

Risk Factors Comparison 2025-02-28 to 2024-02-27 Form: 10-K

Legend: **New Text** ~~Removed Text~~ Unchanged Text **Moved Text Section**

Risks Related to Our Financial Position and Need for Additional Capital We have incurred significant losses since inception, expect to incur significant **expenses and increasing losses for at least this year**, and may never achieve or maintain profitability. We have incurred significant annual net operating losses in every year since our inception. We expect to continue to incur **significant expenses** net operating losses for **the foreseeable future** at least this year. Our net losses were \$ **197.9 million**, \$ 528.6 million ~~and~~ \$ 652.2 million and \$ 746.4 million for the years ended December 31, **2024**, 2023 ~~and~~ 2022 and 2021 respectively. As of December 31, **2023-2024**, we had an accumulated deficit of \$ **2.3, 8.0** billion. While we ~~are now have~~ **begun to generate** ~~generating substantial~~ revenue from sales of SYFOVRE and EMPAVELI, we have primarily financed our operations to date through the sale of our common stock in our public offerings, the sale of convertible notes, private placements of our preferred stock prior to our initial public offering, the development funding agreement with SFJ Pharmaceuticals Group, or SFJ, **the financing agreement with Sixth Street Lending Partners, or Sixth Street**, and the collaboration agreement with Swedish Orphan Biovitrum AB (Publ), or Sobi. We have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and our clinical trials across several disease indications, and the commercialization of SYFOVRE and EMPAVELI. Our operating results may fluctuate significantly from quarter to quarter and year to year. Net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital. We expect to continue to incur significant expenses **for the foreseeable future** and **may incur** operating losses for at least this year. We anticipate that ~~we~~ **our expenses** will continue to incur **increasing expenses** if and as we: • continue to commercialize EMPAVELI **for the treatment of PNH in the United States and commercialize SYFOVRE for the treatment of GA in the United States, Australia, and select other jurisdictions;** • **prioritize the ongoing development of systemic pegcetacoplan and focus our research initiatives on high potential opportunities**; • establish and ~~maintain~~ **continue to build** sales, marketing, distribution and other commercial infrastructure for EMPAVELI and SYFOVRE and any other products for which we may obtain marketing approval; • prepare to submit additional applications for regulatory approval **for SYFOVRE** outside the United States ~~and continue to build a commercial infrastructure for SYFOVRE for GA in the United States and worldwide;~~ • continue to develop and conduct **research and preclinical and** clinical trials of systemic pegcetacoplan for the treatment of C3G and IC-MPGN and other of our **current** product candidates; • ~~initiate and continue research and preclinical and clinical development efforts for any future product candidates;~~ • ~~seek to identify and develop additional product candidates for complement-dependent diseases;~~ • seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials, if any; • ~~require the~~ **continue to** manufacture of commercial quantities of our approved products and ~~larger quantities of~~ **to manufacture our** product candidates for clinical development and, potentially, commercialization; • maintain, expand and protect our intellectual property portfolio; • hire and retain ~~additional personnel, such as clinical, quality control and scientific personnel;~~ • add operational, financial and management information systems ~~and personnel, including personnel to support our product development and help us comply with our obligations as a public company;~~ and • add equipment and physical infrastructure to support our research and development programs. Our ability to become and remain profitable depends on our ability to generate significant product revenue. Our ability to generate significant revenue will require us to successfully commercialize EMPAVELI and SYFOVRE in the approved ~~jurisdictions~~ **indications**. While we ~~have begun to generate~~ **generated** product revenue from sales of EMPAVELI ~~in since May 2021 and~~ **2021 and** SYFOVRE in March 2023, **we have not generated sufficient revenue to achieve profitability and** there can be no assurance that we will generate sufficient revenue to achieve profitability **in the next several years, and or at all. Even if we may not** achieve profitability ~~for several years, if at all~~ **there can be no assurance that we will be able to maintain profitability**. The successful commercialization of ~~both our approved~~ products is subject to many risks. There are numerous examples of unsuccessful product launches and failures to meet expectations of market potential, including by pharmaceutical companies with more experience and resources than us. We do not anticipate our revenue from sales of EMPAVELI ~~for the treatment of PNH alone~~ will be sufficient for us to become profitable for several years, if at all. Our prospects depend ~~primarily~~ **substantially** upon the commercial success of SYFOVRE. Successful commercialization will require manufacturing, marketing and selling our approved products, satisfying any post-marketing requirements and obtaining reimbursement for our products from private insurance or government payors. Because of the uncertainties and risks associated with these activities, we are unable to accurately predict the timing and amount of revenues, and if or when we might achieve profitability. We and any collaborators may never succeed in these activities and, even if we do, or any collaborators do, we may never generate revenues that are large enough for us to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our pipeline of product candidates or continue our operations. A decline in the value of our company could cause our stockholders to lose all or part of their investment. We **are continuing** ~~have obtained marketing approval for EMPAVELI for the treatment of PNH in the United States and for SYFOVRE for the treatment of GA in the United States but have not yet~~ consistently demonstrated an ability to successfully conduct **devote significant resources to support our ongoing** commercial activities ~~We obtained our first~~ **related to product manufacturing,** marketing approval for the use, **sales and distribution** of **EMPAVELI for systemic pegcetacoplan to treat adult patients with PNH in the United States and began to sell SYFOVRE for GA, and if our cash, cash equivalents, and cash generated from sales of** EMPAVELI for the treatment of PNH in May 2021.

Our collaborator Sobi obtained marketing approval in the European Union, Saudi Arabia and Australia for the use of pegcetacoplan to treat patients with PNH. We obtained marketing approval for the use of intravitreal pegcetacoplan to treat patients with GA in the United States in February 2023 and began to commercialize SYFOVRE **are** in March 2023. Prior to obtaining these approvals, our operations were limited to financing and staffing our company, developing our technology, conducting preclinical research and clinical trials of our product candidates and preparing for a commercial launch. We have not **sufficient** yet consistently demonstrated an ability to **fund** conduct sales and marketing activities necessary for successful product commercialization. Accordingly, our stockholders should consider our prospects in light of the costs, uncertainties, delays and difficulties frequently encountered by biopharmaceutical companies such as ours - **our planned expenditures** . Any predictions made about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing pharmaceutical products. We may encounter unforeseen expenses, **we** difficulties, complications, delays and other known or unknown factors in achieving our business objectives. We will need to continue to transition from a company with a development focus to a company capable of supporting commercial activities. We may not be successful in such a transition. We expect our financial **finance** condition and operating results to continue to fluctuate significantly from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. Accordingly, our stockholders should not rely upon the results of any quarterly or **our cash** annual periods as indications of future operating performance. We may need **needs** substantial additional funding to allow us to support both our systemic and ophthalmological programs through **external sources of funds** clinical development and commercial launch, and if we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts. Developing and commercializing pharmaceutical products, including conducting preclinical studies and clinical trials and preparing for commercial launch, is a very time- consuming, expensive and uncertain process that takes years to complete. We have consumed substantial amounts of cash since our inception. For example, in the years ended December 31, **2024, 2023, and 2022 and 2021**, we used net cash of \$ **87.9 million, \$ 594.7 million, and \$ 513.7 million and \$ 563.1 million** respectively, in our operating activities substantially all of which related to research and, development **and commercialization** activities. As of December 31, **2023-2024**, our cash and cash equivalents were \$ **351-411 . 2-3** million. We expect our expenses to continue, particularly as we **continue to** commercialize EMPAVELI and SYFOVRE, **initiate new clinical trials and initiate new** **prioritize the ongoing development of pegcetacoplan and focus our** research and **preclinical development efforts** **initiatives on high potential opportunities** . In addition, as we **continue to** commercialize EMPAVELI and SYFOVRE, and if we obtain marketing approval of pegcetacoplan in other indications or jurisdictions or for our other product candidates, we expect we will incur significant **additional** commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing, manufacturing and distribution are not the responsibility of the **a** collaborator . **Furthermore, we continue to incur significant costs associated with operating as a public company** . We believe that our cash and cash equivalents as of December 31, **2023-2024** and the cash that we anticipate **generating from the unwind of the capped call transactions**, together with the cash that we anticipate will be generated from sales of EMPAVELI and SYFOVRE will be sufficient to fund our projected operating expenses and capital expenditure requirements for at least the next 12 months, as well as our anticipated longer- term cash requirements and obligations. Our expectations regarding our short- term and long- term funding requirements are based on assumptions that may prove to be wrong, and we may need additional capital resources to fund our operating plans and capital expenditure requirements. We are devoting substantial resources to the **commercialization of commercial infrastructure for SYFOVRE for GA** . We are also devoting substantial resources to **the preparation for commercialization of EMPAVELI for the treatment of C3G and IC-MPGN, our planned Phase 3 clinical trials of EMPAVELI for the treatment of FSGS and DGF, and** the development of our product candidates. Because of the numerous risks and uncertainties associated with the commercialization of EMPAVELI and SYFOVRE and development of other product candidates, and because the extent to which we may enter into collaborations with third parties for any of these activities is unknown, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with the research, development and commercialization. Our future funding requirements and long- term capital requirements will depend on many factors, including: • our ability to successfully commercialize and sell **EMPAVELI in the United States and SYFOVRE in the United States, Australia and select other jurisdictions;** • **the cost of and our ability to obtain regulatory approvals of SYFOVRE outside of** the United States; • the cost of and our ability **obtain regulatory approvals of SYFOVRE outside of the United States and to build a commercial infrastructure for SYFOVRE for GA in the United States and worldwide;** • the cost of and our ability to effectively establish and maintain, the commercial infrastructure and manufacturing capabilities required to support the commercialization of EMPAVELI **for PNH**, systemic pegcetacoplan and SYFOVRE and any other products for which we receive marketing approval including product sales, medical affairs, marketing, manufacturing and distribution; • the scope, progress, timing, costs and results of clinical trials of, and research and preclinical development efforts for systemic pegcetacoplan, SYFOVRE and our other product candidates; • our ability to maintain a productive collaborative relationship with Sobi with respect to systemic pegcetacoplan, including our ability to achieve milestone payments under our agreement with Sobi; • our ability to identify additional collaborators for any of our product candidates and the terms and timing of any collaboration agreement that we may establish for the development and any commercialization of such product candidates; • the number and characteristics of **future** product candidates that we pursue and their development requirements; • the outcome, timing and costs of clinical trials and of seeking regulatory approvals of pegcetacoplan in other jurisdictions and indications and other product candidates we may pursue; • the costs of commercialization activities for any of our product candidates that receive marketing approval to the extent such costs are not the responsibility of any collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities; • subject to receipt of marketing approval, revenue, if any, received from commercial sales of pegcetacoplan in other jurisdictions and indications and our other product candidates; • our headcount growth and associated

costs as we expand our research and development and establish a commercial infrastructure; • the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims; • the effect of competing technological and market developments; • the effect of public health crises, including pandemics and epidemics, on the healthcare system and the economy generally and on our clinical trials and other operations specifically; • our ability to obtain adequate reimbursement for EMPAVELI and SYFOVRE in the United States or any other product we commercialize; and • the costs of operating as a public company. If our cash, and cash equivalents, and the cash generated from sales of EMPAVELI and SYFOVRE are not sufficient to fund our planned expenditures, we will need to finance our cash needs through external sources of funds, which may include equity offerings, debt financings, collaborations, strategic alliances or licensing arrangements. We currently do not have any committed external sources of funds. If we are unable to generate sufficient funds from sales of EMPAVELI and SYFOVRE or raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates. We expect our to continue to incur significant expenses to increase in connection with our planned operations. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, the ownership interest of our then- existing stockholders may be diluted, and the terms of these securities could include liquidation or other preferences and anti- dilution protections that could adversely affect the rights of our common stockholders. In addition, additional debt financing, if available, would result in fixed payment obligations and may involve agreements that include grants of security interests on our assets and restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. Future debt securities or other financing arrangements could contain similar or more restrictive negative covenants. In addition, securing financing could require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day- to- day activities, which may adversely affect our management' s ability to oversee the development of our product candidates. In 2021 and 2022, we completed several privately negotiated exchanges with holders of our outstanding 3.5 % senior convertible notes due 2026, or the Convertible Notes, under which we issued approximately 12.9 million shares of common stock in exchange for approximately \$ 425.4 million in aggregate principal amount of Convertible Notes. The effective price per share of the common stock issued in the exchange transactions was lower than the trading price of our common stock on the Nasdaq Global Select Market at the time of settlement of the exchanges. We may in the future exchange additional principal amount of our Convertible Notes and the effective price per share of the common stock may be lower than the trading price at such time. If we raise additional funds through collaborations or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. The We are required to make substantial payments to SFJ pursuant to our development funding agreement as a result of receiving regulatory approval of EMPAVELI for the treatment of PNH. If we do not have sufficient funding or cash flow from our business to meet our payment obligations under the development funding agreement, SFJ could exercise its remedies as a holder of a first priority security interest in our assets and our business could be materially harmed. We are required to make substantial payments to SFJ pursuant to our development funding agreement as a result of obtaining regulatory approvals for EMPAVELI in the United States and the EU. We have paid SFJ a total of \$ 94.0 million through December 31, 2023, and we are obligated to pay an aggregate of \$ 366.0 million as of December 31, 2023 in eight semi- annual payments through December 31, 2027. Our ability to make these required payments depends on our future performance and the future performance of Sobi, which is subject to economic, financial, competitive and other factors beyond our control. Our business may not generate cash flow from operations in the future sufficient to meet our obligations under the development funding agreement. If we are unable to generate such cash flow or to obtain additional funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources on acceptable terms of or our at all, we could default on our payment obligations to SFJ. Our payment obligations to SFJ could have significant consequences for our security holders and our business, results of operations and financial condition by, among other things: • limiting our ability to obtain additional financing; • requiring the dedication of a substantial portion of our cash flow from operations to service our meet our obligations under the development funding agreement, which will reduce the amount of cash available for other purposes; and • limiting our flexibility to plan for, or react to, changes in our business; Our business may not generate sufficient funds, and we may otherwise be unable to maintain sufficient cash reserves, to pay amounts due to SFJ, and our cash needs may increase in the future. We have granted SFJ a first priority security interest in all of our assets other than our intellectual property and the license agreements to which we are a party. If we are unable to meet our payment obligations to SFJ, SFJ may exercise its remedies as a holder of a first priority security interest, which would result in a loss of our assets and our business would be materially harmed. Our indebtedness could limit the cash flow available for our operations, expose us to risks that could adversely affect our operations and limit our ability to plan for or respond to changes in our business, and If we are unable to comply with restrictions in our debt financial financing condition and results agreements, the repayment of operations and impair our ability to satisfy our obligations existing indebtedness could be accelerated. As of December 31, 2024, we had \$ 375 million of indebtedness under the Convertible Notes. We had our financing agreement with Sixth Street an and aggregate of approximately \$ 93.9 million principal amount of the Convertible Notes outstanding and held by third parties as of December 31, 2023-2024. We may also incur additional indebtedness to meet future financing needs. Our Under our financing agreement, or the Sixth Street Financing Agreement, by and among us,

certain of our subsidiaries, the lenders party thereto and Sixth Street, as the administrative agent for the lenders, we have incurred a substantial amount of debt, which could adversely affect our business. In May 2024, we drew down the senior secured term loan facility, or the Credit Facility, of \$ 375. 0 million. The Credit Facility also includes a potential additional \$ 100. 0 million draw at our option upon satisfaction of a \$ 50. 0 million minimum cash requirement and a requirement that our trailing three- month sales of SYFOVRE were at least \$ 180. 0 million prior to the \$ 100. 0 million draw. Among other permissions, we are permitted, on terms and conditions set forth on the Sixth Street Financing Agreement, to enter into a separate asset- based financing arrangement with a third party in an amount of up to \$ 100. 0 million, which amount is increased to \$ 200. 0 million upon certain sales or market capitalization thresholds, and to have outstanding convertible unsecured notes in an amount equal to the greater of \$ 400. 0 million and 10 % of our market capitalization, but not to exceed \$ 600. 0 million. The Sixth Street Financing Agreement requires us to make certain payments of interest over time and contains several other negative covenants that, subject to certain exceptions, restrict indebtedness, liens, investments (including acquisitions), fundamental changes, asset sales and licensing transactions, dividends, modifications to material agreements, payment of subordinated indebtedness, and other matters customarily restricted in such agreements. Among other requirements of the Sixth Street Financing Agreement, we and our subsidiaries party to the Sixth Street Financing Agreement must maintain liquidity of at least \$ 50. 0 million if our market capitalization is below \$ 3. 0 billion. We are also subject to restrictions on sales and licensing transactions with respect to our core intellectual property, defined to include SYFOVRE, EMPAVELI, and other pegcetacoplan product assets, subject to certain exceptions, including certain transactions related to areas outside the United States and Europe. These and other terms in the Sixth Street Financing Agreement could restrict have significant negative consequences for our security holders and ability to grow our business, results of operations and financial condition by, among other things: • increasing our vulnerability to adverse economic and industry conditions; • requiring the dedication of a substantial portion of our cash flow from operations to service our indebtedness, which will reduce the amount of cash available for other purposes; • diluting the interests of our existing stockholders as a result of issuing shares of our common stock in exchange for upon conversion of the Convertible Notes; and • placing us at a possible competitive disadvantage with competitors that are less leveraged than us we believe would be beneficial to or our have better access to capital. Our business may not generate sufficient funds, and we may otherwise be unable to maintain sufficient cash reserves, to pay amounts due under the Convertible Notes, and our cash needs may increase in the future. Servicing the Convertible Notes will require a significant amount of cash, and we may not have sufficient cash flow from our business to make payments on the Convertible Notes. Our ability to make scheduled payments of the principal of, to pay interest on or to refinance the Convertible Notes depends on our future performance, which is subject to economic, financial, competitive and other factors beyond our control. Our business may not generate cash flow from operations in the future sufficient to service the Convertible Notes. If we are unable to generate cash flow, we may be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining additional equity capital on terms that may be unfavorable to us or highly dilutive. Our ability to refinance our indebtedness will depend on the capital markets and our financial condition at the time we seek to refinance such indebtedness. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations. Furthermore, We may not have the funds necessary to settle conversions of the Convertible Notes in cash or to repurchase the Convertible Notes upon a fundamental change, and our future debt may contain limitations on our ability to pay cash upon conversion or repurchase of the Convertible Notes. Holders holders of the Convertible Notes have the right to require us to repurchase all or a portion of their Convertible Notes upon the occurrence of a fundamental change at a price equal to the principal amount of the Convertible Notes to be repurchased, plus accrued and unpaid interest. In addition, upon conversion of the Convertible Notes, unless we elect to deliver solely shares of our common stock to settle such conversion (other than paying cash in lieu of delivering any fractional share), we will be required to make cash payments in respect of the Convertible Notes being converted. However, we may not have enough available cash or be able to obtain financing at the time we are required to make repurchases of Convertible Notes surrendered therefor or Convertible Notes being converted. In addition, our ability to repurchase the Convertible Notes or to pay cash upon conversions of the Convertible Notes may be limited by law, by regulatory authority or by agreements governing our future indebtedness. Our failure to repurchase Convertible Notes at a time when the repurchase is required by the indenture or to pay any cash payable on future conversions of the Convertible Notes as required by the indenture would constitute a default under the indenture. A default under the indenture or the fundamental change itself could also lead to a default under agreements governing our existing or future indebtedness. If the repayment of the related indebtedness were to be accelerated after any applicable notice or grace periods, we may not have sufficient funds to repay the indebtedness and repurchase the Convertible Notes or make cash payments upon conversions thereof. Our indebtedness could A conditional conversion feature of the Convertible Notes, if triggered, may adversely affect our business in the following ways, among other things: make it more difficult for us to satisfy our contractual and commercial commitments; require us to use a substantial portion of our cash flow from operations to pay interest and principal when due, which would reduce funds available for working capital, capital expenditures and other general corporate purposes; limit our ability to obtain additional financial financing condition. In for working capital, capital expenditures, acquisitions and the other investments or general corporate purposes; heighten our vulnerability event a conditional conversion feature of the Convertible Notes is triggered, holders of Convertible Notes will be entitled to convert downturns in our business, our industry or in the Convertible Notes general economy; place us at any time during specified periods at a disadvantage compared to those of our competitors that may have proportionately less debt; limit management' s discretion in operating our business; and limit our flexibility in planning for, or reacting to, changes in our business, their the industry in which we operate or the general economy. Our business may not generate cash flows from option operations in the future that are sufficient to service our debt and support our growth strategies. If one or

