GB and restrict our ability to generate revenue and achieve and sustain profitability. While the Trade and Cooperation Agreement provides for the tariff-free trade of medicinal products between the UK and the EU there are additional non-tariff costs to such trade which did not exist prior to the end of the Transition Period, and shipments between the UK and the EU are more likely to be delayed compared to the position prior to Brexit. Further, should the UK further diverge from the EU from a regulatory perspective in relation to medicinal products, this could lead to a more complex and costly regulatory burden on us. In addition, while the Trade and Cooperation Agreement provides for mutual recognition of GMP inspections and certificates, it does not provide for contain wholesale mutual recognition of UK and EU pharmaceutical rules and product standards, for example in relation to batch testing and pharmacovigilance, which remain subject to further

bilateral discussions. Therefore, additional batch testing between the EU and UK markets and other divergent or duplicative regulatory obligations may be required, which could result in additional expense and supply chain delays. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the UK or the EU for mavodelpar and any future product candidates, or incur significant additional expenses to operate our business, which could significantly and materially harm or delay our ability to generate revenues or achieve profitability of our business. Any further changes in international trade, tariff and import/export regulations as a result of Brexit or otherwise may impose unexpected duty costs or other non-tariff barriers on us. These developments, or the perception that any of them could occur, may significantly reduce global trade and, in particular, trade between the EU and the UK. It is also possible that Brexit may negatively affect our ability to attract and retain employees in the UK, particularly those from the EU.

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of mavodelpar and any future product candidates.

We face an inherent risk of product liability as a result of the clinical testing of mavodelpar and any future product candidates and will face an even greater risk if we commercialize any products. For example, we may be sued if mavodelpar or any future product candidates causes or is perceived to cause injury or is found to be otherwise unsuitable during clinical testing, manufacturing, marketing, or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability, and a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of mavodelpar. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for mavodelpar and any future product candidates;
- injury to our reputation;
- withdrawal of clinical trial participants;
- initiation of investigations by regulatory authorities;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing, or promotional restrictions;
- loss of revenue;
- exhaustion of any available insurance and our capital resources;
- the inability to commercialize mavodelpar and any future product candidates; or
- a decline in our share price.

Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop. We currently carry an aggregate of up to $7.0 million of product liability insurance covering our clinical trials. Although we maintain such insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. If we determine that it is prudent to increase our product liability coverage due to the commercial launch of any approved product, we may be unable to obtain such increased coverage on acceptable terms, or at all. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We will have to pay any amounts awarded by a court or negotiated in a

settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

Our ability to utilize our net operating loss (NOL) carryforwards and certain other tax attributes may be limited.

We have incurred substantial losses during our history and do not expect to become profitable in the near future, and we may never achieve profitability. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset future taxable income, if any, until such unused losses expire (if at all). See Note 10, *Income Taxes* of Notes to Consolidated Financial Statements included in our Annual Report on Form 10-K for year ended December 31, 2022 for further discussion.

Under federal tax legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act (the Tax Act), as modified by the Coronavirus Aid, Relief, and Economic Security Act (the CARES Act) federal NOL carryforwards generated in tax years beginning after December 31, 2017 may be carried forward indefinitely but, in the case of tax years beginning after 2020, may only be used to offset 80% of our taxable income annually. Our NOLs and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service (IRS) and state tax authorities and may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a rolling three-year period in excess of 50 percentage points (by value), as defined under Section 382 of the Internal Revenue Code of 1986, as amended. Our ability to utilize our NOL carryforwards and other tax attributes to offset future taxable income or tax liabilities may be limited as a result of ownership changes. Similar rules may apply under state tax laws. Such limitations could result in the expiration of our carryforwards before they can be utilized and, if we are profitable, our future cash flows could be adversely affected due to our increased taxable income or tax liability. We may have experienced ownership changes in the past and may experience ownership changes as a result of future offerings and/or subsequent changes in our stock ownership (some of which are outside our control). In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

Changes in tax laws or regulations that are applied adversely to us or our customers may have a material and adverse effect on our business, cash flow, financial condition or results of operations.

The Tax Act enacted many significant changes to the U.S. tax laws. Future guidance from the IRS and other tax authorities with respect to the Tax Act may affect us, and certain aspects of the Tax Act could be repealed or modified in future legislation. For example, the CARES Act modified certain provisions of the Tax Act. Changes in corporate tax rates, the realization of net deferred tax assets relating to our U.S. operations, the taxation of foreign earnings and the deductibility of expenses under the Tax Act or future tax reform legislation could have a material impact on the value of our deferred tax assets, could result in significant one-time charges in the current or future taxable years and could increase our future U.S. tax expense. For example, the recently enacted Inflation Reduction Act of 2022 (IRA) includes provisions that will impact the U.S. federal income taxation of corporations, including imposing a minimum tax on the book income of certain large corporations and an excise tax on certain corporate stock repurchases that would be imposed on the corporation repurchasing such stock. The foregoing items, as well as any other future changes in tax laws, could have a material adverse effect on our business, cash flow, financial condition or results of operations. In addition, it is uncertain if and to what extent various states will conform to the Tax Act, the CARES Act, the IRA, or any newly enacted federal tax legislation.

Tax authorities may disagree with our positions and conclusions regarding certain tax positions, resulting in unanticipated costs, taxes or non-realization of expected benefits.

A tax authority may disagree with tax positions that we have taken, which could result in increased tax liabilities. For example, the IRS or another tax authority could challenge our allocation of income by tax jurisdiction and the amounts paid between our affiliated companies pursuant to our intercompany arrangements and transfer pricing policies, including amounts paid with respect to our intellectual property development. Similarly, a tax authority could assert that we are subject to tax in a jurisdiction where we believe we have not established a

taxable nexus, often referred to as a "permanent establishment" under international tax treaties, and such an assertion, if successful, could increase our expected tax liability in one or more jurisdictions. A tax authority may take the position that material income tax liabilities, interest and penalties are payable by us, in which case, we expect that we might contest such assessment. Contesting such an assessment may be lengthy and costly and if we were unsuccessful in disputing the assessment, the implications could increase our anticipated effective tax rate, where applicable.

Adverse developments affecting the financial services industry, such as actual events or concerns involving liquidity, defaults or non-performance by financial institutions could adversely affect our current financial condition and projected business operations.*

Events involving limitations to liquidity, defaults, non-performance or other adverse developments that affect financial institutions, transactional counterparties or other companies in the financial services industry, or concerns or rumors about any events of these kinds or other similar risks, have in the past and may in the future lead to market-wide liquidity problems. For example, on March 10, 2023, Silicon Valley Bank (SVB) was closed by the California Department of Financial Protection and Innovation, and the Federal Deposit Insurance Corporation (FDIC) was appointed as receiver. Subsequently, the FDIC announced that all deposits with SVB are fully insured. Similarly, on March 12, 2023, Signature Bank Corp. and Silvergate Capital Corp. were each swept into receivership and on May 1, 2023, First Republic Bank was swept into receivership. We have moved any cash or other deposits previously held at SVB US (a division of First Citizens Bank) to other financial institutions and all accounts associated with SVB UK (a division of HSBC) were fully transitioned over to HSBC. We have access to all of our cash and other deposits previously held at SVB. We do not anticipate any material impact on our financial condition or operations as a result of SVB's circumstances. Additionally, the failure of a bank, or other adverse conditions in the financial or credit markets impacting financial institutions at which we maintain balances or with which we do business, could adversely impact our liquidity and financial performance. There can be no assurance that our deposits in excess of the FDIC or other comparable insurance limits will be backstopped by the U.S. or any applicable foreign government in the future or that any bank or financial institution with which we do business will be able to obtain needed liquidity from other banks, government institutions or by acquisition in the event of a future failure or liquidity crisis. In addition, if any of our partners or parties with whom we conduct business are unable to access funds due to the status of their financial institution, such parties' ability to pay their obligations to us or to enter into new commercial arrangements requiring additional payments to us could be adversely affected.

Investor concerns regarding the U.S. or international financial systems could result in less favorable commercial financing terms, including higher interest rates or costs and tighter financial and operating covenants, or systemic limitations on access to credit and liquidity sources, thereby making it more difficult for us to acquire financing on acceptable terms or at all. Our inability to acquire financing on acceptable terms or at all may materially harm our business, financial condition, results of operations and prospects.

Risks Related to Our Reliance on Third Parties

We depend on the vTv License Agreement, and termination of this license could result in the loss of significant rights, which would harm our business.

We are dependent on technology, patents, know-how, and proprietary materials, both our own and licensed from others. We entered into the vTv License Agreement in December 2017 pursuant to which we were granted an exclusive, worldwide, sublicensable license under vTv Therapeutics intellectual property relating to vTv Therapeutics' PPARδ agonist program, to develop, manufacture and commercialize PPARδ agonists and products containing such PPARδ agonists, including mavodelpar, or licensed products, for any therapeutic, prophylactic or diagnostic application in humans. Any termination of this license will result in the loss of significant rights and will restrict our ability to develop and commercialize mavodelpar.

We are generally also subject to all of the same risks with respect to protection of intellectual property that we license, as we are for intellectual property that we own, which are described below under "Risks Related to Our Intellectual Property." If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize products could suffer.

We rely on third parties to conduct, supervise, and monitor our clinical trials. If these third parties do not successfully carry out their contractual duties, meet rigorously enforced regulatory requirements, or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize mavodelpar.*

We currently rely on, and intend to continue relying on, third-party CROs in connection with our clinical trials for mavodelpar. We control or will control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our clinical trials is conducted in accordance with applicable protocol, legal, regulatory, and scientific standards, and our reliance on our CROs does not relieve us of our regulatory responsibilities. We and our CROs are required to comply with GCPs, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of these CROs fail to comply with applicable GCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Upon inspection, such regulatory authorities may determine that our clinical trials do not comply with the GCP regulations. In addition, our clinical trials must be conducted with drug product produced under cGMP regulations and will require a large number of test subjects. Our failure or any failure by our CROs to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of our CROs violates federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Our CROs are not our employees and, except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our ongoing preclinical, clinical and nonclinical programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could affect their performance on our behalf. If our CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed, or terminated and we may not be able to complete development of, obtain regulatory approval for or successfully commercialize mavodelpar and any future product candidates. As a result, our financial results and the commercial prospects for mavodelpar and any future product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Switching or adding CROs involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Although we carefully manage our relationships with our CROs, we may encounter challenges or delays in the future and these delays or challenges may have a material adverse impact on our business, prospects, financial condition, and results of operations.

In addition, quarantines, shelter-in-place, and similar government orders, or the perception that such orders, shutdowns or other restrictions on the conduct of business operations could occur, related to infectious diseases could impact personnel at our CROs, which could disrupt our clinical timelines, which could have a material adverse impact on our business, prospects, financial condition, and results of operations.

We rely completely on third parties to manufacture our preclinical and clinical drug supplies and we intend to rely on third parties to produce commercial supplies of mavodelpar and any future product candidates, if

approved, and these third parties may fail to obtain and maintain regulatory approval for their facilities, fail to provide us with sufficient quantities of drug product or fail to do so at acceptable quality levels or prices.*

We do not currently have nor do we plan to acquire the infrastructure or capability internally to manufacture our clinical drug supplies for use in the conduct of our clinical trials, and we lack the resources and the capability to manufacture mavodelpar and any future product candidates on a clinical or commercial scale. Instead, we rely on contract manufacturers for such production.

We do not currently have any long-term agreement with a manufacturer to produce raw materials, active pharmaceutical ingredients (APIs) and the finished products of mavodelpar used in our current product format and we rely on single-source suppliers for clinical supply of API and drug product of mavodelpar. We intend to enter into agreements for commercial production with third-party suppliers. Our reliance on third-party suppliers and manufacturers, including single-source suppliers, could harm our ability to develop mavodelpar or commercialize it, if approved. Further, any delay in identifying and qualifying a manufacturer for commercial production could delay the potential commercialization of mavodelpar and any future product candidates, and, in the event that we do not have sufficient product to complete our clinical trials, it could delay such trials.

The facilities used by our contract manufacturers to manufacture mavodelpar and any future product candidates must be approved by the applicable regulatory authorities, including the FDA, pursuant to inspections that will be conducted after an NDA or comparable foreign regulatory marketing application is submitted. We currently do not control the manufacturing process of mavodelpar and are completely dependent on our contract manufacturing partners for compliance with the FDA's cGMP requirements for manufacture of both the active drug substances and finished drug product. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the FDA's strict regulatory requirements, they will not be able to secure or maintain FDA approval for the manufacturing facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or any other applicable regulatory authority does not approve these facilities for the manufacture of mavodelpar or any future product candidates or if it withdraws any such approval in the future, or if our suppliers or contract manufacturers decide they no longer want to supply or manufacture for us, we may need to find alternative manufacturing facilities, in which case we might not be able to identify manufacturers for clinical or commercial supply on acceptable terms, or at all, which would significantly impact our ability to develop, obtain regulatory approval for, or market mavodelpar and any future product candidates.

In addition, the manufacture of pharmaceutical products is complex and requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, particularly in scaling up and validating initial production and absence of contamination. These problems include difficulties with production costs and yields, quality control, including stability of the product, quality assurance testing, operator error, shortages of qualified personnel, as well as compliance with strictly enforced federal, state, and foreign regulations. Furthermore, if contaminants are discovered in our supply of mavodelpar or any future product candidates or in the manufacturing facilities, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. Any stability or other issues relating to the manufacture of mavodelpar may occur in the future. In addition, quarantines, shelter-in-place, and similar government orders, or the perception that such orders, shutdowns, or other restrictions on the conduct of business operations could occur, related to infectious diseases could impact personnel at our third-party manufacturing facilities upon which we rely, or the availability or cost of materials, which could disrupt the supply chain for our product candidates. Additionally, our manufacturers may experience manufacturing difficulties due to resource constraints or as a result of labor disputes or unstable political environments. If our manufacturers were to encounter any of these difficulties, or otherwise fail to comply with their contractual obligations, our ability to provide our product candidate to patients in clinical trials would be jeopardized. Any delay or interruption in the supply of clinical trial supplies could delay the completion of clinical trials, increase the costs associated with maintaining clinical trial programs and, depending upon the period of delay, require us to commence new clinical trials at additional expense or terminate clinical trials completely.

If we or our third-party manufacturers use hazardous substances in a manner that causes injury or violates applicable law, we may be liable for damages.

Our research and development activities involve the controlled use of potentially hazardous substances by our third-party manufacturers. Our manufacturers are subject to federal, state, and local laws and regulations in the United States governing the use, manufacture, storage, handling and disposal of medical, radioactive and hazardous materials. Although we believe that our manufacturers' procedures for using, handling, storing and disposing of these materials comply with legally prescribed standards, we cannot completely eliminate the risk of contamination or injury resulting from hazardous materials. As a result of any such contamination or injury, we may incur liability or local, city, state or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability could exceed our resources. We do not have any insurance for liabilities arising from hazardous materials. Compliance with applicable environmental laws and regulations is expensive, and current or future environmental regulations may impair our research, development, and production efforts, which could harm our business, prospects, financial condition, or results of operations.

Risks Related to Our Intellectual Property

Our success depends on our ability to obtain and maintain sufficient intellectual property protection for mavodelpar, any future product candidates, and other proprietary technologies.*

Our commercial success will depend in part on our ability to obtain and maintain a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to mavodelpar, any future product candidates, and other proprietary technologies we develop. If we are unable to obtain or maintain patent protection with respect to mavodelpar, any future product candidates, and other proprietary technologies we may develop, our business, financial condition, results of operations, and prospects could be materially harmed.

We generally seek to protect our products and product candidates and related inventions and improvements that we consider important to our business. We own a portfolio of U.S. and non-U.S. patent applications for mavodelpar and have licensed rights to a number of U.S. and non-U.S. patents and patent applications for mavodelpar. Some of our owned and licensed patents and patent applications cover or relate to mavodelpar, including composition of matter, uses to treat particular conditions and methods of manufacturing.

We have developed and continue to expand our patent portfolio for mavodelpar. We have licensed from vTv Therapeutics eight issued patents in the United States and 19 issued patents in foreign countries covering composition of matter of mavodelpar, among other things, which are expected to expire in 2026, absent any patent term adjustments or extensions. Additionally, we have licensed four issued patents in the United States, six issued patents in foreign countries, one pending application in the United States, and one pending application in Europe, from vTv Therapeutics covering methods of using mavodelpar, which are expected to expire in 2034, absent any patent term adjustments or extensions.

In addition to the licensed vTv Therapeutics patents and applications relating to mavodelpar, we have filed our own patent applications. We co-own one pending application in the United States and five pending applications in foreign countries, and own four pending applications in the United States, one pending international patent application, an issued patent in a foreign country, and over 25 pending applications in foreign countries, directed to various methods of use of mavodelpar. These pending patent applications, if issued, would be expected to expire between 2040 and 2043, absent any patent term adjustments or extensions. We also own two issued patents in the United States, two pending applications in the United States, and over 25 pending applications in foreign countries directed to methods of manufacturing, and crystalline forms (polymorphs) of mavodelpar. The issued patents, and pending patent applications if issued, are expected to expire in 2041, absent any patent term adjustments or extensions. Patents related to mavodelpar may be eligible for patent term extensions in certain jurisdictions, including up to five years in both the United States and the EU, upon approval of

a commercial use of the corresponding product by a regulatory agency in the jurisdiction where the patent was granted.

Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless, and until, patents issue from such applications, and then only to the extent the issued claims cover such technology. There can be no assurance that our patent applications or the patent applications of our future licensors will result in patents being issued or that issued patents will afford sufficient protection against competitors with similar technology, nor can there be any assurance that the patents issued will not be infringed, designed around or invalidated by third parties.

Even issued patents may later be found invalid or unenforceable or may be modified or revoked in proceedings instituted by third parties before various patent offices or in courts. The degree of future protection for our and our licensors' proprietary rights is uncertain. Only limited protection may be available and may not adequately protect our rights or permit us to gain or keep any competitive advantage. These uncertainties and/or limitations in our ability to properly protect the intellectual property rights relating to our product candidates could have a material adverse effect on our financial condition and results of operations.

We cannot be certain that the claims in our U.S. pending patent applications, corresponding international patent applications and patent applications in certain foreign territories, or those of our future licensors, will be considered patentable by the United States Patent and Trademark Office (USPTO), courts in the United States or by the patent offices and courts in foreign countries, nor can we be certain that the claims in our future issued patents will not be found invalid or unenforceable if challenged.

In addition, although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors, potential partners, and other third parties, any of these parties may breach such agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection.

Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.*

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our potential future collaborators will be successful in protecting our product candidates by obtaining and defending patents. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment, and other similar provisions during the patent process. Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on any issued patents and/or applications are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm and rely on our outside counsel to pay these fees due to foreign patent agencies. While an inadvertent lapse may sometimes be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If such event were to occur, our competitors might be able to enter the

market with similar or identical products or technology earlier than should otherwise have been the case, which would have a material adverse effect on our business, financial condition, results of operations, and prospects.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patent rights are of limited duration. The term of any individual patent depends on applicable law in the country where the patent is granted. In the United States, provided all maintenance fees are timely paid, a patent generally has a term of 20 years from its application filing date or earliest claimed non-provisional filing date. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such product candidates are commercialized. Even if patents covering our product candidates are obtained, once the patent term has expired for a product, we may be open to competition from generic products. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing product candidates similar or identical to ours. Upon issuance in the United States, the term of a patent can be increased by patent term adjustment, which is based on certain delays caused by the USPTO, but this increase can be reduced or eliminated based on certain delays caused by the patent applicant during patent prosecution. The term of a United States patent may also be shortened if the patent is terminally disclaimed over an earlier-filed patent. Extensions may be available under certain circumstances, but the term of a patent and, correspondingly, the protection it affords is limited. A patent term extension (PTE) based on regulatory delay may be available in the United States. However, only a single patent can be extended for each marketing approval, and any patent can be extended only once, for a single product. Moreover, the scope of protection during the period of the PTE does not extend to the full scope of the claim, but instead only to the scope of the claim covering the product as approved. Laws governing analogous PTEs in foreign jurisdictions vary widely, as do laws governing the ability to obtain multiple patents from a single patent family. Additionally, we may not receive an extension if we fail to exercise due diligence during the testing phase or regulatory review process, apply within applicable deadlines, fail to apply prior to expiration of relevant patents or otherwise fail to satisfy applicable requirements. If we are unable to obtain PTE or restoration, or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product will be shortened and our competitors may obtain approval of competing products following our patent expiration and may take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data to launch their product earlier than might otherwise be the case, which could materially adversely affect our business, financial condition, results of operations and prospects.

Furthermore, our patents covering certain components of our product candidates may expire prior to the commercialization of our product candidates or soon thereafter. As a result, third parties may be able to utilize these components of our products after expiration of these patents.

Even if we or our licensors obtain patents covering our product candidates, when the terms of all patents covering a product expire, our business may become subject to competition from competitive products, including generic products. Given the amount of time required for the development, testing, and regulatory review and approval of new product candidates, patents protecting such candidates may expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. For example, we have licensed patents from vTv Therapeutics that cover composition of matter of mavodelpar, which are set to expire in 2026, absent any patent term adjustments or extensions.

If we do not obtain patent term extension for mavodelpar, our business may be materially harmed.

Depending upon the timing, duration, and specifics of any FDA marketing approval of mavodelpar, or any future product candidate we may develop, one or more of patents issuing from our U.S. patent applications may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Action of 1984 (Hatch-Waxman Amendments). The Hatch-Waxman Amendments permit a PTE of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot

extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. Similar patent term restoration provisions to compensate for commercialization delay caused by regulatory review are also available in certain foreign jurisdictions, such as in Europe under Supplemental Protection Certificate (SPC). If we encounter delays in our development efforts, including our clinical trials, the period of time during which we could market mavodelpar and any future product candidates under patent protection would be reduced. Additionally, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents, or otherwise fail to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration, or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product will be shortened and our competitors may obtain approval of competing products following our patent expiration, and our revenue may be materially reduced. Further, if this occurs, our competitors may take advantage of our investment in development and trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case.

We cannot ensure that patent rights relating to inventions described and claimed in our pending patent applications will issue or that patents based on our patent applications will not be challenged and rendered invalid and/or unenforceable.*

We have pending U.S., international (*i.e.*, PCT), and other foreign patent applications in our portfolio relating to mavodelpar. However, we cannot predict:

- if and when patents may issue based on our patent applications;
- the scope of protection of any patent issuing based on our patent applications;
- whether the claims of any patent issuing based on our patent applications will provide protection against competitors,
- whether or not third parties will find ways to invalidate or circumvent our patent rights;
- whether or not others will obtain patents claiming aspects similar to those covered by our patents and patent applications;
- whether we will need to initiate litigation or administrative proceedings to enforce and/or defend our patent rights which will be costly whether we win or lose; and/or
- whether the patent applications that we own or in-license will result in issued patents with claims that cover our product candidates or uses thereof.

We cannot be certain that the claims in our pending patent applications directed to our product candidates, as well as technologies relating to our research programs will be considered patentable by the USPTO or by patent offices in foreign countries. One aspect of the determination of patentability of our inventions depends on the scope and content of the “prior art,” information that was or is deemed available to a person of skill in the relevant art prior to the priority date of the claimed invention. There may be prior art of which we are not aware that may affect the patentability of our patent claims or, if issued, affect the validity or enforceability of a patent claim relevant to our business. There is no assurance that there is not prior art of which we are aware, but which we do not believe is relevant to our business, which may, nonetheless, ultimately be found to limit our ability to make, use, sell, offer for sale or import our products that may be approved in the future, or impair our competitive position. Even if the patents do issue based on our patent applications, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, patents in our portfolio may not adequately exclude third parties from practicing relevant technology or prevent others from designing around our claims. If the breadth or strength of our intellectual property position with respect to our product candidates is threatened, it could dissuade companies from collaborating with us to develop and threaten our ability to commercialize our

product candidates. In the event of litigation or administrative proceedings, we cannot be certain that the claims in any of our issued patents will be considered valid by courts in the United States or foreign countries.

Derivation proceedings may be necessary to determine priority of inventions, and an unfavorable outcome may require us to cease using the related technology or to attempt to license rights from the prevailing party.

Derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our future licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of derivation proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with such proceedings could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our development programs, license necessary technology from third parties or enter into development or manufacturing partnerships that would help us bring our product candidates to market.

If the scope of any patent protection we obtain is not sufficiently broad, or if we lose any of our patent protection and/or other market exclusivity, our ability to prevent our competitors from commercializing similar or identical product candidates may be adversely affected.

The patent position of biotechnology and pharmaceutical companies is highly uncertain and involves complex legal, scientific, and factual questions and has been the subject of frequent litigation in recent years. As a result, the issuance, scope, validity, enforceability, and commercial value of our patent rights are highly uncertain. Our patent applications may not result in patents being issued which protect mavodelpar, any future product candidates, and other proprietary technologies we may develop or which effectively prevent others from commercializing competitive technologies and products. Further, no consistent policy regarding the breadth of claims allowed in pharmaceutical patents has emerged to date in the United States and in many jurisdictions outside of the United States. Changes in either the patent laws or interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property. Accordingly, we cannot predict the breadth of claims that may be enforced in the patents that may be issued from the applications we currently or may in the future own or license from third parties. Further, if any patents we obtain or license are deemed invalid and unenforceable, our ability to commercialize or license our technology could be adversely affected.

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if patent applications we own or in-license in the future issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Any patents that we own or in-license may be challenged or circumvented by third parties or may be narrowed or invalidated as a result of challenges by third parties. Consequently, we do not know whether our product candidates will be protectable or remain protected by valid and enforceable patents. Our competitors or other third parties may be able to circumvent our patents or the patents of our future licensors by developing similar or alternative technologies or products in a non-infringing manner which could materially adversely affect our business, financial condition, results of operations and prospects.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents or the patents of our future licensors may be challenged in the courts or patent offices in the United States and abroad. We may be subject to a third-party pre-issuance submission of prior art to the USPTO, or become involved in opposition, derivation, revocation, reexamination, post-grant review (PGR) and inter partes review (IPR), or other similar proceedings challenging our owned patent rights. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate or render unenforceable, our patent rights, allow third parties to commercialize our product candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent

rights. Moreover, our patents or the patents of our future licensors may become subject to post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge our priority of invention or other features of patentability with respect to our patents and patent applications and those of our future licensors. Such challenges may result in loss of patent rights, loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our product candidates. Such proceedings also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. In addition, if the breadth or strength of protection provided by our patents and patent applications or the patents and patent applications of our future licensors is threatened, regardless of the outcome, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

The patent application process is subject to numerous risks and uncertainties, and there can be no assurance that we or any of our actual or potential future collaborators will be successful in protecting mavodelpar, any future product candidates, and other proprietary technologies and their uses by obtaining, defending and enforcing patents. These risks and uncertainties include the following:

- the USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process, the noncompliance with which can result in abandonment or lapse of a patent or patent application, and partial or complete loss of patent rights in the relevant jurisdiction;
- patent applications may not result in any patents being issued;
- patents that may be issued or in-licensed may be challenged, invalidated, modified, revoked, circumvented, found to be unenforceable, or may otherwise not provide any competitive advantage;
- our competitors, many of whom have substantially greater resources than we do and many of whom have made significant investments in competing technologies, may seek or may have already obtained patents that will limit, interfere with, or eliminate our ability to make, use and sell our potential product candidates;
- other parties may have designed around our claims or developed technologies that may be related or competitive to our platform, may have filed or may file patent applications and may have received or may receive patents that overlap or conflict with our patent applications, either by claiming the same composition of matter, methods or formulations or by claiming subject matter that could dominate our patent position;
- any successful opposition to any patents owned by or licensed to us could deprive us of rights necessary for the practice of our technologies or the successful commercialization of any products or product candidates that we may develop;
- because patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that we or our licensors were the first to file any patent application related to mavodelpar, any future product candidates, and other proprietary technologies and their uses;
- an interference proceeding can be provoked by a third party or instituted by the USPTO to determine who was the first to invent any of the subject matter covered by the patent claims of our applications for any application with an effective filing date before March 16, 2013;
- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns; and

- countries other than the United States may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop, and market competing product candidates in those countries.