more holders elect to convert their Convertible Notes, unless we elect **are unable to generate such** satisfy our conversion obligation by delivering solely shares of our common stock (other than paying cash flows in lieu of delivering any fractional share), we would **may** be required to **adopt one** settle a portion or all of our **or more alternatives** conversion obligation in cash, **such** which could adversely affect our liquidity. One of the conditional conversion features of the Convertible Notes has been triggered from time and time at the end of fiscal quarters, including as **obtaining additional equity capital on terms that may** of December 31, 2023, and a result the Convertible Notes are convertible at the option of the holders, in whole or in part, until March 31, 2024. Whether the Convertible Notes will be convertible in any future period **onerous or highly dilutive, selling assets, or restructuring debt. Our ability to refinance our indebtedness** will depend on the **capital markets and satisfaction of this condition or our financial** another conversion condition at such time. **We may** In addition, even if holders do not elect **be able to engage in any of** convert their Convertible Notes during a period when the **these** notes are convertible **activities or engage in these activities on desirable terms**, we **which** could be required under applicable accounting rules to reclassify all or a portion of the outstanding principal amount of the Convertible Notes as a current rather than long-term liability, which would result in a **default** material reduction of our net working capital. As of September 20, 2023, we may redeem for cash all or a portion of the Convertible Notes, at our option, if the last reported sale price of our common stock has been at least 130 % of the conversion price then in effect for at least 20 trading days (whether or not consecutive), including the trading day immediately preceding the date on **our debt obligations** which we provide a notice of redemption, during any 30 consecutive trading day period ending on, and including, the trading day immediately preceding the date on which we provide notice of redemption. The redemption price will be equal to 100 % of the principal amount of the Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date. If we call any Convertible Notes for redemption, it will constitute a “make-whole fundamental change” with respect to such Convertible Notes, in which case the conversion rate applicable to the conversion of such Notes, if converted in connection with the redemption, will be increased in certain circumstances. We expect to issue a notice of full redemption of the \$93.9 million of aggregate principal amount of the Convertible Notes on February 27, 2024. We expect that holders will elect to convert their Convertible Notes and that no Convertible Notes will be redeemed for cash. In the event that a holder delivers a conversion notice as provided in the indenture related to the Convertible Notes, we intend to settle conversions by delivering shares of common stock. We have substantial accounts receivable, and any delays in collecting accounts receivable or the failure to collect accounts receivable could have a material adverse effect on our cash flows and results of operations. Our accounts receivable balance was \$ **206.264.49** million as of December 31, **2023-2024**, which primarily consisted of **EMPAVELI and SYFOVRE product sales receivable (s) and licensing and other revenue receivables from our collaboration with Sobi**. While we monitor the financial performance and creditworthiness of our customers and provide reserves against trade receivables for expected credit losses that may result from a customer’s failure to pay, no assurances can be made that we will not experience delays in collecting payments, that we will collect the payments due to us. **Sales to a small number of distributors account or for that substantially all** our reserves will be sufficient **gross revenue related to SYFOVRE during the year ended December 31, 2024. Distributors require industry- standard payment terms, including an extended time period for distributors to make payments to Apellis**. Any failures to receive cash payments **due to us** could have a material adverse effect on our results of operations and cash flows. **We do not have a reserve related to expected credit losses against our accounts receivable balance.** Risks Related to the Commercialization and Product Development Our business **is and prospects are** substantially dependent on the success of SYFOVRE and EMPAVELI and the successful development and commercialization of pegcetacoplan in other jurisdictions and disease indications, **including C3G and IC- MPGN**. If we are unable **to continue** to successfully commercialize SYFOVRE and EMPAVELI, or develop, obtain marketing approval for or successfully commercialize **systemic** pegcetacoplan in other indications and jurisdictions, either alone or through a collaboration, or if we experience significant delays in doing so, our business could be harmed. We are investing a significant portion of our efforts and financial resources to fund the commercialization of SYFOVRE and EMPAVELI and development of **systemic** pegcetacoplan in other disease indications and jurisdictions. Our prospects are substantially dependent on our ability, **or that of Sobi or any future collaborator,** to successfully commercialize EMPAVELI in the United States and SYFOVRE worldwide **and to develop, obtain marketing approval for and successfully commercialize systemic pegcetacoplan in additional disease indications**. SYFOVRE is currently only approved in the United States **and Australia. We did not obtain regulatory approval in the European Union, which has adversely affected our business and prospects**. We cannot be certain that we will be able to obtain regulatory approval for, and successfully commercialize, SYFOVRE in any additional jurisdiction. We are also dependent on the success of pegcetacoplan in clinical development and our ability to obtain additional marketing approvals for pegcetacoplan in one or more other indications. Pursuant to our agreement with Sobi, we have granted to Sobi the exclusive right to commercialize systemic pegcetacoplan outside the United States. Our prospects are dependent on **the success of pegcetacoplan, including C3G and IC- MPGN, and** our ability, **to obtain additional marketing approvals or for that of pegcetacoplan in these or other indications. Pursuant to our agreement with Sobi or any future collaborator, we have granted** to successfully commercialize EMPAVELI and **Sobi the exclusive right** to develop, obtain marketing approval for and successfully commercialize systemic pegcetacoplan in additional disease indications **outside the United States**. All of our product candidates other than pegcetacoplan are in **pre-clinical-early stages of** development. The success of EMPAVELI in PNH, SYFOVRE in GA and pegcetacoplan in **C3G and IC- MPGN and in** other disease indications will depend on several factors, including the following: • **our ability to successfully commercialize of EMPAVELI in the United States and SYFOVRE worldwide, including establishing sales, marketing and distribution capabilities for EMPAVELI and SYFOVRE;** • commercial acceptance by patients, the medical community and third-party payors of EMPAVELI in PNH, SYFOVRE in GA, pegcetacoplan in **C3G and IC- MPGN and in** other indications, if approved, and other product candidates, if approved; • **initiation and** successful recruitment of patients, enrollment in and completion of our ongoing and planned clinical trials; •

initiation and successful recruitment of patients, including our planned Phase 3 enrollment in and completion of additional clinical trials with systemic pegcetacoplan in the second half of 2025 for the treatment of FSGS and DGF; • safety, tolerability and efficacy profiles that are satisfactory to the FDA, EMA or any comparable foreign regulatory authority for marketing approval; • our ability to identify success criteria and endpoints for our clinical trials and otherwise design our clinical trials such that the FDA, EMA, and other regulatory authorities will be able to determine the clinical efficacy and safety profile of any product candidates we may develop; • timely receipt of our ability to submit and obtain marketing approvals for from applicable regulatory authorities, including approval of SYFOVRE from the EMA in additional jurisdictions; • the extent of any required post- marketing approval commitments to applicable regulatory authorities; • establishment of supply arrangements with third- party suppliers and manufacturers of for raw materials and, drug intermediates, and; • establishment of arrangements with third- party manufacturers to obtain finished products that are appropriately packaged for sale; • obtaining pegcetacoplan drug product from third- party manufacturers of sufficient quality to be used in our clinical trials and for commercial sale; • developing, validating and maintaining a commercially viable manufacturing process that is compliant with current good manufacturing practices, or cGMPs; • the performance of Sobi and any future collaborators; • obtaining and maintaining patent, trade secret protection and regulatory exclusivity, both in the United States and internationally; • protection of our rights in our intellectual property portfolio; • successful launch of commercial sales following any marketing approval; • a continued acceptable safety profile following any marketing approval; • our ability to compete with other therapies; and • obtaining and maintaining healthcare coverage and adequate reimbursement. Many of these factors are beyond our control, including the results of clinical development, the regulatory submission approval process, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts of our collaborators, including Sobi. If we are unable to successfully commercialize EMPAVELI in the United States for PNH, C3G and IC- MPGN, SYFOVRE worldwide in the United States and select other jurisdictions for GA, or to develop, receive marketing approval for and successfully commercialize pegcetacoplan in other indications or jurisdictions on our own or with a collaborator, or experience delays as a result of any of these factors or otherwise, our business could be substantially harmed. We or others may later discover that EMPAVELI or SYFOVRE is less effective than previously believed or causes safety issues that were not previously identified in clinical trials, which could compromise our ability, or that of our collaborators, to market the product. Clinical trials of our product candidates are conducted in carefully defined sets of patients who have agreed to enter into clinical trials. Consequently, it is possible that our clinical trials, or those of our collaborators, may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify safety issues that may be observed once the product has been commercialized. If safety problems occur or are identified after with EMPAVELI or SYFOVRE or one of our with any other products product of ours that reaches the market, if any, reaches the market, the FDA or comparable non- U. S. regulatory authorities may require that we amend the labeling of our product, recall our product, or even withdraw approval for our product. A small number of patients treated with SYFOVRE in the real world have experienced retinal vasculitis, a severe form of intraocular inflammation. All suspected retinal vasculitis events reported to us are independently evaluated and adjudicated by two external sources: a panel of four retina /uveitis experts and an independent reading center as well as our internal safety and medical teams. We continue to work with the retinal community to investigate potential contributing factors. We plan to continue to submit all adverse events reported to us to the FDA consistent with reporting guidelines for drug manufacturers. We cannot provide any assurances that the FDA and the retinal community will believe that the expected benefits of SYFOVRE treatment outweigh its potential risks to patients in light of these reported events or other events that might arise or that our applications for marketing approval of SYFOVRE in other jurisdictions will not be adversely impacted by these events. A change in the perception of the benefit / risk profile of SYFOVRE may reduce market acceptance of the product and our product revenues may be adversely affected. If, following approval of a product candidate, we, or others, discover that the a product is less effective than previously believed or causes safety issues that were not previously identified, such as the reported events of retinal vasculitis following SYFOVRE treatment, any of the following events could occur: • the target patient population may be less willing to try, and physicians may be less willing to prescribe, the product; • regulatory authorities may withdraw their approval of the product or seize the product; • we, or our collaborators, may be required to recall the product, change the way the product is administered or conduct additional clinical trials; • additional restrictions may be imposed on the marketing of, or the manufacturing processes for, the particular product; • we may be subject to fines, injunctions or the imposition of civil or criminal penalties; • regulatory authorities may require the addition of labeling statements, such as a “ black box ” warning or a contraindication; • we, or our collaborators, may be required to create a Medication Guide outlining the risks of the previously unidentified side effects for distribution to patients; • we, or our collaborators, could be sued and held liable for harm caused to patients; • the product may become less competitive; and • our reputation may suffer. Any of these events could harm our business and operations, affect sales of our products and negatively impact our stock price. We may fail to achieve the degree of market acceptance by physicians, patients, third- party payors and others in the medical community necessary for commercial success, of EMPAVELI or SYFOVRE, in which case we may not generate significant revenues or become profitable, and the market opportunity for these products may be smaller than we estimate. We may fail to gain sufficient market acceptance by physicians, patients, third- party payors and others in the medical community necessary for commercial success of EMPAVELI or SYFOVRE. Our A key part of our commercial strategy for EMPAVELI for PNH is to maintain targeting at switching patients from currently on treatment with eculizumab or ravulizumab to and drive growth by reinforcing EMPAVELI 's differentiated efficacy profile, long- term data and real- world experience. Physicians are often reluctant to switch their patients from existing therapies even when new and potentially more effective or convenient treatments enter the market. Further, patients often acclimate to the therapy that they are currently taking and do not want to switch unless their physicians recommend switching products or, they are required to switch therapies due to lack of reimbursement for existing therapies, or they are not responding well to their existing therapy. If patients

decide to switch their therapy, many may prefer an orally administered therapy, such as iptacopan, for convenience reasons. There are no current treatment options approved for C3G and IC- MPGN. We anticipate that a competitive product, iptacopan, may be approved and marketed prior to EMPAVELI. Our commercial strategy for EMPAVELI for C3G and IC- MPGN is to raise awareness on disease diagnosis, understand the important role of C3 as a driver of disease, recognize the limitations of symptom management, and drive understanding of EMPAVELI' s differentiated efficacy profile. If physicians and patients choose to initiate therapy, EMPAVELI is a self- administered subcutaneous injection and may not be the preferred route of administration. Our commercial strategy for SYFOVRE is to educate the ophthalmology and retina communities on the urgency to diagnose and treat GA, and to establish SYFOVRE as the preferred product due to its differentiated efficacy, flexible dosing options, and real- world utilization. The commercial efforts are also aimed at increasing the breadth of SYFOVRE utilization and experience among treating retina physicians and ensuring broad and sustained access with payors.

Efforts to educate the medical community and third- party payors on the benefits of our products and product candidates may require significant resources and may not be successful. If EMPAVELI, SYFOVRE, or any of our product candidates for which we obtain marketing approval do **not** achieve an adequate level of market acceptance, we may not generate significant revenues and we may not become profitable. The degree of market acceptance of EMPAVELI, SYFOVRE, or our other product candidates for which we obtain marketing approval, will depend on a number of factors, including: • the efficacy and safety of the product; • the potential advantages of the product compared to competitive therapies; • the prevalence and severity of any side effects; • the clinical indications for which the product is approved; • whether the product is designated under physician treatment guidelines as a first-, second- or third- line therapy; • the price at which the product is offered for sale; • the product' s convenience and ease of administration compared to alternative treatments; • the willingness of the target patient population to try, and of physicians to prescribe, the product; • limitations or warnings, including distribution or use restrictions contained in the product' s approved labeling; • the strength of sales, marketing and distribution support; • the approval of other new products for the same indications; • the timing of market introduction of our approved products as well as competitive products; • adverse publicity about the product or favorable publicity about competitive products; • potential product liability claims; • changes in the standard of care for the targeted indications for the product; and • availability and amount of coverage and reimbursement from government payors, managed care plans and other third- party payors. In addition, the potential market opportunity for EMPAVELI in PNH, SYFOVRE in GA, or in any other indication is difficult to precisely estimate. Our estimates of the potential market opportunity for EMPAVELI in PNH, **C3G and IC- MPGN, FSGS and DGF, and** SYFOVRE in GA, or in other indications include several key assumptions based on our industry knowledge, industry publications, **scientific literature**, third- party research reports and other surveys. However, no independent source has verified such assumptions. If any of these assumptions proves to be inaccurate, then the actual market for EMPAVELI in PNH **and C3G and IC- MPGN**, SYFOVRE in GA, or any other **future** indication could be smaller than our estimates of potential market opportunity. If the actual market for EMPAVELI in PNH, SYFOVRE in GA, in other **future** indications is smaller than we expect, our product revenue may be limited, and it may be more difficult for us to achieve or maintain profitability. We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do. The development and commercialization of new products is highly competitive, as described in “ Business- Competition, ” above. We face significant competition with respect to each of EMPAVELI and SYFOVRE. We expect that we, and our collaborators, will face significant competition from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide with respect to any of our product candidates that we, or our collaborators, may seek to develop or commercialize in the future, including from therapies that act through the complement system and therapies that use different approaches. Our competitors may succeed in developing, acquiring or licensing technologies and products that are more effective, have fewer side effects or more tolerable side effects or are less costly than EMPAVELI, SYFOVRE, or any product candidates that we are currently developing or that we may develop, which could render EMPAVELI, SYFOVRE, or our product candidates obsolete and noncompetitive. EMPAVELI targets a market that is already served by a competitor with significantly greater financial resources than us. The principal competitors for EMPAVELI for the treatment of PNH, are eculizumab (marketed as Soliris) and ravulizumab (marketed as Ultomiris), C5 inhibitors developed and marketed by Alexion AstraZeneca Rare Disease, or AstraZeneca. Furthermore, in December 2023, the FDA approved iptacopan (marketed as Fabhalta), an orally administered factor B inhibitor developed by Novartis, for the treatment of PNH. This product may have a competitive advantage if prescribers and patients prefer to utilize an oral medication rather than an injected medication. Prior to the approval of EMPAVELI, eculizumab and ravulizumab were the only drugs approved for the treatment of PNH. These products have widespread acceptance among clinicians, patients and payors. Eculizumab and ravulizumab may also compete with EMPAVELI in other indications in our systemic programs. In 2022, AstraZeneca also obtained approval for a subcutaneous version of ravulizumab, currently in phase 3 clinical trial. SYFOVRE was the first approved product in the United States for the treatment of GA. In August 2023, the FDA approved avacincaptad pegol ( , marketed as Izervay), a complement C5 inhibitor developed by Astellas Pharma Inc., for the treatment of GA. Our commercial opportunity 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 any products that we, or our collaborators, may develop. Our competitors also may obtain FDA or other marketing approval for their products before we, or our collaborators, are able to obtain approval for ours, which could result in our competitors establishing a strong market position before we, or our collaborators, are able to enter the market. Many of our existing and potential future competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining marketing approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early- stage companies may also

prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, the development of our product candidates. If clinical trials of our product candidates fail to satisfactorily demonstrate safety and efficacy to the FDA and other regulators, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of these product candidates. We are not permitted to commercialize, market, promote or sell any product candidate in the United States without obtaining marketing approval from the FDA. Foreign regulatory authorities, such as the EMA, impose similar requirements. We have received approval for SYFOVRE for the treatment of patients with GA in the United States **and Australia**, but there is no assurance that we will receive regulatory approvals for SYFOVRE for the treatment of GA in other jurisdictions. For example, in ~~January~~ 2024, the **European Commission** CHMP of the EMA adopted a negative opinion on the MAA for SYFOVRE in the European Union - ~~While we are seeking re-examination of the MAA, despite positive recommendations from the~~ ~~we cannot be certain that such re-examination will be successful, and~~ ~~ad hoc expert groups convened by~~ ~~we may be required to conduct additional clinical trials of SYFOVRE in order to obtain marketing approval in the~~ **EMA and a significant number of dissenting votes from** European Union **member states**. Because regulators in other jurisdictions, ~~including those in the Access Consortium countries (including the United Kingdom, Switzerland, Australia and Canada)~~ are influenced by decisions of the FDA and the EMA, negative opinion by the FDA or the EMA may adversely impact the prospects for approval in other jurisdictions. We have received approval for EMPAVELI for the treatment of patients with PNH in several jurisdictions, but there is no assurance that we will receive regulatory approvals for EMPAVELI in other indications, **including C3G and IC- MPGN**. Clinical testing is expensive, is difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. The clinical development of our product candidates is susceptible to the risk of failure inherent at any stage of product development, including failure to demonstrate efficacy in a clinical trial or across a broad population of patients, the occurrence of adverse events that are severe or medically or commercially unacceptable, failure to comply with protocols or applicable regulatory requirements and determination by the FDA or any comparable foreign regulatory authority that a product candidate may not continue development or is not approvable. It is possible that even if one or more of our product candidates has a beneficial effect, that effect will not be detected during clinical evaluation as a result of one or more of a variety of factors, including the size, duration, design, measurements, conduct or analysis of our clinical trials. Conversely, as a result of the same factors, our clinical trials may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any. Similarly, in our clinical trials we may fail to detect toxicity or intolerability caused by our product candidates, or mistakenly believe that our product candidates are toxic or not well tolerated when that is not in fact the case. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in earlier development, and we cannot be certain that we will not face additional setbacks. It is possible that any of our development programs may be placed on full or partial clinical hold by regulatory authorities at any point, which would delay and possibly prevent further development of our product candidates. Any inability to successfully complete preclinical and clinical development could result in additional costs to us and impair our ability to generate revenues from product sales, regulatory and commercialization milestones, and royalties. Moreover, if we are required to conduct additional clinical trials or other testing of our product candidates beyond the trials and testing that we contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing or the results of these trials or tests are unfavorable, uncertain or are only modestly favorable, or there are unacceptable safety concerns associated with our product candidates, we may: • incur additional unplanned costs; • be delayed in obtaining marketing approval for our product candidates; • not obtain marketing approval at all; • obtain approval for indications or patient populations that are not as broad as intended or desired; • obtain approval with labeling that includes significant use or distribution restrictions or significant safety warnings, including boxed warnings; • be subject to additional post-marketing testing or other requirements; or • be required to remove the product from the market after obtaining marketing approval. Under our collaboration with Sobi, we are relying on Sobi to conduct certain clinical trials of systemic pegcetacoplan and seek regulatory approval for systemic pegcetacoplan outside the United States. If Sobi or any future collaborator are unable to successfully complete clinical trials of our product candidates and obtain regulatory approvals on a timely basis, or at all, our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties may be materially impaired. In addition, investigators for our clinical trials and other service providers may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services, including equity awards and option grants, and may have other financial interests in our company. We are required to collect and provide financial disclosure notifications or certifications for our clinical investigators to the FDA. If the FDA concludes that a financial relationship between us and a clinical investigator has created a conflict of interest or otherwise affected interpretation of the trial, the FDA may 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 and may ultimately lead to the denial of marketing approval of our current and future product candidates. Our failure to successfully complete clinical trials of our product candidates and to demonstrate the efficacy and safety necessary to obtain regulatory approval to market any of our product candidates would significantly harm our business. Adverse events or undesirable side effects caused by, or other unexpected properties of, any of our product candidates may be identified during clinical development that could delay or prevent their marketing approval or limit their use. Adverse events or undesirable side effects caused by, or other unexpected properties of, our product candidates could cause us, or any collaborator conducting clinical trials of our product candidates such as Sobi, an institutional review board or regulatory authorities to interrupt, delay or halt clinical trials of one or more of our product candidates and could result in a more restrictive

label, or the delay or denial of marketing approval by the FDA or comparable foreign regulatory authorities. For example, by design pegcetacoplan has immunosuppressive effects and, in some cases, may be administered to patients with underlying significantly compromised health. Administration of our product candidates could make patients more susceptible to infection. ~~In our Phase 3 trials of pegcetacoplan in patients with GA, the most common adverse reactions ($\geq 5\%$) reported in patients receiving SYFOVRE were ocular discomfort, neovascular AMD, vitreous floaters, and conjunctival hemorrhage. Rates of ischemic optic neuropathy events were higher in the monthly group as compared to the every-other-month and sham groups. The rate and severity of endophthalmitis and intraocular inflammation were generally in line with reported studies of other intravitreal therapies.~~ If any of our product candidates is associated with adverse events or undesirable side effects or has properties that are unexpected, we, or our collaborators, may abandon development or limit development of that product candidate to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause undesirable or unexpected side effects that prevented further development of the compound. In addition, clinical trials by their nature utilize a sample of the potential patient population. However, with a limited number of subjects and limited duration of exposure, rare and severe side effects of our product candidates may only be uncovered when a significantly larger number of patients are exposed to the product. If we, or any collaborator conducting clinical trials of any of our product candidates such as Sobi, experience any of a number of possible unforeseen events in connection with clinical trials of our product candidates, potential clinical development, marketing approval or commercialization of our product candidates could be delayed or prevented. We, or our collaborators, may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent clinical development, marketing approval or commercialization of our product candidates, including:

- clinical trials of our product candidates may produce unfavorable or inconclusive results;
- we, or our collaborators, may decide, or regulators may require us or them, to conduct additional clinical trials or abandon product development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we, or our collaborators, anticipate, patient enrollment in these clinical trials may be slower than we, or our collaborators, anticipate or participants may drop out of these clinical trials at a higher rate than we, or our collaborators, anticipate;
- the cost of planned clinical trials of our product candidates may be greater than we anticipate;
- our third-party contractors or those of our collaborators, including those manufacturing our product candidates or components or ingredients thereof or conducting clinical trials on our behalf or on behalf of our collaborators, may deviate from the trial protocol, fail to comply with regulatory requirements or fail to meet their contractual obligations to us or our collaborators in a timely manner or at all;
- regulators or institutional review boards may not authorize us, our collaborators or our or their investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we, or our collaborators, may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- patients that enroll in a clinical trial may misrepresent their eligibility to do so or may otherwise not comply with the clinical trial protocol, resulting in the need to drop the patients from the clinical trial, increase the needed enrollment size for the clinical trial or extend the clinical trial's duration;
- we, or our collaborators, may have to delay, suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate;
- regulators or institutional review boards may require that we, or our collaborators, or our or their investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or their standards of conduct, a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate or findings of undesirable effects caused by a chemically or mechanistically similar product or product candidate;
- the FDA or comparable foreign regulatory authorities may disagree with our, or our collaborators', clinical trial designs or our or their interpretation of data from preclinical studies and clinical trials;
- the FDA or comparable foreign regulatory authorities may fail to approve or subsequently find fault with the manufacturing processes or facilities of third-party manufacturers with which we, or our collaborators, enter into agreements for clinical and commercial supplies;
- the supply or quality of raw materials, drug intermediates or manufactured product candidates, other products evaluated in our clinical trials or other materials necessary to conduct clinical trials of our product candidates may be insufficient, inadequate or not available at an acceptable cost, or we may experience interruptions in supply; 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 to obtain marketing approval. Product development costs for us will increase if we experience delays in testing or pursuing marketing approvals and we may be required to obtain additional funds to complete clinical trials and prepare for possible commercialization of our product candidates. We do not know whether any preclinical tests or clinical trials will begin as planned, will need to be restructured, or will be completed on schedule or at all. Significant preclinical study or clinical trial delays also could shorten our periods during which we, or our collaborators, may have the exclusive right to commercialize our product candidates or allow our competitors, or the competitors of our collaborators, to bring products to market before we, or our collaborators, do and impair our ability, or the ability of our collaborators, to successfully commercialize our product candidates and may harm our business and results of operations. In addition, many of the factors that lead to clinical trial delays may ultimately lead to the denial of marketing approval of any of our product candidates. If we, or any collaborator conducting clinical trials of any of our product candidates such as Sobi, experience delays or difficulties in the enrollment of patients in clinical trials, our or their receipt of necessary regulatory approvals could be delayed or prevented. We, or our collaborators, may not be able to initiate or continue clinical trials for any of our product candidates if we, or they, are unable to locate and enroll a sufficient number of eligible patients to participate in clinical trials as required by the FDA or comparable foreign regulatory authorities. Patient enrollment is a significant factor in the timing of clinical trials, and is affected by many factors, including:
- the size and nature of the patient population;
- the severity of the disease under investigation;
- the proximity of patients to clinical sites;
- the patient referral

practices of physicians; • the eligibility criteria for the trial; • the design of the clinical trial; • efforts to facilitate timely enrollment; • competing clinical trials; and • clinicians' and patients' perceptions as to the potential advantages and risks of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating. For example, in January 2024, we and Sobi agreed to cease clinical development of systemic pegcetacoplan for patients with **cold agglutinin disease, or CAD**, due to the decreased medical need in CAD and the limited number of patients eligible for the CASCADE trial. Many of the indications for which we are developing product candidates are rare diseases with small patient populations, and many of those patients are treated with other therapies or products. Further, there are only a limited number of specialist physicians that regularly treat patients with these rare diseases and major clinical centers that support such treatment are concentrated in a few geographic regions. In addition, other companies are conducting clinical trials and have announced plans for future clinical trials that are seeking, or are likely to seek, to enroll patients with these rare diseases and patients are generally only able to enroll in a single trial at a time. Both patients and their physicians may be reluctant to forgo, discontinue or otherwise alter existing, approved life- saving therapeutic approaches. Given the severe and life- threatening nature of these indications and the expectation that many patients will be on treatment with other therapies or products, we may encounter difficulty in recruiting a sufficient number of patients for our trials including in particular our planned clinical trials. The small population of patients, competition for these patients, the nature of the disease and limited trial sites may make it difficult for us to enroll enough patients to complete our clinical trials of pegcetacoplan in a timely and cost-effective manner. Our inability, or the inability of our collaborators, to enroll a sufficient number of patients for our, or their, clinical trials could result in significant delays or may require us or them to abandon one or more clinical trials altogether. Enrollment delays in our, or their, clinical trials may result in increased development costs for our product candidates, delay or halt the development of and approval processes for our product candidates and jeopardize our, or our collaborators', ability to commence sales of and generate revenues from our product candidates, which could cause the value of our company to decline and limit our ability to obtain additional financing, if needed. Results of preclinical studies and Phase 1 and Phase 2 clinical trials may not be predictive of results of later clinical trials and preliminary or interim results of clinical trials do not necessarily predict final results. The outcome of preclinical studies and Phase 1 and Phase 2 clinical trials may not be predictive of the success of later clinical trials, and preliminary or interim results of clinical trials do not necessarily predict final results. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late- stage clinical trials after achieving positive results in earlier stages of clinical development, and we could face similar setbacks. Similarly, the design of a clinical trial can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. In addition, preclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval for the product candidates. Even if we, or our collaborators, believe that the results of clinical trials for our product candidates warrant marketing approval, the FDA or comparable foreign regulatory authorities may disagree and may not grant marketing approval of our product candidates. In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. If we fail to receive positive results in clinical trials of our product candidates, the development timeline and regulatory approval and commercialization prospects for our most advanced product candidates, and, correspondingly, our business and financial prospects would be negatively impacted. If we fail to develop and commercialize other product candidates, we may be unable to grow our business. Although the development and commercialization of pegcetacoplan is our primary focus, as part of our growth strategy, we are developing a pipeline of product candidates for the treatment of complement- dependent diseases, **including APL- 3007, which is a siRNA, and our FcRn gene editing treatment that we are developing through our collaboration with Beam. These product candidates utilize different mechanisms of action than EMPAVELI and SYFOVRE and we do not have experience conducting clinical trials of product candidates with such mechanisms of action**. These other product candidates will require additional, time-consuming and costly development efforts prior to commercial sale, including preclinical studies, clinical trials and approval by the FDA and / or 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 effective for approval by regulatory authorities. In addition, there can be no assurance that any such products that are approved will be manufactured or produced economically, successfully commercialized or widely accepted in the marketplace or be more effective than other commercially available alternatives. ~~We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success. Because we have limited financial and managerial resources, we intend to focus on developing product candidates for specific indications that we identify as most likely to succeed, in terms of both their potential for marketing approval and commercialization. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that may prove to have greater commercial potential. 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 and product candidates for specific indications may not yield any commercially viable product candidates. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to the product candidate. If the commercial launch of EMPAVELI in the United States for PNH and of SYFOVRE in the United States for GA~~, for each of which we recruited a sales force and established marketing, market access

and medical affairs teams and distribution capabilities is not successful for any reason, we could incur substantial costs and our investment would be lost if we cannot retain or reposition our sales, marketing, market access and medical affairs personnel. To achieve commercial success for EMPAVELI and SYFOVRE, we have expended and anticipate that we will continue to expend significant resources to support our sales force, marketing, market access and medical affairs teams and distribution capabilities. There are risks involved with establishing our own sales, marketing, distribution, training and support capabilities. For example, recruiting and training sales and marketing personnel is expensive and time consuming and could delay our ability to focus on other priorities. If the commercial launch of EMPAVELI or SYFOVRE is not successful for any reason, this would be costly, and our investment would be lost if we cannot retain or reposition our sales, marketing, market access and medical affairs personnel or terminate on favorable terms any agreements entered into with third parties to support our commercialization efforts. Factors that may inhibit our efforts to commercialize EMPAVELI or SYFOVRE on our own in the United States include: • our inability to train and retain adequate numbers of effective sales, marketing, training and support personnel; • the inability of sales personnel to obtain access to physicians, including key opinion leaders, or to educate an adequate number of physicians of • the benefits of EMPAVELI or SYFOVRE over alternative treatment options; • the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with • more extensive or integrated product offerings; and • unforeseen costs and expenses associated with establishing and maintaining an independent sales, marketing, training and support organization. If our salesforce, marketing, market access and medical affairs teams and distribution capabilities fail, or are otherwise unsuccessful, it would materially adversely impact the commercial launch of EMPAVELI or SYFOVRE, impact our ability to generate revenue and harm our business. If we are unable to **maintain establish sales, marketing and distribution capabilities or our enter into sales, marketing and distribution arrangements with third parties, we may not be successful in commercializing EMPAVELI and SYFOVRE. Similarly, if we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution arrangements with third parties, we may not be successful in commercializing EMPAVELI, SYFOVRE, pegcetacoplan in other indications or any of our other product candidates for which we obtain marketing approval.** We have built a sales, marketing and distribution infrastructure in the United States to support commercialization of EMPAVELI and SYFOVRE. We are building focused capabilities to commercialize SYFOVRE in GA and EMPAVELI in PNH, **C3G** and **other indications-IC-MPGN** where we believe that the medical specialists for such indications are sufficiently concentrated to allow us to effectively promote the product with a targeted sales team. The development of sales, marketing and distribution capabilities requires substantial resources, is time- consuming and could delay any product launch. In addition, we may not be able to hire or retain a sales force in the United States that is sufficient in size or has adequate expertise in the medical markets that we plan to target. If we are unable to establish or retain a sales force and marketing and distribution capabilities, our operating results may be adversely affected. If a potential partner has development or commercialization expertise that we believe is particularly relevant to one of our products, then we may seek to collaborate with that potential partner even if we believe we could otherwise develop and commercialize the product independently. In certain indications, we may seek to enter into collaborations that we believe may contribute to our ability to advance development and ultimately commercialize our product candidates. We may also seek to enter into collaborations where we believe that realizing the full commercial value of our development programs will require access to broader geographic markets or the pursuit of broader patient populations or indications. As a result of entering into arrangements with third parties to perform sales, marketing and distribution services, our product revenues or the profitability of these product revenues may be lower, perhaps substantially lower, than if we were to directly market and sell products in those markets. Furthermore, we may be unsuccessful in entering into the necessary arrangements with third parties or may be unable to do so on terms that are favorable to us. In addition, we may have little or no control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales, marketing and distribution capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing EMPAVELI, SYFOVRE, or our other product candidates that receive marketing approval. We have granted exclusive commercialization rights for systemic pegcetacoplan outside of the United States to Sobi under our agreement with Sobi. If Sobi is unable to meet its contractual obligations, we may be forced to focus our efforts internally to commercialize systemic pegcetacoplan outside of the United States without the assistance of a commercialization partner or seek another commercialization partner, either of which would result in us incurring greater expenses and could cause a delay in market penetration while we expand our commercial operations or seek an alternative commercialization partner. Such costs may exceed the increased revenues we would receive from direct systemic pegcetacoplan sales outside of the United States, at least in the near term. We would also be forced to declare a breach of the agreement with Sobi and seek a termination of the agreement which could result in an extended and uncertain dispute with Sobi, including arbitration or litigation, any of which would be costly. If the FDA or comparable foreign regulatory authorities approve generic versions of any of our products that receive marketing approval, or such authorities do not grant our products appropriate periods of data exclusivity before approving generic versions of our products, the sales of our products could be adversely affected. Once an NDA is approved, the product covered thereby becomes a “ reference- listed drug ” in the FDA’s publication, “ Approved Drug Products with Therapeutic Equivalence Evaluations, ” or the Orange Book. Manufacturers may seek approval of generic versions of reference-listed drugs through submission of an **ANDA in the United States. In support of an** abbreviated new drug application, or **ANDA, in the United States. In support of an** ANDA, a generic manufacturer need not conduct clinical trials. Rather, the applicant generally must show that its product has the same active ingredient (s), dosage form, strength, route of administration and conditions of use or labeling as the reference- listed drug and that the generic version is bioequivalent to the reference- listed drug, meaning it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference- listed drug and companies that produce generic products are generally able to offer them at lower prices. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded

product or reference- listed drug may be typically lost to the generic product. The FDA may not approve an ANDA for a generic product until any applicable period of non- patent exclusivity for the reference- listed drug has expired. The Federal Food, Drug, and Cosmetic Act, or FDCA, provides a period of five years of non- patent exclusivity for a new drug containing a new chemical entity, or NCE. Specifically, in cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification that a patent covering the reference- listed drug is either invalid or will not be infringed by the generic product, in which case the applicant may submit its application four years following approval of the reference- listed drug. **Pegcetacoplan received its first approval from the FDA in May 2021.** It is unclear whether the FDA will treat the active ingredients in our product candidates as NCEs and, therefore, afford them five years of NCE data exclusivity if they are approved. If any product we develop does not receive five years of NCE exclusivity, the FDA may approve generic versions of such product three years after its date of approval, subject to the requirement that the ANDA applicant certifies to any patents listed for our products in the Orange Book. Manufacturers may seek to launch these generic products following the expiration of the applicable marketing exclusivity period, even if we still have patent protection for our product. Competition that our products may face from generic versions of our products could negatively impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on our investments in those product candidates. EMPAVELI, SYFOVRE, or any product candidate that we or any collaborator, such as Sobi, commercialize may become subject to unfavorable pricing regulations, third- party payor reimbursement practices or healthcare reform initiatives, any of which could harm our business. The commercial success of EMPAVELI, SYFOVRE, or any **of** our product candidates that we or any collaborator, such as Sobi, commercialize will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by third- party payors, including government health administration authorities and private health coverage insurers. If coverage and reimbursement is not available, or reimbursement is available only to limited levels, we, or our collaborators, may not be able to successfully commercialize EMPAVELI, SYFOVRE, or any other product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us, or our collaborators, to establish or maintain pricing sufficient to realize a sufficient return on our or their investments. In the United States, no uniform policy of coverage and reimbursement for products exists among third- party payors and coverage and reimbursement for 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 our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. There is significant uncertainty related to third- party payor coverage and reimbursement of newly approved drugs. The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drug products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we, or our collaborators, might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay commercial launch of the product, possibly for lengthy time periods, which may negatively impact the revenues we are able to generate from the sale of the product in that country. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost- effectiveness of our product or product candidate to other available therapies. Adverse pricing limitations may hinder our ability or the ability of our collaborators to recoup our or their investment in one or more products or product candidates, even if our product candidates obtain marketing approval. Patients who are provided medical treatment for their conditions generally rely on third- party payors to reimburse all or part of the costs associated with their treatment. Therefore, our ability, and the ability of our collaborators, to commercialize EMPAVELI, SYFOVRE, or any of our product candidates will depend in part on the extent to which coverage and reimbursement for these products and related treatments will be available from third- party payors. Third- party payors decide which medications they will cover and establish reimbursement levels. The healthcare industry is acutely focused on cost containment, both in the United States and abroad. Government authorities and other third- party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications, which could affect our ability or that of our collaborators to sell EMPAVELI, SYFOVRE, or our product candidates profitably. These payors may not view our products, if any, as cost- effective, and coverage and reimbursement may not be available to our customers, or those of our collaborators, or may not be sufficient to allow our products, if any, to be marketed on a competitive basis. Cost- control initiatives could cause us, or our collaborators, to decrease the price we, or they, might establish for products, which could result in lower than anticipated product revenues. If the prices for our products, if any, decrease or if governmental and other third- party payors do not provide coverage or adequate reimbursement, our prospects for revenue and profitability will suffer. The commercial potential of our products depends in part on reimbursement by government health administration authorities, private health insurers and other organizations. If we, or any collaborator that is commercializing our product candidates such as Sobi are unable to obtain coverage or reimbursement for our products, as monotherapy or in combination with other therapies, including possible combinations with eculizumab or ravulizumab, at the levels anticipated, our financial condition could be harmed. Additionally, if new compounds currently in development by potential competitors, including biosimilars of eculizumab or ravulizumab, obtain marketing approval, there may be downward pressure on reimbursement levels for therapies in our target disease areas, which could have a negative impact on our ability to achieve and maintain profitability. There may also be delays in obtaining coverage and reimbursement for newly approved drugs, such as EMPAVELI, SYFOVRE, and coverage may be more limited for EMPAVELI and SYFOVRE than the indication for which the drug is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for reimbursement does not

imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Reimbursement rates may vary, by way of example, according to the use of the product and the clinical setting in which it is used. Reimbursement rates may also be based on reimbursement levels already set for lower cost drugs or may be incorporated into existing payments for other services. In addition, increasingly, third-party payors are requiring higher levels of evidence of the benefits and clinical outcomes of new technologies and are challenging the prices charged. We cannot be sure that coverage will be available for any product candidate that we, or any collaborator, including Sobi, commercialize and, if available, that the reimbursement rates will be adequate. Further, the net reimbursement for drug products may be subject to additional reductions if there are changes to laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. An inability to promptly obtain coverage and adequate payment rates from both government-funded and private payors for any of our product candidates for which we, or our collaborator, obtain marketing approval could significantly harm our operating results, our ability to raise capital needed to commercialize products and our overall financial condition. Product liability lawsuits against us could divert our resources, cause us to incur substantial liabilities and limit commercialization of EMPAVELI, SYFOVRE, and any other products that we may develop. We face an inherent risk of product liability claims as a result of the commercial sale of EMPAVELI and SYFOVRE, and the clinical testing of our product candidates despite obtaining appropriate informed consents from our clinical trial participants. For example, we may be sued if any product we develop allegedly causes 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 or 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 our product candidates. Regardless of the merits or eventual outcome, liability claims may result in: • decreased demand for EMPAVELI, SYFOVRE, and any other product candidates that we may develop; • injury to our reputation and significant negative media attention; • withdrawal of clinical trial participants; • significant costs to defend resulting litigation; • substantial monetary awards to trial participants or patients; • loss of revenue; • reduced resources of our management to pursue our business strategy; and • the inability to successfully commercialize EMPAVELI, SYFOVRE, or any other products that we may develop. Although we maintain product liability and clinical trial insurance coverage in the amount of up to \$ 50.0 million in the aggregate, this insurance may not fully cover potential liabilities that we may incur. The cost of any litigation or other proceeding, even if resolved in our favor, could be substantial. We may need to increase our insurance coverage as we continue to commercialize EMPAVELI, SYFOVRE, and commercialize any other product candidate that receives marketing approval. In addition, insurance coverage is becoming increasingly expensive. If we are unable to maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and sale of EMPAVELI, SYFOVRE, and our other product candidates, which could harm our business, financial condition, results of operations and prospects. Our internal information technology systems, or those of any contractors, consultants, vendors, business partners or other third parties, may fail or suffer security breaches, which could result in a material disruption of our product development programs, compromise sensitive information related to our business or prevent us from accessing critical information, trigger contractual and legal obligations, potentially exposing us to liability, reputational harm or otherwise adversely affecting our business and financial results. We are dependent upon information technology systems, infrastructure and data to operate our business. In the ordinary course of business, we collect, store and transmit large amounts of confidential information, including personal information and information relating to intellectual property, on internal information systems and through the information systems of our contractors, consultants, vendors, business partners or other third parties. It is critical that we, our vendors, collaborators or other contractors or consultants, do so in a secure manner to maintain the availability, security, confidentiality, privacy and integrity of such confidential information. Despite the implementation of security measures, our internal information technology systems and those of third parties are vulnerable to damage from computer viruses, malware, computer hackers, malicious code, employee error, theft or misuse, denial-of-service attacks, sophisticated nation-state supported actors, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Such systems are also vulnerable to service interruptions or to security breaches from inadvertent or intentional actions by our employees, our collaborators, contractors, consultants, vendors, business partners and other third parties, or from cyber-attacks by malicious third parties over the Internet or through other mechanisms. Cyber-attacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cyber-attacks could include the deployment of harmful malware, ransomware, denial of service attacks, unauthorized access to or deletion of files, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information. Cyber-attacks also could include phishing attempts or e-mail fraud to cause payments or information to be transmitted to an unintended recipient. We may not be able to anticipate all types of security threats, and we may not be able to implement preventive measures effective against all such security threats. The techniques used by cyber criminals change frequently, may not be recognized until launched, and can originate from a wide variety of sources, including outside groups such as external service providers, organized crime affiliates, terrorist organizations or hostile foreign governments or agencies. We cannot guarantee that the measures we have taken to date, and actions we may take in the future, will be sufficient to prevent any future breaches. While we have not experienced any such material system failure, accident, cyber-attack or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs, clinical trials and business operations, whether due to a loss of our trade secrets or other proprietary or confidential information or other similar disruptions, in addition to possibly requiring substantial expenditures of resources to remedy. For example, the loss of clinical trial data from clinical trials could result in delays or termination of our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. In addition,

as risks with respect to our information systems continue to evolve, we will incur additional costs to maintain the security of our information systems and comply with evolving laws and regulations pertaining to cybersecurity and related areas. To the extent that any disruption or security breach were to result in a loss of, or damage to, our or our vendors', collaborators' or other contractors' or consultants' data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, including litigation exposure, penalties and fines, we could become the subject of regulatory action or investigation, enrollment in our clinical trials could be negatively affected, our competitive position and reputation could be harmed and the further development and commercialization of our product candidates could be delayed. As a result of such an event, we may be in breach of our contractual obligations. Furthermore, any such event that leads to unauthorized access, use, or disclosure of personal information, including personal information regarding our customers or employees, could harm our reputation, compel us to comply with federal and / or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information, which could result in significant legal and financial exposure and reputational damages. Any of the above could have a material adverse effect on our business, financial condition, results of operations or prospects. The financial exposure from the events referenced above could either not be insured against or not be fully covered through any insurance that we maintain and could have a material adverse effect on our business, financial condition, results of operations or prospects. In addition, we cannot be sure that our existing insurance coverage will continue to be available on acceptable terms or that our insurers will not deny coverage as to any future claim. There can be no assurance that the limitations of liability in our contracts would be enforceable or adequate or would otherwise protect us from liabilities or damages as a result of the events referenced above.