The patent prosecution process is expensive, time-consuming, and complex, and we may not be able to file, prosecute, or maintain all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach such agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection for such output. In addition, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our inventions and the prior art allow our inventions to be patentable over the prior art. Furthermore, publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in any of our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

Intellectual property rights are uncertain and do not necessarily address all potential threats to our competitive advantage.*

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. If we do not adequately protect our intellectual property and proprietary technology, competitors may be able to use mavodelpar, any future product candidates, and other proprietary technologies and erode or negate any competitive advantage we may have, which could have a material adverse effect on our financial condition and results of operations. For example:

- others may be able to make compounds that are similar to mavodelpar and any future product candidates but that are not covered by the claims of our patents;
- we might not have been the first to make the inventions covered by our pending patent applications;
- we might not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- any patents that we obtain may not provide us with any competitive advantages;
- it is possible that the pending patent applications we own or license will not lead to issued patents;
- issued patents that we own or license may be held invalid or unenforceable, as a result of legal challenges by our competitors;
- we may not develop additional proprietary technologies that are patentable;
- our competitors might conduct research and development activities in countries where we do not have patent rights or where patent protection is weak and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we cannot ensure that any of our patents, or any of our pending patent applications, if issued, or those of our licensors, will include claims having a scope sufficient to protect our products;

- there may be significant pressure on the U.S. government and international governmental bodies to limit the scope of patent protection both inside and outside the United States for disease treatments that prove successful, as a matter of public policy regarding worldwide health concerns;
- countries other than the United States may have patent laws less favorable to patentees than those upheld by U.S. courts, allowing foreign competitors a better opportunity to create, develop and market competing product candidates;
- we cannot ensure that we will be able to successfully commercialize our products on a substantial scale, if approved, before the relevant patents that we own or license expire; or
- the patents of others may have an adverse effect on our business.

Others have filed, and in the future are likely to file, patent applications covering products and technologies that are similar, identical or competitive to ours or important to our business. We cannot be certain that any patent application owned by a third party will not have priority over patent applications filed or in-licensed by us, or that we or our licensors will not be involved in interference, opposition or invalidity proceedings before U.S. or non-U.S. patent offices.

We cannot be certain that the claims in our issued patents and pending patent applications covering mavodelpar or any future product candidates will be considered patentable by the USPTO, courts in the United States, or by patent offices and courts in foreign countries. Furthermore, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property internationally.

The strength of patents in the biotechnology and pharmaceutical fields involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover mavodelpar and any future product candidates in the United States or in foreign countries. Even if such patents do successfully issue, third parties may challenge the ownership, validity, enforceability, or scope thereof, which may result in such patents being narrowed, invalidated, or held unenforceable. Any successful opposition to our patents could deprive us of exclusive rights necessary for the successful commercialization of mavodelpar and any future product candidates. Furthermore, even if they are unchallenged, our patents may not adequately protect our intellectual property, provide exclusivity for mavodelpar or any future product candidates or prevent others from designing around our claims. If the breadth or strength of protection provided by the patents we hold with respect to mavodelpar or any future product candidates is threatened, it could dissuade companies from collaborating with us to develop, or threaten our ability to commercialize, mavodelpar or any future product candidates.

Composition of matter patents for pharmaceutical product candidates, in particular patents with claims covering the molecular structure of the active pharmaceutical ingredient, often provide the strongest form of intellectual property protection for those types of products, as such patents provide protection without regard to any variations in formulation, method of use, or manufacturing process of the product. While we have an exclusive license to compositions of matter patents covering the molecular structure of mavodelpar, those patents will expire, absent patent term adjustment or extension, before the expiration of any regulatory exclusivity period that we may receive for mavodelpar. We also own two issued patents in the United States, that are expected to expire in 2041, absent any patent term adjustments or extensions, one pending application in the United States, and 15 pending applications in foreign countries directed to polymorphs of mavodelpar. We cannot be certain that the claims in our pending patent applications directed to the polymorphs of mavodelpar will be considered patentable by patent offices in foreign countries, or that the claims in any of our issued patents will be considered valid and enforceable by courts in the United States or foreign countries. Method of use patents protect the use of a product for the specified method. This type of patent does not prevent a competitor from making and marketing a product that is identical to our product for an indication that is outside the scope of the patented method. Moreover, even if competitors do not actively promote their product for our targeted indications, physicians may prescribe these products “off-label.” Although off-label prescriptions may infringe or contribute to the infringement of method of

use patents, the practice is common and such infringement is difficult to prevent or prosecute. Method of synthesis patents protect the method used to manufacture a product. This type of patent does not prevent a competitor from making and marketing a product that is identical to our product so long as it is made in a different way.

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications or those of our future licensors and the enforcement or defense of our issued patents or those of our future licensors.

In September 2011, the Leahy-Smith America Invents Act, or America Invents Act, was signed into law. The America Invents Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. In particular, under the Leahy-Smith Act, the United States transitioned in March 2013 to a "first inventor to file" system in which, assuming that other requirements of patentability are met, the first inventor to file a patent application will be entitled to the patent regardless of whether a third party was first to invent the claimed invention. A third party that files a patent application in the USPTO after March 2013 but before us could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Furthermore, our ability to obtain and maintain valid and enforceable patents depends on whether the differences between our technology and the prior art allow our technology to be patentable over the prior art. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we may not be certain that we or our future licensors are the first to either (1) file any patent application related to our product candidates or (2) invent any of the inventions claimed in the patents or patent applications.

The Leahy-Smith Act also includes a number of significant changes that affect the way patent applications will be prosecuted and also may affect patent litigation. These include allowing third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including PGR, IPR, and derivation proceedings. An adverse determination in any such submission or proceeding could reduce the scope or enforceability of, or invalidate, our patent rights, which could adversely affect our competitive position.

Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Thus, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications or those of our future licensors and the enforcement or defense of our issued patents or those of our future licensors, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

For U.S. patent applications in which claims are entitled to a priority date before March 16, 2013, an interference proceeding can be provoked by a third party or instituted by the USPTO to determine who was the first to invent any of the subject matter covered by the patent claims of our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our participation in an interference proceeding may fail and, even if successful, may result in substantial costs and distract our management and other employees.

Changes in U.S. patent law, or patent laws in other countries, could diminish the value of patents in general, thereby impairing our ability to protect mavodelpar, any future product candidates, and other proprietary technologies.

As is the case with other biotechnology and pharmaceutical companies, our success is heavily dependent on intellectual property, particularly on obtaining and enforcing patents. Obtaining and enforcing patents in the pharmaceutical industry involves a high degree of technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. Therefore, our patent rights may be affected by developments or uncertainty in U.S. or foreign patent statutes, patent case law, USPTO rules and regulations or the rules and regulations of foreign patent offices. In addition, the United States may, at any time, enact changes to U.S. patent law and regulations, including by legislation, by regulatory rulemaking, or by judicial precedent, that adversely affect the scope of patent protection available and weaken the rights of patent owners to obtain patents, enforce patent infringement and obtain injunctions and/or damages. For example, over the past several years the Court of Appeals for the Federal Circuit and the Supreme Court issued various opinions, and the USPTO modified its guidance for practitioners on multiple occasions, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Other countries may likewise enact changes to their patent laws in ways that adversely diminish the scope of patent protection and weaken the rights of patent owners to obtain patents, enforce patent infringement, and obtain injunctions and/or damages. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. We cannot predict the breadth of claims that may be allowed or enforced in our patents or in third-party patents, and whether Congress or other foreign legislative bodies may pass patent reform legislation that is unfavorable to us.

Further, the United States and other governments may, at any time, enact changes to law and regulation that create new avenues for challenging the validity of issued patents. For example, the America Invents Act created new administrative post-grant proceedings, including post-grant review, inter partes review, and derivation proceedings that allow third parties to challenge the validity of issued patents. This applies to all of our U.S. patents, even those issued before March 16, 2013. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in U.S. federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

After March 2013, under the America Invents Act, the United States transitioned to a first inventor to file system in which, assuming that the other statutory requirements are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third-party was the first to invent the claimed invention. A third party that files a patent application in the USPTO after March 2013, but before we file an application covering the same invention, could therefore be awarded a patent covering an invention of ours even if we had made the invention before it was made by such third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensors were the first to either (i) file any patent application related to our product candidates and other proprietary technologies we may develop or (ii) invent any of the inventions claimed in our or our licensor's patents or patent applications. Even where we have a valid and enforceable patent, we may not be able to exclude others from practicing the claimed invention where the other party can show that they used the invention in commerce before our filing date or the other party benefits from a compulsory license. However, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may not be able to protect our intellectual property rights throughout the world.*

Patents are of national or regional effect. Filing, prosecuting, and defending patents on mavodelpar, any future product candidates, and other proprietary technologies we develop in all countries throughout the world would be prohibitively expensive. In addition, the laws of some foreign countries do not protect intellectual property rights in the same manner and to the same extent as laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but enforcement of such patent protection is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

The requirements for patentability may differ in certain countries. For example, unlike other countries, China has a heightened requirement for patentability, and specifically requires a detailed description of medical uses of a claimed drug. In India, unlike the United States, there is no link between regulatory approval for a drug and its patent status. In addition to India, certain countries in Europe and developing countries, including China, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, some countries limit the enforceability of patents against government agencies or government contractors.

In those countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we own or license.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology or pharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. As an example, European applications have the option, upon grant of a patent, of becoming a Unitary Patent which is subject to the jurisdiction of the Unitary Patent Court (UPC). The option of a Unitary Patent is a significant change in European patent practice. As the UPC is a new court system, there is no precedent for the court, increasing the uncertainty. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly, and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Further, the standards applied by the USPTO and foreign patent offices in granting patents are not always applied uniformly or predictably. As such, we do not know the degree of future protection that we will have on our technologies, products and product candidates. While we will endeavor to try to protect our technologies, products and product candidates with intellectual property rights such as patents, as appropriate, the process of obtaining patents is time consuming, expensive and unpredictable.

Further, geo-political actions in the United States and in foreign countries could increase the uncertainties and costs surrounding the prosecution or maintenance of our patent applications or those of any current or future licensors and the maintenance, enforcement or defense of our issued patents or those of any current or future licensors. For example, the United States and foreign government actions related to Russia's invasion of Ukraine may limit or prevent filing, prosecution and maintenance of patent applications in Russia. Government actions may

also prevent maintenance of issued patents in Russia. These actions could result in abandonment or lapse of our patents or patent applications, resulting in partial or complete loss of patent rights in Russia. If such an event were to occur, it could have a material adverse effect on our business. In addition, a decree was adopted by the Russian government in March 2022, allowing Russian companies and individuals to exploit inventions owned by patentees that have citizenship or nationality in, are registered in, or have predominately primary place of business or profit-making activities in the United States and other countries that Russia has deemed unfriendly without consent or compensation. Consequently, we would not be able to prevent third parties from practicing our inventions in Russia or from selling or importing products made using our inventions in and into Russia. Accordingly, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

We may become subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may be subject to claims that former employees (including former employees of our licensors), collaborators or other third parties have an interest in our patent rights, trade secrets, or other intellectual property as an inventor or co-inventor. The failure to name the proper inventors on a patent application can result in the patents issuing thereon being unenforceable. For example, we may have inventorship disputes arise from conflicting views regarding the contributions of different individuals named as inventors, the effects of foreign laws where foreign nationals are involved in the development of the subject matter of the patent, conflicting obligations of third parties involved in developing mavodelpar or as a result of questions regarding co-ownership of potential joint inventions. Litigation may be necessary to resolve these and other claims challenging inventorship and/or ownership. Alternatively, or additionally, we may enter into agreements to clarify the scope of our rights in such intellectual property. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business, financial condition, results of operations and prospects. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Our current and future licensors may have relied on third-party consultants or collaborators or on funds from third parties, such that our licensors are not the sole and exclusive owners of the patents we in-licensed. If other third parties have ownership rights or other rights to our in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing, or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Such claims could have a material adverse effect on our business, financial condition, results of operations, and prospects.

We may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in-licenses.

Presently we have intellectual property rights through licenses from third parties, including vTv Therapeutics, related to mavodelpar. Because our program may require the use of additional proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights. In addition, mavodelpar may require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license, on reasonable terms, proprietary

rights related to any compositions, formulations, methods of use, processes or other intellectual property rights from third parties that we identify as being necessary for mavodelpar. In such event, we may be required to expend significant time and resources to develop or license replacement technology, which may not be available. Even if we are able to obtain a license to such proprietary rights, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us.

The patent protection and patent prosecution for mavodelpar and any future product candidates may be dependent on third parties.*

While we normally seek to obtain the right to control prosecution, maintenance and enforcement of the patents relating to our product candidates, there may be times when the filing and prosecution activities for patents and patent applications relating to our product candidates are controlled by our future licensors or collaboration partners. When we obtain licenses from or collaborate with third parties, we may not have the right to control the preparation, filing, and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties, or such activities, if controlled by us, may require the input of such third parties. We may also require the cooperation of our licensors and collaborators to enforce any licensed patent rights, and such cooperation may not be provided. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business, or in compliance with applicable laws and regulations, including by payment of all applicable fees for patents covering our product candidates, which may affect the validity and enforceability of such patents or any patents that may issue from such application. If any of our future licensors or collaboration partners fail to prosecute, maintain and enforce such patents and patent applications in a manner consistent with the best interests of our business, including by payment of all applicable fees for patents covering our product candidates, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, our ability to develop and commercialize those product candidates may be adversely affected and we may not be able to prevent competitors from making, using and selling competing products. In addition, even where we have the right to control patent prosecution of patents and patent applications we have licensed to and from third parties, we may still be adversely affected or prejudiced by actions or inactions of our licensees, our future licensors and their counsel that took place prior to the date upon which we assumed control over patent prosecution.

Moreover, if we do obtain necessary licenses, we will likely have obligations under those licenses, including making royalty and milestone payments, and any failure to satisfy those obligations could give our licensor the right to terminate the license. Termination of a necessary license, or expiration of licensed patents or patent applications, could have a material adverse impact on our business. Our business would suffer if any such licenses terminate, if the licensors fail to abide by the terms of the license, if the licensors fail to enforce licensed patents against infringing third parties, if the licensed patents or other rights are found to be invalid or unenforceable, or if we are unable to enter into necessary licenses on acceptable terms. Furthermore, if any licenses terminate, or if the underlying patents fail to provide the intended exclusivity, competitors or other third parties may gain the freedom to seek regulatory approval of, and to market, products identical or similar to ours. Moreover, our licensors may own or control intellectual property that has not been licensed to us and, as a result, we may be subject to claims, regardless of their merit, that we are infringing or otherwise violating the licensor's rights. In addition, while we cannot currently determine the amount of the royalty obligations we would be required to pay on sales of future products, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in products that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize products, we may be unable to achieve or maintain profitability.

Our rights to develop and commercialize our technology and product candidates may be subject, in part, to the terms and conditions of licenses granted to us by others.

Moreover, some of our owned and in-licensed patents or patent applications in the future may be co-owned with third parties. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties,

including our competitors, and our competitors could market competing products and technology. In addition, we may need the cooperation of any such co-owners of our patents in order to enforce such patents against third parties, and such cooperation may not be provided to us. Furthermore, our owned and in-licensed patents may be subject to retained rights by one or more third parties. Any of the foregoing could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

The licensing and acquisition of third-party proprietary rights is a competitive area, and companies, which may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party proprietary rights that we may consider necessary or attractive in order to commercialize mavodelpar. More established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

It is possible that we may be unable to obtain licenses at a reasonable cost or on reasonable terms, if at all. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to redesign our technology, product candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business, financial condition, results of operations, and prospects significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current technology, manufacturing methods, product candidates, or future methods or products resulting in either an injunction prohibiting our manufacture or future sales, or, with respect to our future sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us, either on reasonable terms, or at all. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment, or at all. If we are unable to successfully obtain rights to required third-party intellectual property rights on commercially reasonable terms, our ability to commercialize our products, and our business, financial condition, and prospects for growth, could suffer.

We may enter into license agreements in the future with others to advance our existing or future research or allow commercialization of our existing or future product candidates. These licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and products in the future.

For example, we may collaborate with U.S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate an exclusive license to any of the institution's proprietary rights in technology resulting from the collaboration. Regardless of such option to negotiate a license, we may be unable to negotiate a license within the specified time frame or under terms that are acceptable to us. If we are unable to do so, the institution may offer, on an exclusive basis, their proprietary rights to other parties, potentially blocking our ability to pursue our program.

In addition, subject to the terms of any such license agreements, we may not have the right to control the preparation, filing, prosecution, maintenance, enforcement, and defense of patents and patent applications covering the technology that we license from third parties. In such an event, we cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced, and defended in a manner consistent with the best interests of our business. If our future licensors fail to prosecute, maintain, enforce, and defend such patents or patent applications, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated, and our right to develop and commercialize any of our future product candidates that are subject of such licensed rights could be adversely affected.

Our future licensors may rely on third-party consultants or collaborators or on funds from third parties such that our future licensors are not the sole and exclusive owners of the patents we in-license. If other third parties have ownership rights to our future in-licensed patents, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties, such as the vTv License Agreement, or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.*

We are a party to the vTv License Agreement under which we are granted intellectual property rights that are important to our business and our only product candidate, mavodelpar. If we fail to comply with our obligations under the license agreement, or we are subject to insolvency, the license agreement may be terminated, in which event we would not be able to develop, commercialize or market mavodelpar.

The agreements under which we license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property that we license in the future prevent or impair our ability to maintain our licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

In spite of our best efforts, our current and future licensor(s) might conclude that we materially breached our license agreements and might therefore terminate the license agreements, thereby removing our ability to develop and commercialize products and technology covered by these license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products identical to ours. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Disputes may arise between us and our licensors regarding intellectual property rights subject to a license agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property rights of the licensor that are not subject to the license agreement;
- our right to sublicense intellectual property rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of mavodelpar, and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

We are generally also subject to all of the same risks with respect to protection of intellectual property that we license as we are for intellectual property that we own, which are described herein. If we or our licensor(s) fail to adequately protect this intellectual property, our ability to develop, manufacture or commercialize products could suffer.

If disputes over intellectual property rights that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, our business, results of operations, financial condition, and prospects may be adversely affected. We may enter into additional licenses in the future and if we fail to comply with obligations under those agreements, we could suffer adverse consequences.

In the future, we may need to obtain additional licenses of third-party technology that may not be available to us or are available only on commercially unreasonable terms, and which may cause us to operate our business in a more costly or otherwise adverse manner that was not anticipated.

From time to time, we may be required to license technologies relating to our therapeutic research programs from additional third parties to further develop or commercialize our product candidates. Should we be required to obtain licenses to any third-party technology, including any such patents required to manufacture, use or sell our product candidates, such licenses may not be available to us on commercially reasonable terms, or at all. The inability to obtain any third-party license required to develop or commercialize any of our product candidates could cause us to abandon any related efforts, which could seriously harm our business and operations.

Any collaboration arrangements that we may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize our products.

Any future collaborations that we enter into may not be successful. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborations are subject to numerous risks, which may include that:

- collaborators have significant discretion in determining the efforts and resources that they will apply to collaborations;
- collaborators may not pursue development and commercialization of our products or may elect not to continue or renew development or commercialization programs based on trial or test results, changes in their strategic focus due to the acquisition of competitive products, availability of funding, or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with mavodelpar and any future product candidates;
- a collaborator with marketing, manufacturing, and distribution rights to one or more products may not commit sufficient resources to or otherwise not perform satisfactorily in carrying out these activities;
- we could grant exclusive rights to our collaborators that would prevent us from collaborating with others;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that causes the delay or termination of the research, development, or commercialization of our current or future products or that results in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated, and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable current or future products;
- collaborators may own or co-own intellectual property covering our products that results from our collaborating with them, and in such cases, we would not have the exclusive right to develop or commercialize such intellectual property; and

- a collaborator's sales and marketing activities or other operations may not be in compliance with applicable laws resulting in civil or criminal proceedings.

Third-party claims alleging intellectual property infringement may prevent or delay our drug discovery and development efforts.*

Our success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. However, our research, development and commercialization activities may be subject to claims that we infringe, misappropriate or otherwise violate patents or other intellectual property rights owned or controlled by third parties. Other entities may have or obtain patents or proprietary rights that could limit our ability to make, use, sell, offer for sale or import our product candidates and products that may be approved in the future, or impair our competitive position. There is a substantial amount of litigation, both within and outside the United States, involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including inter partes review, post grant review, interference and reexamination proceedings before the USPTO, or oppositions and other comparable proceedings in foreign jurisdictions. The implementation of these procedures brings uncertainty to the possibility of challenges to our patents in the future and the outcome of such challenges. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing mavodelpar.

Litigation or other legal proceedings relating to intellectual property claims, with or without merit, is unpredictable and generally expensive and time consuming and, even if resolved in our favor, is likely to divert significant resources from our core business, including distracting our technical and management personnel from their normal responsibilities. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our activities related to mavodelpar may give rise to claims of infringement of the patent rights of others. The biotechnology and pharmaceutical industries have produced a proliferation of patents, and it is not always clear to industry participants, including us, which patents cover various types of products or methods of use. Identification of third-party patent rights that may be relevant to our operations is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We cannot guarantee that any of our patent searches or analyses, including the identification of relevant patents, the scope of patent claims or the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States and abroad that is relevant to our research and other operations or necessary for the commercialization of our product candidates in any jurisdiction. We also cannot provide any assurances that third-party patents do not exist which might be enforced against our current technology, including our research programs, product candidates, their respective methods of use, manufacture and formulations thereof, and could result in either an injunction prohibiting our manufacture or future sales, or, with respect to our future sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties, which could be significant. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. We cannot assure you that any of our current or future product candidates will not infringe existing or future patents. We may not be aware of patents that have already issued that a third party might assert are infringed by one of our current or future product candidates. Nevertheless, we are not aware of any issued patents that will prevent us from marketing mavodelpar.

Third parties, including our competitors, in both the United States and abroad, many of which have substantially greater resources and have made substantial investments in patent portfolios and competing

technologies, may have applied for or obtained or may in the future apply for and obtain, patents that will prevent, limit or otherwise interfere with our ability to make, use and sell our products. We do not always conduct independent reviews of pending patent applications and patents issued to third parties. Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents of which we are currently unaware with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of mavodelpar and any future product candidates. Because patent applications can take many years to issue and may be confidential for 18 months or more after filing, there may be currently pending third-party patent applications which may later result in issued patents that mavodelpar, any future product candidates, and other proprietary technologies may infringe, or which such third parties claim are infringed by the use of our technologies. Parties making claims against us for infringement or misappropriation of their intellectual property rights may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize mavodelpar or future product candidates. Defense of these claims, regardless of their merit, could involve substantial expenses and could be a substantial diversion of management and other employee resources from our business.

If we collaborate with third parties in the development of technology in the future, our collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to litigation or potential liability. Further, collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability. In the future, we may agree to indemnify our commercial collaborators against certain intellectual property infringement claims brought by third parties.

Any claims of patent infringement asserted by third parties would be time-consuming and could:

- result in costly litigation;
- cause negative publicity;
- divert the time and attention of our technical personnel and management;
- cause development delays;
- prevent us from commercializing mavodelpar or any future product candidates until the asserted patent expires or is finally held invalid, unenforceable, or not infringed in a court of law;
- require us to develop non-infringing technology, which may not be possible on a cost-effective basis;
- require us to pay damages to the party whose intellectual property rights we may be found to be infringing, which may include treble damages if we are found to have been willfully infringing such intellectual property;
- require us to pay the attorney's fees and costs of litigation to the party whose intellectual property rights we may be found to be willfully infringing; and/or
- require us to enter into royalty or license agreements, which may not be available on commercially reasonable terms, or at all, or which might be non-exclusive, which could result in our competitors gaining access to the same technology.

If we are sued for patent infringement, we would need to demonstrate that our products or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do either. Proving invalidity or unenforceability is difficult. For example, in the United States, proving invalidity before federal courts requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and divert management's time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, which may not be available, defend an infringement action or challenge the validity or enforceability of the patents in court. Patent litigation is costly and time-consuming. We may not have sufficient

resources to bring these actions to a successful conclusion. In addition, if we do not obtain a license, develop or obtain non-infringing technology, fail to defend an infringement action successfully or have infringed patents declared invalid or unenforceable, we may incur substantial monetary damages, encounter significant delays in bringing mavodelpar and any future product candidates to market and be precluded from developing, manufacturing or selling mavodelpar and any future product candidates.

We do not always conduct independent reviews of pending patent applications and patents issued to third parties. We cannot be certain that any of our or our licensors' patent searches or analyses, including but not limited to the identification of relevant patents, analysis of the scope of relevant patent claims or determination of the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States, Europe and elsewhere that is relevant to or necessary for the commercialization of our product candidates in any jurisdiction, because:

- some patent applications in the United States may be maintained in secrecy until the patents are issued;
- patent applications in the United States and elsewhere can be pending for many years before issuance, or unintentionally abandoned patents or applications can be revived;
- pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, mavodelpar, and any future product candidates or the use of mavodelpar and any future product candidates;
- identification of third-party patent rights that may be relevant to our technology is difficult because patent searching is imperfect due to differences in terminology among patents, incomplete databases, and the difficulty in assessing the meaning of patent claims;
- patent applications in the United States are typically not published until 18 months after the priority date; and
- publications in the scientific literature often lag behind actual discoveries.

Furthermore, the scope of a patent claim is determined by an interpretation of the law, the written disclosure in a patent and the patent's prosecution history and can involve other factors such as expert opinion. Our interpretation of the relevance or the scope of claims in a patent or a pending application may be incorrect, which may negatively impact our ability to market our products. Further, we may incorrectly determine that our technologies, products, or product candidates are not covered by a third-party patent or may incorrectly predict whether a third party's pending patent application will issue with claims of relevant scope. Our determination of the expiration date of any patent in the United States or internationally that we consider relevant may be incorrect, which may negatively impact our ability to develop and market our products or product candidates. Furthermore, we cannot guarantee that any of our patent searches or analyses, including the identification of relevant patents, the scope of patent claims or the expiration of relevant patents, are complete or thorough, nor can we be certain that we have identified each and every third-party patent and pending application in the United States and abroad that is relevant to or necessary for the commercialization of our product candidates in any jurisdiction.