Risks Related to Our Dependence on Third Parties We rely on third parties to conduct our clinical trials. If they do not perform satisfactorily, our business could be harmed. We do not independently conduct clinical trials of our product candidates. We rely, and expect to continue to rely, on third parties, such as contract research organizations, clinical data management organizations, medical institutions and clinical investigators, to conduct our clinical trials of pegcetacoplan and any other product candidate that we develop. Any of these third parties may terminate their engagements with us under certain circumstances. We may not be able to enter into alternative arrangements or do so on commercially reasonable terms. In addition, there is a natural transition period when a new contract research organization begins work. As a result, delays would likely occur, which could negatively impact our ability to meet our expected clinical development timelines and harm our business, financial condition and prospects. Further, although our reliance on these third parties for clinical development activities limits our control over these activities, we remain responsible for ensuring that each of our trials is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards. For example, notwithstanding the obligations of a contract research organization for a trial of one of our product candidates, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as current Good Clinical Practices, or cGCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. The FDA enforces these cGCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites and institutional review boards. If we or our third-party contractors fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving our product candidates, which would delay the marketing approval process. We cannot be certain that, upon inspection, the FDA will determine that any of our clinical trials comply with cGCPs. Similar regulatory requirements apply outside the United States, including the International Council for Harmonisation of Technical Requirements for the Registration of Pharmaceuticals for Human Use, or ICH. We are also required to register clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. Furthermore, the third parties conducting clinical trials on our behalf are not our employees, and except for remedies available to us under our agreements with such contractors, we cannot control whether or not they devote sufficient time, skill and resources to our ongoing development programs. These contractors 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 impede their ability to devote appropriate time to our clinical programs. ~~In addition, these contractors may be adversely affected by the COVID-19 pandemic.~~ If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we may not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates. If that occurs, we will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. In such an event, our financial results and the commercial prospects for any product candidates that we seek to develop could be harmed, our costs could increase and our ability to generate revenues could be delayed, impaired or foreclosed. We contract with third parties for the manufacture, storage and distribution of commercial supply for EMPAVELI, SYFOVRE, and clinical supply for our product candidates and expect to continue to do so in connection with our future development and commercialization efforts. This reliance on third parties increases the risk that we will not have sufficient quantities of pegcetacoplan or our other product candidates or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts. We currently have no manufacturing facilities, and a relatively small number of personnel with manufacturing experience who can oversee the manufacturing process. We rely on contract manufacturers to manufacture, store and distribute both drug substance and drug product required for our clinical trials. We also rely upon contract manufacturers, and potentially collaboration partners to manufacture commercial quantities of EMPAVELI, SYFOVRE, and any of our other product candidates, if approved. We may be unable to establish any agreements with contract manufacturers or to do so on acceptable terms, or to maintain such agreements as we may enter. Even if we are able to establish agreements with contract manufacturers, reliance on contract

manufacturers entails additional risks, including: • manufacturing delays if our third- party contractors give greater priority to the supply of other products over EMPAVELI, SYFOVRE, or our product candidates or otherwise do not satisfactorily perform according to the terms of the agreements between us and them, or if unforeseen events in the manufacturing process arise; • the possible termination or nonrenewal of agreements by our third- party contractors at a time that is costly or inconvenient for us; • the possible breach by the third- party contractors of our agreements with them; • the failure of third- party contractors to comply with applicable regulatory requirements; • the possible mislabeling of clinical supplies, potentially resulting in the wrong dose amounts being supplied or active drug or placebo not being properly identified; • the possibility of clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions, or of drug supplies not being distributed to commercial vendors in a timely manner, resulting in lost sales; and • the possible misappropriation of our proprietary information, including our trade secrets and know- how. We currently rely, and expect to continue to rely, on a small number of third- party contract manufacturers to supply most of our supply of active pharmaceutical ingredients and required finished product for our commercial supply of EMPAVELI and SYFOVRE and for our clinical supply of our product candidates. In particular, we have entered into commercial supply agreements with Bachem **Americas, Inc., or Bachem,** and NOF **Corporation, or NOF,** to purchase a significant portion of our requirements for the pegcetacoplan drug substance and drug intermediaries, respectively ~~over the next five years~~. We have also entered into long- term commercial supply agreements with other suppliers of raw materials, drug intermediaries, drug substance and drug product. We also have a separate supply agreement for the manufacture of the drug product for each of EMPAVELI and SYFOVRE. If any of our existing manufacturers should become unavailable to us for any reason, we may incur delays in identifying or qualifying replacements. We also rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our contract manufacturers or distributors could delay clinical development or marketing approval of our product candidates or commercialization of any resulting products, producing additional losses and depriving us of potential product revenue. ~~For example, in the past we experienced issues associated with the manufacturing process for pegcetacoplan that resulted in delays in the supply of pegcetacoplan. These delays resulted in us incurring additional costs and delays in our PNH development program. Additionally, in October 2018, we announced that we voluntarily implemented a pause in dosing in our clinical trials in patients with GA and wet AMD due to observed cases of non- infectious inflammation in patients treated from a single manufacturing lot of pegcetacoplan ophthalmological drug product that we believe occurred due to an impurity in the active pharmaceutical ingredient.~~ If we experience other issues or delays in the future, our commercial success may be materially and adversely impacted and our development of pegcetacoplan may be materially delayed, and our business adversely affected. Any manufacturing problem, the loss of a contract manufacturer or any loss of storage could be disruptive to our operations, result in lost sales of EMPAVELI and / or SYFOVRE or delay our clinical trials. Accordingly, for example, if Bachem or NOF were to experience manufacturing and supply issues, we would have difficulty in procuring the drug substance or drug intermediates needed for the supply and manufacture of pegcetacoplan. Additionally, we rely on third parties to supply the raw materials needed to manufacture our product candidates. Any reliance on suppliers may involve several risks, including a potential inability to obtain critical materials and reduced control over production costs, delivery schedules, reliability and quality. Any unanticipated disruption to our contract manufacturing caused by problems at suppliers could delay shipment of our product candidates, increase our cost of goods sold and result in lost sales with respect to any approved products. For EMPAVELI, SYFOVRE, and any product candidates that are approved by any regulatory agency, we will need to maintain agreements with third- party contract manufacturers for the commercial production and distribution of those products. It may be difficult for us to reach agreement with a contract manufacturer on satisfactory terms or in a timely manner. In addition, we may face competition for access to manufacturing facilities as there are a limited number of contract manufacturers operating under cGMPs that can manufacture our product candidates. Consequently, we may not be able to reach agreement with third- party manufacturers on satisfactory terms, which could delay our commercialization efforts. Third- party manufacturers are required to comply with cGMPs and similar regulatory requirements outside the United States, such as the ICH. Facilities used by our third- party manufacturers must be approved by the FDA after we submit an NDA and before potential approval of the product candidate. Similar regulations apply to manufacturers of our product candidates for use or sale in foreign countries. We do not control the manufacturing process and are completely dependent on our third- party manufacturers for compliance with the applicable regulatory requirements for the manufacture of our product candidates. If our manufacturers cannot successfully manufacture material that conforms to our specifications or the strict regulatory requirements of the FDA and any applicable foreign regulatory authority, they may not be able to meet our supply requirements for clinical and commercial operations and to secure the applicable approval for their manufacturing facilities. If these facilities are not approved for commercial manufacture, we may need to find alternative manufacturing facilities, which could result in delays in obtaining approval for the applicable product candidate. In addition, our manufacturers are subject to ongoing periodic inspections by the FDA and corresponding state and foreign agencies for compliance with cGMPs and similar regulatory requirements both prior to and following the receipt of marketing approval for any of our product candidates. Some of these inspections may be unannounced. Failure by any of our manufacturers to comply with applicable cGMPs or other regulatory requirements could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspensions or withdrawals of approvals, operating restrictions, interruptions in supply and criminal prosecutions, any of which could significantly impact the available supplies of our product candidates and harm our business, financial condition and results of operations. We have developed the EMPAVELI injector, a custom, on- body drug delivery system that would enable patients to self- administer pegcetacoplan through subcutaneous infusion. ~~While the EMPAVELI injector was in development, we used one or more commercially available ambulatory infusion pumps in our ongoing and planned clinical trials and for our commercial launch of EMPAVELI and SYFOVRE.~~ If the EMPAVELI injector becomes unavailable, **we patients** may need to rely upon commercially available ambulatory infusion pumps. Any reliance on third- party infusion pumps may involve several risks, including reduced control over costs, delivery

schedules, reliability and quality. ~~Our~~ **We are developing a single dose, sterilized prefilled syringe for SYFOVRE that will provide physicians with a new way to administer SYFOVRE that requires fewer steps compared to the current and anticipated future dependence administration. If the pre-filled syringe cannot be manufactured in accordance with applicable regulatory requirements or the availability of pre-filled syringe is delayed, physicians may need to rely upon others for the manufacture of EMPAVELI, SYFOVRE, or our product candidates may harm our future profit margins and our ability to commercialize EMPAVELI, SYFOVRE, or any other -- the existing administration method** products that receive marketing approval on a timely and competitive basis. Our prospects for the development and commercialization of our product candidates will depend in part on the success of our collaboration with Sobi and future collaborations. We have entered into a collaboration with Sobi for the global co-development and commercialization outside of the United States of systemic pegcetacoplan and we may seek to enter into additional collaborations for the development and commercialization of certain of our products or product candidates. We may have limited control over the amount and timing of resources that our collaborators, including Sobi, will dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, our collaborators may have the right to abandon research or development projects and terminate applicable agreements, including funding obligations, prior to or upon the expiration of the agreed upon terms. Collaborations involving our product candidates pose a number of risks, including the following: • collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations; • collaborators may not perform their obligations as expected; • collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs, based on clinical trial results, changes in the collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities; • collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing; • collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates; • a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products; • disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive; • 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 potential litigation; • collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; • disputes may arise between the collaborators and us regarding ownership of or other rights in the intellectual property generated in the course of the collaborations; and • collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates. For example, our agreement with Sobi is subject to early termination in the event of any uncured material breach of the agreement or under specific circumstances relating to insolvency. If we do not maintain a productive collaborative relationship with Sobi or if Sobi is unable to meet its contractual obligations or if there is an early termination of the agreement as described above, we would be forced to either establish a commercial infrastructure outside of the United States so that we could undertake the commercialization efforts which had been theretofore undertaken by Sobi or we would need to seek an alternative collaborator. The establishment of a commercial infrastructure and assumption by us of commercialization activities outside of the United States would require substantial resources, financial and otherwise, and could result in us incurring greater expenses than the increase in revenues from our direct sales of systemic pegcetacoplan. It could also cause a delay in market penetration while we expand our commercial operations. Seeking and obtaining an alternative collaborator outside the United States could also adversely impact sales of systemic pegcetacoplan and market penetration outside of the United States. Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If our collaborators, including Sobi are involved in a business combination, it could decide to delay, diminish or terminate the development or commercialization of any product candidate licensed to it by us. ~~We have in the past established, and in the future, may seek to establish, additional collaborations and, if we are not able to establish them on commercially reasonable terms, we may have to alter our development and commercialization plans. We entered into the collaboration agreement with Sobi in October 2020 concerning the development and commercialization of pegcetacoplan and specified other structurally and functionally similar compstatin analogues or derivatives for use systemically or for local non-ophthalmic administration. We may seek to establish one or more additional collaborators for the development and commercialization of one or more of our product candidates. Likely collaborators may include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. In addition, if we are able to obtain marketing approval for product candidates from foreign regulatory authorities, we intend to enter into strategic relationships with international biotechnology or pharmaceutical companies for the commercialization of such product candidates outside of the United States. We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the potential differentiation of our product candidates from competing product candidates, design or results of clinical trials, the likelihood of approval by the FDA or comparable foreign regulatory authorities and the regulatory pathway for any such approval, the potential market for the product candidate, the costs and complexities of manufacturing and delivering the product to patients and the potential of competing products. The~~

collaborator may also consider alternative product candidates or technologies for similar indications that may be available for collaboration and whether such a collaboration could be more attractive than the one with us for our product candidate. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue. Collaborations are complex and time-consuming to negotiate and document. Further, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. Any collaboration agreements that we enter into in the future may contain restrictions on our ability to enter into potential collaborations or to otherwise develop specified product candidates. We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense.

Risks Related to Our Intellectual Property

If we fail to comply with our obligations under our existing and any future intellectual property licenses with third parties, we could lose license rights that are important to our business. We are a party to patent license agreements with The University of Pennsylvania, or Penn, under which we license patent rights relating to a family of compounds for use in all fields. The licensed patent rights include issued U. S. and foreign patents with claims that recite a class of compounds generically covering pegcetacoplan and that specifically recite the active component. We may enter into additional license agreements in the future. Our license agreements with Penn impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with our obligations under these licenses, our licensors may have the right to terminate these license agreements, in which event we might not be able to market any product that is covered by these agreements, or our licensors may convert the license to a non-exclusive license, which could negatively impact the value of the product candidate being developed under the license agreement. Termination of these license agreements or reduction or elimination of our licensed rights may also result in our having to negotiate new or reinstated licenses with less favorable terms. If we are unable to obtain and maintain sufficient patent protection for our product candidates, or if the scope of the patent protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our product candidates may be adversely affected. Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary product candidates. If we do not adequately protect our intellectual property rights, competitors may be able to erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability. To protect our proprietary position, we file patent applications in the United States and abroad related to our product candidates that are important to our business; we also license, or purchase patent applications filed by others. The patent application and approval process is expensive and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. Agreements through which we license patent rights may not give us control over patent prosecution or maintenance, so that we may not be able to control which claims or arguments are presented and may not be able to secure, maintain, or successfully enforce necessary or desirable patent protection from those patent rights. We have not had and do not have primary control over patent prosecution and maintenance for certain of the patents and patent applications we license, and therefore cannot guarantee that these patents and applications will be prosecuted in a manner consistent with the best interests of our business. We cannot be certain that patent prosecution and maintenance activities by our licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents. We, or any partners, collaborators, or licensees, may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Therefore, we may miss potential opportunities to strengthen our patent position. Moreover, in some circumstances, we might not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering any technology that we may license from third parties in the future. These patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business. Our license agreements with Penn provide that Penn has the right under certain circumstances to control the preparation, prosecution and maintenance of the underlying patent rights. It is possible that defects of form in the preparation or filing of our patents or patent applications may exist, or may arise in the future, for example with respect to proper priority claims, inventorship, claim scope, or patent term adjustments. If we or our partners, collaborators, licensees, or licensors, whether current or future, fail to establish, maintain or protect such patents and other intellectual property rights, such rights may be reduced or eliminated. If our partners, collaborators, licensees, or licensors are not fully cooperative or disagree with us as to the prosecution, maintenance or enforcement of any patent rights, such patent rights could be compromised. If there are material defects in the form, preparation, prosecution, or enforcement of our patents or patent applications, such patents may be invalid and / or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business. The patent position of biotechnology and pharmaceutical companies generally is highly uncertain. No consistent policy regarding the breadth of claims allowed in biotechnology and pharmaceutical patents has emerged to date in the United States or in many foreign jurisdictions. In addition, the determination of patent rights with respect to pharmaceutical compounds commonly involves complex legal and factual questions, which has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issue from such applications. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent.

However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. 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 were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. Similarly, we cannot be certain that parties from whom we do or may license, or purchase patent rights were the first to make relevant claimed inventions or were the first to file for patent protection for them. If third parties have filed patent applications on inventions claimed in our patents or applications on or before March 15, 2013, an interference proceeding in the United States can be initiated by such third parties to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. If third parties have filed such applications after March 15, 2013, a derivation proceeding in the United States can be initiated by such third parties to determine whether our invention was derived from theirs. Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our patents or pending patent applications may be challenged in the courts or patent offices in the United States and abroad. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found. If such prior art exists, it may be used to invalidate a patent, or may prevent a patent from issuing from a pending patent application. For example, such patent filings may be subject to a third-party preissuance submission of prior art to the U. S. Patent and Trademark Office, or USPTO, or to other patent offices around the world. Alternately or additionally, we may become involved in post-grant review procedures, oppositions, derivations, proceedings, reexaminations, inter partes review or interference proceedings, in the United States or elsewhere, challenging patents or patent applications in which we have rights, including patents on which we rely to protect our business. An adverse determination in any such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, 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 technology and products. In addition, 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. Furthermore, 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. As a result, the inventorship or ownership of our intellectual property may be challenged in the future. Pending and future patent applications may not result in patents being issued which protect our business, in whole or in part, or which effectively prevent others from commercializing competitive products. Our issued patents or any patents that may issue in the future may be invalidated or interpreted narrowly, such that they fail to provide us with any significant competitive advantage. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the United States. For example, patent laws in various jurisdictions, including significant commercial markets such as Europe, restrict the patentability of methods of treatment of the human body more than U. S. law does. Issued patents that we have or may obtain, or license may not provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors may seek to market generic versions of any approved products by submitting ANDAs to the FDA in which they claim that patents owned or licensed by us are invalid, unenforceable or not infringing. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid or unenforceable or find that our competitors are competing in a non-infringing manner. Thus, even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives. Pursuant to the terms of some of our license agreements with third parties, some of our third-party licensors have the right, but not the obligation in certain circumstances to control enforcement of our licensed patents or defense of any claims asserting the invalidity of these patents. Even if we are permitted to pursue such enforcement or defense, we will require the cooperation of our licensors, and cannot guarantee that we would receive it and on what terms. We cannot be certain that our licensors will allocate sufficient resources or prioritize their or our enforcement of such patents or defense of such claims to protect our interests in the licensed patents. If we cannot obtain patent protection, or enforce existing or future patents against third parties, our competitive position and our financial condition could suffer. If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be negatively impacted, and our business would be harmed. In addition to the protection afforded by patents, we also rely on trade secret protection for certain aspects of our intellectual property. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, consultants, independent contractors, advisors, contract manufacturers, suppliers and other third parties. We also enter into confidentiality and invention or patent assignment agreements with employees and certain consultants. Any party with whom we have executed such an agreement may breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. Further, 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 third party, or those to whom they communicate such technology or information, from using that technology or

information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our business and competitive position could be harmed. We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful. Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent's claims narrowly or 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 the invention. An adverse outcome in a litigation or proceeding involving one or more of our patents could limit our ability to assert those patents against those parties or other competitors and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Similarly, 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. Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could adversely affect the price of shares of our common stock. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings. If we are sued for infringing intellectual property rights of third parties, such litigation could be costly and time consuming and could prevent or delay us from developing or commercializing our product candidates. Our commercial success depends, in part, on our ability to develop, manufacture, market and sell our products without infringing the intellectual property and other proprietary rights of third parties. Third parties may have U. S. and non- U. S. issued patents and pending patent applications relating to compounds and methods of use for the treatment of the disease indications for which we are developing our products or product candidates or relating to the use of complement inhibition that may cover our product candidates or approach to complement inhibition. For example, we are aware of a U. S. patent with claims that could be construed to cover pegcetacoplan. Although we believe that these claims, if construed to cover pegcetacoplan, would be invalid due to various prior art disclosures available more than a year before the priority date of the U. S. patent, there are no assurances that a court would agree. If any third- party patents or patent applications are found to cover our products or product candidates or their methods of use or our approach to complement inhibition, we may not be free to manufacture or market our products or product candidates as planned without obtaining a license, which may not be available on commercially reasonable terms, or at all. There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings regarding intellectual property rights with respect to our products or products candidates, including interference proceedings before the USPTO. 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 our products or product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may be accused of infringing. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Accordingly, third parties may assert infringement claims against us based on existing or future intellectual property rights. The outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance. The pharmaceutical and biotechnology industries have produced a significant number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we were sued for patent infringement, we would need to demonstrate that our product candidates, 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 this. Proving invalidity is difficult. For example, in the United States, proving invalidity 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 the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could significantly harm our business and operating results. In addition, we may not have sufficient resources to bring these actions to a successful conclusion. 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. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product candidate or product. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non- exclusive, thereby giving our competitors access to the same technologies licensed to us; alternatively, or additionally it could include terms that impede or destroy our ability to compete successfully in the commercial marketplace. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to

have willfully infringed a patent. A finding of infringement could prevent us from commercializing our products or product candidates or force us to cease some of our business operations, which could harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business. Some of our intellectual property that was discovered through government- funded programs may be subject to federal regulation such as “ march- in ” rights, certain reporting requirements, and a preference for U. S. industry. Compliance with such regulations may limit our exclusive rights, subject us to expenditure of resources with respect to reporting requirements and limit our ability to contract with foreign manufacturers. Some of our in- licensed intellectual property with respect to our products and product candidates has been funded in part by the U. S. government and, therefore, would be subject to certain federal regulations pursuant to the Bayh- Dole Act of 1980, or the Bayh- Dole Act. As a result, the U. S. government may have certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh- Dole Act. The “ march- in ” provisions of the Bayh- Dole Act allow the U. S. government under strictly limited circumstances to require the patent owners to grant exclusive, partially exclusive or non- exclusive rights to third parties for intellectual property discovered through the government- funded program. The U. S. government can exercise its march- in rights if it determines that action is necessary because the patent owner fails to achieve practical application of the new invention or because action is necessary to alleviate health concerns or address the safety needs of the public. Intellectual property discovered under the government- funded program is also subject to certain reporting requirements, compliance with which may require us or our licensors to expend substantial resources. Such intellectual property is also subject to a preference for U. S. industry, which may limit our ability to contract with foreign product manufacturers for products covered by such intellectual property. Penn has requested a waiver of the U. S. manufacturing requirement **in early 2021**, but there can be no assurance that such waiver will be granted. Changes to the patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products. As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Recent patent reform legislation in the United States, including the Leahy- Smith America Invents Act, or the America Invents Act, could increase those uncertainties and costs. The America Invents Act was signed into law on September 16, 2011, and many of the substantive changes became effective on March 16, 2013. The America Invents Act reformed U. S. patent law in part by changing the U. S. patent system from a “ first to invent ” system to a “ first inventor to file ” system, expanding the definition of prior art, and developing a post- grant review system. This legislation changes U. S. patent law in a way that may weaken our ability to obtain patent protection in the United States for those applications filed after March 16, 2013. Further, the America Invents Act created new procedures to challenge the validity of issued patents in the United States, including post- grant review and inter partes review proceedings, which some third parties have been using to cause the cancellation of selected or all claims of issued patents of competitors. For a patent with an effective filing date of March 16, 2013 or later, a petition for post- grant review can be filed by a third party in a nine- month window from issuance of the patent. A petition for inter partes review can be filed immediately following the issuance of a patent if the patent has an effective filing date prior to March 16, 2013. A petition for inter partes review can be filed after the nine- month period for filing a post- grant review petition has expired for a patent with an effective filing date of March 16, 2013 or later. Post- grant review proceedings can be brought on any ground of invalidity, whereas inter partes review proceedings can only raise an invalidity challenge based on published prior art and patents. These adversarial actions at the USPTO review patent claims without the presumption of validity afforded to U. S. patents in lawsuits in U. S. federal courts and use a lower burden of proof than used in litigation in U. S. federal courts. Therefore, it is generally considered easier for a competitor or third party to have a U. S. patent invalidated in a USPTO post- grant review or inter partes review proceeding than invalidated in a litigation in a U. S. federal court. If any of our patents are challenged by a third party in such a USPTO proceeding, there is no guarantee that we or our licensors or collaborators will be successful in defending the patent, which would result in a loss of the challenged patent right to us. The U. S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Additionally, there have been recent proposals for additional changes to the patent laws of the United States and other countries that, if adopted, could impact our ability to enforce our patents. 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 future actions by the U. S. Congress, the U. S. courts, the USPTO and the relevant law- making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. We may not be able to enforce our intellectual property rights throughout the world. Filing, prosecuting and defending patents on our products or product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States are less extensive than those in the United States. The requirements for patentability may differ in certain countries, particularly in developing countries; thus, even in countries where we do pursue patent protection, there can be no assurance that any patents will issue with claims that cover our products. Competitors may use our technologies in jurisdictions where we have not pursued and obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we may obtain patent protection, but where patent enforcement is not as strong as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued or licensed patents and any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing. Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. Additionally, laws of some countries outside of the United States and Europe do not afford intellectual property protection to the same extent as the laws of the United States and Europe. Many companies have

encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. The legal systems of some countries, including India, China and other developing countries, do not favor the enforcement of patents and other intellectual property rights. This could make it difficult for us to stop the infringement of our patents or the misappropriation of our other intellectual property rights. For example, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. Consequently, we may not be able to prevent third parties from practicing our inventions in certain countries outside the United States and Europe. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop and market their own products and, further, may export otherwise infringing products to territories where we have patent protection, if our ability to enforce our patents to stop infringing activities is inadequate. 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. Agreements through which we license patent rights may not give us sufficient rights to permit us to pursue enforcement of our licensed patents or defense of any claims asserting the invalidity of these patents (or control of enforcement or defense) of such patent rights in all relevant jurisdictions as requirements may vary. For instance, under the Sobi collaboration, we retain the primary right to prosecute and defend its patent and other intellectual property rights, but Sobi has the primary right to enforce such rights against competitive infringement outside the United States. Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and resources from other aspects of our business. Moreover, such proceedings could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing 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. Furthermore, while we intend to protect our intellectual property rights in major markets for our products, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our products. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate. If we do not obtain patent term extension and data exclusivity for our products or product candidates we may develop, our business may be materially harmed. Depending upon the timing, duration and specifics of any FDA marketing approval of our products or product candidates we may develop, one or more of our U. S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Action of 1984, or Hatch- Waxman Amendments. The Hatch- Waxman Amendments permit a patent term extension 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 14 years from the date of product approval, only one patent may be extended, and the extension only applies to those claims covering the approved drug, a method for using it, or a method for manufacturing it. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents, or otherwise failing 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 the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations and prospects could be materially harmed. We may be subject to claims by third parties asserting that our employees or we have misappropriated their intellectual property or claiming ownership of what we regard as our own intellectual property. Many of our employees, including our senior management, were previously employed at universities or at other biotechnology or pharmaceutical companies, including some which may be competitors or potential competitors. Some of these employees, including each member of our senior management, executed proprietary rights, non-disclosure, non- competition and non- solicitation agreements, or similar agreements, in connection with such previous employment. Although we try to ensure that our employees do not use the proprietary information or know- how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such third party. Litigation may be necessary to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel or sustain damages. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management. In addition, while we typically require our employees, consultants and contractors who may be involved in the 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 develops intellectual property that we regard as our own, which may result in claims by or against us related to the ownership of such intellectual property. If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to our senior management and scientific personnel. Obtaining and maintaining 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. Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and applications are required to be paid to the USPTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and applications. The USPTO and various non- U. S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process and after a patent has issued. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which non-compliance 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, the failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we fail to maintain the patents and patent applications covering our products and product candidates, our competitive position would be adversely affected. If we are unable to obtain licenses from third parties on commercially reasonable terms or fail to comply with our obligations under such agreements, our business could be harmed. It may be necessary for us to use the patented or proprietary technology of third parties to commercialize our products, in which case we would be required to obtain a license from these third parties. If we are unable to license such technology, or if we are forced to license such technology on unfavorable terms, our business could be materially harmed. If we are unable to obtain a necessary license, we may be unable to develop or commercialize the affected products or product candidates, which could materially harm our business and the third parties owning such intellectual property rights could seek either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and / or other forms of compensation. Even if we are able to obtain a license, it may be non-exclusive, which could enable our competitors to obtain access to the same technologies licensed to us. If we fail to comply with our obligations under license agreements, our counterparties may have the right to terminate these agreements, in which event we might not be able to develop, manufacture or market, or may be forced to cease developing, manufacturing or marketing, any product that is covered by these agreements or may face other penalties under such agreements. Such an occurrence could materially adversely affect the value of the product or product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms, cause us to lose our rights under these agreements, including our rights to important intellectual property or technology, or impede, delay or prohibit the further development or commercialization of one or more product candidates that rely on such agreements. Risks Related to Regulatory Approval and Marketing of Our Product Candidates and Other Legal Compliance Matters The regulatory approval process is expensive, time consuming and uncertain and may prevent us or our collaborators such as Sobi from obtaining approvals for the commercialization of pegcetacoplan or any of our product candidates that we develop. As a result, we cannot predict when or if, and in which territories, we, or our collaborators, will obtain marketing approval to commercialize pegcetacoplan or any other product candidate that we develop. The research, testing, manufacturing, labeling, approval, selling, marketing, promotion, and distribution of products are subject to extensive regulation by the FDA and comparable foreign regulatory authorities. We are not permitted to market our product candidates in the United States or in other countries until we, or they, receive approval of an NDA from the FDA or marketing approval from applicable regulatory authorities outside the United States. Our product candidates are in various stages of development and are subject to the risks of failure inherent in drug development. The FDA approved EMPAVELI **for the treatment of PNH** in May 2021 and the EMA approved ASPAVELI **for the treatment of PNH** in December 2021. **EMPAVELI has also been approved in the United Kingdom, Canada, Japan, Saudi Arabia and Australia**. The FDA approved SYFOVRE **for the treatment of GA** in February 2023 **and the Therapeutic Goods Administration of Australia approved SYFOVRE for the treatment of GA in January 2025. We submitted an sNDA for EMPAVELI for the treatment of C3G and IC-MPGN in the first quarter of 2025**. The process of obtaining marketing approvals, both in the United States and abroad, is lengthy, expensive and uncertain. It may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information, including manufacturing information, to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. The FDA or other regulatory authorities may determine that our product candidates are not safe and effective, only moderately effective or have undesirable or unintended side effects, toxicities or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable. In addition, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. For example, in December 2022, with the passage of Food and Drug Omnibus Reform Act, or FDORA, Congress required sponsors to develop and submit a diversity action plan for each Phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. **In June 2024, as mandated by FDORA, the FDA issued draft guidance outlining the general requirements for DAPs. Unlike most guidance documents issued by the FDA, the DAP guidance when finalized will have the force of law because FDORA specifically dictates that the form and manner for submission of DAPs are specified in FDA guidance. On January 27, 2025, in response to an Executive Order issued by President Trump on January 21, 2025, on Diversity, Equity and Inclusion programs, the FDA removed this draft guidance from its website. The implications of this action are not yet known**. Further, in January 2022, the new Clinical Trials Regulation (EU) No 536 / 2014 became effective in the European Union and replaced the prior Clinical Trials Directive 2001 / 20 / EC. This regulation aims at simplifying and streamlining the authorization, conduct and transparency of clinical trials in the European Union. Under the coordinated procedure for the approval of clinical trials, the sponsor of a clinical trial to be conducted in more than one European Union Member State will only be required to submit a single application for approval. The submission will be made through the Clinical Trials Information System, a clinical trials portal overseen by the EMA and available to clinical trial sponsors, competent authorities of the European Union Member States and the public. **Further In addition**, under the Pediatric Research Equity Act, or PREA, an NDA, biologics license application, or BLA, or supplement to an NDA or BLA for certain drugs and biological products must contain data to assess the safety and effectiveness of the drug or biological product in all relevant pediatric subpopulations and to support dosing and administration for each pediatric

subpopulation for which the product is safe and effective, unless the sponsor receives a deferral or waiver from the FDA. A deferral may be granted for several reasons, including a finding that the product or therapeutic candidate is ready for approval for use in adults before pediatric trials are complete or that additional safety or effectiveness data needs to be collected before the pediatric trials begin. The applicable legislation in the European Union also requires sponsors to either conduct clinical trials in a pediatric population in accordance with a Pediatric Investigation Plan approved by the Pediatric Committee of EMA, or to obtain a waiver or deferral from the conduct of these studies by this Committee. For any of our product candidates for which we are seeking regulatory approval in the United States or the European Union, we cannot guarantee that we will be able to obtain a waiver or alternatively complete any required studies and other requirements in a timely manner, or at all, which could result in associated reputational harm and subject us to enforcement action. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we, or our collaborators, ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable. In addition, to the extent that we seek to develop a combination drug-device product for delivery of a product candidate, or we rely on a previously cleared device to deliver a product candidate, we will also be dependent on FDA clearance or approval of such products. **Moreover, principal investigators for our future clinical trials may serve as scientific advisors or consultants to us 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 a 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 study. 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 one or more of our product candidates.** Under our agreement with Sobi, Sobi is responsible for seeking regulatory approval outside the United States for systemic pegcetacoplan. A delay in obtaining or failure to obtain required approvals and clearances could negatively impact our ability or that of our collaborators, including Sobi, to generate revenue from the particular product candidate, which likely would result in significant harm to our financial position and adversely impact our stock price. Failure to obtain marketing approval in foreign jurisdictions would prevent our product candidates from being marketed abroad. Any approval we are granted for our product candidates in the United States would not assure approval of our product candidates in foreign jurisdictions. In order to market and sell EMPAVELI, SYFOVRE, pegcetacoplan in other indications or any of our other products in the European Union and other foreign jurisdictions, we, and our collaborators, such as Sobi, must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The marketing approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, a product must be approved for reimbursement before the product can be approved for sale in that country. We, and our collaborators, such as Sobi, may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may file for marketing approvals but not receive necessary approvals to commercialize our products in any market. For example, in ~~January-December~~ 2024, the ~~Committee for Medicinal Products for Human Use, or CHMP, of the European~~ **Commission rejected** ~~Medicines Agency adopted a negative opinion on the marketing authorization application, or MAA, for SYFOVRE in the European Union.~~ ~~While we are seeking re-examination of the MAA,~~ **after a negative recommendation** ~~we cannot be certain that such re-examination will be successful, and we may be required to conduct additional clinical trials of SYFOVRE~~ **the Committee for Medicinal Products for Human Use, or CHMP**. Because regulators in other jurisdictions, ~~including those in the Access Consortium countries (including the United Kingdom, Switzerland, Australia and Canada)~~ are influenced by decisions of the FDA and the EMA, a negative opinion by the FDA or the EMA may adversely impact the prospects for approval in other jurisdictions. Additionally, we could face heightened risks with respect to obtaining marketing authorization in the United Kingdom as a result of the withdrawal of the United Kingdom from the European Union, commonly referred to as Brexit. The United Kingdom is no longer part of the European Single Market and EU Customs Union. As of January 1, ~~2021-2025~~, the Medicines and Healthcare products Regulatory Agency, or MHRA, ~~became responsible for supervising medicines and medical devices in Great Britain, comprising England, Scotland and Wales under domestic law, whereas under the terms of the Northern Ireland Protocol, Northern Ireland is currently subject to EU rules. The United Kingdom and European Union have however agreed to the Windsor Framework which fundamentally changes the existing system under the Northern Ireland Protocol, including with respect to the regulation of medicinal products in the United Kingdom. Once implemented, the changes introduced by the Windsor Framework will see the MHRA be responsible for approving all medicinal products destined for the UK market (i. e., Great Britain and Northern Ireland).~~ **At the same time, a new international recognition procedure, or IRP, will apply, which intends to facilitate approval of pharmaceutical products in the United Kingdom. The IRP is open to applicants that have already received and an authorization for the same product from one of the MHRA's specified Reference Regulators, or RRs. The RRs notably include EMA and regulators in the European Union / European Economic Area, or EEA, member states for approvals in the European Union centralized procedure and mutual recognition procedure as will well as the FDA (for no longer have any role in approving medicinal products- product destined for Northern Ireland approvals granted in the U.**

S.). However, the concrete functioning of the IRP is currently unclear. Any delay in obtaining, or an inability to obtain, any marketing **approvals** ~~authorizations, as a result of Brexit or otherwise,~~ may force us **or our collaborators** to restrict or delay efforts to seek regulatory approval in the United Kingdom for our product candidates, which could significantly and materially harm our business. In addition, foreign regulatory authorities may change their approval policies and new regulations may be enacted. For instance, the EU pharmaceutical legislation is currently undergoing a complete review process, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the European Commission in November 2020. The European Commission's proposal for revision of several legislative instruments related to medicinal products (potentially reducing the duration of regulatory data protection, revising the eligibility for expedited pathways, etc.) was published on April 26, 2023. The proposed revisions remain to be agreed and adopted by the European Parliament and European Council and the proposals may therefore be substantially revised before adoption, which is not anticipated before early 2026. The revisions may however have a significant impact on the pharmaceutical industry and our business in the long term. We expect that we will be subject to additional risks in commercializing any of our product candidates that receive marketing approval outside the United States, including tariffs, trade barriers and regulatory requirements; economic weakness, including inflation, or political instability in particular foreign economies and markets; compliance with tax, employment, immigration and labor laws for employees living or traveling outside of the United States; foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country; and workforce uncertainty in countries where labor unrest is more common than in the United States. We intend to conduct certain of our clinical trials globally. However, the FDA and other foreign equivalents may not accept data from such trials, in which case our development plans will be delayed, which could materially harm our business. We have conducted and intend to continue conducting certain of our clinical trials globally. The acceptance by the FDA or other regulatory authorities of study data from clinical trials conducted outside their jurisdiction may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U. S. population and U. S. medical practice; (ii) the trials were performed by clinical investigators of recognized competence and pursuant to good clinical practices, or GCP, regulations; and (iii) the data may be considered valid without the need for an on- site inspection by the FDA, or if the FDA considers such inspection to be necessary, the FDA is able to validate the data through an on- site inspection or other appropriate means. In addition, even where the foreign study data are not intended to serve as the sole basis for approval, the FDA will not accept the data as support for an application for marketing approval unless the study is well- designed and well- conducted in accordance with GCP requirements and the FDA is able to validate the data from the study through an onsite inspection if deemed necessary. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials would be subject to the applicable local laws of the foreign jurisdictions where the trials are conducted. There can be no assurance that the FDA or any comparable foreign regulatory authority will accept data from trials conducted outside of the United States or the applicable jurisdiction. If the FDA or any comparable foreign regulatory authority does not accept such data, it would result in the need for additional trials, which could be costly and time- consuming, and which may result in current or future product candidates that we may develop not receiving approval for commercialization in the applicable jurisdiction. Conducting clinical trials outside the United States also exposes us to additional risks, including risks associated with: • additional foreign regulatory requirements; • foreign exchange fluctuations; • compliance with foreign manufacturing, customs, shipment and storage requirements; • cultural differences in medical practice and clinical research; • diminished protection of intellectual property in some countries; and • interruptions or delays in our trials resulting from geopolitical events, such as war or terrorism. We may seek certain designations for our product candidates, including Breakthrough Therapy, Fast Track and Priority Review in the United States, and PRIME (priority medicines) in the European Union, but we might not receive such designations, and even if we do, such designations may not lead to a faster development or regulatory review or approval process. We may seek certain designations for one or more of our product candidates that could expedite review and approval by the FDA. A Breakthrough Therapy product is defined as a product that is intended, alone or in combination with one or more other products, to treat a serious condition, and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For products that have been designated as Breakthrough Therapies, early and frequent interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development. The FDA may also designate a product for Fast Track review if it is intended, whether alone or in combination with one or more other products, for the treatment of a serious or life- threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. For Fast Track products, sponsors may have greater interactions with the FDA and the FDA may initiate review of sections of a Fast Track product's NDA before the application is complete. This rolling review may be available if the FDA determines, after preliminary evaluation of data submitted by the sponsor, that a Fast Track product may be effective. We may also seek a priority review designation for one or more of our product candidates. If the FDA determines that a product candidate intended to treat a serious condition and, if approved, offers a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A priority review designation shortens the goal for the FDA to review an application within six months, rather than the standard review period of ten months. These designations require a sponsor to submit an application for review and approval by the FDA. Accordingly, even if we believe that one of our product candidates meets the criteria for these designations, the FDA may disagree and instead determine not to make such designation. Further, even if we receive a designation, the receipt of such designation for a product candidate may not result in a faster development or regulatory review or approval process compared to products considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualifies for these designations, the