Our competitors may have filed, and may in the future file, patent applications covering technology similar to ours, and others may have or obtain patents or proprietary rights that could limit our ability to make, use, sell, offer for sale or import mavodelpar and future approved products or impair our competitive position. Numerous third-party U.S. and foreign issued patents and pending patent applications exist in the fields in which we are developing product candidates. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of mavodelpar and any future product candidates. Any such patent application may have priority over our patent applications, which could further require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to ours, we may have to participate in an interference

proceeding declared by the USPTO to determine priority of invention in the United States. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful if, unbeknownst to us, the other party had independently arrived at the same or similar invention prior to our own invention, resulting in a loss of our U.S. patent position with respect to such inventions. Other countries have similar laws that permit secrecy of patent applications and may be entitled to priority over our applications in such jurisdictions.

Some third parties making claims against us may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our business, results of operations, financial condition and prospects.

If we are found to infringe a third party's intellectual property rights, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product, we may have to pay substantial damages, including treble damages and attorneys' fees if we are found to be willfully infringing a third party's patents, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure.

We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of mavodelpar and any future product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize mavodelpar and any future product candidates, which could harm our business significantly. Even if we were able to obtain a license, the rights may be nonexclusive, which may give our competitors access to the same intellectual property.

Although no third party has asserted a claim of patent infringement against us as of the date of this Quarterly Report on Form 10-Q, others may hold proprietary rights that could prevent our product candidates from being marketed. It is possible that a third party may assert a claim of patent infringement directed at any of our product candidates. Any patent-related legal action against us claiming damages and seeking to enjoin commercial activities relating to our product candidates, treatment indications, or processes could subject us to significant liability for damages, including treble damages if we were determined to willfully infringe, and require us to obtain a license to manufacture or market our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. We cannot predict whether we would prevail in any such actions or that any license required under any of these patents would be made available on commercially acceptable terms, if at all. Moreover, even if we or our future strategic partners were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. In addition, we cannot be certain that we could redesign our product candidates, treatment indications, or processes to avoid infringement, if necessary. Accordingly, an adverse determination in a judicial or administrative proceeding, or the failure to obtain necessary licenses, could prevent us from developing and commercializing our product candidates, which could harm our business, financial condition and operating results. In addition, intellectual property litigation, regardless of its outcome, may cause negative publicity and could prohibit us from marketing or otherwise commercializing our product candidates and technology.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming, and unsuccessful. Further, our issued patents could be found invalid or unenforceable if challenged in court, and we may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights.*

Third parties including competitors may infringe, misappropriate or otherwise violate our patents, patents that may issue to us in the future, or the patents of our licensors that are licensed to us. To stop or prevent

infringement or unauthorized use, we may need to or choose to file infringement claims, which can be expensive and time-consuming. We may not be able to stop or prevent, alone or with our licensors, infringement, misappropriation, or other violation of our intellectual property, particularly in countries where the laws may not protect those rights as fully as in the United States.

If we choose to go to court to stop another party from using the inventions claimed in our patents, a court may decide that a patent we own or in-license is not valid, is unenforceable and/or is not infringed by that third party for any number of reasons. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge include an alleged failure to meet any of several statutory requirements for patentability, including lack of novelty, obviousness, obviousness-type double patenting, lack of written description, indefiniteness, or non-enablement. Grounds for an unenforceability assertion could include an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution, i.e., committed inequitable conduct. Third parties may also raise similar claims before the USPTO, even outside the context of litigation, including re-examination, PGR, IPR, and derivation proceedings. Similar mechanisms for challenging the validity and enforceability of a patent exist in foreign patent offices and courts and may result in the revocation, cancellation, or amendment of any foreign patents we or our licensors hold now or in the future. The outcome following legal assertions of invalidity and unenforceability is unpredictable, and prior art could render our patents or those of our licensors invalid. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on such product candidate. Such a loss of patent protection would have a material adverse impact on our business. There is also a risk that, even if the validity of our patents is upheld, the court will decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover such invention, or decide that the other party's use of our patented technology falls under the safe harbor to patent infringement under 35 U.S.C. §271(e)(1).

With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our future licensors, and the patent examiners are unaware during prosecution. There is also no assurance that there is not prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim in our patents and patent applications or the patents and patent applications of our future licensors, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. If a third party were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our technology or platform, or any product candidates that we may develop. Such a loss of patent protection would have a material adverse impact on our business, financial condition, results of operations and prospects.

Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non-exclusive license is offered and our competitors gain access to the same technology. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development or manufacturing partnerships that would help us bring mavodelpar and any future product candidates to market.

Even if resolved in our favor, litigation or other legal proceedings relating to our intellectual property rights may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions, or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or

proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing, or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

Our ability to enforce our patent rights depends on our ability to detect infringement. It may be difficult to detect infringers who do not advertise the components or methods that are used in connection with their products and services. Moreover, it may be difficult or impossible to obtain evidence of infringement in a competitor's or potential competitor's product or service. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded if we were to prevail may not be commercially meaningful.

In addition, if the breadth or strength of protection provided by our patents and patent applications or the patents and patent applications of our future licensors is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

In addition, the issuance of a patent does not give us the right to practice the patented invention. Third parties may have blocking patents that could prevent us from marketing our own patented product(s) and practicing our own patented technologies.

Because of the expense and uncertainty of litigation, we may not be in a position to enforce our intellectual property rights against third parties.

Because of the expense and uncertainty of litigation, we may not be in a position to enforce our intellectual property rights against third parties and we may conclude that even if a third party is infringing our issued patent, any patents that may be issued as a result of our pending or future patent applications or other intellectual property rights, the risk-adjusted cost of bringing and enforcing such a claim or action may be too high or not in the best interest of our company or our stockholders. In addition, the uncertainties associated with litigation could compromise our ability to raise the funds necessary to continue our clinical trials, continue our internal research programs, in-license needed technology or other product candidates, or enter into development partnerships that would help us bring our product candidates to market. In such cases, we may decide that the more prudent course of action is to simply monitor the situation or initiate or seek some other non-litigious action or solution.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

We rely on trade secrets, including unpatented know-how, technology and other proprietary information, to protect our proprietary technologies and maintain our competitive position, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. We rely in part on confidentiality agreements with our employees, outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors, potential partners, and other third parties, to protect our trade secrets and other proprietary information. In addition to contractual measures, we try to protect the confidential nature of our trade secrets and other proprietary information using commonly accepted physical and technological security measures. Despite these efforts, we cannot provide any assurances that all such agreements have been duly executed, and these agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

In addition, such commonly accepted physical and technological security measures may not provide adequate protection for our proprietary information, for example, in the case of misappropriation of a trade secret by an employee, consultant, advisor, or other third party with authorized access. Our security measures may not prevent an employee, outside scientific collaborator, CRO, third-party manufacturer, consultant, advisor, potential partner, and other third party from misappropriating our trade secrets and providing them to a competitor, and recourse we take against such misconduct may not provide an adequate remedy to protect our interests fully. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. Unauthorized parties may also attempt to copy or reverse engineer certain aspects of our products that we consider proprietary. Even though we use commonly accepted security measures, the criteria for protection of trade secrets can vary among different jurisdictions. Further, we may need to share our proprietary information, including trade secrets, with our current and future business partners, collaborators, contractors and others located in countries at heightened risk of theft of trade secrets, including through direct intrusion by private parties or foreign actors, and those affiliated with or controlled by state actors.

Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. Moreover, third parties may still obtain this information or may come upon this or similar information independently, and we would have no right to prevent them from using that technology or information to compete with us. If any of these events occurs or if we otherwise lose protection for our trade secrets, the value of this information may be greatly reduced, and our competitive position would be harmed.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Elements of our product candidates, including processes for their preparation and manufacture, may involve proprietary know-how, and other proprietary information that is not covered by patents, and thus for these aspects we may consider trade secrets, including unpatented know-how, and other proprietary information to be our primary intellectual property. Any disclosure, either intentional or unintentional, by our employees, the employees of third parties with whom we share our facilities or outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors, and vendors that we engage to perform research, clinical trials or manufacturing activities, or misappropriation by third parties (such as through a cybersecurity breach) of our trade secrets or proprietary information could enable competitors to duplicate or surpass our technological achievements, thus eroding our competitive position in our market.

Trade secrets, including unpatented know-how, and other proprietary information, can be difficult to trace, protect and enforce. We require our employees to enter into written employment agreements containing provisions of confidentiality and obligations to assign to us any inventions generated in the course of their employment. We further seek to protect our potential trade secrets, proprietary know-how and information in part, by entering into non-disclosure and confidentiality agreements with parties who are given access to them, such as outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors, and other third parties. With our consultants, advisors, contractors and outside scientific collaborators, these agreements typically include invention assignment obligations. Although we have taken steps to protect our trade secrets and unpatented know-how, we cannot provide any assurances that all such agreements have been duly executed, and any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets and unpatented know-how, and we may not be able to obtain adequate remedies for such breaches. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective.

Trade secrets may be independently developed by others in a manner that could prevent legal recourse by us. Trade secrets will over time be disseminated within the industry through independent development, the publication of journal articles, and the movement of personnel skilled in the art from company to company or

academic to industry scientific positions. Though our agreements with third parties typically restrict the ability of our employees, outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors, potential partners, and other third parties, to publish data potentially relating to our trade secrets, our agreements may contain certain limited publication rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position. Because from time to time we expect to rely on third parties in the development, manufacture, and distribution of our products and provision of our services, we must, at times, share trade secrets with them. Despite employing the contractual and other security precautions described above, the need to share trade secrets increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. If any of these events occurs or if any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, or if we otherwise lose protection for our trade secrets, the value of this information may be greatly reduced and our competitive position would be harmed and we would have no right to prevent them from using that technology or information to compete with us. If we do not apply for patent protection prior to such publication or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized.

We may be subject to claims that we or our employees, outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors, potential partners, and other third parties have wrongfully used or disclosed alleged confidential information or trade secrets of their former employers.

We have entered into and may enter in the future into non-disclosure and confidentiality agreements to protect the proprietary positions of third parties, such as outside scientific collaborators, CROs, third-party manufacturers, consultants, advisors, potential partners, and other third parties. We may become subject to litigation where a third party asserts that we or our employees inadvertently or otherwise breached the agreements and used or disclosed trade secrets or other information proprietary to the third parties. We may also be subject to claims that we have wrongfully hired an employee from a competitor. Defense of such matters, regardless of their merit, could involve substantial litigation expense and be a substantial diversion of employee resources from our business. We cannot predict whether we would prevail in any such actions. Moreover, intellectual property litigation, regardless of its outcome, may cause negative publicity and could prohibit us from marketing or otherwise commercializing our product candidates and technology. Failure to defend against any such claim could subject us to significant liability for monetary damages or prevent or delay our developmental and commercialization efforts, which could adversely affect our business. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to our management team and other employees.

Parties making claims against us may be able to sustain the costs of complex intellectual property litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, operating results, financial condition and prospects.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

Our current or future trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks, and we may be unable to obtain future trademarks or trade names that we intend to use. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other trademarks or trademarks that incorporate variations

of our registered or unregistered trademarks or trade names. During trademark registration proceedings, we may receive rejections of our applications by the USPTO or in other foreign jurisdictions. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the USPTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. We may license our trademarks and trade names to third parties, such as distributors. Though these license agreements may provide guidelines for how our trademarks and trade names may be used, a breach of these agreements or misuse of our trademarks and tradenames by our licensees may jeopardize our rights in or diminish the goodwill associated with our trademarks and trade names. Our efforts to enforce or protect our proprietary rights related to trademarks, trade names, trade secrets, domain names, copyrights, or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could adversely affect our financial condition or results of operations.

Moreover, any name we have proposed to use with mavodelpar in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA (or an equivalent administrative body in a foreign jurisdiction) objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties, and be acceptable to the FDA. Similar requirements exist in Europe. Furthermore, in many countries, owning and maintaining a trademark registration may not provide an adequate defense against a subsequent infringement claim asserted by the owner of a senior trademark. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. If we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Risks Related to Ownership of Our Common Stock

An active, liquid and orderly trading market for our common stock may not be sustained.

Prior to the closing of our initial public offering (IPO) in April 2021, there was no public market for shares of our common stock. An active trading market for our shares may not be sustained. You may not be able to sell your shares quickly or at the market price if trading in shares of our common stock is not active. As a result of these and other factors, you may be unable to resell your shares of our common stock at or above the price at which they were purchased. Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic partnerships or acquire companies or products by using our shares of common stock as consideration.

The price of our stock may be volatile, and you could lose all or part of your investment.*

The trading price of our common stock is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. In addition to the factors discussed in this “Risk Factors” section and elsewhere in this Quarterly Report on Form 10-Q, these factors include:

- the commencement, enrollment or results of our ongoing and planned clinical trials of mavodelpar or any future clinical trials we may conduct for any future product candidates, or changes in the development status of mavodelpar or any future product candidates;

- acceptance by the FDA and EMA of data from our ongoing pivotal STRIDE study or any future clinical trials we conduct;
- any delay in our regulatory filings for mavodelpar and any future product candidates;
- adverse results or delays in clinical trials or preclinical studies;
- our decision to initiate a clinical trial, not to initiate a clinical trial, or to terminate an existing clinical trial;
- adverse regulatory decisions, including failure to receive regulatory approval for mavodelpar and any future product candidates;
- changes in laws or regulations applicable to mavodelpar and any future product candidates, including but not limited to clinical trial requirements for approvals;
- our failure to commercialize mavodelpar and any future product candidates;
- the failure to obtain coverage and adequate reimbursement of mavodelpar and any future product candidates, if approved;
- changes in the structure of healthcare payment systems;
- adverse developments concerning our manufacturers;
- our inability to obtain adequate product supply for any approved drug product or inability to do so at acceptable prices;
- additions or departures of key scientific or management personnel;
- unanticipated serious safety concerns related to the use of mavodelpar and any future product candidates;
- introduction of new products or services offered by us or our competitors, or the release or publication of clinical trial results from competing product candidates;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- the size and growth, if any, of the markets for patients with PMM and LC-FAOD, and other rare genetic mitochondrial diseases that we may target;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- developments with respect to our intellectual property rights;
- our commencement of, or involvement in, litigation; and
- general political and economic conditions, including those resulting from armed conflicts, infectious diseases, and bank failures.

In addition, the stock market in general, and pharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because pharmaceutical companies have

experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

Our executive officers and directors, combined with our stockholders who own more than 5% of our outstanding capital stock, beneficially own shares representing a significant percentage of our common stock. Therefore, these stockholders will have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to significantly influence elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We may seek additional capital through a combination of public or private equity offerings or debt financings, credit or loan facilities, collaborations, strategic alliances, licensing arrangements or a combination of one or more of these funding sources. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a stockholder. The incurrence of indebtedness would result in increased fixed payment obligations and could involve certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights, and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or current or future product candidates, or grant licenses on terms unfavorable to us.

We are an emerging growth company and a smaller reporting company, and the reduced reporting requirements applicable to emerging growth companies and smaller reporting companies may make our common stock less attractive to investors.*

We are an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012 (the JOBS Act). For as long as we continue to be an emerging growth company, we may take advantage of certain exemptions from various public company reporting requirements, including being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure in this Quarterly Report on Form 10-Q, not being required to have our internal control over financial reporting audited by our independent registered public accounting firm under Section 404 of the Sarbanes-Oxley Act of 2002 (Sarbanes-Oxley Act), reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We may take advantage of these exemptions until December 31, 2026 or until we are no longer an emerging growth company, whichever is earlier. We will cease to be an emerging growth company prior to the end of such five-year period if certain earlier events occur, including if we become a "large accelerated filer" as defined in Rule 12b-2 under the Exchange Act, our annual gross revenues exceed $1.235 billion or we issue more than $1.0 billion of non-convertible debt in any three-year period.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have elected to use this extended

transition period under the JOBS Act until the earlier of the date we (i) are no longer an emerging growth company or (ii) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act.

We are also a "smaller reporting company" as defined in the Exchange Act. We may continue to be a smaller reporting company even after we are no longer an emerging growth company, which would allow us to take advantage of many of the same exemptions available to emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation. We will be able to take advantage of these scaled disclosures for so long as our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter, or our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of our second fiscal quarter. Investors may find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act, and the rules and regulations of The Nasdaq Stock Market LLC (Nasdaq). The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal controls over financial reporting. Each fiscal year, we are required to provide a report by our management on, among other things, our internal control over financial reporting as discussed in our Annual Report on Form 10-K filing for that year. The reporting on our assessment of the effectiveness of our internal control over financial reporting needs to include disclosure of any material weaknesses identified in our internal control over financial reporting, as well as a statement that our independent registered public accounting firm has audited the effectiveness of our internal control over financial reporting. While we qualify as an emerging growth company under SEC rules for fiscal year 2023 and therefore are not required to obtain such an audit for fiscal year 2023, in the event that we qualify as a large accelerated filer or accelerated filer under SEC rules in future years, our independent registered public accounting firm will be required to audit the effectiveness of our internal control over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act (Section 404(b)). Any mandatory or voluntary compliance with Section 404(b) will result in increased costs, expenses, and management resources. However, for as long as we remain an emerging growth company as defined in the JOBS Act, we intend to take advantage of the exemption permitting us not to comply with the independent registered public accounting firm attestation requirement.

We may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our consolidated financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

We cannot assure you that the measures we have taken to date, and actions we may take in the future, will prevent or avoid potential future material weaknesses. Moreover, our current controls and any new controls that we develop may become inadequate because of changes in conditions in our business. Further, material weaknesses in our disclosure controls and procedures and internal control over financial reporting may be discovered in the future. Any failure to develop or maintain effective internal control over financial reporting or any difficulties encountered in their implementation or improvement could harm our operating results or cause us to fail to meet our reporting obligations and may result in a restatement of our financial statements for prior periods, which could cause the price of our common stock to decline. In addition, if we are not able to continue to meet these requirements, we may not be able to remain listed on Nasdaq.

We have incurred and will continue to incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.*

As a public company, we have incurred and will continue to incur significant legal, accounting, and other expenses that we did not incur as a private company. We are subject to the reporting requirements of the Exchange Act, which require, among other things, that we file with the SEC annual, quarterly, and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC and Nasdaq to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act (Dodd-Frank Act) was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as "say on pay" and proxy access. Emerging growth companies and smaller reporting companies are exempted from certain of these requirements, but we may be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to continue to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition, results of operations and prospects. The increased costs will decrease our net income or increase our consolidated net loss, and may require us to reduce costs in other areas of our business or increase the prices of our products or services. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

Sales of a substantial number of shares of our common stock in the public market could cause our stock price to fall.*

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. As of November 9, 2023, there were 33,311,787 shares of our common stock outstanding.

In addition, shares of common stock that are either subject to outstanding options, restricted stock units or performance-based restricted stock units or reserved for future issuance under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, and Rule 144 and Rule 701 under the Securities Act of 1933, as amended (the Securities Act). If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

Further, the holders of 16,819,283 shares of our common stock are entitled to rights with respect to the registration of their shares under the Securities Act. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by affiliates, as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.*

We expect that we will need significant additional capital in the future to continue our planned operations, including conducting clinical trials, commercialization efforts, expanded research and development activities, and costs associated with operating a public company. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities, investors may be materially diluted by subsequent sales. Such sales may also result in material dilution to our existing stockholders, and new investors could gain rights, preferences and privileges senior to the holders of our common stock. As of September 30, 2023, the remaining capacity under the 2022 ATM Facility was approximately $18.8 million in shares of common stock. In November 2023, we terminated the 2022 ATM Facility. In November 2023, we entered into the 2023 ATM Facility with Leerink under which we may offer and sell, from time to time, at our sole discretion, up to $100.0 million in shares of our common stock. We have not yet sold any shares of our common stock under the 2023 ATM Facility.

Pursuant to our 2021 Equity Incentive Plan (the 2021 Plan), our management is authorized to grant stock options and other stock awards to our employees, directors and consultants. Additionally, the number of shares of our common stock reserved for issuance under our 2021 Plan will automatically increase on January 1 of each year through and including January 1, 2031, by 5% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of directors. In addition, pursuant to our 2021 Employee Stock Purchase Plan, the number of shares of our common stock reserved for issuance will automatically increase on January 1 of each calendar year through and including January 1, 2031, by the lesser of (i) 1% of the total number of shares of our common stock outstanding on the last day of the calendar month before the date of the automatic increase, and (ii) 729,174 shares; provided that before the date of any such increase, our board of directors may determine that such increase will be less than the amount set forth in clauses (i) and (ii). Unless our board of directors elects not to increase the number of shares available for future grant each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.

We have never declared or paid any cash dividend on our common stock. We currently anticipate that we will retain future earnings for the development, operation, and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. In addition, future debt instruments may materially restrict our ability to pay dividends on our common stock. Any return to stockholders would therefore be limited to the appreciation, if any, of their stock.

Anti-takeover provisions under our charter documents and Delaware law could delay or prevent a change of control which could limit the market price of our common stock and may prevent or frustrate attempts by our stockholders to replace or remove our current management.

Our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that could delay or prevent a change of control of our company or changes in our board of directors that our stockholders might consider favorable. Some of these provisions include:

- a board of directors divided into three classes serving staggered three-year terms, such that not all members of the board will be elected at one time;
- a prohibition on stockholder action through written consent, which requires that all stockholder actions be taken at a meeting of our stockholders;

- a requirement that special meetings of stockholders be called only by the chairman of the board of directors, the chief executive officer, the president, or by a majority of the total number of authorized directors;
- advance notice requirements for stockholder proposals and nominations for election to our board of directors;
- a requirement that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of not less than two-thirds of all outstanding shares of our voting stock then entitled to vote in the election of directors;
- a requirement of approval of not less than two-thirds of all outstanding shares of our voting stock to amend any bylaws by stockholder action or to amend specific provisions of our certificate of incorporation; and
- the authority of the board of directors to issue preferred stock on terms determined by the board of directors without stockholder approval and which preferred stock may include rights superior to the rights of the holders of common stock.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporate Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These anti-takeover provisions and other provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make it more difficult for stockholders or potential acquirors to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors and could also delay or impede a merger, tender offer, or proxy contest involving our company. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing or cause us to take other corporate actions you desire. Any delay or prevention of a change of control transaction or changes in our board of directors could cause the market price of our common stock to decline.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that the Court of Chancery of the State of Delaware will be the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that the Court of Chancery of the State of Delaware is the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (i) any derivative action or proceeding brought on our behalf; (ii) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers, or other employees to us or our stockholders; (iii) any action or proceeding asserting a claim against us or any of our current or former directors, officers, or other employees, arising out of or pursuant to any provision of the Delaware General Corporation Law, our amended and restated certificate of incorporation or our amended and restated bylaws; (iv) any action or proceeding to interpret, apply, enforce, or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws; (v) any action or proceeding as to which the Delaware General Corporation Law confers jurisdiction to the Court of Chancery of the State of Delaware; and (vi) any action asserting a claim against us or any of our directors, officers, or other employees governed by the internal affairs doctrine, in all cases to the fullest extent permitted by law and subject to the court's having personal jurisdiction over the indispensable parties named as defendants.

These provisions would not apply to suits brought to enforce a duty or liability created by the Exchange Act. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by

different courts, among other considerations, our amended and restated certificate of incorporation and our amended and restated bylaws provide that the federal district courts of the United States of America are the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act, including all causes of action asserted against any defendant named in such complaint. While the Delaware courts have determined that such choice of forum provisions are facially valid and several state trial courts have enforced such provisions and required that suits asserting Securities Act claims be filed in federal court, there is no guarantee that courts of appeal will affirm the enforceability of such provisions and a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated certificate of incorporation and our amended and restated bylaws. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions. If a court were to find either exclusive forum provision in our amended and restated certificate of incorporation and our amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with litigating Securities Act claims in state court, or both state and federal court, which could seriously harm our business, financial condition, results of operations, and prospects.

These exclusive forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers and other employees. If a court were to find either exclusive forum provision contained in our amended and restated certificate of incorporation or amended and restated bylaws to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business.

General Risk Factors

We are subject to U.S. and certain foreign export and import controls, sanctions, embargoes, anti-corruption laws and anti-money laundering laws and regulations. Compliance with these legal standards could impair our ability to compete in domestic and international markets. We can face criminal liability and other serious consequences for violations, which can harm our business.

We are subject to export control and import laws and regulations, including the U.S. Export Administration Regulations, U.S. Customs regulations, and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Controls, and anti-corruption and anti-money laundering laws and regulations, including the FCPA, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act, and other state and national anti-bribery and anti-money laundering laws in the countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees, agents, clinical research organizations, contractors and other collaborators and partners from authorizing, promising, offering, providing, soliciting or receiving, directly or indirectly, improper payments or anything else of value to recipients in the public or private sector. We may engage third parties for clinical trials outside of the United States, to sell our products internationally once we enter a commercialization phase, and/or to obtain necessary permits, licenses, patent registrations and other regulatory approvals. We have direct or indirect interactions with officials and employees of government agencies or government-affiliated hospitals, universities and other organizations. We can be held liable for the corrupt or other illegal activities of our employees, agents, clinical research organizations, contractors and other collaborators and partners, even if we do not explicitly authorize or have actual knowledge of such activities. Any violations of the laws and regulations described above may result in substantial civil and criminal fines and penalties, imprisonment, the loss of export or import privileges, debarment, tax reassessments, breach of contract and fraud litigation, reputational harm, and other consequences.

Business disruptions could seriously harm our future revenues and financial condition and increase our costs and expenses.

Our operations, and those of our CROs and other contractors and consultants, could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on third-party manufacturers to produce mavodelpar. Our ability to obtain clinical supplies of mavodelpar and any future product candidates could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption. Our corporate headquarters is located in California near major earthquake faults and fire zones. The ultimate impact on us, our suppliers and our general infrastructure of being located near major earthquake faults and fire zones and being consolidated in certain geographical areas is unknown, but our operations and financial condition could suffer in the event of a major earthquake, fire or other natural disaster.

If our information technology systems or data, or those of third parties upon which we rely, are or were compromised, we could experience adverse consequences resulting from such compromise, including but not limited to regulatory investigations or actions; litigation; fines and penalties; disruptions of our business operations; reputational harm; loss of revenue or profits; and other adverse consequences.*

In the ordinary course of our business, we and the third parties upon which we rely process sensitive data, and, as a result, we and the third parties upon which we rely face a variety of evolving threats, including but not limited to ransomware attacks, which could cause security incidents.

Cyberattacks, malicious internet-based activity, online and offline fraud, and other similar activities threaten the confidentiality, integrity, and availability of our sensitive data and information technology systems, and those of the third parties upon which we rely. Such threats are prevalent and continue to rise, are increasingly difficult to detect, and come from a variety of sources, including traditional computer “hackers,” threat actors, “hacktivists,” organized criminal threat actors, personnel (such as through theft or misuse), sophisticated nation-states, and nation-state-supported actors.