FDA may later decide that the product candidates no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. ~~In the European Union, we may seek PRIME for some of our product candidates in the future. PRIME is a voluntary program launched by the EMA that is aimed at enhancing the scientific and regulatory support for the development and accelerated assessment of new product candidates that target an unmet medical need. PRIME is aimed to offer early and proactive support to sponsors to optimize the generation of robust data on the product's benefits and risks and enable accelerated regulatory assessment of new marketing applications. To be eligible for PRIME, a product candidate must meet the eligibility criteria in respect to its potential to offer a major therapeutic advantage over existing treatments, or benefit patients who do not have any treatment options. The benefits of PRIME include the appointment of a CHMP rapporteur to provide continued support and help to build knowledge ahead of a MAA, early dialogue and scientific advice at key development milestones, and the potential to qualify products for accelerated review, meaning reduction in the review time for an opinion on approvability to be issued earlier in the application process. PRIME enables an applicant to request parallel EMA scientific advice and health technology assessment advice to facilitate timely market access. We may apply for PRIME and it may not be granted. Even if we receive PRIME designation for any of our product candidates, the designation may not result in a materially faster development process, review or approval compared to conventional EMA procedures. Further, obtaining PRIME designation does not assure or increase the likelihood of EMA's grant of a marketing authorization.~~ Where appropriate, we plan to pursue approval from the FDA, EMA or comparable foreign regulatory authorities through the use of accelerated registration pathways. If we are unable to obtain such approval, we may be required to conduct additional preclinical studies or clinical trials beyond those that we contemplate, which could increase the expense of obtaining, and delay the receipt of, necessary marketing approvals. Even if we receive accelerated approval from the FDA, EMA or comparable regulatory authorities, if our confirmatory trials do not verify clinical benefit, or if we do not comply with rigorous post- marketing requirements, the FDA, EMA or such other regulatory authorities may seek to withdraw accelerated approval. Where appropriate, we plan to pursue accelerated development strategies in areas of medical need. We may seek an accelerated approval pathway for one or more of our product candidates from the FDA, EMA or comparable foreign regulatory authorities. Under the accelerated approval provisions in the Federal Food, Drug, and Cosmetic Act, and the FDA's implementing regulations, the FDA may grant accelerated approval to a product candidate designed to treat a serious or life- threatening condition that provides meaningful therapeutic benefit over available therapies upon a determination that the product candidate has an effect on a surrogate endpoint or intermediate clinical endpoint that is reasonably likely to predict clinical benefit. The FDA considers a clinical benefit to be a positive therapeutic effect that is clinically meaningful in the context of a given disease, such as irreversible morbidity or mortality. For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. An intermediate clinical endpoint is a clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit. The accelerated approval pathway may be used in cases in which the advantage of a new drug over available therapy may not be a direct therapeutic advantage but is a clinically important improvement from a patient and public health perspective. If granted, accelerated approval is usually contingent on the sponsor's agreement to conduct, in a diligent manner, additional post- approval confirmatory studies to verify and describe the drug's clinical benefit. If such post- approval studies fail to confirm the drug's clinical benefit, the FDA may withdraw its approval of the drug. **In addition, there can be no assurance that we will satisfy all FDA requirements, including new provisions, that govern accelerated approval. For example, With with** passage of FDORA in December 2022, Congress modified certain provisions governing accelerated approval of drug and biologic products. Specifically, the new legislation authorized the FDA to require a sponsor to have its confirmatory clinical trial underway before accelerated approval is awarded **and** ~~require a sponsor of a product granted accelerated approval to submit progress reports on its post- approval studies to the FDA (every six months (until the study is completed)~~ **Moreover** ~~and use~~ **FDORA established** expedited procedures **authorizing FDA** to withdraw **an** accelerated approval ~~of an NDA or BLA after the~~ **if certain conditions are met, including where a required** confirmatory **trial study** fails to verify **and describe** the **predicted** product's clinical benefit **or where evidence demonstrates** ~~Further, FDORA requires the~~ **product is not shown to be safe or effective under the conditions of use. The** FDA **may also use such procedures to withdraw an accelerated approval if** ~~publish on its website "the rationale for why a~~ **sponsor fails to conduct any required** post- approval study ~~is not appropriate or necessary~~ **of the product with due diligence, including with respect to " conditions specified by the Secretary. "** whenever it decides not to require such ~~The new procedures include the provision of due notice and an explanation for a study upon granting proposed withdrawal, and opportunities for a meeting with the Commissioner or the Commissioner's designee and a written appeal, among other things. We will need to fully comply with these and other requirements in connection with the development and approval of any product candidate that qualifies for~~ accelerated approval. More recently, in March 2023, the FDA issued draft guidance that outlines its current thinking and approach to accelerated approval. The FDA indicated that the accelerated approval pathway is commonly used for approval of oncology drugs due to the serious and life- threatening nature of cancer. Although single- arm trials have been commonly used to support accelerated approval, a randomized controlled trial is the preferred approach as it provides a more robust efficacy and safety assessment and allows for direct comparisons to an available therapy. To that end, the FDA outlined considerations for designing, conducting, and analyzing data for trials intended to support accelerated approvals of oncology therapeutics. **Subsequently, in December 2024 and January 2025, the FDA issued additional draft guidances relating to accelerated approval. These guidances describe FDA's views on what it means to conduct a confirmatory trial with due diligence and how the agency plans to interpret whether such a study needs to be underway at the time of approval.** While ~~this~~ **these guidance guidances is are** currently only in draft form and will **ultimately** not be legally binding even when finalized, we will need to consider the FDA's ~~guidance guidances~~ **guidance guidances** if we seek accelerated approval for any of our products in

the future. ~~In the European Union, a “conditional” marketing authorization may be granted in cases where all the required safety and efficacy data are not yet available. A conditional marketing authorization is subject to conditions to be fulfilled for generating missing data or ensuring increased safety measures. A conditional marketing authorization is valid for one year and has to be renewed annually until fulfillment of all relevant conditions. Once the applicable pending studies are provided, a conditional marketing authorization can become a “standard” marketing authorization. However, if the conditions are not fulfilled within the timeframe set by the EMA, the marketing authorization will cease to be renewed.~~ Prior to seeking accelerated approval, we will seek feedback from the FDA, EMA or comparable foreign regulatory authorities and will otherwise evaluate our ability to seek and receive such accelerated approval. There can be no assurance that after our evaluation of the feedback and other factors we will decide to pursue or submit an NDA for accelerated approval or any other form of expedited development, review or approval. Similarly, there can be no assurance that after subsequent feedback from the FDA, EMA or comparable foreign regulatory authorities, we will continue to pursue or apply for accelerated approval or any other form of expedited development, review or approval, even if we initially decide to do so. Furthermore, if we decide to submit an application for accelerated approval or under another expedited regulatory designation (i. e., Fast Track designation, Breakthrough Therapy designation or orphan drug designation), there can be no assurance that such submission or application will be accepted or that any expedited development, review or approval will be granted on a timely basis, or at all. The FDA, EMA or other comparable foreign regulatory authorities could also require us to conduct further studies prior to considering our application or granting approval of any type. A failure to obtain accelerated approval or any other form of expedited development, review or approval for our product candidate would result in a longer time period to commercialization of such product candidate, could increase the cost of development of such product candidate and could harm our competitive position in the marketplace. We, or our collaborators, may not be able to obtain orphan drug designation or orphan drug exclusivity for our product candidates and, even if we do, that exclusivity may not prevent the FDA or the EMA from approving other competing products. Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200, 000 individuals annually in the United States. The FDA has granted orphan drug designation to pegcetacoplan for the treatment of PNH and for the treatment of C3 glomerulopathy. We, or our collaborators, may seek orphan drug designations for pegcetacoplan for other indications and for other product candidates and may be unable to obtain such designations. Even if we, or our collaborators, obtain orphan drug designation for a product candidate, such as is the case for pegcetacoplan for the treatment of PNH **and C3G**, we, or they, may not be able to obtain orphan drug exclusivity for that product candidate. Generally, a product with orphan drug designation only becomes entitled to orphan drug exclusivity if it receives the first marketing approval for the indication for which it has such designation, in which case the FDA or the EMA will be precluded from approving another marketing application for the same drug for that indication for the applicable exclusivity period. The applicable exclusivity period is seven years in the United States and ten years in Europe. The European exclusivity period can be reduced to six years if 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 or the EMA 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 disease or condition. Even if we, or our collaborators, obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. The FDA and Congress may further reevaluate the Orphan Drug Act and its regulations and policies. This may be particularly true in light of a decision from the Court of Appeals for the 11th Circuit in September 2021 finding that, for the purpose of determining the scope of exclusivity, the term “same disease or condition” means the designated “rare disease or condition” and could not be interpreted by the FDA to mean the “indication or use.” Thus, the court concluded, orphan drug exclusivity applies to the entire designated disease or condition rather than the “indication or use.” Although there have been legislative proposals to overrule this decision, they have not been enacted into law. On January 23, 2023, the FDA announced that, in matters beyond the scope of that court order, the FDA will continue to apply its existing regulations tying orphan- drug exclusivity to the uses or indications for which the orphan drug was approved. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted. The FDA, EMA and other regulatory authorities actively enforce the laws and regulations prohibiting the promotion of off- label uses. If any of our product candidates are approved and we are found to have improperly promoted off- label uses of those products, we may become subject to significant liability. The FDA, EMA and other regulatory authorities strictly regulate the promotional claims that may be made about prescription products, such as our product candidates, if approved. In particular, a product may not be promoted in the United States for uses that are not approved by the FDA as reflected in the product’ s approved labelling, or in other jurisdictions for uses that differ from the labelling or uses approved by the applicable regulatory authorities. While physicians may prescribe products for off- label uses, the FDA, EMA and other regulatory authorities actively enforce laws and regulations that prohibit the promotion of off- label uses by companies, including promotional communications made by companies’ sales force with respect to off- label uses that are not consistent with the approved labelling, and a company that is found to have improperly promoted off- label uses may be subject to significant civil, criminal and administrative penalties. Notwithstanding the regulatory restrictions on off- label promotion, the FDA and other regulatory authorities allow companies to engage in truthful, non- misleading, and non- promotional scientific communications concerning their products in certain circumstances. For example, in October 2023, the FDA published

draft guidance outlining the agency's non-binding policies governing the distribution of scientific information on unapproved uses to healthcare providers. This draft guidance calls for such communications to be truthful, non-misleading, factual, and unbiased and include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use. In addition, under some relatively recent guidance from the FDA and the Pre-Approval Information Exchange Act, or PIE Act, signed into law as part of the Consolidated Appropriations Act of 2023, companies may also promote information that is consistent with the prescribing information and proactively speak to formulary committee members of payors regarding data for an unapproved drug or unapproved uses of an approved drug. We may engage in these discussions and communicate with healthcare providers, payors and other constituencies in compliance with all applicable laws, regulatory guidance and industry best practices. We will need to carefully navigate the FDA's various regulations, guidance and policies, along with recently enacted legislation, to ensure compliance with restrictions governing promotion of our products. **We will also need to observe the FDA's various regulations, guidance and policies, along with recently enacted legislation, to ensure compliance with restrictions governing promotion of our products. In September 2021, the FDA published final regulations which describe the types of evidence that the Agency will consider in determining the intended use of a drug or biologic. In addition, in January 2025, the FDA published final guidance outlining its policies governing the distribution of scientific information to healthcare providers about unapproved uses of approved products. The final guidance calls for such communications to be truthful, non-misleading and scientifically sound and to include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use of the approved product. If a company engages in such communications consistent with the guidance's recommendations, the FDA indicated that it will not treat such communications as evidence of unlawful promotion of a new intended use for the approved product.** If we are found to have promoted such off-label uses, we may become subject to significant liability. The federal government has levied large civil and criminal fines against companies for alleged improper promotion of off-label use and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition. Even if we, or our collaborators, obtain marketing approvals for our product candidates, the terms of approvals and ongoing regulation of our products may limit how we manufacture and market our products, which could impair our ability to generate revenue. Once marketing approval has been granted, an approved product and its manufacturer and marketer are subject to ongoing review and extensive regulation. We, and our collaborators, must therefore comply with requirements concerning advertising and promotion for any of our product candidates which we or they market. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we and our collaborators will not be able to promote any products we develop for indications or uses for which they are not approved. We are limited to promoting EMPAVELI and SYFOVRE in accordance with their approved label in each jurisdiction and may not promote them for any indication other than as stated on the label. The label for Aspaveli in the European Union is more limited than the label for EMPAVELI in the United States. EMPAVELI, SYFOVRE, and any other product candidates for which we, or our collaborators, obtain marketing approval in the future could be subject to post-marketing restrictions or withdrawal from the market and we, or our collaborators, may be subject to substantial penalties if we, or they, fail to comply with regulatory requirements or if we, or they, experience unanticipated problems with our products following approval. EMPAVELI, SYFOVRE, and any other product candidates for which we, or our collaborators, obtain marketing approval, as well as the manufacturing processes, post-approval studies and measures, labeling, advertising and promotional activities for such product, among other things, will be subject to ongoing requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. For EMPAVELI, SYFOVRE, and any other product candidate that is granted marketing approval, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a Risk Evaluation and Mitigation Strategy. In addition, manufacturers of approved products and those manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to cGMPs, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, our contract manufacturers, our collaborators and their contract manufacturers could be subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with cGMPs. The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. The FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we, or our collaborators, do not market any of our product candidates for which we, or they, receive marketing approval for only their approved indications, we, or they, may be subject to warnings or enforcement action for off-label marketing. Violation of the FDCA and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws. In addition, later discovery of previously unknown adverse events or other problems with our products or their manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such products,

manufacturers or manufacturing processes; • restrictions on the labeling or marketing of a product; • restrictions on product distribution or use; • requirements to conduct post- marketing studies or clinical trials; • warning letters or untitled letters; • withdrawal of the products from the market; • refusal to approve pending applications or supplements to approved applications that we submit; • recall of products; • restrictions on coverage by third- party payors; • fines, restitution or disgorgement of profits or revenues; • suspension or withdrawal of marketing approvals; • refusal to permit the import or export of products; • product seizure; or • injunctions or the imposition of civil or criminal penalties. ~~In addition, our ability to maintain approval and market our new drug products may be impacted by ongoing litigation challenging the FDA's approval of mifepristone.~~ Specifically, on April 7, 2023, the U. S. District Court for the Northern District of Texas stayed the approval by the FDA of mifepristone, a drug product which was originally approved in 2000 and whose distribution is governed by various conditions adopted under a Risk Evaluation and Mitigation Strategies, or REMS. In reaching that decision, the district court made a number of findings that may negatively impact the development, approval and distribution of drug products in the U. S. Among other determinations, the district court held that plaintiffs were likely to prevail in their claim that FDA had acted arbitrarily and capriciously in approving mifepristone without sufficiently considering evidence bearing on whether the drug was safe to use under the conditions identified in its labeling. Further, the district court read the standing requirements governing litigation in federal court as permitting a plaintiff to bring a lawsuit against the FDA in connection with its decision to approve an NDA or establish requirements under a REMS based on a showing that the plaintiff or its members would be harmed to the extent that FDA's drug approval decision effectively compelled the plaintiffs to provide care for patients suffering adverse events caused by a given drug. On April 12, 2023, the district court decision was stayed, in part, by the U. S. Court of Appeals for the Fifth Circuit. Thereafter, on April 21, 2023, the U. S. Supreme Court entered a stay of the district court's decision, in its entirety, pending disposition of the appeal of the district court decision in the Court of Appeals for the Fifth Circuit and the disposition of any petition for a writ of certiorari to or the Supreme Court. The Court of Appeals for the Fifth Circuit held oral argument in the case on May 17, 2023 and, on August 16, 2023, issued its decision. The court declined to order the removal of mifepristone from the market, finding that a challenge to the FDA's initial approval in 2000 is barred by the statute of limitations. But the Appeals Court did hold that plaintiffs were likely to prevail in their claim that changes allowing for expanded access of mifepristone that FDA authorized in 2016 and 2021 were arbitrary and capricious. On September 8, 2023, the Justice Department and a manufacturer of mifepristone filed petitions for a writ of certiorari, requesting that asked the U. S. Supreme Court to review the Appeals Court decision. On December 13, 2023, the Supreme Court granted these petitions for writ of certiorari for the appeals court decision. If we, and our collaborators, are not able to comply with post- approval regulatory requirements, we, and our collaborators, could have the marketing approvals for our products withdrawn by regulatory authorities and our, or our collaborators', ability to market any future products **for which we receive marketing approval** could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition. **There is substantial uncertainty as to how, if at all, the Trump administration will seek to modify or revise the requirements and policies of the FDA and other regulatory agencies with jurisdiction over our product candidates. The impending uncertainty could present new challenges or potential opportunities as we navigate the clinical development and approval process for our product candidates**. Inadequate funding for the FDA, the SEC and other government agencies, including from government shut downs, or other disruptions to these agencies' operations, could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business. The ability of the FDA 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 agency have fluctuated in recent years as a result. Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and / or approved by necessary government agencies, which would adversely affect our business. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable. Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and / or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years the U. S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical 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. Further, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations. In addition, disruptions may still result also from the recent COVID- 19 pandemic or any similar event that may occur in the future. During the recent COVID- 19 pandemic, a number of companies announced receipt of complete response letters due to the FDA's inability to complete required inspections for their applications. In the event of a ~~resurgence of the recent COVID-19 pandemic or a~~ similar public health emergency in the future, the FDA may not be able to continue its current pace and review timelines could be extended. Regulatory authorities outside the United States facing similar circumstances may adopt similar restrictions or other policy measures in response to ~~the recent COVID-19 pandemic or~~ a similar public health emergency and may also experience delays in their regulatory activities. If a prolonged government shutdown or other disruption 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. Future shutdowns or other disruptions could also affect other government agencies such as the SEC, which may also impact our business by delaying review of our public filings, to the extent such review is necessary, and our ability to access the public markets. Current and

future legislation may increase the difficulty and cost for us and our collaborators to obtain reimbursement of and commercialize our product candidates and affect the prices we, or they, may obtain. In the United States and some foreign jurisdictions, there have been and continue to be a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of our collaborators, to profitably sell EMPAVELI, SYFOVRE, or any other products for which we, or they, obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or our collaborators, may receive for any approved products. If reimbursement of our products is unavailable or limited in scope, our business could be materially harmed. In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care Education Reconciliation Act, or collectively the ACA. Since enactment of the ACA, there have been and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Act in 2017, Congress repealed the “individual mandate.” The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. On June 17, 2021, the U. S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results. Other legislative changes have been adopted since the ACA was enacted, including aggregate reductions to Medicare payments to providers of up to 2 % per fiscal year, which went into effect in April 2013 and will remain in effect through 2031. Under current legislation, the actual reductions in Medicare payments may vary up to 4 %. The Consolidated Appropriations Act, or CAA, which was signed into law by President Biden in December 2022, made several changes to sequestration of the Medicare program. Section 1001 of the CAA delays the 4 % Statutory Pay-As-You-Go Act of 2010, or PAYGO, sequester for two years, through the end of calendar year 2024. Triggered by the enactment of the American Rescue Plan Act of 2021, the 4 % cut to the Medicare program would have taken effect in January 2023. The CAA’s health care offset title includes Section 4163, which extends the 2 % Budget Control Act of 2011 Medicare sequester for six months into fiscal year 2032 and lowers the payment reduction percentages in fiscal years 2030 and 2031. The Trump Administration also took executive actions to undermine or delay implementation of the ACA, including directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers or manufacturers of pharmaceuticals or medical devices. On January 28, 2021, however, President Biden revoked those Orders and issued a new Executive Order which directs federal agencies to reconsider rules and other policies that limit Americans’ access to health care and consider actions that will protect and strengthen that access. Under this Order, federal agencies are directed to re-examine policies that undermine protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the ACA that may reduce coverage or undermine the programs, including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and the ACA; and policies that reduce affordability of coverage or financial assistance, including for dependents. In the European Union, on December 13, 2021, Regulation No 2021/2282 on Health Technology Assessment, or HTA, amending Directive 2011/24/EU, was adopted. While the Regulation entered into force in January 2022, it will only begin to apply from January 2025 onwards, with preparatory and implementation-related steps to take place in the interim. Once applicable, it will have a phased implementation depending on the concerned products. The Regulation intends to boost cooperation among EU member states in assessing health technologies, including new medicinal products as well as certain high-risk medical devices, and provide the basis for cooperation at the EU level for joint clinical assessments in these areas. It will permit EU member states to use common health technology assessment, or HTA, tools, methodologies, and procedures across the European Union, working together in four main areas, including joint clinical assessment of the innovative health technologies with the highest potential impact for patients; joint scientific consultations whereby developers can seek advice from HTA authorities; identification of emerging health technologies to identify promising technologies early, and continuing voluntary cooperation in other areas. Individual EU member states will continue to be responsible for assessing non-clinical (e. g., economic, social, ethical) aspects of health technology, and making decisions on pricing and reimbursement. The prices of prescription pharmaceuticals in the United States and foreign jurisdictions are subject to considerable legislative and executive actions and could impact the prices we obtain for our products, if and when approved. The prices of prescription pharmaceuticals have also been the subject of considerable discussion in the United States. There have been several recent U. S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to pharmaceutical pricing, review the relationship between pricing and manufacturer patient programs, and reduce the costs of pharmaceuticals under Medicare and Medicaid. In August 2020, President Trump issued several executive orders intended to lower the costs of prescription products and certain provisions in these orders have been incorporated into regulations. These regulations include an interim final rule implementing a most favored nation model for prices that would tie Medicare Part B payments for certain physician-administered pharmaceuticals to the lowest price paid in other economically advanced countries, effective January 1, 2021. That rule, however, has been subject to a nationwide preliminary injunction and, on December 29, 2021, CMS issued a final rule to rescind it. With issuance of this rule, CMS stated that it will explore all options to incorporate value into payments for Medicare Part B pharmaceuticals and improve beneficiaries’ access to evidence-based care. In addition, in October 2020, HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program, or SIP, to import certain prescription drugs from Canada into the United States. That regulation was challenged in a lawsuit by the Pharmaceutical Research and Manufacturers of America, or PhRMA, but the case was dismissed by a federal district court in February 2023 after the court found that PhRMA did not have standing to sue HHS. Nine states have passed laws allowing for

the importation of drugs from Canada. Certain of these states have submitted Section 804 Importation Program proposals and are awaiting FDA approval. On January 5, 2023, the FDA approved Florida's plan for Canadian drug importation. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a new safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers, the implementation of which has been delayed until January 1, 2032 by the Inflation Reduction Act of 2022, or IRA. On July 9, 2021, President Biden signed Executive Order 14063, which focuses on, among other things, the price of pharmaceuticals. The Order directs HHS to create a plan within 45 days to combat "excessive pricing of prescription pharmaceuticals and enhance domestic pharmaceutical supply chains, to reduce the prices paid by the federal government for such pharmaceuticals, and to address the recurrent problem of price gouging." On September 9, 2021, HHS released its plan to reduce pharmaceutical prices. The key features of that plan are to: (a) make pharmaceutical prices more affordable and equitable for all consumers and throughout the health care system by supporting pharmaceutical price negotiations with manufacturers; (b) improve and promote competition throughout the prescription pharmaceutical industry by supporting market changes that strengthen supply chains, promote biosimilars and generic drugs, and increase transparency; and (c) foster scientific innovation to promote better healthcare and improve health by supporting public and private research and making sure that market incentives promote discovery of valuable and accessible new treatments. On August 16, the IRA, was signed into law by President Biden. The new legislation has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single- source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high- cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Part D drugs in 2027, 15 Part B or Part D drugs in 2028, and 20 Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least nine years and biologics that have been licensed for 13 years, but it does not apply to drugs and biologics that have been approved for a single rare disease or condition. Nonetheless, since CMS may establish a maximum price for these products in price negotiations, we would be fully at risk of government action if our products are the subject of Medicare price negotiations. Moreover, given the risk that could be the case, these provisions of the IRA may also further heighten the risk that we would not be able to achieve the expected return on our drug products or full value of our patents protecting our products if prices are set after such products have been on the market for nine years. Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated \$ 4, 000 a year in 2024 and, thereafter beginning in 2025, at \$ 2, 000 a year. **On August 15, 2024, HHS published the results of the first Medicare drug price negotiations for ten selected drugs. On January 17, 2025, CMS announced its selection of 15 additional drugs covered by Part D for the second cycle of negotiations.** On June 6, 2023, Merck & Co. filed a lawsuit against the HHS and CMS asserting that, among other things, the IRA's Drug Price Negotiation Program for Medicare constitutes an uncompensated taking in violation of the Fifth Amendment of the Constitution. Subsequently, a number of other parties also filed lawsuits in various courts with similar constitutional claims against the HHS and CMS. **We expect that HHS has generally won the substantive disputes in these cases. Certain of these cases are now on appeal. Litigation** involving these and other provisions of the IRA will continue, with unpredictable and uncertain results. **Accordingly, while it is currently unclear how the IRA will be effectuated, we cannot predict with certainty what impact any federal or state health reforms will have on us, but such changes could impose new or more stringent regulatory requirements on our activities or result in reduced reimbursement for our products, any of** At the state level, individual states are increasingly aggressive in passing legislation and implementing 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. **This is increasingly true with respect to products approved pursuant to the accelerated approval pathway. State Medicaid programs and other payers are developing strategies and implementing significant coverage barriers, or refusing to cover these products outright, arguing that accelerated approval drugs have insufficient or limited evidence despite meeting the FDA's standards for accelerated approval.** In addition, regional health care organizations 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 health care programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures. **On June 6, 2023,..... results of operations, and financial condition.** In addition, in some countries, including member states of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take a significant amount of time after receipt of marketing approval for a product. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic, and regulatory developments may further complicate

pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various EU member states and parallel distribution, or arbitrage between low- priced and high- priced member states, can further reduce prices, and in certain instances render commercialization in certain markets infeasible or disadvantageous from a financial perspective. In some countries, we or our collaborators may be required to conduct a clinical trial or other studies that compare the cost- effectiveness of our product and / or our product candidates to other available products in order to obtain or maintain reimbursement or pricing approval. Publication of discounts by third party payors or government authorities may lead to further pressure on the prices or reimbursement levels. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, the commercial launch of our product and / or product candidates could be delayed, possibly for lengthy periods of time, we or our collaborators may not launch at all in a particular country, we may not be able to recoup our investment in one or more product candidates, and there could be a material adverse effect on our business. Our relationships with customers and third- party payors, among others, will be subject to applicable anti- kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to penalties, including criminal sanctions, civil penalties, contractual damages, reputational harm, fines, disgorgement, exclusion from participation in government healthcare programs, curtailment or restricting of our operations, and diminished profits and future earnings. Healthcare providers, physicians and third- party payors will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our current and future arrangements with healthcare providers, and third- party payors and customers, if any, will subject us to broadly applicable fraud and abuse and other healthcare laws and regulations. The laws and regulations may constrain the business or financial arrangements and relationships through which we conduct clinical research, market, sell and distribute any products for which we obtain marketing approval. These include the following: Anti- Kickback Statute. The federal Anti- Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration (including any kickback, bribe or rebate), directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease or order of a good, facility, item or service for which payment may be made under a federal healthcare program such as Medicare and Medicaid; False Claims Laws. The federal false claims and civil monetary penalties laws, including the federal civil False Claims Act, impose criminal and civil penalties, including through civil whistleblower or qui tam actions against individuals or entities for, among other things, knowingly presenting or causing to be presented false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per- claim penalties; HIPAA. The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for, among other things, executing a scheme, or making materially false statements in connection with the delivery of or payment for health care benefits, items, or services. Additionally, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations on covered entities and their business associates that perform certain functions or activities that involve the use or disclosure of protected health information on their behalf, including mandatory contractual terms and technical safeguards, with respect to maintaining the privacy, security and transmission of individually identifiable health information; Transparency Requirements. The federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics, and medical supplies for which payment is available under Medicare, Medicaid, or the Children’s Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to payments or transfers of value made to certain healthcare providers and teaching hospitals, as well as information regarding ownership and investment interests held by physicians and their immediate family members; Analogous State and Foreign Laws. Analogous state and foreign fraud and abuse laws and regulations, such as state anti- kickback and false claims laws, can apply to sales or marketing arrangements, and claims involving healthcare items or services reimbursed by non- governmental third- party payors, and are generally broad and are enforced by many different federal and state agencies as well as through private actions. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. Additionally, some state and local laws require the registration of pharmaceutical sales representatives in the jurisdiction. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not pre- emptied by HIPAA, thus complicating compliance efforts. Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, imprisonment and the curtailment or restructuring of our operations, any of which could adversely affect our business, financial condition, results of operations and prospects. The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the European Union. The provision of benefits or advantages to physicians is also governed by the national anti- bribery laws of European Union Member States, such as the U. K. Bribery Act 2010. Violation of these laws could result in substantial fines and imprisonment. Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician’s employer, his or her competent professional organization and / or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct applicable in the European Union Member States. Failure to comply with

these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment. Efforts to ensure that our business arrangements with third parties, and our business generally, will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, individual imprisonment, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm, and the curtailment or restructuring of our operations. Defending against any such actions can be costly, time-consuming and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. Further, if any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Liabilities they incur pursuant to these laws could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations and prospects. With the passage of the CREATES Act, we are exposed to possible litigation and damages by competitors who may claim that we are not providing sufficient quantities of our approved products on commercially reasonable, market-based terms for testing in support of their ANDAs and 505 (b) (2) applications. In December 2019, ~~former~~ President Trump signed legislation intended to facilitate the development of generic and biosimilar products. The bill, previously known as the CREATES Act, authorizes sponsors of abbreviated new drug applications, or ANDAs, and 505 (b) (2) applications to file lawsuits against companies holding NDAs that decline to provide sufficient quantities of an approved reference drug on commercially reasonable, market-based terms. Drug products on FDA's drug shortage list are exempt from these new provisions unless the product has been on the list for more than six continuous months, or the FDA determines that the supply of the product will help alleviate or prevent a shortage. To bring an action under the statute, an ANDA or 505 (b) (2) sponsor must take certain steps to request the reference product, which, in the case of products covered by a Risk Evaluation and Mitigation Strategy with elements to assure safe use, include obtaining authorization from the FDA for the acquisition of the reference product. If the sponsor does bring an action for failure to provide a reference product, there are certain affirmative defenses available to the NDA holder, which must be shown by a preponderance of evidence. If the sponsor prevails in litigation, it is entitled to a court order directing the NDA holder to provide, without delay, sufficient quantities of the applicable product on commercially reasonable, market-based terms, plus reasonable attorney fees and costs. Additionally, the new statutory provisions authorize a federal court to award the product developer an amount "sufficient to deter" the NDA holder from refusing to provide sufficient product quantities on commercially reasonable, market-based terms if the court finds, by a preponderance of the evidence, that the NDA holder did not have a legitimate business justification to delay providing the product or failed to comply with the court's order. For the purposes of the statute, the term "commercially reasonable, market-based terms" is defined as (1) the nondiscriminatory price at or below the most recent wholesale acquisition cost for the product, (2) a delivery schedule that meets the statutorily defined timetable, and (3) no additional conditions on the sale. Although we intend to comply fully with the terms of these new statutory provisions, we are still exposed to potential litigation and damages by competitors who may claim that we are not providing sufficient quantities of our approved products on commercially reasonable, market-based terms for testing in support of ANDAs and 505 (b) (2) applications. Such litigation would subject us to additional litigation costs, damages and reputational harm, which could lead to lower revenues. The CREATES Act may enable generic competition with EMPAVELI, SYFOVRE, and any of our product candidates, if approved, which could impact our ability to maximize product revenue. Compliance with global privacy and data security requirements could result in additional costs and liabilities to us or inhibit our ability to collect and process data globally, and the failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations. We are subject to data privacy and protection laws and regulations that apply to the collection, transmission, storage and use of personally-identifying information, which among other things, impose certain requirements relating to the privacy, security and transmission of personal information, including comprehensive regulatory systems in the United States, European Union and United Kingdom. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. Failure to comply with any of these laws and regulations could result in enforcement action against us, including fines, claims for damages by affected individuals, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects. There are numerous U. S. federal and state laws and regulations related to the privacy and security of personal information. In particular, regulations promulgated pursuant to HIPAA establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information, and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information and ensure the confidentiality, integrity and availability of electronic protected health information. Determining whether protected health information has been handled in compliance with applicable privacy standards and our contractual obligations can be complex and may be subject to changing interpretation. These obligations may be applicable to some or all of our business activities now or in the future. If we are unable to properly protect the privacy and security of protected health information, we could be found to have breached our contracts. Further, if we fail to comply with applicable privacy laws, including applicable HIPAA privacy and security standards, we could face civil and criminal penalties. HHS enforcement activity can result in financial liability and reputational harm, and responses to such enforcement activity can

consume significant internal resources. In addition, state attorneys general are authorized to bring civil actions seeking either injunctions or damages in response to violations that threaten the privacy of state residents. We cannot be sure how these regulations will be interpreted, enforced or applied to our operations. In addition to the risks associated with enforcement activities and potential contractual liabilities, our ongoing efforts to comply with evolving laws and regulations at the federal and state level may be costly and require ongoing modifications to our policies, procedures and systems. ~~In 2018, California passed into law the California Consumer Privacy Act, or the CCPA, which took effect on January 1, 2020 and imposed many requirements on businesses that process the personal information of California residents. Many of the CCPA's requirements are similar to those found in the General Data Protection Regulation, or GDPR, including requiring businesses to provide notice to data subjects regarding the information collected about them and how such information is used and shared, and providing data subjects the right to request access to such personal information and, in certain cases, request the erasure of such personal information. The CCPA also affords California residents the right to opt-out of "sales" of their personal information. The CCPA contains significant penalties for companies that violate its requirements. In November 2020, California voters passed a ballot initiative for the California Privacy Rights Act, or the CPRA, which went into effect on January 1, 2023 and significantly expanded the CCPA to incorporate additional GDPR-like provisions including requiring that the use, retention, and sharing of personal information of California residents be reasonably necessary and proportionate to the purposes of collection or processing, granting additional protections for sensitive personal information, and requiring greater disclosures related to notice to residents regarding retention of information. The CPRA also created a new enforcement agency—the California Privacy Protection Agency—whose sole responsibility is to enforce the CPRA, which will further increase compliance risk. The provisions in the CPRA may apply to some of our business activities. In addition to California, at least eleven other states have passed comprehensive privacy laws similar to the CCPA and CPRA. These laws are either in effect or will go into effect sometime before the end of 2026. Like the CCPA and CPRA, these laws create obligations related to the processing of personal information, as well as special obligations for the processing of "sensitive" data, which includes health data in some cases. Some of the provisions of these laws may apply to our business activities. There are also states that are strongly considering or have already passed comprehensive privacy laws during the 2023 legislative sessions that will go into effect in 2024 and beyond, including New Hampshire and New Jersey. Other states will be considering similar laws in the future, and Congress has also been debating passing a federal privacy law. There are also states that are specifically regulating health information that may affect our business. For example, Washington state passed a health privacy law in 2023 that will regulate the collection and sharing of health information, and the law also has a private right of action, which further increases the relevant compliance risk. Connecticut and Nevada have also passed similar laws regulating consumer health data and additional states are considering such legislation for 2024. These laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products. Similar to the laws in the United States, there are significant privacy and data security laws that apply in Europe and other countries. The collection, use, disclosure, transfer, or other processing of personal data, including personal health data, regarding individuals who are located in the EEA, and the processing of personal data that takes place in the EEA, is regulated by the GDPR, which went into effect in May 2018 and which imposes obligations on companies that operate in our industry with respect to the processing of personal data and the cross-border transfer of such data. The GDPR imposes onerous accountability obligations requiring data controllers and processors to maintain a record of their data processing and policies. If our or our partners' or service providers' privacy or data security measures fail to comply with the GDPR requirements, we may be subject to litigation, regulatory investigations, enforcement notices requiring us to change the way we use personal data and / or fines of up to 20 million Euros or up to 4 % of the total worldwide annual turnover of the preceding financial year, whichever is higher, as well as compensation claims by affected individuals, negative publicity, reputational harm and a potential loss of business and goodwill. The GDPR places restrictions on the cross-border transfer of personal data from the European Union to countries that have not been found by the EC to offer adequate data protection legislation, such as the United States. There are ongoing concerns about the ability of companies to transfer personal data from the EU to other countries. In July 2020, the Court of Justice of the European Union, or the CJEU, invalidated the EU-U.S. Privacy Shield, one of the mechanisms used to legitimize the transfer of personal data from the EEA to the U.S. The CJEU decision also drew into question the long-term viability of an alternative means of data transfer, the standard contractual clauses, for transfers of personal data from the EEA to the U.S. While we were not self-certified under the Privacy Shield, this CJEU decision may lead to increased scrutiny on data transfers from the EEA to the U.S. generally and increase our costs of compliance with data privacy legislation as well as our costs of negotiating appropriate privacy and security agreements with our vendors and business partners.~~ ~~In October 2022, President Biden signed an executive order to implement the EU-U.S. Data Privacy Framework, which serves as a replacement to the EU-U.S. Privacy Shield. The European Commission adopted the adequacy decision on July 10, 2023. The adequacy decision permits U.S. companies who self-certify to the EU-U.S. Data Privacy Framework to rely on it as a valid data transfer mechanism for data transfers from the European Union to the United States. However, some privacy advocacy groups have already suggested that they will be challenging the EU-U.S. Data Privacy Framework. If these challenges are successful, they may not only impact the EU-U.S. Data Privacy Framework, but also further limit the viability of the standard contractual clauses and other data transfer mechanisms. The uncertainty around this issue has the potential to impact our business. Furthermore, while the Data Protection Act of 2018 in the United Kingdom that "implements" and complements the EU's GDPR has achieved Royal Assent on May 23, 2018 and is now effective in the United Kingdom, it is still unclear whether transfer of data from the EEA to the United Kingdom will remain lawful under GDPR. The Trade and Cooperation Agreement provides for a transitional period during which the United Kingdom will be treated like a European Union member state in relation to processing and transfers of personal data for four months from January 1, 2021. This may be extended by two further months. After such period, the United Kingdom will be a "third country" under the GDPR unless the European Commission adopts an adequacy decision in respect~~

~~of transfers of personal data to the United Kingdom. The United Kingdom has already determined that it considers all of the EU 27 and EEA member states to be adequate for the purposes of data protection, ensuring that data flows from the United Kingdom to the EU/EEA remain unaffected.~~

Beyond GDPR, there are privacy and data security laws in a growing number of countries around the world. While many loosely follow GDPR as a model, other laws contain different or conflicting provisions. These laws will impact our ability to conduct our business activities, including both our clinical trials and the sale and distribution of commercial products, through increased compliance costs, costs associated with contracting and potential enforcement actions. While we continue to address the implications of the recent changes to data privacy regulations, data privacy remains an evolving landscape at both the domestic and international level, with new regulations coming into effect and continued legal challenges, and our efforts to comply with the evolving data protection rules may be unsuccessful. It is possible that these laws may be interpreted and applied in a manner that is inconsistent with our practices. We must devote significant resources to understanding and complying with this changing landscape. Failure to comply with laws regarding data protection would expose us to risk of enforcement actions taken by data protection authorities in the EEA and elsewhere and carries with it the potential for significant penalties if we are found to be non-compliant. Similarly, failure to comply with federal and state laws in the U. S. regarding privacy and security of personal information could expose us to penalties under such laws. Any such failure to comply with data protection and privacy laws could result in government- imposed fines or orders requiring that we change our practices, claims for damages or other liabilities, regulatory investigations and enforcement action, litigation and significant costs for remediation, any of which could adversely affect our business. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our business, financial condition, results of operations or prospects. A variety of risks associated with international operations could materially adversely affect our business. As we engage in significant cross-border and international activities, we will be subject to risks related to international operations, including:

- different regulatory requirements for initiating clinical trials and maintaining approval of drugs in foreign countries;
- reduced protection for intellectual property rights in certain countries;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, political instability or open conflict in particular foreign economies and markets;
- differing and multiple payor reimbursement regimes, government payers or patient self- pay systems;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations of doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in North America;
- controlled substance legislation differs between countries and legislation in certain countries may restrict, limit, or delay our ability to manufacture and / or transport our product candidates;
- likelihood of potential or actual violations of domestic and international anti- corruption laws, such as the U. S. Foreign Corrupt Practices Act and the U. K. Bribery Act, or of U. S. and international import, export and re- export control and sanctions laws and regulations, which likelihood may increase with an increase of operations in foreign jurisdictions, directly or indirectly through third parties (whose corrupt or other illegal conduct may subject us to liability), which may involve interactions with government agencies or government- affiliated hospitals, universities and other organizations, such as conducting clinical trials, selling our products, and obtaining necessary permits, licenses, patent registrations, and other regulatory approvals
- tighter restrictions on privacy and data protection, and more burdensome obligations associated with the collection, use and retention of data, including clinical data and genetic material, may apply in jurisdictions outside of North America;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- business interruptions resulting from geopolitical actions, including war, terrorism, and civil and political unrest (such as the ongoing conflicts in the Middle East and between Russia and Ukraine), or natural disasters including earthquakes, typhoons, floods and fires; and
- supply and other disruptions resulting from the impact of public health epidemics, including the COVID- 19 pandemic, on our strategic partners, third- party manufacturers, suppliers and other third parties upon which we rely. Laws and regulations governing our international operations may preclude us from developing, manufacturing, and selling certain products outside of the United States and require us to develop and implement costly compliance programs. As we expand our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or FCPA, prohibits any U. S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring us to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The FCPA is enforced by the Department of Justice and the SEC. Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospital clinics, universities and similar institutions are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials, regulatory approvals, sales and marketing and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions. Because the FCPA applies to indirect payments, the use of third parties and other collaborators can increase potential FCPA risk, as we could be held liable for the acts of third parties that do not comply with the FCPA' s requirements. The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA can result in significant civil and criminal penalties. Indictment alone under the FCPA can lead to suspension of the right to do business with the U. S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long- term

disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U. S. exchanges for violations of the FCPA's accounting provisions. Like the FCPA, the UK Bribery Act and other anti-corruption laws throughout the world similarly prohibit offers and payments made to obtain improper business advantages, including offers or payments to healthcare professionals and other government and non-government officials. These other anti-corruption laws also can result in substantial financial penalties and other collateral consequences. Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U. S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. As we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs. The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The SEC also may suspend or bar issuers from trading securities on U. S. exchanges for violations of the FCPA's accounting provisions. We are subject to governmental export and import controls that could impair our or our collaborators' ability to compete in international markets due to licensing requirements and subject us or them to liability if we or they are not in compliance with applicable laws. Our products 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. Exports of our products outside of the United States must be made in compliance with these laws and regulations. If we or our collaborators fail to comply with these laws and regulations, we or they and certain of our or their employees could be subject to substantial civil or criminal penalties, including the possible loss of export or import privileges; fines, which may be imposed on us or our collaborators and the respective responsible employees or managers; and, in extreme cases, the incarceration of responsible employees or managers. We have conducted, and continue to conduct, clinical trials in various jurisdictions, including Russia and other Eastern European countries. In response to the conflict between Russia and Ukraine, the United States, the European Union, and other jurisdictions have imposed economic sanctions and other restrictions against certain officials, individuals, entities, regions, and industries in Russia, Ukraine, and Belarus. Such sanctions, and any further restrictions that may be promulgated, could adversely impact our ability to conduct our clinical program in certain jurisdictions. We will continue to closely monitor the geopolitical situation in Ukraine and its impact on our clinical trial operations. In addition, changes in our products or changes in applicable export or import laws and regulations may create delays in the introduction, provision, or sale of our products in international markets, prevent customers from using our products or, in some cases, prevent the export or import of our products to certain countries, governments or persons altogether. Any limitation on our ability to export, provide, or sell our products could adversely affect our business, financial condition and results of operations.