Some actors now engage and are expected to continue to engage in cyber-attacks, including without limitation nation-state actors for geopolitical reasons and in conjunction with military conflicts and defense activities. During times of war and other major conflicts, we and the third parties upon which we rely, may be vulnerable to a heightened risk of these attacks, including retaliatory cyber-attacks, that could materially disrupt our systems and operations, supply chain, and ability to produce, sell and distribute our services. Further, the loss of clinical trial data from completed or ongoing clinical trials could result in delays in, or cancellations of any regulatory approval or clearance efforts and significantly increase our costs to recover or reproduce the data, and subsequently commercialize the product. Additionally, theft of our intellectual property or proprietary business information could require substantial expenditures to remedy.

We and the third-parties upon which we rely are subject to a variety of evolving threats including but not limited to social-engineering attacks (including through deep fakes, which may be increasingly more difficult to identify as fake, and phishing attacks), malicious code (such as viruses and worms), malware (including as a result of advanced persistent threat intrusions), denial of service attacks, credential stuffing attacks, credential harvesting, personnel misconduct or error, ransomware attacks, supply-chain attacks, software bugs, server malfunctions, software or hardware failures, loss of data or other information technology assets, adware, telecommunications failures, earthquakes, fires, floods, attacks enhanced or facilitated by AI, and other similar threats.

In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, ability to provide our products or services, loss of sensitive data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments. Remote work has become more common and has increased risks to our

information technology systems and data, as more of our employees utilize network connections, computers and devices outside our premises or network, including working at home, while in transit and in public locations.

Additionally, future or past business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

In addition, our reliance on third-party service providers could introduce new cybersecurity risks and vulnerabilities, including supply-chain attacks, and other threats to our business operations. We rely on third-party service providers and technologies to operate critical business systems to process sensitive data in a variety of contexts, including, without limitation, cloud-based infrastructure, data center facilities, encryption and authentication technology, employee email, and other functions. We also rely on third-party service providers to provide other products, services, parts, or otherwise to operate our business. Our ability to monitor these third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. If our third-party service providers experience a security incident or other interruption, we could experience adverse consequences. While we may be entitled to damages if our third-party service providers fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award. In addition, supply-chain attacks have increased in frequency and severity, and we cannot guarantee that third parties' infrastructure in our supply chain or our third-party partners' supply chains have not been compromised.

Any of the previously identified or similar threats could cause a security incident or other interruption that could result in unauthorized, unlawful, or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive data or our information technology systems, or those of the third parties upon whom we rely. A security incident or other interruption could disrupt our ability (and that of third parties upon whom we rely) to provide our products.

We may expend significant resources or modify our business activities (including our clinical trial activities) to try to protect against security incidents. Additionally, certain data privacy and security obligations may require us to implement and maintain specific security measures or industry-standard or reasonable security measures to protect our information technology systems and sensitive data.

While we have implemented security measures designed to protect against security incidents, there can be no assurance that these measures will be effective. We take steps to detect and remediate vulnerabilities, but we may not be able to detect and remediate all vulnerabilities because the threats and techniques used to exploit the vulnerability change frequently and are often sophisticated in nature. Therefore, such vulnerabilities could be exploited but may not be detected until after a security incident has occurred. Unremediated high risk or critical vulnerabilities pose material risks to our business. Further, we may experience delays in developing and deploying remedial measures designed to address any such identified vulnerabilities.

Applicable data privacy and security obligations may require us to notify relevant stakeholders of security incidents. Such disclosures are costly, and the disclosures or the failure to comply with such requirements could lead to adverse consequences.

If we (or a third party upon whom we rely) experience a security incident or are perceived to have experienced a security incident, we may experience adverse consequences such as government enforcement actions (for example, investigations, fines, penalties, audits, and inspections); additional reporting requirements and/or oversight; restrictions on processing sensitive data (including personal data); litigation (including class claims); indemnification obligations; negative publicity; reputational harm; monetary fund diversions; interruptions in our operations (including availability of data); financial loss; and other similar harms. Security incidents and attendant consequences may prevent or cause customers to stop using our products or services, deter new

customers from using our products or services, the development and commercialization of mavodelpar could be delayed, and negatively impact our ability to grow and operate our business. Likewise, we rely on third parties to conduct clinical trials, and similar incidents relating to their information technology systems or data could also have a material adverse effect on our business.

Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations. We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices, that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims.

In addition to experiencing a security incident, third parties may gather, collect, or infer sensitive data about us from public sources, data brokers, or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position. Additionally, sensitive data of the Company could be leaked, disclosed, or revealed as a result of or in connection with our employee's, personnel's, or vendor's use of generative AI technologies.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock depends in part on the research and reports that securities or industry analysts publish about us or our business. If one or more of the analysts who covers us downgrades our stock or publishes inaccurate or unfavorable research about our business, our stock price may decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

Unregistered Sales of Equity Securities

None.

Issuer Purchases of Equity Securities

On October 30, 2023, pursuant to a Common Stock Repurchase Agreement with vTv Therapeutics, we repurchased 576,443 shares of our common stock from vTv Therapeutics at a price of $7.64 per share for an aggregate purchase price of approximately $4.4 million. The repurchase was made directly in a private, non-underwritten transaction. Subsequently, we retired the repurchased shares.

Use of Proceeds

We commenced our IPO pursuant to the registration statement on Form S-1 (File No. 333-254534) that was declared effective on April 8, 2021 and registered an aggregate of 7,187,500 shares of our common stock. On April 13, 2021, we completed our IPO and sold 6,250,000 shares of our common stock at a public offering price of $15.00 per share for aggregate gross proceeds of $93.8 million before deducting underwriters' discounts and commissions and offering-related expenses. Net proceeds, after deducting underwriting discounts and commissions of $6.6 million and offering expenses of approximately $2.6 million, were $84.6 million. Jefferies LLC, Leerink and Piper Sandler & Co. acted as joint book-running managers.

As of September 30, 2023, we have used approximately $23.8 million of the net proceeds from our IPO. We have invested the remaining net proceeds in highly liquid money market funds and short-term investments. The remaining net proceeds from the IPO will be used, together with our cash, cash equivalents, and short-term

investments, to fund continued research and development of mavodelpar in patients with PMM and LC-FAOD, other clinical trials and preclinical studies, and commercial readiness preparations, and to provide funds for working capital and other general purposes. None of the offering proceeds were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning 10.0% or more of any class of our equity securities or to any other affiliates.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

None.

Item 5. Other Information

Entry into New Sales Agreement

On November 13, 2023, we entered into an at-the-market equity offering sales agreement (the 2023 Sales Agreement) with Leerink Partners LLC (Leerink), pursuant to which we may offer and sell, from time to time at our sole discretion, up to an aggregate amount of $100.0 million of our common stock through Leerink (the 2023 ATM Facility). We are not required to sell shares under the 2023 Sales Agreement. Sales of our common stock, if any, under the 2023 Sales Agreement may be made in any transactions that are deemed to be "at the market offerings" as defined in Rule 415 under the Securities Act. The 2023 Sales Agreement contains customary representations, warranties and agreements, indemnification rights and obligations of the parties and termination provisions. We will pay Leerink a commission equal to 3.0% of the aggregate gross proceeds of any shares of common stock sold through it pursuant to the 2023 Sales Agreement. No sales may be made under the 2023 Sales Agreement until a prospectus supplement relating to the 2023 ATM Offering is filed with the SEC.

The 2023 ATM Offering is being made under a prospectus supplement dated November 13, 2023, and related prospectus to be filed with the SEC pursuant to our shelf registration statement on Form S-3 (Registration No. 333-264616).

A copy of the 2023 Sales Agreement is attached as Exhibit 10.1 to this Quarterly Report on Form 10-Q. A copy of the opinion of Cooley LLP relating to the validity of the securities issued in the 2023 ATM Offering is filed as Exhibit 5.1 to this Quarterly Report on Form 10-Q.

Termination of Prior Sales Agreement

On November 13, 2023, we and Leerink agreed to terminate the sales agreement that we entered into with Leerink on May 2, 2022 (the Prior Sales Agreement), effective November 13, 2023. Under the Prior Sales Agreement, we were permitted to offer and sell, from time to time at our sole discretion, shares of our common stock, having an aggregate offering price of up to $20.0 million through Leerink by any method permitted by law deemed to be an "at the market offering" as defined in Rule 415 of the Securities Act. Prior to such termination, we had sold and issued approximately 500,000 shares of our common stock pursuant to the Prior Sales Agreement at a weighted-average price of $2.48 per share, resulting in aggregate gross proceeds to us of $1.2 million. In connection with the termination of the Prior Sales Agreement, we terminated the prospectus, dated May 9, 2022, related to the $20.0 million of shares of our common stock issuable pursuant to the terms of the Prior Sales Agreement.

Item 6. Exhibits

EXHIBIT INDEX

Exhibit No.	Description
3.1	Amended and Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on April 13, 2021).
3.2	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's Current Report on Form 8-K, filed with the SEC on April 13, 2021).
4.1	Form of Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-254534), filed with the SEC on April 5, 2021).
4.2	Amended and Restated Investors' Rights Agreement, by and among the Registrant and certain of its stockholders, dated December 9, 2020 (incorporated by reference to Exhibit 4.2 to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-254534), filed with the SEC on March 19, 2021).
5.1	Opinion of Cooley LLP.
10.1	Sales Agreement, dated November 13, 2023, by and between the Registrant and Leerink Partners LLC.
31.1	Certification of Principal Executive Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Principal Financial Officer Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*	Certification of Principal Executive Officer and Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (formatted in Inline XBRL and contained in Exhibit 101)

* The certification attached as Exhibit 32.1 accompanies this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, and shall not be deemed "filed" by the Registrant for purposes of Section 18 of the Securities Exchange Act of 1934, as amended.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Date: November 13, 2023

RENEO PHARMACEUTICALS, INC.

By:	/s/ Gregory J. Flesher
Name:	Gregory J. Flesher
Title:	President and Chief Executive Officer (Principal Executive Officer)

By:	/s/ Jennifer P. Lam
Name:	Jennifer P. Lam
Title:	Senior Vice President, Finance and Administration (Principal Financial and Accounting Officer)

Jason L. Kent
+1 212 479 6044
jkent@cooley.com

Exhibit 5.1

November 13, 2023

Reneo Pharmaceuticals, Inc.
18575 Jamboree Road, Suite 275-S
Irvine, CA 92612

Ladies and Gentlemen:

We have acted as counsel to Reneo Pharmaceuticals, Inc., a Delaware corporation (the "***Company***"), in connection with the offering by the Company of up to $100 million shares (the "***Shares***") of its common stock, par value $0.0001 per share (the "Common Stock"), pursuant to the Registration Statement on Form S-3 (File No. 333-264616) (the "***Registration Statement***") filed with the Securities and Exchange Commission (the "***Commission***") under the Securities Act of 1933, as amended (the "***Securities Act***"), the prospectus included in the Registration Statement (the "***Base Prospectus***") and the prospectus supplement dated November 13, 2023 filed with the Commission pursuant to Rule 424(b) under the Securities Act (together with the Base Prospectus, the "***Prospectus***"). The Shares are to be sold by the Company in accordance with that certain Sales Agreement, dated November 13, 2023, by and between the Company and Leerink Partners (the "***Agreement***"), as described in the Prospectus.

In connection with this opinion, we have examined and relied upon the Registration Statement and the Prospectus, the Agreement, the Company's certificate of incorporation and bylaws, each as currently in effect, and such other records, documents, certificates, memoranda and other instruments as in our judgment are necessary or appropriate to enable us to render the opinion expressed below. We have assumed the genuineness of all signatures; the authenticity of all documents submitted to us as originals; the conformity to originals of all documents submitted to us as copies; the accuracy, completeness and authenticity of certificates of public officials; and the due authorization, execution and delivery of all documents by all persons other than the Company where authorization, execution and delivery are prerequisites to the effectiveness thereof. As to certain factual matters, we have relied upon a certificate of an officer of the Company and have not independently verified such matters.

We have assumed (i) that each sale of Shares will be duly authorized by the Board of Directors of the Company, a duly authorized committee thereof or a person or body pursuant to an authorization granted in accordance with Section 152 of the General Corporation Law of the State of Delaware (the "***DGCL***"), (ii) that no more than $100.0 million Shares will be sold under the Agreement pursuant to the Prospectus and (iii) that the price at which the Shares are sold will equal or exceed the par value of the Common Stock. We express no opinion to the extent that future issuances of securities of the Company, anti-dilution adjustments to outstanding securities of the Company or other matters cause the number of shares of Common Stock issuable under the Agreement to exceed the number of shares available for issuance by the Company.

Our opinion is expressed only with respect to the DGCL. We express no opinion to the extent that any other laws are applicable to the subject matter hereof and express no opinion and provide no assurance as to compliance with any federal or state securities law, rule or regulation.

On the basis of the foregoing, in reliance thereon and subject to the qualifications set forth herein, we are of the opinion that the Shares, when sold and issued against payment therefor in accordance with the Agreement, the Registration Statement and the Prospectus, will be validly issued, fully paid and nonassessable.

Our opinion is limited to the matters expressly set forth in this letter, and no opinion should be implied, or may be inferred, beyond the matters expressly stated. This opinion speaks only as to law and facts in effect or existing as of the date hereof, and we undertake no obligation or responsibility to update or supplement this letter to reflect any facts or circumstances that may hereafter come to our attention or any changes in law that may hereafter occur.

We consent to the reference to our firm under the caption “Legal Matters” in the Prospectus and to the filing of this opinion as an exhibit to the Company’s Current Report on Form 8-K to be filed with the Commission for incorporation by reference into the Registration Statement. In giving such consent, we do not thereby admit that we are in the category of persons whose consent is required under Section 7 of the Securities Act or the rules and regulations of the Commission thereunder.

Sincerely,

Cooley LLP

By: /s/ Jason L. Kent
Jason L. Kent

Exhibit 10.1

RENEO PHARMACEUTICALS, INC.
Shares of Common Stock
($0.0001 par value per share)

SALES AGREEMENT

November 13, 2023

LEERINK PARTNERS LLC
1301 Avenue of the Americas, 12th Floor
New York, New York 10019

Ladies and Gentlemen:

Reneo Pharmaceuticals, Inc., a Delaware corporation (the "**Company**"), confirms its agreement (this "**Agreement**") with Leerink Partners LLC (the "**Agent**"), as follows:

1. Issuance and Sale of Shares. The Company agrees that, from time to time during the term of this Agreement, on the terms and subject to the conditions set forth herein, it may issue and sell through the Agent up to $100,000,000 of shares of common stock, $0.0001 par value per share, of the Company (the "**Common Stock**"), subject to the limitations set forth in Section 5(c) (the "**Placement Shares**"). Notwithstanding anything to the contrary contained herein, the parties hereto agree that compliance with the limitation set forth in this Section 1 on the aggregate gross sales price of Placement Shares that may be issued and sold under this Agreement from time to time shall be the sole responsibility of the Company, and that the Agent shall have no obligation in connection with such compliance. The issuance and sale of Placement Shares through the Agent will be effected pursuant to the Registration Statement (as defined below) filed by the Company with the Securities and Exchange Commission (the "**Commission**") on May 2, 2022 and declared effective by the Commission on May 9, 2022, although nothing in this Agreement shall be construed as requiring the Company to issue any Placement Shares.

The Company has prepared and has filed, in accordance with the provisions of the Securities Act of 1933, as amended, and the rules and regulations thereunder (collectively, the "**Securities Act**"), with the Commission a registration statement on Form S-3 (File No. 333-264616), including a base prospectus (the "**Base Prospectus**"), relating to certain securities, including the Common Stock, to be issued from time to time by the Company, and which incorporates by reference documents that the Company has filed or will file in accordance with the provisions of the Securities Exchange Act of 1934, as amended, and the rules and regulations thereunder (collectively, the "**Exchange Act**"). The Company has prepared a sales prospectus supplement specifically relating to the Placement Shares (the "**Prospectus Supplement**" and, together with the Base Prospectus, the "**Sales Prospectus**") which will be filed as part of such registration statement. The Company will furnish to the Agent, for use by the Agent, copies of the Sales Prospectus, as supplemented by any prospectus supplement, relating to the Placement Shares. Except where the context otherwise requires, such registration statement, when it became effective, including all documents filed as part thereof or incorporated by reference therein, and including any information contained in a Prospectus (as defined below) subsequently filed with the Commission pursuant to Rule 424(b) under the

Securities Act or deemed to be a part of such registration statement pursuant to Rule 430B or Rule 462(b) under the Securities Act, is herein called the "**Registration Statement.**" The Base Prospectus, including all documents incorporated therein by reference, included in the Registration Statement, as supplemented by the Prospectus Supplement, in the form in which such prospectus and/or Prospectus Supplement have most recently been filed by the Company with the Commission pursuant to Rule 424(b) under the Securities Act, together with any "issuer free writing prospectus," as defined in Rule 433 under the Securities Act ("**Rule 433**"), relating to the Placement Shares that (i) is required to be filed with the Commission by the Company or (ii) is exempt from filing pursuant to Rule 433(d)(5)(i), in each case in the form filed or required to be filed with the Commission or, if not required to be filed, in the form retained in the Company's records pursuant to Rule 433(g), is herein called the "**Prospectus**." Any reference herein to the Registration Statement, the Prospectus or any amendment or supplement thereto shall be deemed to refer to and include the documents incorporated by reference therein (the "**Incorporated Documents**"), and any reference herein to the terms "amend," "amendment" or "supplement" with respect to the Registration Statement or the Prospectus shall be deemed to refer to and include the filing after the execution hereof of any document with the Commission deemed to be incorporated by reference therein. For purposes of this Agreement, all references to the Registration Statement, the Prospectus or to any amendment or supplement thereto shall be deemed to include the most recent copy filed with the Commission pursuant to the Electronic Data Gathering Analysis and Retrieval System ("**EDGAR**").

2. Placements. Each time that the Company wishes to issue and sell any Placement Shares through the Agent hereunder (each, a "**Placement**"), it will notify the Agent by email notice (or other method mutually agreed to in writing by the parties) (each such notice, a "**Placement Notice**") containing the parameters in accordance with which it desires such Placement Shares to be sold, which at a minimum shall include the maximum number or amount of Placement Shares to be sold, the time period during which sales are requested to be made, any limitation on the number or amount of Placement Shares that may be sold in any one Trading Day (as defined in Section 3) and any minimum price below which sales may not be made, a form of which containing such minimum sales parameters is attached hereto as **Schedule 1**. The Placement Notice must originate from one of the individuals authorized to act on behalf of the Company and set forth on **Schedule 2** (with a copy to each of the other individuals from the Company listed on such **Schedule 2**), and shall be addressed to each of the individuals from the Agent set forth on **Schedule 2**, as such **Schedule 2** may be updated by either party from time to time by sending a written notice containing a revised **Schedule 2** to the other party in the manner provided in Section 12 (including by email correspondence to each of the individuals of the Company set forth on **Schedule 2**, if receipt of such correspondence is actually acknowledged by any of the individuals to whom the notice is sent, other than via auto-reply). The Placement Notice shall be effective upon receipt by the Agent unless and until (i) in accordance with the notice requirements set forth in Section 4, the Agent declines to accept the terms contained therein for any reason, in its sole discretion, within two Trading Days of the date the Agent receives the Placement Notice, (ii) in accordance with the notice requirements set forth in Section 4, the Agent suspends sales under the Placement Notice for any reason in its sole discretion, (iii) the entire amount of the Placement Shares has been sold pursuant to this Agreement, (iv) in accordance with the notice requirements set forth in Section 4, the Company suspends sales under or terminates the Placement Notice for any reason in its sole discretion, (v) the Company issues a subsequent Placement Notice and explicitly indicates that its parameters supersede those contained in the earlier dated Placement Notice or (vi) this Agreement has been terminated pursuant to the provisions of Section 11. The amount of any discount, commission or other compensation to be paid by the Company to the Agent in connection with the sale of the Placement Shares effected through the Agent shall be calculated in accordance with the terms set forth in **Schedule 3**. It is expressly acknowledged and agreed that neither the Company nor the Agent will have any obligation whatsoever with respect to a Placement or any Placement Shares unless

and until the Company delivers a Placement Notice to the Agent and the Agent does not decline such Placement Notice pursuant to the terms set forth above, and then only upon the terms specified therein and herein. In the event of a conflict between the terms of this Agreement and the terms of a Placement Notice, the terms of the Placement Notice will control with respect to the matters covered thereby.

3. Sale of Placement Shares by the Agent. On the basis of the representations and warranties herein contained and subject to the terms and conditions herein set forth, including Section 5(c), upon the Agent's acceptance of the terms of a Placement Notice as provided in Section 2, and unless the sale of the Placement Shares described therein has been declined, suspended or otherwise terminated in accordance with the terms of this Agreement, the Agent, for the period specified in the Placement Notice, will use its commercially reasonable efforts consistent with its normal trading and sales practices and applicable state and federal laws, rules and regulations and the rules of the Nasdaq Global Market ("**Nasdaq**") to sell such Placement Shares up to the number or amount specified in, and otherwise in accordance with the terms of, such Placement Notice. The Agent will provide written confirmation to the Company (including by email correspondence to each of the individuals of the Company set forth on **Schedule 2**, if receipt of such correspondence is actually acknowledged by any of the individuals to whom the notice is sent, other than via auto-reply) no later than the opening of the Trading Day (as defined below) immediately following the Trading Day on which it has made sales of Placement Shares hereunder setting forth the number or amount of Placement Shares sold on such Trading Day, the volume-weighted average price of the Placement Shares sold and the Net Proceeds (as defined below) payable to the Company. Unless otherwise specified by the Company in a Placement Notice, the Agent may sell Placement Shares by any method permitted by law deemed to be an "at the market offering" as defined in Rule 415(a)(4) of the Securities Act, including sales made directly on or through Nasdaq, on or through any other existing trading market for the Common Stock or to or through a market maker. If expressly authorized by the Company (including in a Placement Notice), the Agent may also sell Placement Shares in negotiated transactions. Notwithstanding the provisions of Section 6(ww), except as may be otherwise agreed by the Company and the Agent, the Agent shall not purchase Placement Shares on a principal basis pursuant to this Agreement unless the Company and the Agent enter into a separate written agreement setting forth the terms of such sale. The Company acknowledges and agrees that (i) there can be no assurance that the Agent will be successful in selling Placement Shares, (ii) the Agent will incur no liability or obligation to the Company or any other person or entity if it does not sell Placement Shares for any reason other than a failure by the Agent to use its commercially reasonable efforts consistent with its normal trading and sales practices and applicable state and federal laws, rules and regulations and the rules of Nasdaq to sell such Placement Shares as required under this Agreement and (iii) the Agent shall be under no obligation to purchase Placement Shares on a principal basis pursuant to this Agreement unless the Company and the Agent enter into a separate written agreement setting forth the terms of such sale. For the purposes hereof, "**Trading Day**" means any day on which the Common Stock is purchased and sold on the principal market on which the Common Stock is then listed or quoted.

4. Suspension of Sales.

(a) The Company or the Agent may, upon notice to the other party in writing (including by email correspondence to each of the individuals of the other party set forth on **Schedule 2**, if receipt of such correspondence is actually acknowledged by any of the individuals to whom the notice is sent, other than via auto-reply) or by telephone (confirmed immediately by email correspondence to each of the individuals of the other party set forth on **Schedule 2**), suspend any sale of Placement Shares; *provided*, *however*, that such suspension shall not affect or impair either party's obligations with respect to any Placement Shares sold hereunder prior to the receipt of such notice. While a suspension pursuant to this Section 4(a) is in effect, any obligation under Sections 7(m), 7(n), 7(o), and 7(p) with respect to the

delivery of certificates, opinions, or comfort letters to the Agent, shall be waived; provided, that upon delivery of a Placement Notice following a Representation Date, the Company shall be subject to the obligations set forth in Sections 7(m), 7(n), 7(o), and 7(p) as applicable. Each of the parties agrees that no such notice under this Section 4 shall be effective against the other party unless notice is sent by one of the individuals named on **Schedule 2** hereto to the other party in writing (including by email correspondence to each of the individuals of the other party set forth on **Schedule 2**, if receipt of such correspondence is actually acknowledged by any of the individuals to whom the notice is sent, other than via auto-reply).

(b) Notwithstanding any other provision of this Agreement, during any period in which the Company is, or could be deemed to be, in possession of material non-public information, the Company and the Agent agree that (i) no sale of Placement Shares will take place, (ii) the Company shall not request the sale of any Placement Shares and shall cancel any effective Placement Notices instructing the Agent to make any sales and (iii) the Agent shall not be obligated to sell or offer to sell any Placement Shares.

5. Settlement and Delivery of the Placement Shares.

(a) Settlement of Placement Shares. Unless otherwise specified in the applicable Placement Notice, settlement for sales of Placement Shares will occur on the second Trading Day (or such earlier day as is industry practice or as is required for regular-way trading) following the date on which such sales are made (each, a "**Settlement Date**"). The amount of proceeds to be delivered to the Company on a Settlement Date against receipt of the Placement Shares sold (the "**Net Proceeds**") will be equal to the aggregate gross sales price received by the Agent at which such Placement Shares were sold, after deduction of (i) the Agent's commission, discount or other compensation for such sales payable by the Company pursuant to Section 2 hereof, (ii) any other amounts due and payable by the Company to the Agent hereunder pursuant to Section 7(g) hereof and (iii) any transaction fees imposed by any governmental or self-regulatory organization in respect of such sales.

(b) Delivery of Placement Shares. On or before each Settlement Date, the Company will issue the Placement Shares being sold on such date and will, or will cause its transfer agent to, electronically transfer such Placement Shares by crediting the Agent's or its designee's account (provided the Agent shall have given the Company written notice of such designee prior to the Settlement Date) at The Depository Trust Company through its Deposit and Withdrawal at Custodian System ("**DWAC**") or by such other means of delivery as may be mutually agreed upon by the parties hereto, which in all cases shall be duly authorized, freely tradeable, transferable, registered shares of Common Stock in good deliverable form. On each Settlement Date, the Agent will deliver the related Net Proceeds in same day funds to an account designated by the Company on or prior to the Settlement Date. The Agent shall be responsible for providing DWAC instructions or other instructions for delivery by other means with regard to the transfer of the Placement Shares being sold. In addition to and in no way limiting the rights and obligations set forth in Section 9(a) hereto, the Company agrees that if the Company or its transfer agent (if applicable), defaults in its obligation to deliver duly authorized, freely tradeable, transferable, registered Placement Shares in good deliverable form by 2:30 P.M., New York City time, on a Settlement Date (other than as a result of a failure by the Agent to provide instructions for delivery), the Company will (i) take all necessary action to cause the full amount of any Net Proceeds that were delivered to the Company's account with respect to such settlement, together with any costs incurred by the Agent and/or its clearing firm in connection with recovering such Net Proceeds, to be immediately returned to the Agent or its clearing firm no later than 5:00 P.M., New York City time, on such Settlement Date, by wire transfer of immediately available funds to an account designated by the Agent or its clearing firm, (ii)

indemnify and hold the Agent and its clearing firm harmless against any loss, claim, damage, or expense (including reasonable legal fees and expenses), as incurred, arising out of or in connection with such default by the Company or its transfer agent (if applicable) and (iii) pay to the Agent any commission, discount or other compensation to which it would otherwise have been entitled absent such default. Certificates for the Placement Shares, if any, shall be in such denominations and registered in such names as the Agent may request in writing one Business Day (as defined below) before the applicable Settlement Date. Certificates for the Placement Shares, if any, will be made available by the Company for examination and packaging by the Agent in New York City not later than 12:00 P.M., New York City time, on the Business Day prior to the applicable Settlement Date.

(c) Limitations on Offering Size. Under no circumstances shall the Company cause or request the offer or sale of any Placement Shares if, after giving effect to the sale of such Placement Shares, the aggregate number or gross sales proceeds of Placement Shares sold pursuant to this Agreement would exceed the lesser of: (i) the number or dollar amount of shares of Common Stock registered pursuant to, and available for offer and sale under, the Registration Statement pursuant to which the offering of Placement Shares is being made, (ii) the number of authorized but unissued shares of Common Stock of the Company (less shares of Common Stock issuable upon exercise, conversion or exchange of any outstanding securities of the Company or otherwise reserved from the Company's authorized capital stock), (iii) the number or dollar amount of shares of Common Stock permitted to be offered and sold by the Company under Form S-3 (including General Instruction I.B.6. thereof, if such instruction is applicable), (iv) the number or dollar amount of shares of Common Stock that the Company's board of directors or a duly authorized committee thereof is authorized to issue and sell from time to time, and notified to the Agent in writing, or (v) the dollar amount of shares of Common Stock for which the Company has filed the Prospectus. Under no circumstances shall the Company cause or request the offer or sale of any Placement Shares pursuant to this Agreement at a price lower than the minimum price authorized from time to time by the Company's board of directors or a duly authorized committee thereof, and notified to the Agent in writing. Notwithstanding anything to the contrary contained herein, the parties hereto acknowledge and agree that compliance with the limitations set forth in this Section 5(c) on the number or dollar amount of Placement Shares that may be issued and sold under this Agreement from time to time shall be the sole responsibility of the Company, and that the Agent shall have no obligation in connection with such compliance.

6. Representations and Warranties of the Company. The Company represents and warrants to, and agrees with, the Agent that, as of the date of this Agreement, and as of (i) each Representation Date (as defined in Section 7(m)), (ii) each date on which a Placement Notice is given, (iii) any date on which Placement Shares are sold pursuant to this Agreement and (iv) each Settlement Date (each such time or date referred to in clauses (i) through (iv), an "**Applicable Time**"):

(a) The Company and the transactions contemplated by this Agreement meet the requirements for and comply with the conditions for the use of Form S-3 (including General Instructions I.A and I.B.6.) under the Securities Act. The Registration Statement has been filed with and declared effective by the Commission under the Securities Act prior to the issuance of any Placement Notices by the Company. At the time the Registration Statement was declared effective and, subsequent thereto, at the time the Company's then most recent Annual Report on Form 10-K was filed with the Commission, the Company met the then- applicable requirements for use of Form S-3 (including General Instructions I.A and I.B.6.) under the Securities Act. The Registration Statement meets, and the offering and sale of Placement Shares as contemplated hereby comply with, the requirements of Rule 415(a)(1)(x) under the Securities Act. The Agent is named as the agent engaged by the Company in the section entitled "Plan of Distribution" in the Prospectus. The Company has not received, and has no notice from the Commission

of, any notice pursuant to Rule 401(g)(1) under the Securities Act objecting to the use of the shelf registration statement form. No stop order of the Commission preventing or suspending the use of the Prospectus, or the effectiveness of the Registration Statement, has been issued, and no proceedings for such purpose are pending before or, to the knowledge of the Company, threatened by the Commission. Copies of the Registration Statement, the Prospectus, any such amendments or supplements to any of the foregoing and all Incorporated Documents that were filed with the Commission on or prior to the date of this Agreement have been delivered, or are available through EDGAR, to the Agent and its counsel.

(b) Each of the Registration Statement and any post-effective amendment thereto, at the time it became or becomes effective, at each deemed effective date with respect to the Agent pursuant to Rule 430B(f)(2) under the Securities Act and as of each Applicable Time, complied, complies and will comply in all material respects with the requirements of the Securities Act and did not, does not and will not contain any untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the statements therein not misleading, except that the representations and warranties set forth in this sentence do not apply to Agent's Information (as defined below). The Prospectus and any amendment or supplement thereto, when so filed with the Commission under Rule 424(b) under the Securities Act, complied, complies and as of each Applicable Time will comply in all material respects with the requirements of the Securities Act, and the Prospectus or issuer free writing prospectus (or any amendments or supplements to any of the foregoing) furnished to the Agent for use in connection with the offering of the Placement Shares was identical to the electronically transmitted copies thereof filed with the Commission pursuant to EDGAR, except to the extent permitted by Regulation S-T. Neither the Prospectus nor any amendment or supplement thereto, as of its date and as of each Applicable Time, included, includes or will include an untrue statement of a material fact or omitted, omits or will omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading, except that the representations and warranties set forth in this sentence do not apply to Agent's Information. Each Incorporated Document heretofore filed, when it was filed (or, if any amendment with respect to any such document was filed, when such amendment was filed), conformed in all material respects with the requirements of the Exchange Act and were filed on a timely basis with the Commission, and any further Incorporated Documents so filed and incorporated after the date of this Agreement will be filed on a timely basis and, when so filed, will conform in all material respects with the requirements of the Exchange Act; no such Incorporated Document when it was filed (or, if an amendment with respect to any such document was filed, when such amendment was filed), contained an untrue statement of a material fact or omitted to state a material fact required to be stated therein or necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading; and no such Incorporated Document, when it is filed, will contain an untrue statement of a material fact or will omit to state a material fact required to be stated therein or necessary in order to make the statements therein, in light of the circumstances under which they were made, not misleading.

(c) (i) At the time of filing the Registration Statement and (ii) at the time of the execution of this Agreement (with such date being used as the determination date for purposes of this clause (ii)), the Company was not and is not an "ineligible issuer" (as defined in Rule 405), without taking account of any determination by the Commission pursuant to Rule 405 that it is not necessary that the Company be considered an ineligible issuer.

(d) The Company is an "emerging growth company," as defined in Section 2(a) of the Securities Act (an "**Emerging Growth Company**").

(e) Each issuer free writing prospectus, as of its issue date and as of each Applicable Time, did not, does not and will not include any information that conflicted, conflicts or will conflict with the information contained in the Registration Statement or the Prospectus, including any Incorporated Document deemed to be a part thereof that has not been superseded or modified. Each issuer free writing prospectus that the Company has filed, or is required to file, pursuant to Rule 433 or that was prepared by or on behalf of or used by the Company complies or will comply in all material respects with the requirements of the Securities Act.

(f) The Company has not distributed and, prior to the later to occur of each Settlement Date and completion of the Agent's distribution of the Placement Shares under this Agreement, will not distribute any offering material in connection with the offering and sale of the Placement Shares other than the Registration Statement, the Prospectus or any Permitted Free Writing Prospectus (as defined below).

(g) The interactive data in eXtensible Business Reporting Language included or incorporated by reference in the Registration Statement and the Prospectus fairly presents the information called for in all material respects and has been prepared in accordance with the Commission's rules and guidelines applicable thereto.

(h) The Company is subject to and in compliance in all material respects with the reporting requirements of Section 13 or Section 15(d) of the Exchange Act. The Common Stock is registered pursuant to Section 12(b) of the Exchange Act and is listed on Nasdaq, and the Company has taken no action designed to, or reasonably likely to have the effect of, terminating the registration of the Common Stock under the Exchange Act or delisting the Common Stock from Nasdaq, nor has the Company received any notification that the Commission or Nasdaq is contemplating terminating such registration or listing. The Company is in compliance with the current listing standards of Nasdaq. The Company has filed a Notification of Listing of Additional Shares with Nasdaq with respect to the Placement Shares.

(i) No person (as such term is defined in Rule 1-02 of Regulation S-X promulgated under the Securities Act) has the right to act as an underwriter or as a financial advisor to the Company in connection with the offer and sale of the Placement Shares hereunder, whether as a result of the filing or effectiveness of the Registration Statement or the sale of the Placement Shares as contemplated hereby or otherwise. Except for the Agent, there is no broker, finder or other party that is entitled to receive from the Company or any of its Subsidiaries (as defined below) any brokerage or finder's fee or other fee or commission as a result of any transactions contemplated by this Agreement.

(j) The Company has been duly organized and is validly existing as a corporation in good standing under the laws of the State of Delaware, with full corporate power and authority to acquire, own, lease and operate its properties, and to lease the same to others, and to conduct its business as described in the Registration Statement and the Prospectus and to enter into and perform its obligations under this Agreement. The Company is duly qualified to transact business as a foreign corporation and is in good standing in the State of California and under the laws of each other jurisdiction that requires such qualification, whether by reason of the ownership or leasing of property or the conduct of business, except to the extent that the failure to be so qualified or in good standing could not reasonably be expected, individually or in the aggregate, to have a material adverse effect on the condition (financial or otherwise), earnings, results of operations, business, properties, operations, assets, liabilities or prospects of the Company and its Subsidiaries, taken as a whole, whether or not arising from transactions in the ordinary course of business (a "**Material Adverse Effect**").

(k) Each of the Company's "subsidiaries" (for purposes of this Agreement, as defined in Rule 405 under the Securities Act) (each, a "**Subsidiary**" and collectively, the "**Subsidiaries**") has been duly organized and is validly existing in good standing (where such concept exists) under the laws of the jurisdiction of its organization and has full power and authority to acquire, own, lease and operate its properties, and to conduct its business as described in the Registration Statement and the Prospectus. Each Subsidiary is duly qualified to transact business and is in good standing (where such concept exists) under the laws of each jurisdiction that requires such qualification, whether by reason of the ownership or leasing of property or the conduct of business, except to the extent that the failure to be so qualified or in good standing could not reasonably be expected, individually or in the aggregate, to have a Material Adverse Effect. All of the issued and outstanding share capital or other equity or ownership interests of each Subsidiary has been duly authorized and validly issued, is fully paid and nonassessable, has been issued in compliance with federal state and securities laws and is owned by the Company, directly or through other wholly-owned Subsidiaries, free and clear of any security interest, mortgage, pledge, lien, encumbrance or adverse claim. The Company does not own or control, directly or indirectly, any corporation, association or other entity, other than the Subsidiaries listed on Exhibit 21.1 to the Company's most recent Annual Report on Form 10-K filed with the Commission. No Subsidiary is prohibited or restricted, directly or indirectly, from paying dividends to the Company, from making any other distribution with respect to such Subsidiary's equity securities, from repaying to the Company or any other Subsidiary any amounts that may from time to time become due under any loans or advances to such Subsidiary from the Company or from transferring any property or assets to the Company or to any other Subsidiary.

(l) The Company's authorized and outstanding equity capitalization is as set forth in the Company's Quarterly Report on Form 10-Q for the most recent fiscal quarter, as of the dates referred to therein (subject, in each case, to the issuance of Placement Shares under this Agreement, the issuance of shares of Common Stock upon exercise of options and warrants and/or the settlement of restricted stock units disclosed as outstanding as of the date hereof in the Registration Statement and the Prospectus and the grant of options and restricted stock units under existing Stock Plans (as defined below) described in the Registration Statement and the Prospectus) and as may be modified by the Prospectus; the capital stock of the Company conforms in all respects to the description thereof contained in the Registration Statement and the Prospectus as of the date stated therein; the outstanding shares of Common Stock have been duly and validly authorized and issued and are fully paid and nonassessable; and, except as set forth in the Registration Statement and the Prospectus, no options, warrants or other rights to purchase, agreements or other obligations to issue, or rights to convert any obligations into or exchange any securities for, shares of capital stock of or ownership interests in the Company are outstanding. The descriptions of the Company's equity incentive plan, stock option plans and other stock plans or arrangements described in the Prospectus and in effect as of the date hereof (collectively, the "**Stock Plans**") and the options or other rights granted thereunder, set forth in the Registration Statement and the Prospectus accurately and fairly present, in all material respects, the information required to be shown with respect to such Stock Plans and the options or other rights granted thereunder.

(m) The Placement Shares have been duly authorized for issuance and sale pursuant to this Agreement and, when issued and delivered by the Company against payment therefor pursuant to this Agreement, will be validly issued, fully paid and nonassessable and will conform in all material respects to the description thereof contained in the Prospectus. The issuance and sale of the Placement Shares as contemplated hereby shall not be subject to any preemptive rights, rights of first refusal or other similar rights to subscribe for or purchase the Placement Shares. When issued and delivered by the Company against payment therefor pursuant to this Agreement, the purchasers of the Placement Shares issued and sold hereunder will acquire good, marketable and valid title to such Placement Shares, free and clear of

all pledges, liens, security interests, charges, claims or encumbrances. The issuance and sale of the Placement Shares as contemplated hereby will not cause any holder of any share capital, securities convertible into or exchangeable or exercisable for share capital or options, warrants or other rights to purchase share capital or any other securities of the Company to have any right to acquire any preferred shares of the Company. There are no restrictions upon the voting or transfer of the Common Stock under the Company's amended and restated certificate of incorporation or amended and restated bylaws or any agreement or other instrument to which the Company is a party or otherwise filed as an exhibit to the Registration Statement.

(n) There is no franchise, contract or other document of a character required to be described in the Registration Statement or Prospectus, or to be filed as an exhibit to the Registration Statement, which is not described or filed as required.

(o) There is no statute, regulation, contract, agreement or other document required to be described in the Registration Statement, Prospectus or in any Incorporated Document, or to be filed as an exhibit to the Registration Statement or any Incorporated Document which is not described or filed as required. The statements set forth or incorporated by reference in the Prospectus, insofar as they purport to constitute summaries of the terms of the statutes, regulations, contracts, agreements or other documents described and filed, constitute accurate summaries of the terms thereof in all material respects. Neither the Company nor any of its subsidiaries has sent or received any communication regarding termination of, or intent not to renew or render performance under, any of the contracts or agreements referred to or described in the Prospectus or any free writing prospectus, or referred to or described in, or filed as an exhibit to, the Registration Statement, or any Incorporated Document, and no such termination or non-renewal has been threatened by the Company or any of its Subsidiaries or, to the Company's knowledge, any other party to any such contract or agreement, which threat of termination or non-renewal has not been rescinded as of the date hereof.

(p) This Agreement has been duly authorized, executed and delivered by the Company and constitutes a valid and legally binding obligation of the Company, enforceable against the Company in accordance with its terms, except as enforceability, including rights of indemnification, may be limited by bankruptcy, insolvency, fraudulent conveyance, reorganization, moratorium and other similar laws relating to or affecting creditors' rights generally and by general principles of equity. This Agreement conforms in all material respects to the descriptions thereof in the Registration Statement and the Prospectus.

(q) The Company is not and, after giving effect to the offering and sale of the Placement Shares and the application of the proceeds thereof as described in the Registration Statement and the Prospectus, will not be an "investment company" as defined in the Investment Company Act of 1940, as amended.

(r) No consent, approval, license, permit, qualification, authorization or other order or decree of, or registration or filing with, any court or other governmental, taxing or regulatory authority or agency, is required for the Company's execution, delivery and performance of this Agreement or consummation of the transactions contemplated hereby or by the Registration Statement and the Prospectus (including the issuance and sale of the Placement Shares hereunder), except such as have been already obtained or made or as may be required under the Securities Act, applicable state securities or Blue Sky laws, applicable rules of Nasdaq, or Rule 5110 of the Financial Industry Regulatory Authority, Inc. ("**FINRA**").

(s) Neither the execution and delivery by the Company of this Agreement or the issuance and sale of the Placement Shares nor the consummation of any other of the transactions herein contemplated nor the fulfillment of the terms hereof will conflict with, result in a breach or violation of, or imposition of any lien, charge or encumbrance upon any property or assets of the Company or any of its Subsidiaries pursuant to, (i) the charter or bylaws, or similar organizational documents, of the Company or any of its Subsidiaries, (ii) the terms of any indenture, contract, lease, mortgage, deed of trust, note agreement, loan agreement or other agreement, obligation, condition, covenant or instrument to which the Company or any of its Subsidiaries is a party or bound or to which its or their property is subject, or (iii) any statute, law, rule, regulation, judgment, order or decree applicable to the Company or any of its Subsidiaries of any court, regulatory body, administrative agency, governmental body, arbitrator or other authority having jurisdiction over the Company or any of its Subsidiaries or any of its or their properties, except in the case of clauses (ii) and (iii) for any such breach, violation or imposition as would not reasonably be expected, individually or in the aggregate, to result in a Material Adverse Effect.

(t) Subsequent to the respective dates as of which information is given in the Registration Statement and the Prospectus and except as set forth in the Registration Statement or the Prospectus: (i) there has been no material adverse change, or any development that could reasonably be expected to result in a material adverse change, in the condition (financial or otherwise), earnings, results of operations, business, properties, operations, assets, liabilities or prospects of the Company and its Subsidiaries, taken as a whole, whether or not arising from transactions in the ordinary course of business; (ii) neither the Company nor its Subsidiaries has (A) incurred any material liability or obligation, indirect, direct or contingent, including without limitation any losses or interference with its business from fire, explosion, flood, earthquakes, accident or other calamity, whether or not covered by insurance, or from any strike, labor dispute or court or governmental action, order or decree, that are material, individually or in the aggregate, to the Company and its Subsidiaries, considered as one entity, (B) entered into any material transactions not in the ordinary course of business or (C) issued or granted any shares of the Company's capital stock or securities convertible into or exchangeable or exercisable for or that represent the right to receive shares of the Company's capital stock other than under the Stock Plans; and (iii) there has not been any material decrease in the share capital or any material increase in any short-term or long-term indebtedness of the Company or any of its Subsidiaries and there has been no dividend or distribution of any kind declared, paid or made by the Company or, except for dividends paid to the Company or another Subsidiary, by any Subsidiary on any class of shares, or any repurchase or redemption by the Company or any of its Subsidiaries of any class of shares.

(u) There are no persons (as such term is defined in Rule 1-02 of Regulation S-X promulgated under the Securities Act) with registration or other similar rights to have any equity or debt securities of the Company registered for sale under the Registration Statement or included in the offering contemplated by this Agreement, except for such rights as have been duly waived in a writing previously furnished to the Agent.

(v) The financial statements included or incorporated by reference in the Registration Statement and the Prospectus, together with the related notes and schedules, present fairly, in all material respects, the consolidated financial position of the Company and the Subsidiaries as of the dates indicated and the consolidated results of operations, cash flows and changes in stockholders' equity of the Company and the Subsidiaries for the periods specified and have been prepared in compliance with the requirements of the Securities Act and Exchange Act and in conformity with United States generally accepted accounting principles ("**GAAP**") applied on a consistent basis during the periods involved (except as otherwise noted therein). To the extent applicable, any pro forma financial statements, information or data included or incorporated by reference in the Registration Statement and the Prospectus comply with the requirements

of Regulation S-X of the Securities Act, including, without limitation, Article 11 thereof, fairly present the information set forth herein, and the assumptions used in the preparation of such pro forma financial statements and data are reasonable, the pro forma adjustments used therein are appropriate to give effect to the circumstances referred to therein and the pro forma adjustments have been properly applied to the historical amounts in the compilation of those statements and data.

(w) No action, suit or proceeding by or before any court or governmental agency, authority or body or any arbitrator involving the Company or any of its Subsidiaries or its or their property is pending or, to the knowledge of the Company, threatened that would be reasonably expected to have, individually or in the aggregate (i) a material adverse effect on the performance of this Agreement or the consummation of any of the transactions contemplated hereby or (ii) a Material Adverse Effect, except as set forth in or contemplated in the Registration Statement and the Prospectus (exclusive of any amendment or supplement thereto).

(x) Each of the Company and each of its Subsidiaries owns or leases all such properties as are necessary to the conduct of its operations as presently conducted, except as would not reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect.

(y) Neither the Company nor any of its Subsidiaries is in violation or default of (i) any provision of its charter or bylaws, or similar organizational documents, (ii) the terms of any indenture, contract, lease, mortgage, deed of trust, note agreement, loan agreement or other agreement, obligation, condition, covenant or instrument to which it is a party or bound or to which its property is subject, or (iii) any statute, law, rule, regulation, judgment, order or decree of any court, regulatory body, administrative agency, governmental body, arbitrator or other authority having jurisdiction over the Company or such subsidiary or any of its properties, as applicable, except in the case of clauses (ii) and (iii) for any such violation or default as would not reasonably be expected, individually or in the aggregate, to have a Material Adverse Effect.

(z) Ernst & Young LLP, who has certified certain financial statements of the Company and its consolidated Subsidiaries and delivered its report with respect to the audited consolidated financial statements and schedules included in the Registration Statement and the Prospectus, is an independent registered public accounting firm with respect to the Company within the meaning of the Securities Act and the applicable published rules and regulations thereunder.

(aa) The Company has filed all tax returns that are required to be filed by it or has requested extensions thereof (except in any case in which the failure to so file would not have a Material Adverse Effect, except as set forth in or contemplated in the Registration Statement and the Prospectus (exclusive of any amendment or supplement thereto)) and has paid all taxes required to be paid by it and any other assessment, fine or penalty levied against it, to the extent that any of the foregoing is due and payable, except for any such assessment, fine or penalty that is currently being contested in good faith or as would not reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect, and except as set forth in or contemplated in the Registration Statement and the Prospectus (exclusive of any amendment or supplement thereto).

(bb) No labor problem or dispute with the employees of the Company or any of its Subsidiaries exists or, to the knowledge of the Company, is threatened or imminent, and the Company is not aware of any existing or imminent labor disturbance by the employees of any of its or its Subsidiaries' principal suppliers that would reasonably be expected to have, individually or in the aggregate, a Material

Adverse Effect, except as set forth in or contemplated in the Registration Statement and the Prospectus (exclusive of any amendment or supplement thereto).

(cc) The Company and each of its Subsidiaries are insured by insurers of recognized financial responsibility against such losses and risks and in such amounts as the Company believes are customary in the businesses in which they are engaged; all policies of insurance and fidelity or surety bonds insuring the Company or any of its Subsidiaries or their respective businesses, assets, employees, officers and directors are in full force and effect; the Company and its Subsidiaries are in compliance with the terms of such policies and instruments in all material respects; and there are no material claims by the Company or any of its Subsidiaries under any such policy or instrument as to which any insurance company is denying liability or defending under a reservation of rights clause; neither the Company nor any such subsidiary has been refused any insurance coverage sought or applied for; and neither the Company nor any such subsidiary has any reason to believe that it will not be able to renew its existing insurance coverage as and when such coverage expires or to obtain similar coverage from similar insurers as may be necessary to continue its business at a cost that would not reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect, except as set forth in or contemplated in the Registration Statement and the Prospectus (exclusive of any amendment or supplement thereto).

(dd) No subsidiary of the Company is currently prohibited, directly or indirectly, from paying any dividends to the Company, from making any other distribution on such Subsidiary's capital stock, from repaying to the Company any loans or advances to such subsidiary from the Company or from transferring any of such subsidiary's property or assets to the Company or any other Subsidiary of the Company, except as described in or contemplated by the Registration Statement and the Prospectus (exclusive of any amendment or supplement thereto).

(ee) The Company and its Subsidiaries possess all licenses, certificates, permits and other authorizations issued by all applicable authorities necessary to conduct their respective businesses, except for any such failure to possess as would not reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect; and neither the Company nor any such subsidiary has received any notice of proceedings relating to the revocation or modification of any such certificate, authorization or permit which, singly or in the aggregate, if the subject of an unfavorable decision, ruling or finding, would reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect, except as set forth in or contemplated in the Registration Statement and the Prospectus (exclusive of any amendment or supplement thereto).

(ff) The Company and each of its Subsidiaries, considered together as one entity, maintain a system of internal control over financial reporting designed to provide reasonable assurance that (i) transactions are executed in accordance with management's general or specific authorizations; (ii) transactions are recorded as necessary to permit preparation of financial statements in conformity with GAAP and to maintain asset accountability; (iii) access to assets is permitted only in accordance with management's general or specific authorization; (iv) the recorded accountability for assets is compared with the existing assets at reasonable intervals and appropriate action is taken with respect to any differences; and (v) interactive data in eXtensible Business Reporting Language included in the Registration Statement and the Prospectus fairly presents the information called for in all material respects and is prepared in accordance with the Commission's rules and guidelines applicable thereto. The Company and its Subsidiaries' internal controls over financial reporting are effective and the Company and its Subsidiaries are not aware of any material weakness in their internal controls over financial reporting.

(gg) The Company and its Subsidiaries, considered together as one entity, maintain "disclosure controls and procedures" (as such term is defined in Rule 13a-15(e) under the Exchange Act); such disclosure controls and procedures are effective; the Company (on a consolidated basis with its Subsidiaries) has carried out evaluations of the effectiveness of its disclosure controls and procedures as required by Rule 13a-15 of the Exchange Act.

(hh) The Company has not taken, directly or indirectly (without giving effect to the activities of the Agent), any action designed to or that would constitute or that might reasonably be expected to cause or result in, under the Exchange Act or otherwise, stabilization or manipulation of the price of any security of the Company to facilitate the sale or resale of the Placement Shares.

(ii) The Company and its Subsidiaries (i) are in compliance with all applicable foreign, U.S. federal, state and local laws and regulations relating to the protection of human health and safety, the environment or hazardous or toxic substances or wastes, pollutants or contaminants ("**Environmental Laws**"), (ii) have received and are in compliance with all permits, licenses or other approvals required of them under applicable Environmental Laws to conduct their respective businesses and (iii) have not received notice of any actual or potential liability under any Environmental Law, except where such non-compliance with Environmental Laws, failure to receive required permits, licenses or other approvals, or liability would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect, except as set forth in or contemplated in the Registration Statement and the Prospectus (exclusive of any amendment or supplement thereto). Except as set forth in the Registration Statement and the Prospectus, neither the Company nor any of its Subsidiaries has been named as a "potentially responsible party" under the Comprehensive Environmental Response, Compensation, and Liability Act of 1980, as amended.

(jj) On the basis of any periodic review of the effect of Environmental Laws on the business, operations and properties of the Company and its Subsidiaries, the Company has reasonably concluded that the costs and liabilities associated therewith would not, singly or in the aggregate, reasonably be expected to have a Material Adverse Effect, except as set forth in or contemplated in the Registration Statement and the Prospectus (exclusive of any amendment or supplement thereto).

(kk) Nothing has come to the attention of the Company that has caused the Company to believe that the statistical and market-related data included in the Registration Statement and the Prospectus is not based on or derived from sources that are reliable and accurate in all material respects, and, to the extent required by such sources, the Company has obtained the written consent to the use of such data from such sources.

(ll) None of the following events has occurred or exists: (i) a failure to fulfill the obligations, if any, under the minimum funding standards of Section 302 of the United States Employee Retirement Income Security Act of 1974, as amended ("**ERISA**"), and the regulations and published interpretations thereunder with respect to a Plan (as defined herein), determined without regard to any waiver of such obligations or extension of any amortization period, which such failure would reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect; (ii) an audit or investigation by the Internal Revenue Service, the U.S. Department of Labor, the Pension Benefit Guaranty Corporation or any other federal or state governmental agency or any foreign regulatory agency with respect to the employment or compensation of employees by any of the Company or any of its Subsidiaries that would reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect; (iii) any breach of any contractual obligation, or any violation of law or applicable qualification standards, with respect to the employment or compensation of employees by the Company or any of its Subsidiaries that

would reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect. None of the following events has occurred or is reasonably likely to occur: (i) a material increase in the aggregate amount of contributions required to be made to all Plans in the current fiscal year of the Company and its Subsidiaries compared to the amount of such contributions made in the most recently completed fiscal year of the Company and its Subsidiaries, other than increases in the ordinary course resulting from an increase in the number of eligible participants in such Plans or increase resulting from increased participation by eligible participants, in such Plans; (ii) a material increase in the "accumulated post-retirement benefit obligations" (within the meaning of Statement of Financial Accounting Standards 106) of the Company and its Subsidiaries compared to the amount of such obligations in the most recently completed fiscal year of the Company and its Subsidiaries; (iii) any event or condition giving rise to a liability under Title IV of ERISA that would reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect; or (iv) the filing of a claim by one or more employees or former employees of the Company or any of its Subsidiaries related to their employment that would reasonably be expected to have a Material Adverse Effect. For purposes of this paragraph, the term "**Plan**" means a plan (within the meaning of Section 3(3) of ERISA) subject to Title IV of ERISA with respect to which the Company or any of its Subsidiaries may have any liability.

(mm) There is and has been no failure on the part of the Company and any of the Company's directors or officers, in their capacities as such, to comply with any provision of the Sarbanes-Oxley Act of 2002, as amended, and the rules and regulations promulgated in connection thereunder (the "**Sarbanes-Oxley Act**"), including Section 402 relating to loans and Sections 302 and 906 relating to certifications.

(nn) Neither the Company nor any of its Subsidiaries nor, to the knowledge of the Company, any director, officer, agent, employee, affiliate or other person acting on behalf of the Company or any of its Subsidiaries has violated or is in violation of the Foreign Corrupt Practices Act of 1977 or the U.K. Bribery Act 2010, each as may be amended, or similar applicable anti-corruption law of any other relevant jurisdiction (collectively, the "**Anti-Corruption Laws**"); and the Company and its Subsidiaries have instituted and maintain policies and procedures to ensure compliance therewith. No part of the proceeds of the offering will be used, directly or indirectly, in violation of the Anti-Corruption Laws.

(oo) The operations of the Company and its Subsidiaries are and have been conducted at all times in compliance with applicable financial recordkeeping and reporting requirements and the money laundering statutes and the rules and regulations thereunder and any related or similar rules, regulations or guidelines, issued, administered or enforced by any governmental agency (collectively, the "**Money Laundering Laws**") and no action, suit or proceeding by or before any court or governmental agency, authority or body or any arbitrator involving the Company or any of its Subsidiaries with respect to the Money Laundering Laws is pending or, to the knowledge of the Company, threatened.

(pp) Neither the Company nor any of its Subsidiaries nor, to the knowledge of the Company, any director, officer, agent, employee or affiliate of the Company or any of its Subsidiaries (i) is, or is controlled or more than 50% owned in the aggregate by or is acting on behalf of, one or more individuals or entities that are currently the subject of any sanctions administered or enforced by the United States (including any administered or enforced by the Office of Foreign Assets Control of the U.S. Department of the Treasury, the U.S. Department of State or the Bureau of Industry and Security of the U.S. Department of Commerce), the United Nations Security Council, the European Union, a member state of the European Union (including sanctions administered or enforced by His Majesty's Treasury of the United Kingdom) or other relevant sanctions authority (collectively, "**Sanctions**" and such persons, "**Sanctioned Persons**" and each such person, a "**Sanctioned Person**"), (ii) is located, organized or resident in a country or territory that is, or whose government is, the subject of Sanctions that broadly

prohibit dealings with that country or territory (collectively, "**Sanctioned Countries**" and each, a "**Sanctioned Country**") or (iii) will, directly or indirectly, use the proceeds of this offering, or lend, contribute or otherwise make available such proceeds to any subsidiary, joint venture partner or other individual or entity in any manner that would result in a violation of any Sanctions by, or could result in the imposition of Sanctions against, any individual or entity (including any individual or entity participating in the offering, whether as an underwriter, advisor, investor or otherwise).

(qq) Neither the Company nor any of its Subsidiaries has engaged in any dealings or transactions with or for the benefit of a Sanctioned Person, or with or in a Sanctioned Country, since May 2, 2018, nor does the Company or any of its Subsidiaries have any plans to engage in dealings or transactions with or for the benefit of a Sanctioned Person, or with or in a Sanctioned Country.

(rr) The Company and its Subsidiaries own, or have obtained valid and enforceable licenses for, the inventions, patent applications, patents, trademarks, trade names, service names, copyrights, trade secrets, domain names, technology, know-how and other intellectual property (including all registrations and applications for registration of any of the foregoing and all goodwill associated with any of the foregoing) described in the Registration Statement and the Prospectus as being owned ("**Company Owned Intellectual Property**") or licensed by them ("**Company Licensed Intellectual Property**") or which are necessary for the conduct of their respective businesses (collectively, "**Intellectual Property**"). To the Company's knowledge: (i) there are no third parties who have rights to any Intellectual Property, except for customary reversionary rights of third-party licenses with respect to Intellectual Property that is disclosed in the Registration Statement and the Prospectus as licensed to the Company or one or more of its Subsidiaries; (ii) all Company Owned Intellectual Property is free and clear of all liens, encumbrances, or defects; (iii) there is no infringement by third parties of any Intellectual Property; (iv) the Company and its Subsidiaries are not infringing or misappropriating the intellectual property rights of third parties; and (v) the Company and its Subsidiaries are the sole owners of the Company Owned Intellectual Property and have the valid and enforceable right to use the Intellectual Property without the obligation to obtain consent to sublicense and without a duty of accounting to the co-owner, as applicable. The Company and its Subsidiaries have taken reasonable steps necessary to secure assignments to their title, rights and interests in the Company Owned Intellectual Property from their employees, consultants, agents and contractors and to the Company's knowledge, no employee of the Company or its Subsidiaries is in or has been in violation of any term of any employment contract, patent disclosure agreement, invention assignment agreement, noncompetition agreement, non-solicitation agreement, nondisclosure agreement or any restrictive covenant to or with a former employer where the basis of such violation relates to such employee's employment with the Company or its Subsidiaries. There is no pending or, to the Company's knowledge, threatened or notices of action, suit, proceeding or claim by others: (A) challenging the Company and its Subsidiaries' rights in or to any Intellectual Property, and the Company and its Subsidiaries are unaware of any facts which would form a reasonable basis for any such action, suit, proceeding or claim; (B) challenging the validity, enforceability or scope of any Intellectual Property, and the Company and its Subsidiaries are unaware of any facts which would form a reasonable basis for any such action, suit, proceeding or claim; or (C) asserting that the Company or its Subsidiaries infringe, misappropriate or otherwise violate, or would, upon the manufacturing or commercialization of any product or service described in the Registration Statement or the Prospectus as under development, infringe, misappropriate or violate, any patent, trademark, trade name, service name, copyright, trade secret or other proprietary rights of others, and the Company and its Subsidiaries are unaware of any facts which would form a reasonable basis for any such action, suit, proceeding or claim. The Company and its Subsidiaries have complied with the terms of each agreement pursuant to which Intellectual Property has been licensed to the Company or its Subsidiaries, and, to the knowledge of the Company, all such agreements are in full force and effect. The product candidates described in the Registration Statement

and the Prospectus as under development by the Company and its Subsidiaries fall within the scope of the claims of one or more patents or patent applications owned by, or exclusively licensed to, the Company or its Subsidiaries. No government funding, facilities or resources of a university, college, other educational institution or research center was used in the development of any Intellectual Property that is owned or purported to be owned by the Company and its Subsidiaries that would confer upon any governmental agency or body, university, college, other educational institution or research center any claim or right of ownership to any such Intellectual Property. The Company and its Subsidiaries have taken commercially reasonable actions in accordance with customary industry practice to maintain and protect all Intellectual Property owned by or exclusively licensed to the Company or its Subsidiaries, including the maintenance and protection of all trade secrets, know-how and other confidential information.

(ss) All patents and patent applications owned by or exclusively licensed to the Company and its Subsidiaries or under which the Company and its Subsidiaries have rights have, to the knowledge of the Company, been duly and properly filed and each issued patent is being diligently maintained and are valid and enforceable. The Company is unaware of any facts that would preclude the issuance of a valid and enforceable patent on any pending patent application included in the Intellectual Property. To the knowledge of the Company, the Company, its affiliates and the parties prosecuting such patent applications have complied with their duty of candor and disclosure to the U.S. Patent and Trademark Office ("**USPTO**") in connection with such patents and patent applications for which it has filing, prosecution, and/or maintenance responsibilities. The Company is not aware of any prior art or public or commercial activity or other facts required to be disclosed to the USPTO that were not disclosed to the USPTO and which would preclude the grant of a patent in connection with any such patent application or would reasonably be expected to form the basis of a finding of invalidity with respect to any patents that have been issued with respect to such patent applications.

(tt) The Company and its Subsidiaries: (i) have operated and currently operate their respective businesses in compliance in all material respects with all Health Care Laws (as defined below) applicable to the ownership, testing, development, manufacture, packaging, processing, use, distribution, storage, import, export or disposal of any of the Company's and its Subsidiaries' product candidates or any product manufactured or distributed by the Company and its Subsidiaries; (ii) have not received any Food and Drug Administration ("**FDA**") Form 483, written notice of adverse finding, warning letter, untitled letter or other correspondence or written notice from any court or arbitrator or governmental or regulatory authority alleging or asserting material non-compliance with (A) any Health Care Laws or (B) or any licenses, certificates, approvals, clearances, exemptions, authorizations, permits and supplements or amendments thereto materially required by any such Health Care Laws ("**Regulatory Authorizations**"); (iii) possess all Regulatory Authorizations materially required to conduct their respective business as currently conducted and such Regulatory Authorizations are valid and in full force and effect and the Company and its Subsidiaries are not in violation, in any material respect, of any term of any such Regulatory Authorizations; (iv) have not received written notice of any claim, action, suit, proceeding, hearing, enforcement, investigation, arbitration or other action from the FDA, the Department of Health and Human Services or any comparable foreign or other regulatory authority to which they are subject (collectively, the "**Applicable Regulatory Authorities**") or any other third party alleging that any product operation or activity is in material violation of any Health Care Laws or Regulatory Authorizations and have no knowledge that the Applicable Regulatory Authorities or any other third party is considering any such claim, litigation, arbitration, action, suit, investigation or proceeding; (v) have not received written notice that any of the Applicable Regulatory Authorities has taken, is taking or intends to take action to limit, suspend, modify or revoke any Regulatory Authorizations and have no knowledge that any of the Applicable Regulatory Authorities is considering such action; (vi) have filed, obtained, maintained or submitted all material reports, documents, forms, notices, applications, records, claims,

submissions and supplements or amendments as required by any Health Care Laws or Regulatory Authorizations and that all such reports, documents, forms, notices, applications, records, claims, submissions and supplements or amendments were, to the Company's knowledge, materially complete and correct on the date filed (or were materially corrected or supplemented by a subsequent submission); (vii) are not a party to or have any ongoing reporting obligations pursuant to any corporate integrity agreements, deferred or non-prosecution agreements, monitoring agreements, consent decrees, settlement orders, plans of correction or similar agreements with or imposed by any Applicable Regulatory Authority; and (viii) have not been, and their respective employees, officers and directors have not been, excluded, suspended or debarred from participation in any government health care program or human clinical research or, to the knowledge of the Company, are subject to a governmental inquiry, investigation, proceeding, or other similar action that could reasonably be expected to result in debarment, suspension, or exclusion.

The term "**Health Care Laws**" means Title XVIII of the Social Security Act, 42 U.S.C. §§ 1395-1395hhh (the Medicare statute); Title XIX of the Social Security Act, 42 U.S.C. §§ 1396-1396v (the Medicaid statute); the Federal Anti-Kickback Statute, 42 U.S.C. § 1320a-7b(b); the civil False Claims Act, 31 U.S.C. §§ 3729 et seq.; the criminal False Claims Act, 42 U.S.C. § 1320a-7b(a); any criminal laws relating to health care fraud and abuse, including but not limited to 18 U.S.C. Sections 286 and 287, and the health care fraud criminal provisions under the Health Insurance Portability and Accountability Act of 1996, 42 U.S.C. §§ 1320d et seq. ("**HIPAA**"); the Civil Monetary Penalties Law, 42 U.S.C. § 1320a-7a; the Physician Payments Sunshine Act, 42 U.S.C. § 1320a-7h; the Exclusions Law, 42 U.S.C. § 1320a-7; HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, 42 U.S.C. §§ 17921 et seq. ("**HITECH**"); the Federal Food, Drug, and Cosmetic Act, 21 U.S.C. §§ 301 et seq.; the regulations promulgated pursuant to such laws; and any similar federal, state and local laws and regulations.

(uu) To the Company's knowledge, the manufacturing facilities and operations of its and its Subsidiaries' suppliers are operated in compliance in all material respects with all applicable statutes, rules, regulations and policies of the Applicable Regulatory Authorities, including the Health Care Laws.

(vv) The studies, tests and preclinical and clinical trials conducted by or on behalf of, or sponsored by, the Company or its Subsidiaries, or in which the Company or its Subsidiaries have participated, that are described in the Registration Statement or the Prospectus, or the results of which are referred to in the Registration Statement or the Prospectus, were and, if still pending, are being conducted in all material respects in accordance with protocols, procedures and controls pursuant to, where applicable, accepted professional and scientific standards for products or product candidates comparable to those being developed by the Company or its Subsidiaries and all applicable statutes, rules and regulations of the FDA and other comparable regulatory agencies outside of the United States to which they are subject, including, without limitation, 21 C.F.R. Parts 50, 54, 56, 58, and 312; the descriptions of the results of such studies, tests and trials contained in the Registration Statement or the Prospectus do not contain any misstatement of a material fact or omit a material fact necessary to make such statements not misleading; the Company has no knowledge of any studies, tests or trials not described in the Registration Statement and the Prospectus the results of which reasonably call into question in any material respect the results of the studies, tests and trials described in the Registration Statement or Prospectus; and the Company and its Subsidiaries have not received any written notices or other correspondence from the FDA or any other foreign, state or local governmental body exercising comparable authority or any Institutional Review Board or comparable authority requiring or threatening the termination, suspension or material modification of any studies, tests or preclinical or clinical trials conducted by or on behalf of, or sponsored by, the Company or its Subsidiaries or in which the Company or its Subsidiaries have

participated, and, to the Company's knowledge, there are no reasonable grounds for the same. Except as disclosed in the Registration Statement and the Prospectus, there has not been any material violation of law or regulation by the Company or its Subsidiaries in their respective product development efforts, submissions or reports to any regulatory authority that could reasonably be expected to require investigation, corrective action or enforcement action.

(ww) There are no debt securities or preferred stock issued or guaranteed by the Company or any of its Subsidiaries that are rated by a "nationally recognized statistical rating organization", as such term is defined in Section 3(a)(62) under the Exchange Act.

(xx) No financial or operational projection or other "forward-looking statement" (as defined by Section 27A of the Securities Act or Section 21E of the Exchange Act) contained in the Prospectus has been made or reaffirmed without a reasonable basis or has been disclosed other than in good faith; *provided that* for documents incorporated by reference in the Prospectus, forward-looking statements speak only as to the date they were made.

(yy) The Company has not distributed and, prior to the later to occur of each Settlement Date and completion of the Agent's distribution of the Placement Shares under this Agreement, will not distribute any offering material in connection with the offering and sale of the Placement Shares other than the Registration Statement, the Prospectus or any Permitted Free Writing Prospectus (as defined below).

(zz) The Company acknowledges and agrees that the Agent has informed the Company that the Agent may, to the extent permitted under the Securities Act and the Exchange Act, purchase and sell shares of Common Stock for its own account while this Agreement is in effect; *provided*, that (i) no such purchase or sales shall take place while a Placement Notice is in effect (except to the extent the Agent may engage in sales of Placement Shares purchased or deemed purchased from the Company as a "riskless principal" or in a similar capacity) and (ii) the Company shall not be deemed to have authorized or consented to any such purchases or sales by the Agent, except as may be otherwise agreed by the Company and the Agent.

(aaa) The Company is not a party to any agreement with an agent or underwriter for any other "at the market offering" or continuous equity transaction. No person (as such term is defined in Rule 1-02 of Regulation S-X promulgated under the Securities Act) has the right to act as an underwriter or as a financial advisor to the Company in connection with the offer and sale of the Placement Shares hereunder, whether as a result of the filing or effectiveness of the Registration Statement or the sale of the Placement Shares as contemplated hereby or otherwise. Except for the Agent, there is no broker, finder or other party that is entitled to receive from the Company or any of its Subsidiaries any brokerage or finder's fee or other fee or commission as a result of any transactions contemplated by this Agreement.

Any certificate signed by any officer of the Company and delivered to the Agent or its counsel in connection with the offering of the Placement Shares shall be deemed a representation and warranty by the Company, as to matters covered thereby, to the Agent.

7. Covenants of the Company. The Company covenants and agrees with the Agent that:

(a) Registration Statement Amendments. After the date of this Agreement and during any period in which the Prospectus relating to any Placement Shares is required to be delivered by the Agent under the Securities Act (including in circumstances where such requirement may be satisfied pursuant to Rule

172 under the Securities Act or a similar rule); (i) the Company will notify the Agent promptly of the time when any subsequent amendment to the Registration Statement, other than Incorporated Documents, has been filed with the Commission and/or has become effective or any subsequent supplement to the Prospectus, other than Incorporated Documents, has been filed and of any request by the Commission for any amendment or supplement to the Registration Statement or Prospectus or for additional information (in each case, insofar as it relates to the transactions contemplated hereby); (ii) the Company will prepare and file with the Commission, promptly upon the Agent's reasonable request, any amendments or supplements to the Registration Statement or Prospectus that, in the Agent's reasonable opinion, may be necessary or advisable in connection with the distribution of the Placement Shares by the Agent (provided, however, that the failure of the Agent to make such request shall not relieve the Company of any obligation or liability hereunder, or affect the Agent's right to rely on the representations and warranties made by the Company in this Agreement and provided, further, that the only remedy the Agent shall have with respect to the failure by the Company to make such filing (but without limiting the Agent's rights under Section 9 hereof) will be to cease making sales under this Agreement until such amendment or supplement is filed); (iii) the Company will not file any amendment or supplement to the Registration Statement or Prospectus, other than Incorporated Documents, relating to the Placement Shares or a security convertible into or exchangeable or exercisable for the Placement Shares unless a copy thereof has been submitted to the Agent within a reasonable period of time before the filing and the Agent has not reasonably objected thereto (provided, however, that the failure of the Agent to make such objection shall not relieve the Company of any obligation or liability hereunder, or affect the Agent's right to rely on the representations and warranties made by the Company in this Agreement and provided, further, that the only remedy the Agent shall have with respect to the failure by the Company to make such filing (but without limiting the Agent's rights under Section 9 hereof) will be to cease making sales under this Agreement until such amendment or supplement is filed) and the Company will furnish to the Agent at the time of filing thereof a copy of any Incorporated Document, except for those documents available via EDGAR; and (iv) the Company will cause each amendment or supplement to the Prospectus, other than Incorporated Documents, to be filed with the Commission as required pursuant to the applicable paragraph of Rule 424(b) of the Securities Act and, in the case of any Incorporated Document, to be filed with the Commission as required pursuant to the Exchange Act, within the time period prescribed (the determination to file or not file any amendment or supplement with the Commission under this Section 7(a), based on the Company's reasonable opinion or reasonable objections, shall be made exclusively by the Company).

(b) Notice of Commission Stop Orders. The Company will advise the Agent, promptly after it receives notice or obtains knowledge thereof, of the issuance or threatened issuance by the Commission of any stop order suspending the effectiveness of the Registration Statement, of the suspension of the qualification of the Placement Shares for offering or sale in any jurisdiction or of the initiation or threatening of any proceeding for any such purpose; and it will promptly use its commercially reasonable efforts to prevent the issuance of any stop order or to obtain its withdrawal if such a stop order should be issued. The Company will advise the Agent promptly after it receives any request by the Commission for any amendments to the Registration Statement or any amendment or supplements to the Prospectus or for additional information related to the offering of the Placement Shares or for additional information related to the Registration Statement or the Prospectus.

(c) Delivery of Prospectus; Subsequent Changes. During any period in which the Prospectus relating to the Placement Shares is required to be delivered by the Agent under the Securities Act with respect to the offer and sale of the Placement Shares (including in circumstances where such requirement may be satisfied pursuant to Rule 172 under the Securities Act or a similar rule), the Company will comply with all requirements imposed upon it by the Securities Act, as from time to time in force, and

will file on or before their respective due dates (taking into account any extensions available under the Exchange Act) all reports and any definitive proxy or information statements required to be filed by the Company with the Commission pursuant to Sections 13(a), 13(c), 14, 15(d) or any other provision of or under the Exchange Act. If during such period any event occurs as a result of which the Prospectus as then amended or supplemented would include an untrue statement of a material fact or omit to state a material fact necessary to make the statements therein, in the light of the circumstances then existing, not misleading, or if during such period it is necessary to amend or supplement the Registration Statement or Prospectus to comply with the Securities Act, the Company will promptly notify the Agent to suspend the offering of Placement Shares during such period and the Company will promptly amend or supplement the Registration Statement or Prospectus (at the expense of the Company) so as to correct such statement or omission or effect such compliance. If the Company has omitted any information from the Registration Statement pursuant to Rule 430B under the Securities Act, it will use its best efforts to comply with the provisions thereof and make all requisite filings with the Commission pursuant to said Rule 430B and to notify the Agent promptly of all such filings if not available on EDGAR.

(d) Listing of Placement Shares. During any period in which the Prospectus relating to the Placement Shares is required to be delivered by the Agent under the Securities Act with respect to the offer and sale of the Placement Shares (including in circumstances where such requirement may be satisfied pursuant to Rule 172 under the Securities Act or a similar rule), the Company will use its commercially reasonable efforts to cause the Placement Shares to be listed on Nasdaq. The Company will timely file with Nasdaq all material documents and notices required by Nasdaq of companies that have or will issue securities that are traded on Nasdaq.

(e) Delivery of Registration Statement and Prospectus. The Company will furnish to the Agent and its counsel (at the expense of the Company) copies of the Registration Statement, the Prospectus (including all Incorporated Documents) and all amendments and supplements to the Registration Statement or Prospectus that are filed with the Commission during any period in which the Prospectus relating to the Placement Shares is required to be delivered under the Securities Act (including all Incorporated Documents filed with the Commission during such period), in each case as soon as reasonably practicable and in such quantities as the Agent may from time to time reasonably request and, at the Agent's request, will also furnish copies of the Prospectus to each exchange or market on which sales of the Placement Shares may be made; *provided*, *however*, that the Company shall not be required to furnish any document (other than the Prospectus) to the Agent to the extent such document is available on EDGAR.

(f) Earnings Statement. The Company will make generally available to its security holders as soon as practicable, but in any event not later than 15 months after the end of the Company's current fiscal quarter, an earnings statement covering a 12-month period that satisfies the provisions of Section 11(a) of and Rule 158 under the Securities Act.

(g) Expenses. The Company, whether or not the transactions contemplated hereunder are consummated or this Agreement is terminated in accordance with the provisions of Section 11 hereunder, will pay all expenses incident to the performance of its obligations hereunder, including expenses relating to (i) the preparation, printing and filing of the Registration Statement and each amendment and supplement thereto, of the Prospectus and of each amendment and supplement thereto and of this Agreement and such other documents as may be required in connection with the offering, purchase, sale, issuance or delivery of the Placement Shares, (ii) the preparation, issuance, sale and delivery of the Placement Shares and any taxes due or payable in connection therewith, (iii) the qualification of the Placement Shares under securities laws in accordance with the provisions of Section 7(w) of this

Agreement, including filing fees (provided, however, that any fees or disbursements of counsel for the Agent in connection therewith shall be paid by the Agent except as set forth in clauses (vii) and (viii) below), (iv) the printing and delivery to the Agent and its counsel of copies of the Prospectus and any amendments or supplements thereto, and of this Agreement, (v) the fees and expenses incurred in connection with the listing or qualification of the Placement Shares for trading on Nasdaq, (vi) the filing fees and expenses, if any, owed to the Commission or FINRA and the fees and expenses of any transfer agent or registrar for the Shares, (vii) the fees and associated expenses of the Agent's outside legal counsel for filings with the FINRA Corporate Financing Department in an amount not to exceed $20,000 (excluding FINRA filing fees referred to in clause (vi) above and in addition to the fees and disbursements referred to in clause (viii) below), and (viii) the reasonable fees and disbursements of the Agent's outside legal counsel (A) in an amount not to exceed $75,000 arising out of executing this Agreement and the Company's delivery of the initial certificate pursuant to Section 7(m) and (B) in an amount not to exceed $15,000 in connection with each Representation Date (as defined below) on which the Company is required to provide a certificate pursuant to Section 7(m) (in addition to the fees and associated expenses referred to in clause (vii) above).

(h) Use of Proceeds. The Company will use the Net Proceeds as described in the Prospectus in the section entitled "Use of Proceeds."

(i) Notice of Other Sales. Without the prior written consent of the Agent, the Company will not, directly or indirectly, offer to sell, sell, contract to sell, grant any option to sell or otherwise dispose of any shares of Common Stock (other than the Placement Shares offered pursuant to this Agreement) or securities convertible into or exchangeable or exercisable for shares of Common Stock, warrants or any rights to purchase or acquire shares of Common Stock during the period beginning on the fifth Trading Day immediately prior to the date on which any Placement Notice is delivered to Agent hereunder and ending on the second Trading Day immediately following the final Settlement Date with respect to Placement Shares sold pursuant to such Placement Notice (or, if the Placement Notice has been terminated or suspended prior to the sale of all Placement Shares covered by a Placement Notice, the date of such suspension or termination); and will not directly or indirectly in any other "at the market offering" or continuous equity transaction offer to sell, sell, contract to sell, grant any option to sell or otherwise dispose of any shares of Common Stock (other than the Placement Shares offered pursuant to this Agreement) or securities convertible into or exchangeable or exercisable for shares of Common Stock, warrants or any rights to purchase or acquire, shares of Common Stock prior to the later of the termination of this Agreement and the sixtieth day immediately following the final Settlement Date with respect to Placement Shares sold pursuant to such Placement Notice; *provided*, that such restrictions shall not apply in connection with the (i) issuance, grant or sale of Common Stock, options or warrants to purchase shares of Common Stock, restricted shares of Common Stock, restricted stock units or other equity awards, or Common Stock issuable upon the exercise of options or other equity awards pursuant to any Stock Plan, (ii) the issuance of securities in connection with an acquisition, merger or sale or purchase of assets, (iii) the issuance or sale of Common Stock pursuant to any dividend reinvestment plan that the Company may adopt from time to time provided the implementation of such is disclosed to the Agent in advance, (iv) any shares of Common Stock issuable upon the exchange, conversion or redemption of securities or the exercise of warrants, options or other rights in effect or outstanding or (v) the issuance or sale of Common Stock, or securities convertible into or exercisable for Common Stock offered and sold in a private placement transaction to vendors, customers or strategic partners if the primary purpose is not a capital raising transaction. Notwithstanding the foregoing provisions, nothing herein shall be construed to restrict the Company's ability, or require the Company to provide notice to the Agent, to file a registration statement under the Securities Act.

(j) Change of Circumstances. The Company will, at any time during a fiscal quarter in which the Company intends to tender a Placement Notice or sell Placement Shares, advise the Agent promptly after it shall have received notice or obtained knowledge of any information or fact that would alter or affect in any material respect any opinion, certificate, letter or other document provided or required to be provided to the Agent pursuant to this Agreement.

(k) Due Diligence Cooperation. During the term of this Agreement, the Company will cooperate with any reasonable due diligence review conducted by the Agent, its affiliates agents and counsel from time to time in connection with the transactions contemplated hereby, including providing information and making available documents and senior corporate officers, during regular business hours and at the Company's principal offices, as the Agent may reasonably request.

(l) Required Filings Relating to Placement of Placement Shares. The Company agrees that on or prior to such dates as the Securities Act shall require with respect to the Placement Shares, the Company will (i) file a prospectus supplement with the Commission under the applicable paragraph of Rule 424(b) under the Securities Act, which prospectus supplement will set forth, within the relevant period, the number or amount of Placement Shares sold through the Agent, the Net Proceeds to the Company and the compensation payable by the Company to the Agent with respect to such Placement Shares, and (ii) deliver such number of copies of each such prospectus supplement to each exchange or market on which such sales were effected as may be required by the rules or regulations of such exchange or market; *provided*, that, unless a prospectus supplement containing such information is required to be filed under the Securities Act, the requirement of this Section 7(l) may be satisfied by Company's inclusion in the Company's Form 10-K or Form 10-Q, as applicable, of the number or amount of Placement Shares sold through the Agent, the Net Proceeds to the Company and the compensation payable by the Company to the Agent with respect to such Placement Shares during the relevant period.

(m) Representation Dates; Certificate. On or prior to the date on which the Company first delivers a Placement Notice pursuant to this agreement (the "**First Placement Notice Date**") and each time the Company:

(i) amends or supplements the Registration Statement or the Prospectus relating to the Placement Shares (other than a prospectus supplement filed in accordance with Section 7(l) of this Agreement) by means of a post-effective amendment, sticker or supplement but not by means of incorporation of document(s) by reference into the Registration Statement or the Prospectus relating to the Placement Shares;

(ii) files an annual report on Form 10-K under the Exchange Act (including any Form 10-K/A containing amended financial information or a material amendment to the previously filed Form 10-K);

(iii) files a quarterly report on Form 10-Q under the Exchange Act; or

(iv) files a current report on Form 8-K containing amended financial information (other than an earnings release that is "furnished" pursuant to Item 2.02 or Item 7.01 of Form 8-K) under the Exchange Act (each date of filing of one or more of the documents referred to in clauses (i) through (iv) shall be a "**Representation Date**"), the Company shall furnish the Agent (but in the case of clause (iv) above only if (1) a Placement Notice is pending or in effect and (2) the Agent requests such certificate within three Business Days after the filing of such Form 8-K with the Commission) with a certificate, in the form attached hereto as **Exhibit 7(m)** (modified, as necessary, to relate to the Registration Statement

and the Prospectus as then amended or supplemented), within two Trading Days of any Representation Date. The requirement to provide a certificate under this Section 7(m) shall be waived for any Representation Date occurring at a time at which no Placement Notice is pending or in effect, which waiver shall continue until the earlier to occur of (1) the date the Company delivers a Placement Notice hereunder (which for such calendar quarter shall be considered a Representation Date) and (2) the next occurring Representation Date. Notwithstanding the foregoing, if the Company subsequently decides to sell Placement Shares following a Representation Date on which the Company relied on the waiver referred to in the previous sentence and did not provide the Agent with a certificate under this Section 7(m), then before the Company delivers a Placement Notice or the Agent sells any Placement Shares pursuant thereto, the Company shall provide the Agent with a certificate, in the form attached hereto as **Exhibit 7(m)**, dated the date of such Placement Notice. Within two Trading Days of each Representation Date, the Company shall have furnished to the Agent such further information, certificates and documents as the Agent may reasonably request.

(n) Legal Opinions. On or prior to the First Placement Notice Date and no less than once per fiscal year, the Company shall cause to be furnished to the Agent the written opinion of Cooley LLP, counsel to the Company, or such other counsel satisfactory to the Agent ("**Company Counsel**"), and on or prior to the First Placement Notice Date and any date which the Company is obligated to deliver a certificate pursuant to Section 7(m) for which no waiver is applicable, the Company shall cause to be furnished to the Agent the written opinion and negative assurance letter of Company Counsel, each in form and substance satisfactory to the Agent and its counsel, dated the date that the opinion and negative assurance letter are required to be delivered, modified, as necessary, to relate to the Registration Statement and the Prospectus as then amended or supplemented; *provided, however*, that in lieu of such opinion and negative assurance letter for subsequent Representation Dates, Company Counsel may furnish the Agent with a letter to the effect that the Agent may rely on a prior opinion or negative assurance letter delivered by such counsel under this Section 7(n) to the same extent as if it were dated the date of such letter (except that statements in such prior opinion or negative assurance letter shall be deemed to relate to the Registration Statement and the Prospectus as amended or supplemented at such Representation Date).

(o) Intellectual Property Opinion. On or prior to the First Placement Notice Date and no less than once per fiscal year, the Company shall cause to be furnished to the Agent the written opinion of Wilson Sonsini Goodrich & Rosati, counsel for the Company with respect to intellectual property matters, or such other intellectual property counsel satisfactory to the Agent ("**Intellectual Property Counsel**"), in form and substance satisfactory to the Agent and its counsel, dated the date that the opinion letter is required to be delivered, modified, as necessary, to relate to the Registration Statement and the Prospectus as then amended or supplemented; *provided*, *however*, that in lieu of such written opinion for subsequent Representation Dates, Intellectual Property Counsel may furnish the Agent with a letter to the effect that the Agent may rely on a prior opinion letter delivered by such counsel under this Section 7(o) to the same extent as if it were dated the date of such opinion letter (except that statements in such prior opinion letter shall be deemed to relate to the Registration Statement and the Prospectus as amended or supplemented at such Representation Date).

(p) Comfort Letter. On or prior to the First Placement Notice Date and on any date which the Company is obligated to deliver a certificate pursuant to Section 7(m) for which no waiver is applicable, the Company shall cause its independent registered public accounting firm (and any other independent accountants whose report is included in the Registration Statement or the Prospectus) to furnish the Agent letters (the "**Comfort Letters**"), dated the date the Comfort Letter is delivered, which shall meet the requirements set forth in this Section 7(p); *provided*, that if requested by the Agent, the Company shall

cause a Comfort Letter to be furnished to the Agent within 10 Trading Days of the occurrence of any material transaction or event that necessitates the filing of additional, pro forma, amended or revised financial statements (including any restatement of previously issued financial statements). Each Comfort Letter shall be in form and substance satisfactory to the Agent and each Comfort Letter from the Company's independent registered public accounting firm shall (i) confirm that they are an independent registered public accounting firm within the meaning of the Securities Act and the PCAOB, (ii) state, as of such date, the conclusions and findings of such firm with respect to the financial information and other matters ordinarily covered by accountants' "comfort letters" to underwriters in connection with registered public offerings (the first such letter, the "**Initial Comfort Letter**") and (iii) update the Initial Comfort Letter with any information that would have been included in the Initial Comfort Letter had it been given on such date and modified as necessary to relate to the Registration Statement and the Prospectus, as amended and supplemented to the date of such letter.

(q) Market Activities. The Company will not, directly or indirectly, and will cause its officers, directors and Subsidiaries not to (i) take any action designed to cause or result in, or that constitutes or might reasonably be expected to constitute, the stabilization or manipulation of the price of any security of the Company to facilitate the sale or resale of shares of Common Stock or (ii) sell, bid for, or purchase shares of Common Stock to be issued and sold pursuant to this Agreement in violation of Regulation M, or pay anyone any compensation for soliciting purchases of the Placement Shares other than the Agent; *provided*, *however*, that the Company may bid for and purchase shares of Common Stock in accordance with Rule 10b-18 under the Exchange Act.

(r) Insurance. The Company and its Subsidiaries shall maintain, or cause to be maintained, insurance in such amounts and covering such risks as is reasonable and customary for the business for which it is engaged.

(s) Compliance with Laws. The Company and each of its Subsidiaries shall maintain, or cause to be maintained, all material environmental certificates, authorizations or permits required by federal, state and local law in order to conduct their businesses as described in the Prospectus (collectively, "**Permits**"), and the Company and each of its Subsidiaries shall conduct their businesses, or cause their businesses to be conducted, in substantial compliance with such Permits and with applicable Environmental Laws, except where the failure to maintain or be in compliance with such Permits could not reasonably be expected to result in a Material Adverse Effect.

(t) Investment Company Act. The Company will conduct its affairs in such a manner so as to reasonably ensure that neither it nor any of its Subsidiaries will be or become, at any time prior to the termination of this Agreement, an "investment company," as such term is defined in the Investment Company Act.

(u) Securities Act and Exchange Act. The Company will use its best efforts to comply with all requirements imposed upon it by the Securities Act and the Exchange Act as from time to time in force, so far as necessary to permit the sales of, or dealings in, the Placement Shares as contemplated by the provisions hereof and the Prospectus.

(v) No Offer to Sell. Other than a free writing prospectus (as defined in Rule 405 under the Securities Act) approved in advance by the Company and the Agent, neither the Agent nor the Company (including its agents and representatives, other than the Agent in its capacity as agent) will make, use, prepare, authorize, approve or refer to any written communication (as defined in Rule 405 under the

Securities Act), required to be filed with the Commission, that constitutes an offer to sell or solicitation of an offer to buy Placement Shares hereunder.

(w) Blue Sky and Other Qualifications. The Company will use its commercially reasonable efforts, in cooperation with the Agent, to qualify the Placement Shares for offering and sale, or to obtain an exemption for the Placement Shares to be offered and sold, under the applicable securities laws of such states and other jurisdictions (domestic or foreign) as the Agent may designate and to maintain such qualifications and exemptions in effect for so long as required for the distribution of the Placement Shares (but in no event for less than one year from the date of this Agreement); *provided, however*, that the Company shall not be obligated to file any general consent to service of process or to qualify as a foreign corporation or as a dealer in securities in any jurisdiction in which it is not so qualified or to subject itself to taxation in respect of doing business in any jurisdiction in which it is not otherwise so subject. In each jurisdiction in which the Placement Shares have been so qualified or exempt, the Company will file such statements and reports as may be required by the laws of such jurisdiction to continue such qualification or exemption, as the case may be, in effect for so long as required for the distribution of the Placement Shares (but in no event for less than one year from the date of this Agreement).

(x) Sarbanes-Oxley Act. The Company will maintain and keep accurate books and records reflecting its assets and maintain internal accounting controls in a manner designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with GAAP and including those policies and procedures that (i) pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of the assets of the Company, (ii) provide reasonable assurance that transactions are recorded as necessary to permit the preparation of the Company's financial statements in accordance with GAAP, (iii) that receipts and expenditures of the Company are being made only in accordance with management's and the Company's directors' authorization, and (iv) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of the Company's assets that could have a material effect on its financial statements. The Company will maintain such controls and other procedures, including, without limitation, those required by Sections 302 and 906 of the Sarbanes-Oxley Act, and the applicable regulations thereunder that are designed to ensure that information required to be disclosed by the Company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Commission's rules and forms, including, without limitation, controls and procedures designed to ensure that information required to be disclosed by the Company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the Company's management, including its principal executive officer and principal financial officer, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure and to ensure that material information relating to the Company is made known to it by others within the Company, particularly during the period in which such periodic reports are being prepared.

(y) Emerging Growth Company. The Company will promptly notify the Agent if the company ceases to be an Emerging Growth Company at any time prior to the completion of the Agent's distribution of the Placement Shares pursuant to this Agreement.

(z) Renewal of Registration Statement. If, immediately prior to the third anniversary of the initial effective date of the Registration Statement (the "**Renewal Date**"), any of the Placement Shares remain unsold and this Agreement has not been terminated, the Company will, prior to the Renewal Date, file a new shelf registration statement or, if applicable, an automatic shelf registration statement relating to the Common Stock that may be offered and sold pursuant to this Agreement (which shall include a

prospectus reflecting the number or amount of Placement Shares that may be offered and sold pursuant to this Agreement), in a form satisfactory to the Agent and its counsel, and, if such registration statement is not an automatic shelf registration statement, will use its best efforts to cause such registration statement to be declared effective within 180 days after the Renewal Date. The Company will take all other reasonable actions necessary or appropriate to permit the public offer and sale of the Placement Shares to continue as contemplated in the expired registration statement and this Agreement. From and after the effective date thereof, references herein to the "Registration Statement" shall include such new shelf registration statement or such new automatic shelf registration statement, as the case may be.

(aa) Tax Indemnity. The Company will indemnify and hold harmless the Agent against any documentary, stamp or similar issue tax, including any interest and penalties, on the issue and sale of the Placement Shares.

(bb) Transfer Agent. The Company has engaged and will maintain, at its sole expense, a transfer agent and registrar for the Common Stock.

8. Conditions to the Agent's Obligations. The obligations of the Agent hereunder with respect to a Placement will be subject to the continuing accuracy and completeness of the representations and warranties made by the Company herein, to the due performance by the Company of its obligations hereunder, to the completion by the Agent of a due diligence review satisfactory to the Agent in its reasonable judgment, and to the continuing satisfaction (or waiver by the Agent in its sole discretion) of the following additional conditions:

(a) Registration Statement Effective. The Registration Statement shall be effective and shall be available for all offers and sales of Placement Shares (i) that have been issued pursuant to all prior Placement Notices and (ii) that will be issued pursuant to any Placement Notice.

(b) Prospectus Supplement. The Company shall have filed with the Commission the Prospectus Supplement with respect to the Placement Shares, pursuant to Rule 424(b) under the Securities Act, not later than the Commission's close of business on the second Business Day following the date of this Agreement.

(c) No Material Notices. None of the following events shall have occurred and be continuing: (i) receipt by the Company or any of its Subsidiaries of any request for additional information from the Commission or any other federal or state governmental authority during the period of effectiveness of the Registration Statement, the response to which would require any post-effective amendments or supplements to the Registration Statement or the Prospectus; (ii) the issuance by the Commission or any other federal or state governmental authority of any stop order suspending the effectiveness of the Registration Statement or the initiation of any proceedings for that purpose; (iii) receipt by the Company or any of its Subsidiaries of any notification with respect to the suspension of the qualification or exemption from qualification of any of the Placement Shares for sale in any jurisdiction or the initiation or threatening of any proceeding for such purpose; or (iv) the occurrence of any event that makes any material statement made in the Registration Statement or the Prospectus or any material Incorporated Document untrue in any material respect or that requires the making of any changes in the Registration Statement, the Prospectus or Incorporated Documents so that, in the case of the Registration Statement, it will not contain any untrue statement of a material fact or omit to state any material fact required to be stated therein or necessary to make the statements therein not misleading and, in the case of the Prospectus, so that it will not contain any untrue statement of a material fact or omit to state any material

fact required to be stated therein or necessary to make the statements therein, in the light of the circumstances under which they were made, not misleading.

(d) No Misstatement or Material Omission. The Agent shall not have advised the Company that the Registration Statement or Prospectus, or any amendment or supplement thereto, contains an untrue statement of fact that in the Agent's opinion is material, or omits to state a fact that in the Agent's opinion is material and is required to be stated therein or is necessary to make the statements therein not misleading.

(e) Material Changes. Except as contemplated in the Prospectus, or disclosed in the Company's reports filed with the Commission, there shall not have been any material adverse change, on a consolidated basis, in the authorized capital stock of the Company or any Material Adverse Effect or any development that could reasonably be expected to result in a Material Adverse Effect, or any downgrading in or withdrawal of the rating assigned to any of the Company's securities (other than asset backed securities), if any, by any rating organization or a public announcement by any rating organization that it has under surveillance or review its rating of any of the Company's securities (other than asset backed securities), if any, the effect of which, in the judgment of the Agent (without relieving the Company of any obligation or liability it may otherwise have), is so material as to make it impracticable or inadvisable to proceed with the offering of the Placement Shares on the terms and in the manner contemplated in the Prospectus.

(f) Company Counsel Legal Opinions. The Agent shall have received the opinions and negative assurance letters, as applicable, of Company Counsel and Intellectual Property Counsel required to be delivered pursuant to Section 7(n) and Section 7(o), as applicable, on or before the date on which such delivery of such opinions and negative assurance letters are required pursuant to Section 7(n) and Section 7(o), as applicable.

(g) Agent's Counsel Legal Opinion. The Agent shall have received from Latham & Watkins LLP, counsel for the Agent, such opinion or opinions, on or before the date on which the delivery of the Company Counsel legal opinion is required pursuant to Section 7(n), with respect to such matters as the Agent may reasonably require, and the Company shall have furnished to such counsel such documents as they may request to enable them to pass upon such matters.

(h) Comfort Letter. The Agent shall have received the Comfort Letter required to be delivered pursuant to Section 7(p) on or before the date on which such delivery of such Comfort Letter is required pursuant to Section 7(p).

(i) Representation Certificate. The Agent shall have received the certificate required to be delivered pursuant to Section 7(m) on or before the date on which delivery of such certificate is required pursuant to Section 7(m).

(j) Secretary's Certificate. On or prior to the First Placement Notice Date, the Agent shall have received a certificate, signed on behalf of the Company by the Secretary of the Company and attested to by an executive officer of the Company, dated as of such date and in form and substance satisfactory to the Agent and its counsel, certifying as to (i) the amended and restated certificate of incorporation of the Company, (ii) the amended and restated bylaws of the Company, (iii) the resolutions of the board of directors of the Company or duly authorized committee thereof authorizing the execution, delivery and performance of this Agreement and the issuance and sale of the Placement Shares and (iv) the

incumbency of the officers of the Company duly authorized to execute this Agreement and the other documents contemplated by this Agreement (including each of the officers set forth on **Schedule 2**).

(k) No Suspension. The Common Stock shall be duly listed, and admitted and authorized for trading, subject to official notice of issuance, on Nasdaq. Trading in the Common Stock shall not have been suspended on, and the Common Stock shall not have been delisted from, Nasdaq.

(l) Other Materials. On each date on which the Company is required to deliver a certificate pursuant to Section 7(m), the Company shall have furnished to the Agent such appropriate further information, opinions, certificates, letters and other documents as the Agent may have reasonably requested. All such information, opinions, certificates, letters and other documents shall have been in compliance with the provisions hereof. The Company shall have furnished the Agent with conformed copies of such opinions, certificates, letters and other documents as the Agent may have reasonably requested.

(m) Securities Act Filings Made. All filings with the Commission required by Rule 424(b) or Rule 433 under the Securities Act to have been filed prior to the issuance of any Placement Notice hereunder shall have been made within the applicable time period prescribed for such filing by Rule 424(b) (without reliance on Rule 424(b)(8) of the Securities Act) or Rule 433, as applicable.

(n) Approval for Listing. Either (i) the Placement Shares shall have been approved for listing on Nasdaq, subject only to notice of issuance, or (ii) the Company shall have filed an application for listing of the Placement Shares on Nasdaq at, or prior to, the First Placement Notice Date and Nasdaq shall have reviewed such application and not provided any objections thereto.

(o) FINRA. FINRA shall have raised no objection to the terms of the offering contemplated hereby and the amount of compensation allowable or payable to the Agent as described in the Prospectus.

(p) No Termination Event. There shall not have occurred any event that would permit the Agent to terminate this Agreement pursuant to Section 11(a).

9. Indemnification and Contribution.

(a) Company Indemnification. The Company agrees to indemnify and hold harmless the Agent, its affiliates and their respective partners, members, directors, officers, employees and agents, and each person, if any, who (i) controls the Agent within the meaning of Section 15 of the Securities Act or Section 20 of the Exchange Act or (ii) is controlled by or is under common control with the Agent, in each case from and against any and all losses, claims, liabilities, expenses and damages (including any and all investigative, legal and other expenses reasonably incurred in connection with, and any and all amounts paid in settlement (in accordance with this Section 9)), any action, suit, investigation or proceeding between any of the indemnified parties and any indemnifying parties or between any indemnified party and any third party (including any governmental or self-regulatory authority, or otherwise, or any claim asserted or threatened), as and when incurred, to which the Agent, or any such other person may become subject under the Securities Act, the Exchange Act or other federal or state statutory law or regulation, at common law or otherwise, insofar as such losses, claims, liabilities, expenses or damages arise out of or are based, directly or indirectly, on (x) any untrue statement or alleged untrue statement of a material fact contained in the Registration Statement or the Prospectus (or any amendment or supplement to the Registration Statement or the Prospectus) or in any free writing prospectus or in any application or other document executed by or on behalf of the Company or based on

written information furnished by or on behalf of the Company filed in any jurisdiction in order to qualify the Common Stock under the securities laws thereof or filed with the Commission, (y) the omission or alleged omission to state in any such document a material fact required to be stated therein or necessary to make the statements therein (solely with respect to the Prospectus, in light of the circumstances under which they were made) not misleading or (z) any breach by any of the indemnifying parties of any of their respective representations, warranties or agreements contained in this Agreement; *provided*, *however*, that this indemnity agreement shall not apply to the extent that such loss, claim, liability, expense or damage arises from the sale of the Placement Shares pursuant to this Agreement and is caused, directly or indirectly, by an untrue statement or omission, or alleged untrue statement or omission, made in reliance upon and in conformity with the Agent's Information. This indemnity agreement will be in addition to any liability that the Company might otherwise have.

(b) Agent Indemnification. The Agent agrees to indemnify and hold harmless the Company and its directors and each officer of the Company who signed the Registration Statement, and each person, if any, who (i) controls the Company within the meaning of Section 15 of the Securities Act or Section 20 of the Exchange Act or (ii) is controlled by or is under common control with the Company against any and all loss, liability, claim, damage and expense described in the indemnity contained in Section 9(a), as incurred, but only with respect to untrue statements or omissions, or alleged untrue statements or omissions, made in the Registration Statement (or any amendments thereto) or the Prospectus (or any amendment or supplement thereto) in reliance upon and in conformity with the Agent's Information.

(c) Procedure. Any party that proposes to assert the right to be indemnified under this Section 9 will, promptly after receipt of notice of commencement of any action against such party in respect of which a claim is to be made against an indemnifying party or parties under this Section 9, notify each such indemnifying party of the commencement of such action, enclosing a copy of all papers served, but the omission so to notify such indemnifying party will not relieve the indemnifying party from (i) any liability that it might have to any indemnified party otherwise than under this Section 9 and (ii) any liability that it may have to any indemnified party under the foregoing provision of this Section 9 unless, and only to the extent that, such omission results in the forfeiture of substantive rights or defenses by the indemnifying party. If any such action is brought against any indemnified party and it notifies the indemnifying party of its commencement, the indemnifying party will be entitled to participate in and, to the extent that it elects by delivering written notice to the indemnified party promptly after receiving notice of the commencement of the action from the indemnified party, jointly with any other indemnifying party similarly notified, to assume the defense of the action, with counsel reasonably satisfactory to the indemnified party, and after notice from the indemnifying party to the indemnified party of its election to assume the defense, the indemnifying party will not be liable to the indemnified party for any other legal expenses except as provided below and except for the reasonable costs of investigation subsequently incurred by the indemnified party in connection with the defense. The indemnified party will have the right to employ its own counsel in any such action, but the fees, expenses and other charges of such counsel will be at the expense of such indemnified party unless (1) the employment of counsel by the indemnified party has been authorized in writing by the indemnifying party, (2) the indemnified party has reasonably concluded (based on advice of counsel) that there may be legal defenses available to it or other indemnified parties that are different from or in addition to those available to the indemnifying party, (3) a conflict or potential conflict exists (based on advice of counsel to the indemnified party) between the indemnified party and the indemnifying party (in which case the indemnifying party will not have the right to direct the defense of such action on behalf of the indemnified party) or (4) the indemnifying party has not in fact employed counsel reasonably satisfactory to the indemnified party to assume the defense of such action within a reasonable time after receiving notice of the commencement of the action, in each of which cases the reasonable fees, disbursements and

other charges of counsel will be at the expense of the indemnifying party or parties. It is understood that the indemnifying party or parties shall not, in connection with any proceeding or related proceedings in the same jurisdiction, be liable for the reasonable fees, disbursements and other charges of more than one separate firm (plus local counsel) admitted to practice in such jurisdiction at any one time for all such indemnified party or parties. All such fees, disbursements and other charges will be reimbursed by the indemnifying party promptly after the indemnifying party receives a written invoice relating to such fees, disbursements and other charges in reasonable detail. An indemnifying party will not, in any event, be liable for any settlement of any action or claim effected without its written consent. No indemnifying party shall, without the prior written consent of each indemnified party, settle or compromise or consent to the entry of any judgment in any pending or threatened claim, action or proceeding relating to the matters contemplated by this Section 9 (whether or not any indemnified party is a party thereto), unless such settlement, compromise or consent (1) includes an unconditional release of each indemnified party, in form and substance reasonably satisfactory to such indemnified party, from all liability arising out of such claim, action or proceeding and (2) does not include a statement as to or an admission of fault, culpability or a failure to act by or on behalf of any indemnified party.

(d) Settlement Without Consent if Failure to Reimburse. If an indemnified party shall have requested an indemnifying party to reimburse the indemnified party for reasonable fees and expenses of counsel for which it is entitled to be reimbursed under this Section 9, such indemnifying party agrees that it shall be liable for any settlement of the nature contemplated by Section 9(a) effected without its written consent if (i) such settlement is entered into more than 45 days after receipt by such indemnifying party of the aforesaid request, (ii) such indemnifying party shall have received notice of the terms of such settlement at least 30 days prior to such settlement being entered into and (iii) such indemnifying party shall not have reimbursed such indemnified party in accordance with such request prior to the date of such settlement.

(e) Contribution. In order to provide for just and equitable contribution in circumstances in which the indemnification provided for in the foregoing paragraphs of this Section 9 is applicable in accordance with its terms but for any reason is held to be unavailable or insufficient from the Company or the Agent, the Company and the Agent will contribute to the total losses, claims, liabilities, expenses and damages (including any investigative, legal and other expenses reasonably incurred in connection with, and any amount paid in settlement of, any action, suit, investigation or proceeding or any claim asserted, but after deducting any contribution received by the Company from persons other than the Agent, such as persons who control the Company within the meaning of the Securities Act, officers of the Company who signed the Registration Statement and directors of the Company, who also may be liable for contribution) to which the Company and the Agent may be subject in such proportion as shall be appropriate to reflect the relative benefits received by the Company on the one hand and the Agent on the other hand. The relative benefits received by the Company on the one hand and the Agent on the other hand shall be deemed to be in the same proportion as the total Net Proceeds from the sale of the Placement Shares (before deducting expenses) received by the Company bear to the total compensation received by the Agent from the sale of Placement Shares on behalf of the Company. If, but only if, the allocation provided by the foregoing sentence is not permitted by applicable law, the allocation of contribution shall be made in such proportion as is appropriate to reflect not only the relative benefits referred to in the foregoing sentence but also the relative fault of the Company, on the one hand, and the Agent, on the other hand, with respect to the statements or omission that resulted in such loss, claim, liability, expense or damage, or action, suit, investigation or proceeding in respect thereof, as well as any other relevant equitable considerations with respect to such offering. Such relative fault shall be determined by reference to, among other things, whether the untrue or alleged untrue statement of a material fact or omission or alleged omission to state a material fact relates to information supplied by the Company or the Agent, the intent of the parties and

their relative knowledge, access to information and opportunity to correct or prevent such statement or omission. The Company and the Agent agree that it would not be just and equitable if contributions pursuant to this Section 9(e) were to be determined by *pro rata* allocation or by any other method of allocation that does not take into account the equitable considerations referred to herein. The amount paid or payable by an indemnified party as a result of the loss, claim, liability, expense or damage, or action, suit, investigation or proceeding in respect thereof, referred to above in this Section 9(e) shall be deemed to include, for the purpose of this Section 9(e), any legal or other expenses reasonably incurred by such indemnified party in connection with investigating or defending any such action, suit, investigation, proceeding or claim to the extent consistent with this Section 9. Notwithstanding the foregoing provisions of this Section 9(e), the Agent shall not be required to contribute any amount in excess of the commissions received by it under this Agreement and no person found guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) will be entitled to contribution from any person who was not guilty of such fraudulent misrepresentation. For purposes of this Section 9(e), any person who controls a party to this Agreement within the meaning of Section 15 of the Securities Act or Section 20 of the Exchange Act, any affiliates of the Agent, any partners, members, directors, officers, employees and agents of the Agent and each person that is controlled by or under common control with the Agent will have the same rights to contribution as that party, and each officer of the Company who signed the Registration Statement will have the same rights to contribution as the Company, subject in each case to the provisions hereof. Any party entitled to contribution, promptly after receipt of notice of commencement of any action against such party in respect of which a claim for contribution may be made under this Section 9(e), will notify any such party or parties from whom contribution may be sought, but the omission to so notify will not relieve that party or parties from whom contribution may be sought from any other obligation it or they may have under this Section 9(e) except to the extent that the failure to so notify such other party materially prejudiced the substantive rights or defenses of the party from whom contribution is sought. Except for a settlement entered into pursuant to the last sentence of Section 9(c) hereof or pursuant to Section 9(d) hereof, no party will be liable for contribution with respect to any action or claim settled without its written consent if such consent is required pursuant to Section 9(c) hereof.

10. Representations and Agreements to Survive Delivery. The indemnity and contribution agreements contained in Section 9 of this Agreement and all representations and warranties of the Company herein or in certificates delivered pursuant hereto shall survive, as of their respective dates, regardless of (i) any investigation made by or on behalf of the Agent, any controlling persons, or the Company (or any of their respective officers, directors, employees or controlling persons), (ii) delivery and acceptance of the Placement Shares and payment therefor or (iii) any termination of this Agreement.

11. Termination.

(a) The Agent shall have the right, by giving notice as hereinafter specified, at any time to terminate this Agreement if (i) any Material Adverse Effect, or any development that could reasonably be expected to result in a Material Adverse Effect, has occurred that, in the judgment of the Agent, may materially impair the ability of the Agent to sell the Placement Shares hereunder, (ii) the Company shall have failed, refused or been unable to perform any agreement on its part to be performed hereunder; *provided*, *however*, in the case of any failure of the Company to deliver (or cause another person to deliver) any certification, opinion or letter required under Section 7(m), Section 7(n), Section 7(o) or Section 7(p), the Agent's right to terminate shall not arise unless such failure to deliver (or cause to be delivered) continues for more than 15 calendar days from the date such delivery was required, (iii) any other condition of the Agent's obligations hereunder is not fulfilled, (iv) any suspension or limitation of trading in the Placement Shares or in securities generally on Nasdaq shall have occurred, (v) a general

banking moratorium shall have been declared by any of United States federal or New York authorities, or (vi) there shall have occurred any outbreak or escalation of national or international hostilities or any crisis or calamity, or any change in the United States or international financial markets, or any substantial change or development involving a prospective substantial change in United States or international political, financial or economic conditions that, in the judgment of the Agent, may materially impair the ability of the Agent to sell the Placement Shares hereunder or to enforce contracts for the sale of securities. Any such termination shall be without liability of any party to any other party except that the provisions of Section 7(g), Section 9, Section 10, Section 16 and Section 17 hereof shall remain in full force and effect notwithstanding such termination. If the Agent elects to terminate this Agreement as provided in this Section 11(a), the Agent shall provide the required notice as specified in Section 12.

(b) The Company shall have the right, by giving 10 days' prior notice as hereinafter specified, to terminate this Agreement in its sole discretion at any time after the date of this Agreement. Any such termination shall be without liability of any party to any other party except that the provisions of Section 7(g), Section 9, Section 10, Section 11(f), Section 16 and Section 17 hereof shall remain in full force and effect notwithstanding such termination.

(c) The Agent shall have the right, by giving 10 days' prior notice as hereinafter specified, to terminate this Agreement in its sole discretion at any time after the date of this Agreement. Any such termination shall be without liability of any party to any other party except that the provisions of Section 7(g), Section 9, Section 10, Section 11(f), Section 16 and Section 17 hereof shall remain in full force and effect notwithstanding such termination.

(d) Unless earlier terminated pursuant to this Section 11, this Agreement shall automatically terminate upon the issuance and sale of all of the Placement Shares through the Agent on the terms and subject to the conditions set forth herein; *provided* that the provisions of Section 7(g), Section 9, Section 10, Section 11(f), Section 16 and Section 17 hereof shall remain in full force and effect notwithstanding such termination.

(e) This Agreement shall remain in full force and effect unless terminated pursuant to Sections 11(a), (b), (c), or (d) above or otherwise by mutual agreement of the parties; *provided, however*, that any such termination by mutual agreement shall in all cases be deemed to provide that Section 7(g), Section 9, Section 10, Section 11(f), Section 16 and Section 17 shall remain in full force and effect.

(f) Any termination of this Agreement shall be effective on the date specified in such notice of termination; *provided, however*, that such termination shall not be effective until the close of business on the date of receipt of such notice by the Agent or the Company, as the case may be. If such termination shall occur prior to the Settlement Date for any sale of Placement Shares, such Placement Shares shall settle in accordance with the provisions of this Agreement. Upon termination of this Agreement, the Company shall not be required to pay to the Agent any discount or commission with respect to any Placement Shares not otherwise sold by the Agent under this Agreement; *provided, however*, that the Company shall remain obligated to reimburse the Agent's accountable, actually incurred expenses pursuant to Section 7(g).

12. Notices. All notices or other communications required or permitted to be given by any party to any other party pursuant to the terms of this Agreement shall be in writing, unless otherwise specified in this Agreement, and if sent to the Agent, shall be delivered to:

Leerink Partners LLC
1301 Avenue of the Americas, 12th Floor
New York, New York 10019
Attention: Peter M. Fry
E-mail: peter.fry@leerink.com

with a copy (which shall not constitute notice) to:

Leerink Partners LLC
1301 Avenue of the Americas, 12th Floor
New York, New York 10019
Attention: Stuart R. Nayman, Esq.
E-mail: stuart.nayman@leerink.com

and

Latham & Watkins LLP
12670 High Bluff Drive
San Diego, California 92130
Attention: Matthew T. Bush, Esq.
E-mail: matt.bush@lw.com

and if to the Company, shall be delivered to:

Reneo Pharmaceuticals, Inc.
18575 Jamboree Rd., Suite 275-S
Irvine, California 92612
Attention: Gregory J. Flesher, Chief Executive Officer
E-mail: gflesher@reneopharma.com

with copies (which shall not constitute notice) to:

Cooley LLP
55 Hudson Yards
New York, New York 10001
Attention: Jason L. Kent, Esq.
E-mail: jkent@cooley.com

Each party to this Agreement may change such address for notices by sending to the parties to this Agreement written notice of a new address for such purpose. Each such notice or other communication shall be deemed given (i) when delivered personally on or before 4:30 P.M., New York City time, on a Business Day, or, if such day is not a Business Day, on the next succeeding Business Day, (ii) by Electronic Notice as set forth in the next paragraph, (iii) on the next Business Day after timely delivery to a nationally-recognized overnight courier or (iv) on the Business Day actually received if deposited in the U.S. mail (certified or registered mail, return receipt requested, postage prepaid). For purposes of this Agreement, "**Business Day**" shall mean any day on which the Nasdaq and commercial banks in the City of New York are open for business.

An electronic communication ("**Electronic Notice**") shall be deemed written notice for purposes of this Section 12 if sent to the electronic mail address specified by the receiving party in this Section 12. Electronic Notice shall be deemed received at the time the party sending Electronic Notice receives actual acknowledgment of receipt from the person whom the notice is sent, other than via auto-reply. Any party receiving Electronic Notice may request and shall be entitled to receive the notice on paper, in a nonelectronic form ("**Nonelectronic Notice**"), which shall be sent to the requesting party within 10 days of receipt of the written request for Nonelectronic Notice.

13. Successors and Assigns. This Agreement shall inure to the benefit of and be binding upon the Company and the Agent and their respective successors and the affiliates, controlling persons, officers, directors and other persons referred to in Section 9 hereof. References to any of the parties contained in this Agreement shall be deemed to include the successors and permitted assigns of each such party. Nothing in this Agreement, express or implied, is intended to confer upon any party other than the parties hereto, the persons referred to in the preceding sentence and their respective successors and permitted assigns any rights, remedies, obligations or liabilities under or by reason of this Agreement, except as expressly provided in this Agreement. Neither party may assign its rights or obligations under this Agreement without the prior written consent of the other party; *provided, however*, that the Agent may assign its rights and obligations hereunder to an affiliate of the Agent without obtaining the Company's consent, so long as such affiliate is a registered broker-dealer.

14. Adjustments for Share Splits. The parties acknowledge and agree that all share-related numbers contained in this Agreement shall be adjusted to take into account any share split, share dividend or similar event effected with respect to the Common Stock.

15. Entire Agreement; Amendment; Severability; Waiver. This Agreement (including all schedules (as amended pursuant to this Agreement) and exhibits attached hereto and Placement Notices issued pursuant hereto) constitutes the entire agreement and supersedes all other prior and contemporaneous agreements and undertakings, both written and oral, among the parties hereto with regard to the subject matter hereof. Neither this Agreement nor any term hereof may be amended except pursuant to a written instrument executed by the Company and the Agent; *provided*, *however*, that **Schedule 2** of this Agreement may be amended by either party from time to time by sending a notice containing a revised **Schedule 2** to the other party in the manner provided in Section 12 and, upon such amendment, all references herein to **Schedule 2** shall automatically be deemed to refer to such amended **Schedule 2**. In the event that any one or more of the provisions contained herein, or the application thereof in any circumstance, is held invalid, illegal or unenforceable as written by a court of competent jurisdiction, then such provision shall be given full force and effect to the fullest possible extent that it is valid, legal and enforceable, and the remainder of the terms and provisions herein shall be construed as if such invalid, illegal or unenforceable term or provision was not contained herein, but only to the extent that giving effect to such provision and the remainder of the terms and provisions hereof shall be in accordance with the intent of the parties as reflected in this Agreement. No implied waiver by a party shall arise in the absence of a waiver in writing signed by such party. No failure or delay in exercising any right, power, or privilege hereunder shall operate as a waiver thereof, nor shall any single or partial exercise thereof preclude any other or further exercise thereof or the exercise of any right, power, or privilege hereunder.

16. **GOVERNING LAW AND TIME; WAIVER OF JURY TRIAL. THIS AGREEMENT SHALL BE GOVERNED BY AND CONSTRUED IN ACCORDANCE WITH THE LAWS OF THE STATE OF NEW YORK WITHOUT REGARD TO THE PRINCIPLES OF CONFLICTS OF LAWS. SPECIFIED TIMES OF DAY REFER TO NEW YORK CITY TIME.**

EACH PARTY HEREBY IRREVOCABLY WAIVES, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, ANY AND ALL RIGHT TO TRIAL BY JURY IN ANY LEGAL PROCEEDING ARISING OUT OF OR RELATING TO THIS AGREEMENT OR THE TRANSACTIONS CONTEMPLATED HEREBY.

17. Consent to Jurisdiction. Each party hereby irrevocably submits to the exclusive jurisdiction of the state and federal courts sitting in the City of New York, Borough of Manhattan, for the adjudication of any dispute hereunder or in connection with any of the transactions contemplated hereby, and hereby irrevocably waives, and agrees not to assert in any suit, action or proceeding, any claim that it is not personally subject to the jurisdiction of any such court, that such suit, action or proceeding is brought in an inconvenient forum, or that the venue of such suit, action or proceeding is improper. Each party hereby irrevocably waives personal service of process and consents to process being served in any such suit, action or proceeding by mailing a copy (certified or registered mail, return receipt requested) to such party at the address in effect for notices under Section 12 of this Agreement and agrees that such service shall constitute good and sufficient notice of process and notice thereof. Nothing contained herein shall be deemed to limit in any way any right to serve process in any manner permitted by law.

18. Construction.

(a) The section and exhibit headings herein are for convenience only and shall not affect the construction hereof.

(b) Words defined in the singular shall have a comparable meaning when used in the plural, and vice versa.

(c) The words “hereof,” “hereto,” “herein” and “hereunder” and words of similar import, when used in this Agreement, shall refer to this Agreement as a whole and not to any particular provision of this Agreement.

(d) Wherever the word “include,” “includes” or “including” is used in this Agreement, it shall be deemed to be followed by the words “without limitation.”

(e) References herein to any gender shall include each other gender.

(f) References herein to any law, statute, ordinance, code, regulation, rule or other requirement of any governmental authority shall be deemed to refer to such law, statute, ordinance, code, regulation, rule or other requirement of any governmental authority as amended, reenacted, supplemented or superseded in whole or in part and in effect from time to time and also to all rules and regulations promulgated thereunder.

19. Permitted Free Writing Prospectuses. Each of the Company and the Agent represents, warrants and agrees that, unless it obtains the prior written consent of the other party, which consent shall not be unreasonably withheld, conditioned or delayed, it has not made and will not make any offer relating to the Placement Shares that would constitute an issuer free writing prospectus, or that would otherwise constitute a free writing prospectus (as defined in Rule 405), required to be filed with the Commission. Any such free writing prospectus consented to by the Agent or by the Company, as the case may be, is hereinafter referred to as a “**Permitted Free Writing Prospectus**.” The Company represents and warrants that it has treated and agrees that it will treat each Permitted Free Writing Prospectus as an issuer free writing prospectus, and that it has complied and will comply with the requirements of Rule

433 applicable to any Permitted Free Writing Prospectus, including timely filing with the Commission where required, legending and record keeping.

20. Absence of Fiduciary Relationship. The Company acknowledges and agrees that:

(a) the Agent has been retained to act as sales agent in connection with the sale of the Placement Shares, the Agent has acted at arms' length and no fiduciary or advisory relationship between the Company or any of its respective affiliates, stockholders (or other equity holders), creditors or employees or any other party, on the one hand, and the Agent, on the other hand, has been or will be created in respect of any of the transactions contemplated by this Agreement, irrespective of whether the Agent has advised or is advising the Company on other matters and the Agent has no duties or obligations to the Company with respect to the transactions contemplated by this Agreement except the obligations expressly set forth herein;

(b) the Company is capable of evaluating, and understanding and understands and accepts, the terms, risks and conditions of the transactions contemplated by this Agreement;

(c) neither the Agent nor its affiliates have provided any legal, accounting, regulatory or tax advice with respect to the transactions contemplated by this Agreement and it has consulted its own legal, accounting, regulatory and tax advisors to the extent it has deemed appropriate;

(d) the Company has been advised and is aware that the Agent and its affiliates are engaged in a broad range of transactions which may involve interests that differ from those of the Company and that the Agent and its affiliates have no obligation to disclose such interests and transactions to the Company by virtue of any fiduciary, advisory or agency relationship or otherwise; and

(e) the Company waives, to the fullest extent permitted by law, any claims it may have against the Agent or its affiliates for breach of fiduciary duty or alleged breach of fiduciary duty in connection with the transactions contemplated by this Agreement and agrees that the Agent and its affiliates shall have no liability (whether direct or indirect) to the Company in respect of such a fiduciary claim or to any person asserting a fiduciary duty claim on behalf of or in right of the Company, including stockholders (or other equity holders), creditors or employees of the Company.

21. Recognition of the U.S. Special Resolution Regimes. In the event that the Agent is a Covered Entity and becomes subject to a proceeding under a U.S. Special Resolution Regime, the transfer from the Agent of this Agreement, and any interest and obligation in or under this Agreement, will be effective to the same extent as the transfer would be effective under the U.S. Special Resolution Regime if this Agreement, and any such interest and obligation, were governed by the laws of the United States or a state of the United States.

In the event that the Agent is a Covered Entity and the Agent or a BHC Act Affiliate of the Agent becomes subject to a proceeding under a U.S. Special Resolution Regime, Default Rights under this Agreement that may be exercised against the Agent are permitted to be exercised to no greater extent than such Default Rights could be exercised under the U.S. Special Resolution Regime if this Agreement were governed by the laws of the United States or a state of the United States.

For purposes of this Agreement, (A) "BHC Act Affiliate" has the meaning assigned to the term "affiliate" in, and shall be interpreted in accordance with, 12 U.S.C. § 1841(k); (B) "Covered Entity" means any of the following: (i) a "covered entity" as that term is defined in, and interpreted in accordance

with, 12 C.F.R. § 252.82(b); (ii) a "covered bank" as that term is defined in, and interpreted in accordance with, 12 C.F.R. § 47.3(b); or (iii) a "covered FSI" as that term is defined in, and interpreted in accordance with, 12 C.F.R. § 382.2(b); (C) "Default Right" has the meaning assigned to that term in, and shall be interpreted in accordance with, 12 C.F.R. §§ 252.81, 47.2 or 382.1, as applicable; and (D) "U.S. Special Resolution Regime" means each of (i) the Federal Deposit Insurance Act and the regulations promulgated thereunder and (ii) Title II of the Dodd-Frank Wall Street Reform and Consumer Protection Act and the regulations promulgated thereunder.

22. Counterparts. This Agreement may be executed in two or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. Delivery of an executed Agreement by one party to the other may be made by facsimile or electronic transmission. Counterparts may be delivered via facsimile, electronic mail (including any electronic signature covered by the U.S. federal ESIGN Act of 2000, Uniform Electronic Transactions Act, the Electronic Signatures and Records Act or other applicable law, e.g., www.docusign.com) or other transmission method and any counterpart so delivered shall be deemed to have been duly and validly delivered and be valid and effective for all purposes.

23. Use of Information. The Agent may not provide any information gained in connection with this Agreement and the transactions contemplated by this Agreement, including due diligence, to any third party other than its legal counsel advising it on this Agreement and the transactions contemplated by this Agreement unless expressly approved by the Company in writing.

24. Termination of Existing Sales Agreement. Effective as of immediately prior to the execution of this Agreement, the Company and Leerink Partners LLC hereby terminate the Sales Agreement dated as of May 2, 2022 (the "**Existing Sales Agreement**"), by and between the Company and Leerink Partners LLC, pursuant to Section 11(e) of the Existing Sales Agreement.

25. Agent's Information. As used in this Agreement, "**Agent's Information**" means solely the following information in the Registration Statement and the Prospectus: [the first and third sentences of the eighth paragraph and the tenth paragraph] under the heading "Plan of Distribution" in the Sales Prospectus specifically relating to the Placement Shares.

All references in this Agreement to the Registration Statement, the Prospectus or any amendment or supplement to any of the foregoing shall be deemed to include the copy filed with the Commission pursuant to EDGAR. All references in this Agreement to financial statements and schedules and other information that is "contained," "included" or "stated" in the Registration Statement or the Prospectus (and all other references of like import) shall be deemed to mean and include all such financial statements and schedules and other information that is incorporated by reference in the Registration Statement or the Prospectus, as the case may be.

All references in this Agreement to "supplements" to the Prospectus shall include any supplements, "wrappers" or similar materials prepared in connection with any offering, sale or private placement of any Placement Shares by the Agent outside of the United States.

[Remainder of Page Intentionally Blank]

If the foregoing correctly sets forth the understanding between the Company and the Agent, please so indicate in the space provided below for that purpose, whereupon this letter shall constitute a binding agreement between the Company and the Agent.

Very truly yours,

RENEO PHARMACEUTICALS, INC.

By: /s/Gregory J. Flesher
Name: Gregory J. Flesher
Title: President and Chief Executive Officer

ACCEPTED as of the date first-above written:

LEERINK PARTNERS LLC

By: /s/Peter Fry
Name: Peter Fry
Title: Senior Managing Director

[*Signature Page to Sales Agreement*]

SCHEDULE 1

FORM OF PLACEMENT NOTICE

From: [NAME]
[TITLE]
Reneo Pharmaceuticals, Inc.

Cc: [NAME]

To: Leerink Partners LLC

Subject: Leerink Partners—At the Market Offering—Placement Notice

Ladies and Gentlemen:

Pursuant to the terms and subject to the conditions contained in the Sales Agreement, dated November [13], 2023 (the "**Agreement**"), by and between Reneo Pharmaceuticals, Inc., a Delaware corporation (the "**Company**"), and Leerink Partners LLC ("**Leerink Partners**"), I hereby request on behalf of the Company that Leerink Partners sell up to [ ] shares of common stock, $0.0001 par value per share, of the Company (the "**Shares**"), at a minimum market price of $[ ] per share[; *provided* that no more than [ ] Shares shall be sold in any one Trading Day (as such term is defined in Section 3 of the Agreement)]. Sales should begin [on the date of this Placement Notice] and end on [DATE] [until all Shares that are the subject of this Placement Notice are sold].

SCHEDULE 2

The Company

Gregory Flesher

Leerink Partners

Murphy Gallagher (murphy.gallagher@leerink.com)
Liz Meeks (liz.meeks@leerink.com)

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SCHEDULE 3

Compensation

The Company shall pay Leerink Partners LLC compensation in cash equal to 3.0% of the gross proceeds from the sales of Placement Shares pursuant to the terms of the Sales Agreement of which this **Schedule 3** forms a part.

Exhibit 7(m)

OFFICERS' CERTIFICATE

Each of Gregory J. Flesher, the duly qualified and elected President and Chief Executive Officer of Reneo Pharmaceuticals, Inc., a Delaware corporation (the "**Company**"), and Jennifer P. Lam, the duly qualified and elected Principal Financial and Accounting Officer of the Company, does hereby certify in his and her respective capacity and on behalf of the Company, pursuant to Section 7(m) of the Sales Agreement, dated November [13], 2023 (the "**Sales Agreement**"), by and between the Company and Leerink Partners LLC, that, after due inquiry, to the best of the knowledge of the undersigned:

(i) The representations and warranties of the Company in Section 6 of the Sales Agreement (A) to the extent such representations and warranties are subject to qualifications and exceptions contained therein relating to materiality or Material Adverse Effect, are true and correct on and as of the date hereof with the same force and effect as if expressly made on and as of the date hereof, except for those representations and warranties that speak solely as of a specific date and which were true and correct as of such date, and (B) to the extent such representations and warranties are not subject to any qualifications or exceptions relating to materiality or Material Adverse Effect, are true and correct in all material respects as of the date hereof as if made on and as of the date hereof with the same force and effect as if expressly made on and as of the date hereof, except for those representations and warranties that speak solely as of a specific date and which were true and correct as of such date.

(ii) The Company has complied with all agreements and satisfied all conditions on its part to be performed or satisfied pursuant to the Sales Agreement at or prior to the date hereof.

Capitalized terms used but not defined herein shall have the meanings ascribed to them in the Sales Agreement.

[Remainder of Page Intentionally Blank]

IN WITNESS WHEREOF, each of the undersigned, in such individual's respective capacity as President and Chief Executive Officer or Principal Financial and Accounting Officer of the Company, has executed this Officers' Certificate on behalf of the Company.

By: /s/ Gregory J. Flesher
Name: Gregory J. Flesher
Title: President and Chief Executive Officer
Date:

By: /s/ Jennifer P. Lam
Name: Jennifer P. Lam
Title: Principal Financial and Accounting Officer
Date:

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED

I, Gregory J. Flesher, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Reneo Pharmaceuticals, Inc. (the "registrant");

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 13, 2023

By:	/s/ Gregory J. Flesher
Name:	Gregory J. Flesher
Title:	President and Chief Executive Officer
	(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER PURSUANT TO
RULES 13a-14(a) AND 15d-14(a) UNDER THE SECURITIES EXCHANGE ACT OF 1934, AS AMENDED

I, Jennifer P. Lam, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Reneo Pharmaceuticals, Inc. (the "registrant");
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
    a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
    b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
    c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
    d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
    a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
    b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 13, 2023

By:	/s/ Jennifer P. Lam
Name:	Jennifer P. Lam
Title:	Senior Vice President, Finance and Administration (Principal Financial and Accounting Officer)

Exhibit 32.1

Statement Pursuant to 18 U.S.C. Section 1350,
As required by Section 906 of the Sarbanes-Oxley Act of 2002

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), Gregory J. Flesher, President and Chief Executive Officer of Reneo Pharmaceuticals, Inc. (the "Company"), and Jennifer P. Lam, Senior Vice President, Finance and Administration of the Company, each hereby certifies that, to the best of his or her knowledge:

(1) The Company's Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2023, to which this Certification is attached as Exhibit 32.1 (the "Quarterly Report"), fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and

(2) The information contained in the Quarterly Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 13, 2023	By:	/s/ Gregory J. Flesher
	Name:	Gregory J. Flesher
	Title:	President and Chief Executive Officer (Principal Executive Officer)

Date: November 13, 2023	By:	/s/ Jennifer P. Lam
	Name:	Jennifer P. Lam
	Title:	Senior Vice President, Finance and Administration (Principal Financial and Accounting Officer)

This certification accompanies the Quarterly Report to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Exchange Act (whether made before or after the date of the Quarterly Report), irrespective of any general incorporation language contained in such filing.