Changes in U. S. and international trade policies may adversely impact our business and operating results. The U. S. government has recently made statements and taken, or has contemplated taking, certain actions that may lead to potential changes to U. S. and international trade policies, including imposing tariffs and export control restrictions affecting products manufactured outside the United States. Some of our manufacturers and suppliers are located outside the United States. Any unfavorable government policies on international trade, such as export controls, capital controls or tariffs, may increase the cost of manufacturing our product candidates and platform materials, affect the demand for our drug products (if and once approved), the competitive position of our product candidates, and import or export of raw materials and finished product candidate used in our and our collaborators' preclinical studies and clinical trials, particularly with respect to any product candidates and materials that we import. If any new tariffs, export controls, legislation and / or regulations are implemented, or if existing trade agreements are renegotiated or, in particular, if either the U. S. or any foreign government takes retaliatory trade actions, such changes could have an adverse effect on our business, financial condition and results of operations. Moreover, trade tensions and conflicts between the United States and China in particular have been escalating in recent years and, as such, we are exposed to the possibility of product supply disruption and increased costs and expenses in the event of changes to the laws, rules, regulations and policies of the governments of the U. S. or China, or due to geopolitical unrest and unstable economic conditions. Certain Chinese biotechnology companies may become subject to trade restrictions, sanctions, other regulatory requirements or proposed legislation by the U. S. government, which could restrict or even prohibit our ability to work with such entities, thereby potentially disrupting their supply of material to us. The recently proposed BIOSECURE Act introduced in the House of Representatives, as well as a substantially similar bill in the Senate, targets certain Chinese biotechnology companies. If these bills become law, or similar laws are passed, they would have the potential to severely restrict the ability of companies to contract with certain Chinese biotechnology companies of concern without losing the ability to contract with, or otherwise received funding from, the U. S. government. Such disruptions could have adverse effects on the development of our product candidates and our business operations.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business. We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we

could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations. We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us. In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions. Our employees or consultants may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation. We are exposed to the risk of employee fraud or other misconduct, including intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign regulatory authorities, comply with manufacturing standards, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Employee or consultant misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. This could include violations of HIPAA, other U. S. federal and state law, and requirements of non- U. S. jurisdictions, including the European Union Data Protection Directive. It is not always possible to identify and deter employee or consultant misconduct, and the precautions we take to detect and prevent this activity 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, standards, regulations, guidance or codes of conduct. 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 and results of operations, including the imposition of significant fines or other sanctions.

Risks Related to Employee Matters and Managing Growth Our future success depends on our ability to retain our executive team and to attract, retain and motivate qualified personnel. We are highly dependent on the pharmaceutical research and development and business development expertise of our executive team, including Cedric Francois, M. D., Ph. D., our President and Chief Executive Officer. The members of our executive team are employed "at will," meaning any of them may terminate his or her employment with us at any time with or without notice and for any reason or no reason. In the future, we may be dependent on other members of our management, scientific and development team. Our ability to compete in the biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. Our industry has experienced a high rate of turnover of management personnel in recent years. If we lose one or more of our executive officers or other key employees, our ability to implement our business strategy successfully could be seriously harmed. Furthermore, replacing executive officers or other key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain marketing approval of and commercialize products successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key employees on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. We rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by other entities and may have commitments under consulting or advisory contracts with those entities that may limit their availability to us. If we are unable to continue to attract and retain highly qualified personnel, our ability to develop and commercialize our product candidates will be limited.

~~Our corporate restructuring and workforce reduction announced in August 2023, may not result in anticipated savings, could result in total costs and expenses that are greater than expected and could disrupt our business. In August 2023, we announced that we were conducting a corporate restructuring and cost savings initiatives involving a headcount reduction of approximately 225 employees, or approximately 25% of our then current workforce. We have estimated that the restructuring and related cost reduction initiatives will result in up to \$ 300 million in total cost savings through 2024. However, these estimates are subject to several assumptions, and actual results may differ. We may not realize, in full or in part, the anticipated benefits and savings from this restructuring due to unforeseen difficulties, delays or unexpected costs. If we are unable to realize the expected cost savings from the announced restructuring, our operating results and financial condition could be adversely affected. The workforce reduction may be disruptive to our operations and could yield unanticipated consequences, such as attrition beyond planned staff reductions, increased difficulties in our day-to-day operations and reduced employee morale, as well as result in weaknesses in our infrastructure and operations, and may increase the risk that we become unable to comply with legal and regulatory requirements. Our workforce reductions could also harm ability to attract and retain qualified management, scientific, clinical, and / or manufacturing personnel. Any failure to attract or retain qualified personnel could prevent us from successfully commercializing SYFOVRE and EMPAVELI and may adversely affect the development of our product candidates. Our employees, independent contractors, consultants, collaborators and contract research organizations may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation. We are exposed to the risk that our employees, independent contractors, consultants, collaborators and contract research organizations may engage in fraud or other misconduct, including intentional failures to comply with FDA regulations or similar regulations of comparable non- U. S. regulatory authorities, to provide accurate information to the FDA or comparable non- U. S. regulatory authorities, to comply with manufacturing standards we have established, to comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations~~

established and enforced by comparable non- U. S. regulatory authorities, to report financial information or data accurately or to disclose unauthorized activities to us. Such misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter misconduct, and the precautions we take to detect and prevent this activity 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, standards or regulations. These risks may be particularly acute given the rapid growth in the size of our company. 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 and results of operations, including the imposition of significant criminal, civil and administrative sanctions including monetary penalties, damages, fines, disgorgement, individual imprisonment, and exclusion from participation in government funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non- compliance with these laws, reputational harm, and we may be required to curtail or restructure our operations. We may engage in acquisitions that could disrupt our business, cause dilution to our stockholders or reduce our financial resources. In the future, we may enter into transactions to acquire other businesses, products or technologies. Because we have not made any acquisitions to date, our ability to do so successfully is unproven. If we do identify suitable candidates, we may not be able to make such acquisitions on favorable terms, or at all. Any acquisitions we make may not strengthen our competitive position, and these transactions may be viewed negatively by customers or investors. We may decide to incur debt in connection with an acquisition or issue our common stock or other equity securities to the stockholders of the acquired company, which would reduce the percentage ownership of our existing stockholders. We could incur losses resulting from undiscovered liabilities of the acquired business that are not covered by the indemnification we may obtain from the seller. In addition, we may not be able to successfully integrate the acquired personnel, technologies and operations into our existing business in an effective, timely and non- disruptive manner. Acquisitions may also divert management attention from day- to- day responsibilities, increase our expenses and reduce our cash available for operations and other uses. We cannot predict the number, timing or size of future acquisitions or the effect that any such transactions might have on our operating results.

Risks Related to Ownership of Our Common Stock An active trading market for our common stock may not be sustainable. If an active trading market is not sustained, our ability to raise capital in the future may be impaired. Our shares began trading on the Nasdaq Global Select Market on November 9, 2017. There is a risk that an active trading market for our shares may not be sustained, which could put downward pressure on the market price of our common stock and thereby affect the ability of stockholders to sell their shares. An inactive trading market for our common stock may also impair our ability to raise capital to continue to fund our operations by selling shares and impair our ability to acquire other companies or technologies by using our shares as consideration. The trading price of our common stock is highly volatile, which could result in substantial losses for our stockholders. The trading price of our common stock has been, and is likely to continue to be, highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their common stock at or above the price they paid for their common stock. The market price for our common stock may be influenced by many factors, including: • our success in commercializing EMPAVELI and SYFOVRE **and obtaining regulatory approval of EMPAVELI in additional indications and jurisdictions and SYFOVRE in additional jurisdictions**; • the timing and results of clinical trials of pegcetacoplan and any other product candidates; • the success of existing or new competitive products or technologies; • results of discussions with regulatory authorities and regulatory actions with respect to our product candidates or our competitors' products and product candidates; • announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments; • commencement or termination of collaborations for our development programs; • failure or discontinuation of any of our product candidates or development programs; • results of clinical trials of product candidates of our competitors; • regulatory or legal developments in the United States and other countries; • developments or disputes concerning patent applications, issued patents or other proprietary rights; • the recruitment or departure of key personnel; • the level of expenses related to any of our product candidates or clinical development programs; • the results of our efforts to develop additional product candidates or products; • actual or anticipated changes in estimates as to financial results or development timelines; • announcement or expectation of additional financing efforts; • sales of our common stock by us, our insiders or other stockholders; • variations in our financial results or those of companies that are perceived to be similar to us; • short positions, hedging or other transactions in our securities in connection with our Convertible Notes; • changes in estimates or recommendations by securities analysts, if any, that cover our stock; • changes in the structure of healthcare payment systems; • market conditions in the pharmaceutical and biotechnology sectors; • general economic, industry and market conditions; and • the other factors described in this “ Risk Factors ” section. For example, the trading price of our common stock experienced significant volatility in ~~2023-2024~~. On ~~June 13~~ **January 9, 2023-2024**, the closing price of our common stock on the Nasdaq Global Select Market was \$ ~~93-72~~ **31-47** and on ~~August 7~~ **October 10, 2023-2024**, the closing price of our common stock on the Nasdaq Global Select Market was \$ ~~23-27~~ **65-14**. Following periods of volatility in the market price of a company' s stock, securities class- action litigation has often been instituted against that company. We and certain of our current and former executive officers have been named as defendants in purported class action lawsuits following our announcement of the initial, top- line results. We and our chief executive officer have been named as defendants in lawsuits that could result in substantial costs and divert management' s attention. We ~~and~~, our chief executive officer, ~~and our~~ **directors** have been named as defendants in a purported class action lawsuit initiated in 2023 that alleges, among other things, that the defendants violated Sections 10 (b) and / or 20 (a) of the Exchange Act and Rule 10b- 5 promulgated thereunder by

misrepresenting and / or omitting certain material facts related to the design of SYFOVRE' s clinical trials and the risks associated with SYFOVRE' s commercial adoption. The plaintiffs seek, among other relief, compensatory damages and equitable relief in favor of the alleged class of plaintiffs against all defendants, including interest, and reasonable costs and expenses incurred by plaintiffs, including attorneys' and expert fees. **We, our chief executive officer, and our directors have also been named as defendants in a purported stockholder derivative lawsuit initiated in 2024 that alleges, among other things that the defendants breached fiduciary duties, were unjustly enriched, committed corporate waste, and violated Section 14 (a) of the Exchange Act based on the same facts. The plaintiffs seek, among other relief, monetary and punitive damages, and costs, including attorneys' fees.** The outcome of the matter described above cannot be predicted with certainty. However, we intend to vigorously defend against the litigation. We are unable, however, to predict the outcome of these matters at this time. Moreover, any conclusion of these matters in a manner adverse to us and for which we incur substantial costs or damages not covered by our directors' and officers' liability insurance would have a material adverse effect on our financial condition and business. In addition, the litigation could adversely impact our reputation and divert management attention and resources from other priorities, including the execution of business plans and strategies that are important to our ability to grow our business, any of which could have a material adverse effect on our business. Additional similar lawsuits might be filed. See " Part II, Item 1- Legal Proceedings ". We have broad discretion in the use of our funds and may not use them effectively. Our management will have broad discretion in the application of our cash and cash equivalents and could spend our funds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could harm our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest our funds in a manner that does not produce income or that loses value. ~~We incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to compliance initiatives and corporate governance practices. As a public company, we incur significant legal, accounting and other expenses. The Sarbanes- Oxley Act of 2002, the Dodd- Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Global Select Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote and will need to continue to devote, a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations increase our legal and financial compliance costs and make some activities more time- consuming and costly.~~ If we identify a material weakness in our internal control over financial reporting, it could have an adverse effect on our business and financial results and our ability to meet our reporting obligations could be negatively affected, each of which could negatively affect the trading price of our common stock. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected on a timely basis. Accordingly, a material weakness increases the risk that the financial information we report contains material errors. We regularly review and update our internal controls, disclosure controls and procedures, and corporate governance policies. In addition, we are required under the Sarbanes- Oxley Act of 2002 to report annually on our internal control over financial reporting. Our system of internal controls, however well- designed and operated, is based in part on certain assumptions and includes elements that rely on information from third parties. Our system can provide only reasonable, not absolute, assurances that the objectives of the system are met. If we, or our independent registered public accounting firm, determine that our internal controls over financial reporting are not effective, or we discover areas that need improvement in the future, these shortcomings could have an adverse effect on our business and financial results, and the price of our common stock could be negatively affected. If we cannot conclude that we have effective internal control over our financial reporting, or if our independent registered public accounting firm is unable to provide an unqualified opinion regarding the effectiveness of our internal control over financial reporting, investors could lose confidence in the reliability of our financial statements, which could lead to a decline in our stock price. Failure to comply with reporting requirements could also subject us to sanctions and / or investigations by the SEC, The Nasdaq Stock Market or other regulatory authorities. A sale of a substantial number of shares of our common stock could cause the market price of our common stock to decline significantly, even if our business is doing well. 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 of common stock intend to sell shares, could reduce the market price of our common stock. We have registered all shares of common stock that we may issue under our equity compensation plans. As of December 31, 2023, we had options to purchase an aggregate of 8, ~~663,048~~, ~~776 shares~~ **307 shares** of our common stock outstanding, of which options to purchase 6, ~~712,713~~, ~~201,203~~ shares were vested and ~~43,301,061~~, ~~126,810~~ outstanding unvested restricted stock units that upon vesting would result in the issuance of ~~43,301,961~~, ~~126,810~~ shares of our common stock. We also ~~had~~ **have** pre- funded warrants to purchase ~~2,380,80~~, 956 shares of our common stock outstanding. The shares issuable upon exercise or vesting can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates. Moreover, holders of an aggregate of 10, 778, 303 shares of our common stock have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. If these additional shares 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. Changes in tax laws or in their interpretation could adversely affect our business and financial condition. Changes in tax law could adversely affect our business or financial condition. For example, on December 22, 2017, the U. S. government enacted legislation, commonly referred to as the Tax Cuts and Jobs Act, or the TCJA, that significantly revised the Internal Revenue Code of 1986, as amended, or the Code. The TCJA, as amended by the Coronavirus Aid, Relief, and Economic Security Act, or CARES Act, among other things, contained significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35 % to a flat rate of 21 % for taxable years

beginning after December 31, 2020 and limitation of the deduction for net operating losses to 80 % of current year taxable income for losses arising in taxable years beginning after December 31, 2017 (though any such net operating losses may be carried forward indefinitely). In addition, beginning in 2022, the TCJA eliminates the option to deduct research and development expenditures currently and generally requires corporations to capitalize and amortize them over five years or 15 years (for expenditures attributable to foreign research). As part of Congress' response to the COVID- 19 pandemic, in addition to the CARES Act, economic relief legislation was enacted in 2020 and 2021 containing tax provisions. The IRA, which was signed into law in August 2022, also introduced new tax provisions, including a one percent excise tax imposed on certain stock repurchases by publicly traded corporations. Regulatory guidance under the TCJA, the IRA, and additional legislation is and continues to be forthcoming, and such guidance could ultimately increase or lessen the impact of these laws on our business and financial condition. Congress may enact additional legislations, some of which could have an impact on our company. In addition, it is uncertain if and to what extent various states will conform to such legislation. The enactment of some or all of the recommendations set forth or that may be forthcoming in the Organization for Economic Cooperation and Development' s ("~~OECD~~,") or OECD,") project on " Base Erosion and Profit Shifting " by tax authorities in the countries in which we operate, could unfavorably impact our effective tax rate. These initiatives focus on common international principles for the entitlement to tax global corporate profits and enactment of minimum global tax rate of 15 %. Many countries have or are in the process of enacting legislation intended to implement the OECD Global Anti- Base Erosion, ~~or GloBEModel~~ ("~~GloBE~~") Model Rules effective on January 01, 2024. The impact on the Company will depend on the timing of implementation, the exact nature of each country' s GloBE legislation, guidance, and regulations thereon and their application by the tax authorities either prospectively or retrospectively. We might not be able to utilize a significant portion of our net operating loss carryforwards and research and development tax credit carryforwards. As of December 31, ~~2023-2024~~, we had both federal and state net operating loss carryforwards of \$ ~~494-422~~ . ~~1-5~~ million and \$ ~~524-621~~ . ~~0-8~~ million, respectively, and federal and state research and development tax credit carryforwards of \$ ~~85-107~~ . ~~7-8~~ million and \$ ~~20-26~~ . ~~2-4~~ million, respectively. Federal net operating loss carryforward generated post- 2017 in the amount of \$ ~~449-420~~ . ~~8-9~~ million may be carried forward indefinitely. The remaining net operating loss and research and development tax credit carryforward will begin to expire in 2025. These net operating loss and tax credit carryforwards could expire unused and be unavailable to offset future income tax liabilities. Under the TCJA, as modified by the CARES Act, federal net operating losses incurred in 2018 and in future years may be carried forward indefinitely, but the deductibility of such federal net operating losses in 2021 and future years is limited. Certain states have also enacted temporary suspension or limitation of the utilization of net operating loss carryforwards. In addition, under Section 382 of the Code, and corresponding provisions of state law, if a corporation undergoes an " ownership change, " which is generally defined as a greater than 50 % change, by value, in its equity ownership over a three- year period, the corporation' s ability to use its pre- change net operating loss carryforwards and other pre- change tax attributes to offset its post- change income may be limited. We experienced a Section 382 ownership change in September 2015, which imposes annual limitations on our use of pre- change net operating loss carryforwards and other pre- change tax attributes. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. We have determined that our research and development credit carryforwards are also limited. These limitations upon our historical net operating loss and tax credit carryforwards may harm our future operating results by effectively increasing our future tax obligations. Refer to Note ~~14-13~~ . " Income Taxes, " of the consolidated financial statements included in this Annual Report on Form 10- K for additional information related to our accounting for income taxes. Taxing authorities could challenge our historical and future tax positions or our allocation of taxable income among our subsidiaries, and tax laws to which we are subject could change in a manner adverse to us. We operate through various subsidiaries in a number of countries throughout the world. Consequently, we are subject to tax laws, treaties, and regulations in the countries in which we operate, and these laws and treaties are subject to interpretation. We have taken, and will continue to take, tax positions based on our interpretation of such tax laws. Our transfer pricing arrangements are not generally binding on applicable tax authorities. The price charged for products, services, or the royalty rates and other amounts paid for intellectual property rights, could be challenged by the various tax authorities, resulting in additional tax liability, interest, and / or penalties. There can be no assurance that a taxing authority will not have a different interpretation of applicable law and assess us with additional taxes. If we are assessed with additional taxes, this may result in a material adverse effect on our results of operations and / or financial condition. Any changes to existing accounting pronouncements or taxation rules or practices may cause adverse fluctuations in our reported results of operations or affect how we conduct our business. A change in accounting pronouncements or taxation rules or practices can have a significant effect on our reported results and may affect our reporting of transactions completed before the change is effective. New accounting pronouncements, taxation rules and varying interpretations of accounting pronouncements or taxation rules have occurred in the past and may occur in the future. The change to existing rules, future changes, if any, or the need for us to modify a current tax or accounting position may adversely affect our reported financial results or the way we conduct our business. We do not anticipate paying any cash dividends on our capital stock in the foreseeable future. Accordingly, stockholders must rely on capital appreciation, if any, for any return on their investment. We ~~have~~ never declared nor paid cash dividends on our capital stock. We currently plan to retain all of our future earnings, if any, to finance the operation, development and growth of our business. In addition, the terms of ~~our development funding~~ **the Sixth Street Financing agreement Agreement with SFJ**, precludes us from paying dividends, and any future debt or credit agreements may also preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be our stockholders' sole source of gain for the foreseeable future. Concentration of ownership of our common stock among our executive officers and directors, entities associated with our executive officers and directors and our largest stockholders may allow these stockholders to significantly influence matters submitted to our stockholders for approval, as well as our management and affairs. As of February ~~20-28~~ , ~~2024-2025~~ , our executive officers and directors, and entities associated or affiliated with our executive officers

and directors, in the aggregate, beneficially owned shares representing approximately ~~18.16~~ **16.04**% of our outstanding common stock, including one of our largest stockholders, Morningside Venture Investments Ltd., which beneficially owned approximately 10. ~~7.3~~ **7.3**% of our outstanding common stock. As a result, if these stockholders were to choose to act together, they may have the ability to significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, could substantially influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of ownership may: • delay, defer or prevent a change in control; • entrench our management or the board of directors; or • impede a merger, consolidation, takeover or other business combination involving us that other stockholders may desire. Some of these persons or entities may have interests different than those of our other investors. For example, because many of these stockholders purchased their shares at prices substantially below the price at which other investors purchased shares and have held their shares for a longer period, they may be more interested in selling our company to an acquirer than other investors or they may want us to pursue strategies that deviate from the interests of other stockholders. Provisions in our corporate charter documents and under Delaware law may prevent or frustrate attempts by our stockholders to change our management or hinder efforts to acquire a controlling interest in us. Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions: • establish a classified board of directors such that all members of the board are not elected at one time; • allow the authorized number of our directors to be changed only by resolution of our board of directors; • limit the manner in which stockholders can remove directors from the board; • establish advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted on at stockholder meetings; • require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent; • limit who may call a special meeting of stockholders; • authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a “poison pill” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and • require the approval of the holders of at least 75 % of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws. Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of the State of Delaware, which prohibits a person who owns in excess of 15 % of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15 % of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. This could discourage, delay or prevent someone from acquiring us or merging with us, whether or not it is desired by, or beneficial to, our stockholders. This could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in the best interests of our stockholders. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock. If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our share price and trading volume could decline. The trading market for our common stock will likely depend in part on the research and reports that securities or industry analysts publish about us or our business. We do not have any control over these analysts. There can be no assurance that analysts will continue to cover us or provide favorable coverage. Securities or industry analysts may elect not to provide research coverage of our common stock, and such lack of research coverage may negatively impact the market price of our common stock. In the event we do have analyst coverage, if one or more analysts downgrade our stock or change their opinion of our stock, our share price would likely decline. In addition, if one or more analysts cease coverage of our company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which could cause our share price or trading volume to decline. Our restated certificate of incorporation designates the state courts in the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the federal court for the District of Delaware, as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against our company and our directors, officers and employees. Our restated certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery does not have jurisdiction, the federal district court for the District of Delaware) will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or employees to our company or our stockholders, any action asserting a claim against us arising pursuant to any provision of the General Corporation Law of the State of Delaware or our certificate of incorporation or bylaws, or any action asserting a claim against us governed by the internal affairs doctrine. This exclusive forum provision will not apply to actions arising under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended. This exclusive forum provision may limit the ability of our stockholders to bring a claim in a judicial forum that such stockholders find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees.