Risk Factors Comparison 2024-02-27 to 2023-03-09 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 our inception. We expect to incur losses over the next several years and may never achieve or maintain profitability. Since inception, we have incurred significant operating losses. Our net loss was $ ~~109~~ **97** million for the year ended December 31, ~~2022~~ **2023** and $ ~~80~~ **109** million for the year ended December 31, ~~2021~~ **2022** . As of December 31, ~~2022~~ **2023** , we had an accumulated deficit of $ ~~412~~ **509** million. To date, we have funded our operations primarily from the sale of shares of our capital stock and from upfront payments received under our collaboration and license agreements. We have devoted substantially all of our financial resources and efforts to research and development, including clinical trials and preclinical studies. We are still in the early stages of development of our product candidates, and we have not completed development of any product candidates. We expect to continue to incur significant expenses and operating losses over the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we: • continue our clinical development of losmapimod and **pociredir** ~~seek to resolve the clinical hold on FTX- 6058~~ ; • continue our ongoing preclinical studies; • advance clinical- stage product candidates through later stage trials, such as REACH, the Phase 3 clinical trial of losmapimod for the treatment of FSHD; • pursue the discovery of drug targets for other genetically- defined rare diseases and the subsequent development of any resulting product candidates **, including for Diamond Blackfan Anemia under our recent license agreement with CAMP4** ; • seek regulatory approvals for any product candidates that successfully complete clinical trials; • scale up our manufacturing processes and capabilities, or arrange for a third party to do so on our behalf, to support our clinical trials of our product candidates and commercialization of any of our product candidates for which we may obtain marketing approval; • establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain regulatory approval; • acquire or in- license products, product candidates, technologies and / or data referencing rights **, such as our recent agreement with CAMP4** ; • make any milestone payments to affiliates of **GlaxoSmithKline plc, or** GSK, under our right of reference and license agreement with GSK upon the achievement of specified clinical or regulatory milestones **, or to CAMP4 under our license agreement with CAMP4** ; • maintain, expand, enforce, defend and protect our intellectual property; • hire additional clinical, quality control and scientific personnel; and • add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts and our operations as a public company. To become and remain profitable, we must succeed in developing, and eventually commercializing, a product or products that generate significant revenue. The ability to achieve this success will require us to be effective in a range of challenging activities, including completing preclinical testing and clinical trials of our product candidates, discovering additional product candidates, obtaining regulatory approval for these product candidates and manufacturing, marketing and selling any products for which we may obtain regulatory approval. We are only in the preliminary stages of most of these activities. We may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to achieve profitability. Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. Our expenses will increase if, among other things: • we are required by the FDA, the EMA, or other regulatory authorities to perform trials or studies in addition to, or different than, those expected ~~(including as may be required to address the recent clinical hold on FTX- 6058)~~ ; • there are any delays in completing our clinical trials or the development of any of our product candidates, such as the ~~recent~~ clinical hold imposed by **the** FDA on the ~~FTX- 6058~~ **Investigational New Drug, or** IND **, application for pociredir in SCD, which was lifted in August 2023** ; or • there are any third- party challenges to our intellectual property or we need to defend against any intellectual property- related claim. 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 depress 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 even continue our operations. A decline in the value of our company could also cause our stockholders to lose all or part of their investment. We will need substantial additional funding. 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. We expect to devote substantial financial resources to our ongoing and planned activities, particularly as we continue our ongoing and planned clinical trials of losmapimod and **pociredir** ~~FTX- 6058~~ , continue research and development and initiate additional clinical trials of, and seek regulatory approval for, these and other product candidates. We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance our preclinical activities and clinical trials. In addition, if we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product manufacturing, sales, marketing and distribution. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. Given current uncertainty in the capital markets and other factors, such funding may not be available on terms favorable to us or at all. If we are unable to raise capital when needed or on acceptable terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts. Our future capital requirements will depend on many factors, including: • the progress, costs and results of our ongoing clinical trials of losmapimod, including REACH, the Phase 3 clinical trial of losmapimod for the treatment of FSHD, which **completed** ~~enrolled~~ **enrollment** ~~its first patient~~ in ~~June~~ **September** ~~2022~~ **2023** , and our ~~ability to resolve the clinical hold on the~~ Phase 1b **clinical** trial of ~~FTX- 6058~~ **pociredir in SCD** ; • additional planned clinical trials; • the

scope, progress, costs and results of discovery research, preclinical development, laboratory testing and clinical trials for our current product candidates in additional indications or for any future product candidates that we may pursue; • the number of and development requirements for other product candidates that we pursue; • the costs, timing and outcome of regulatory review of our product candidates; • our ability to enter into contract manufacturing arrangements for supply of active pharmaceutical ingredient, or API, and manufacture of our product candidates and the terms of such arrangements; • the success of our collaboration with MyoKardia **or under our recent license agreement with CAMP4** ; • our ability to establish and maintain additional strategic collaborations, licensing or other arrangements and the financial terms of such arrangements; • the payment or receipt of milestones, royalties and other collaboration- based revenues, if any; • the costs and timing of future commercialization activities, including product manufacturing, sales, marketing and distribution, for any of our product candidates for which we may receive marketing approval; • the amount and timing of revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval; • the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property and proprietary rights and defending any intellectual property- related claims; • the ~~impact of the ongoing COVID- 19 pandemic on our business and operations; and • the~~ extent to which we acquire or in- license other products, product candidates, technologies or data referencing rights. As of December 31, ~~2022~~ **2023** , we had cash, cash equivalents, and marketable securities of approximately $ ~~202~~ **236** . ~~9~~ **2** million. We believe that our cash, cash equivalents, and marketable securities as of December 31, ~~2022, together with the net proceeds from our public offering completed in January~~ 2023 ~~,~~ will enable us to fund our operating expenses and capital expenditure requirements into ~~mid- 2025~~ **2026** . However, we have based this estimate on assumptions that may prove to be wrong, and our operating plan may change as a result of many factors currently unknown to us. As a result, we could deplete our capital resources sooner than we currently expect. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time- consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Commercial revenues, if any, will not be derived unless and until we can achieve sales of products, which we do not anticipate for ~~many~~ **several** years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all, and may become even more difficult to obtain due to rising interest rates and the current downturn in the U. S. capital markets and the biotechnology sector in general. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans. If adequate funds are not available to us on a timely basis, we may be required to delay, limit, reduce or terminate preclinical studies, clinical trials or other development activities for one or more of our product candidates or discovery stage programs or delay, limit, reduce or terminate our establishment of sales and marketing capabilities or other activities that may be necessary to commercialize our product candidates. We may also choose to further realign our operations to achieve additional operational efficiencies beyond **the** ~~our recently announced~~ strategic realignment **commenced in August 2022** . Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and marketing, distribution or licensing arrangements. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights as common stockholders. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, selling or licensing our assets, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings 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. We have in the past relied **, and in the future anticipate we will rely,** in part on sales of our common stock through ~~an~~ at- the- market, or ATM, offering ~~program~~ **programs** . Increased volatility and decreases in market prices of equity securities generally and of our common stock in particular may have an adverse impact on our willingness and / or ability to continue to sell our common stock through ~~our~~ **an** ATM offering program. Decreases in these sales could affect the cost or availability of equity capital, which could in turn have an adverse effect on our business, including current operations, future growth, revenues, net income and the market prices of our common stock. In May 2022, we established **an** ~~a new~~ ATM offering program to sell shares of our common stock having an aggregate offering price of up to $ 50. 0 million from time to time. ~~In~~ **We suspended this program in** January 2023 ~~,~~ immediately prior to commencing our underwritten public offering of common stock, ~~we suspended our use of~~ and **then** terminated **this** ~~the prospectus supplement related to the~~ ATM offering program **in February 2024 in anticipation of entering into a new ATM offering program to sell shares of our common stock having an aggregate offering price of up to $ 100. 0 million from time to time promptly after filing this annual report on Form 10- K** . We may not make any sales of securities pursuant to ~~the~~ **any** ATM offering program unless and until ~~we file~~ a ~~new prospectus supplement~~ **registration statement with respect to the shares being offered thereunder becomes effective** . However, given the overall volatility in the capital markets, even if a new ~~prospectus supplement is filed~~ **ATM offering program becomes available to us** , we may not be willing or able ~~to continue~~ to raise equity capital through ~~our~~ **an** ATM offering program. We may, therefore, need to turn to other sources of funding that may have terms that are not favorable to us, or reduce our business operations given capital constraints.

Alternative financing arrangements could involve issuances of one or more types of securities, including common stock, preferred stock, convertible debt, warrants to acquire common stock or other securities. These securities could be issued at or below the then prevailing market price for our common stock. In addition, if we issue debt securities, the holders of the debt would have a claim to our assets that would be superior to the rights of stockholders until the principal, accrued and unpaid interest and any premium or make- whole has been paid. In addition, if we borrow funds and / or issue debt securities through a subsidiary, the lenders and / or holders of those debt securities would have a right to payment that would be effectively senior to our equity ownership in the subsidiary, which would adversely affect the rights of holders of both our equity securities and, if any, our debt and debt securities. Interest on any newly- issued debt securities and / or newly- incurred borrowings would increase our operating costs and reduce our net income, and these impacts may be material. If the issuance of new securities results in diminished rights to holders of our common stock, the market price of our common stock could be materially and adversely affected. Should the financing we require to sustain our working capital needs be unavailable or prohibitively expensive when we require it, the consequences could result in a material adverse effect on our business, operating results, financial condition and prospects. Our limited operating history may make it difficult for stockholders to evaluate the success of our business to date and to assess our future viability. We commenced activities in 2015 and are a clinical- stage biotechnology company. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, establishing our intellectual property, building our discovery platform, identifying drug targets and potential product candidates, in- licensing assets, producing drug substance and drug product material for use in clinical trials and conducting preclinical studies and clinical trials. We have not yet demonstrated our ability to successfully develop any product candidate, obtain regulatory approvals, manufacture a commercial scale product or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, any predictions stockholders make 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 products. In addition, as our business grows, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition at some point from a company with a research and development focus to a company capable of supporting commercial activities. We may not be successful in such a transition. We expect our financial condition and operating results 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, stockholders should not rely upon the results of any quarterly or annual periods as indications of future operating performance. Our business was negatively impacted by the ~~ongoing~~ COVID- 19 pandemic and may in the future be impacted by any future pandemics. In addition, **the effects of** ~~this pandemic may continue to, and~~ any future pandemics may, adversely impact economies worldwide, which could result in adverse effects on our business and operations. We experienced enrollment delays in our ReDUX4 clinical trial due to the COVID- 19 pandemic as the clinical trial sites for our ReDUX4 clinical trial temporarily postponed trial- related activities. We also saw temporary disruptions in other business activities due to a temporary reduction in workforce presence at our Cambridge research facility ~~. Although our employees have returned to work~~ , ~~there are a number of vaccines available,~~ and ~~many restrictions have been lifted, there is still uncertainty about the overall impact of~~ COVID- 19 **had a significant** ~~on our business, as well as its continuing~~ impact on economies worldwide. Future pandemics may arise, and they, like COVID- 19, could impact our company, our CMOs and contract research organizations, or CROs, creating disruptions that affect our ability to initiate and complete preclinical studies or clinical trials, disrupt our supply chain for our research and development activities, and disrupt any then planned or ongoing clinical trials for any number of reasons. Any future pandemics could similarly impact patient recruitment or retention for clinical trials, or result in resources being redirected in a way that adversely impacts our ability to progress regulatory approvals and protect our intellectual property. In addition, as with COVID- 19 pandemic, we may face impediments to regulatory meetings and approvals due to recommended safety measures intended to limit in- person interactions in any future pandemic. The ~~ongoing~~ COVID- 19 pandemic ~~already~~ caused significant disruptions in the financial markets ~~, and it may continue to,~~ and any future pandemic could similarly ~~,~~ cause such disruptions, which could impact our ability to raise additional funds through public offerings and may also impact the volatility of our stock price and trading in our stock. We cannot be certain what the overall impact of ~~the ongoing COVID- 19 pandemic or~~ any future pandemic will be on our business. The extent of ~~the impact of COVID- 19 and~~ any future pandemic on our business, financial condition, results of operations and prospects will depend on future developments that are uncertain. Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition. The rules dealing with U. S. federal, state, and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and the U. S. Treasury Department. Changes to tax laws (which changes may have retroactive application), including with respect to net operating losses and research and development tax credits, could adversely affect us or holders of our common stock. In recent years, many such changes have been made and changes are likely to continue to occur in the future. **For example, under Section 174 of the U. S. Internal Revenue Code of 1986, as amended, or the Code, in taxable years beginning after December 31, 2021, expenses that are incurred for research and development in the United States will be capitalized and amortized, which may have an adverse effect on our cash flow.** Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. We urge investors to consult with their legal and tax advisers regarding the implications of potential changes in tax laws on an investment in our common stock. Our ability to use our net operating losses and research and development tax credit carryforwards to offset future taxable income may be subject to certain limitations. As of December 31, ~~2022~~ **2023** , we had federal and state net operating loss carryforwards of $ ~~275~~ **312. 3 million and $ 317** . 1 million ~~and $ 272. 6 million~~ , respectively, which begin to expire in 2036. Approximately $ ~~251~~ **288** . ~~5~~ **7** million of the federal net operating losses can be carried forward indefinitely. As of December 31, ~~2022~~ **2023** , we also had federal orphan drug credits of $ ~~14~~ **23** . ~~6~~ **8** million, which begin to expire in 2040. As of December 31, ~~2022~~ **2023** , we also had federal and state research and development tax credit carryforwards of $ **7.** 6 ~~. 9~~ million and $ 4. ~~0~~ **9**

million, respectively, which begin to expire in 2035 and 2030, respectively. These net operating loss and tax credit carryforwards could expire unused and be unavailable to offset future income tax liabilities. In general, under Section 382 of ~~the U. S. Internal Revenue Code of 1986, as amended, or~~ the Code, and corresponding provisions of state law, a corporation that undergoes an " ownership change, " which is generally defined as a greater than 50 % change, by value, in its equity ownership by certain stockholders over a three- year period, is subject to limitations on its ability to utilize its pre- change net operating losses and research and development tax credit carryforwards to offset future taxable income. We conducted an analysis under Section 382 **of the Code** to determine if historical changes in ownership through December 31, 2021 would limit or otherwise restrict our ability to utilize our pre- change net operating losses and research and development tax credit carryforwards to offset future taxable income. As a result of the analysis, we do not believe that there are any significant limitations on our ability to utilize our net operating losses and research and development tax credit carryforwards to offset future taxable income. However, we may experience such ownership changes in the future (which may be outside our control). As a result, if, and to the extent that, we earn net taxable income, our ability to use our pre- change net operating losses and research and development tax credit carryforwards to offset such taxable income may be subject to limitations. Our net operating losses or credits may also be impaired under state law. We have a history of cumulative losses and anticipate that we will continue to incur significant losses in the foreseeable future; thus, we do not know whether or when we will generate taxable income necessary to utilize our net operating losses or research and development tax credit carryforwards. **Adverse developments affecting the financial services industry, such as actual events or concerns involving liquidity, defaults or non- performance by financial institutions or transactional counterparties, could adversely affect our current and projected business operations and financial condition and results of operations. Events involving limited liquidity, defaults, non- performance or other adverse developments that affect financial institutions, transactional counterparties or other companies in the financial services industry or the financial services industry generally, or concerns or rumors about any events of these kinds or other similar risks, have in the past and may in the future lead to market- wide liquidity problems. In March 2023, a number of banks (e. g., Silicon Valley Bank, Signature Bank and Silvergate Capital Corp.) were placed into receivership, followed by First Republic Bank in May 2023. Although the Federal Deposit Insurance Corporation, or FDIC, and others have taken steps to reduce risk to uninsured depositors, borrowers under credit agreements, letters of credit and certain other financial instruments with such banks or any other financial institution that is placed into receivership by the FDIC may be unable to access undrawn amounts thereunder. Even though we assess our banking relationships as we believe necessary or appropriate, our access to funding sources and other credit arrangements in amounts adequate to finance or capitalize our current and projected future business operations could be significantly impaired by factors affecting the financial services industry or economy in general, such as these recent bank failures. These factors could also include, among others, liquidity constraints or failures, the ability to perform obligations under various types of financial, credit or liquidity agreements or arrangements, disruptions or instability in the financial services industry or financial markets, or concerns or negative expectations about the prospects for companies in the financial services industry and the supervision thereof. In addition, investor concerns regarding the United States or international financial systems could result in less favorable commercial financing terms, including higher interest rates or costs and tighter financial and operating covenants, or systemic limitations on access to credit and liquidity sources, thereby making it more difficult for us to acquire financing on acceptable terms or at all. Any decline in available funding or access to our cash and liquidity resources could, among other risks, adversely impact our ability to meet our operating expenses, financial obligations or fulfill our other obligations, result in breaches of our contractual obligations or result in violations of federal or state wage and hour laws, which could have material adverse effect on our liquidity and on our business, financial condition or results of operations.** Risks Related to the Discovery and Development of our Product Candidates We are early in our development efforts, and we only have two clinical- stage product candidates. If we are unable to commercialize our product candidates or experience significant delays in doing so, our business will be materially harmed. We are early in our development efforts, and we have advanced only two product candidates into clinical trials, losmapimod for the treatment of FSHD, and **pociredir** ~~FTX- 6058~~ for the treatment of SCD **(which was** ~~, although the latter program is currently~~ on clinical hold **between February and August 2023)** . We have invested substantially all of our efforts and financial resources in ~~our proprietary product engine to identify~~ **identifying** and ~~validate~~ **validating and conducting clinical trials on** cellular drug targets that can potentially modulate gene expression to address the root cause of genetically- defined rare diseases. Our ability to generate product revenues, which we do not expect will occur for ~~many~~ **several** years, if ever, will depend heavily on the successful development, regulatory approval and eventual commercialization of our product candidates. The success of our product candidates will depend on several factors, including the following: • successfully completing preclinical studies and clinical trials; • allowance by the FDA or other regulatory agencies of the INDs, clinical trial applications, or CTAs, or other regulatory filings for losmapimod, **pociredir** ~~FTX- 6058~~ and future product candidates ~~, including our ability to resolve the current clinical hold on FTX- 6058 for SCD~~ ; • expanding and maintaining a workforce of experienced scientists and others to continue to develop our product candidates; • applying for and receiving marketing approvals from applicable regulatory authorities; • obtaining and maintaining intellectual property protection and regulatory exclusivity for our product candidates; • making arrangements with third- party manufacturers for, or establishing, commercial manufacturing capabilities; • establishing sales, marketing and distribution capabilities and successfully launching commercial sales of the products, if and when approved, whether alone or in collaboration with others; • acceptance of the products, if and when approved, by patients, the medical community and third- party payors; • effectively competing with other therapies; • obtaining and maintaining coverage, adequate pricing and adequate reimbursement from third- party payors, including government payors; • maintaining, enforcing, defending and protecting our rights in our intellectual property portfolio; • not infringing, misappropriating or otherwise violating others' intellectual property or proprietary rights; and • maintaining a continued acceptable safety profile of the

products following receipt of any regulatory approvals. If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully develop and commercialize our product candidates, which would materially harm our business. We may not be successful in our efforts to ~~use our product engine to~~ build a pipeline of product candidates. **Our current** ~~A key element of our~~ strategy is **focused on developing small molecules** to **improve** ~~use our proprietary product engine to identify and validate cellular drug targets that can potentially modulate gene expression to address~~ the **lives** ~~root cause~~ of **patients with** genetically ~~-~~defined rare diseases ~~, with an initial focus on identifying small molecules specific to the identified cellular target~~. Even if we are successful in identifying drug targets and potential product candidates, such candidates that we identify may not be suitable for clinical development, including as a result of being shown to have harmful side effects or other characteristics that indicate that they are unlikely to receive marketing approval and achieve market acceptance. Identifying, developing, obtaining regulatory approval for and commercializing additional product candidates will require substantial additional funding and is prone to the risks of failure inherent in product development. We cannot provide stockholders any assurance that we will be able to successfully identify additional product candidates ~~with our product engine~~, including as a result of our collaboration with MyoKardia, advance any additional product candidates through the development process or successfully commercialize any such additional product candidates. Regulatory authorities have substantial discretion in the approval process and may cause delays in the approval or rejection of an application. As a result of these factors, it is difficult for us to predict the time and cost of product candidate development. There can be no assurance that any development problems we experience in the future related to our **discovery technologies** ~~proprietary product engine~~ or any of our research or development programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. If we do not successfully identify, develop, obtain regulatory approval for and commercialize product candidates ~~based upon our technological approach~~, we will not be able to generate product revenues. Clinical drug development involves a lengthy and expensive process, with an uncertain outcome. The results of preclinical studies and early clinical trials may not be predictive of future results. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates. We have two product candidates in clinical development. The risk of failure for each of our product candidates is high. It is impossible to predict when or if any of our product candidates will prove effective or safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. We have not yet completed a pivotal clinical trial of any product candidate. Clinical trials may fail to demonstrate that our product candidates are safe for humans and effective for indicated uses. For example, **pociredir** ~~FTX- 6058~~, our clinical trial stage candidate to treat SCD, is an **embryonic ectoderm development, or** ~~EEDi~~ - **EED, inhibitor**. EED is a member of the PRC2 complex, which also includes EZH2. There are approved products in the EZH2 class of medications and their approved labeling outlines safety risks, including an increased risk of malignancies. In the event that **pociredir** ~~FTX- 6058~~ has similar safety risks as other PRC2 medications, this could impact its acceptance. Even if the clinical trials are successful, 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. Before we can commence clinical trials for a product candidate, we must complete extensive preclinical testing and studies that support our planned INDs and other regulatory filings in the United States and abroad. We cannot be certain of the timely completion or outcome of our preclinical testing and studies and cannot predict if the FDA or other regulatory agencies will accept our proposed clinical programs or if the outcome of our preclinical testing and studies will ultimately support the further development of our current or future product candidates. As a result, we cannot be sure that we will be able to submit INDs or similar applications for our preclinical programs on the timelines we expect, if at all, and we cannot be sure that submission of INDs or similar applications will result in the FDA or other regulatory authorities allowing clinical trials to begin or continue. ~~On~~ **For example, in** February ~~23,~~ 2023, the FDA imposed a clinical hold on our IND for **pociredir** ~~FTX- 6058~~ in SCD. **We** ~~While we intend to work~~ **worked** diligently to resolve the hold as soon as possible, **and in August 2023,** ~~there is no guarantee that~~ FDA **lifted** ~~will allow~~ the **clinical hold** ~~trial to resume in a timely manner or at all~~. ~~Furthermore, product~~ **Product** candidates are subject to continued preclinical safety studies, which may be conducted concurrent with our clinical testing. The outcomes of these safety studies may delay the launch of or enrollment in future clinical trials and could impact our ability to continue to conduct our clinical trials. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, or at all. For example, **we revised the inclusion and exclusion criteria of** our clinical trial of ~~FTX-6058 is currently on~~ **pociredir in SCD to address the clinical** hold **imposed by the FDA,** and there can be no certainty as to ~~when it~~ **whether we** will ~~resume, if at all~~ **be successful in completing the clinical trial with its revised design**. A failure of one or more clinical trials can occur at any stage of testing, which may result from a multitude of factors, including, but not limited to, flaws in study design, dose selection issues, placebo effects, patient enrollment criteria and failure to demonstrate favorable safety or efficacy traits. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and preliminary or interim results of a clinical trial do not necessarily predict final results. For example, our product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials. A lack of clinical benefit may be due to insufficient dosing or for other reasons. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late- stage clinical trials even after achieving promising results in preclinical testing and earlier- stage clinical trials, and we cannot be certain that we will not face similar setbacks. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

Furthermore, the failure of any of our product candidates to demonstrate safety and efficacy in any clinical trial could negatively impact the perception of our other product candidates and / or cause the FDA or other regulatory authorities to require additional testing before approving any of our product candidates. As described in Item 1 " Business — Licenses and ~~Collaborations~~ **Collaboration** -- Right of Reference and License Agreement with GlaxoSmithKline " in **our Annual Report on Form 10- K for the year ended December 31, 2023, or** this Annual Report on Form 10- K, we have entered into a right of reference and license agreement, as amended, with affiliates of GSK. Although losmapimod was originally evaluated by GSK in nearly 3, 600 subjects, GSK did not evaluate losmapimod in FSHD or in any other muscular dystrophy, and most of the subjects in these trials were given a dose that was lower than our planned dosage of 15 mg of losmapimod twice per day. Accordingly, the safety data generated from GSK' s clinical trials of losmapimod may not be predictive or indicative of the results of our clinical trials. Similarly, while we believe the safety data from GSK' s clinical trials may, in part, support the safety database for losmapimod, GSK evaluated a limited number of subjects at a dose of 15 mg twice daily. We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including: • regulators or institutional review boards, or IRBs, may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site; • we may experience delays in reaching, or fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites; • regulators may decide the design of our clinical trials is flawed, for example if our trial protocol does not evaluate treatment effects in trial subjects for a sufficient length of time; • clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs; • we may be unable to establish clinical endpoints that applicable regulatory authorities would consider clinically meaningful, or, if we seek accelerated approval, biomarker efficacy endpoints that applicable regulatory authorities would consider likely to predict clinical benefit; • preclinical testing may produce results based on which we may decide, or regulators may require us, to conduct additional preclinical studies before we proceed with certain clinical trials, limit the scope of our clinical trials, halt ongoing clinical trials or abandon product development programs; • the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate; • our third- party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all; • we may decide, or regulators or IRBs may require us, to suspend or terminate clinical trials of our product candidates for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks; • regulators or IRBs may require us to perform additional or unanticipated clinical trials to obtain approval or we may be subject to additional post- marketing testing requirements to maintain regulatory approval; • regulators may revise the requirements for approving our product candidates, or such requirements may not be as we anticipate; • the cost of clinical trials of our product candidates may be greater than we anticipate; • the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; • our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or IRBs to suspend or terminate the trials; • unforeseen global instability, including political instability, such as the Russian invasion of Ukraine **or recent hostilities in Israel and Gaza Strip** , or instability from an outbreak of pandemic or contagious disease ~~, such as the ongoing COVID- 19 pandemic,~~ in or around the countries in which we conduct our clinical trials, could delay the commencement or rate of completion of our clinical trials; and • regulators may withdraw their approval of a product or impose restrictions on its distribution, such as in the form of a risk evaluation and mitigation strategy, or REMS. For example, in response to the ~~ongoing~~ COVID- 19 pandemic, the clinical trial sites for our ReDUX4 trial temporarily postponed trial-related activities, impacting our clinical trial execution plans, and we cannot be certain that we will not face other postponements or similar difficulties in the future. **Further** ~~In addition~~ , ~~on~~ **in** February ~~23,~~ 2023, the FDA imposed a clinical hold on our IND for **pociredir** ~~FTX- 6058~~ in SCD **, which halted our clinical trial until the FDA lifted the clinical hold in August 2023** . If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may: • 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 or a REMS that includes significant use or distribution restrictions or safety warnings; • be subject to additional post- marketing testing requirements; or • have the product removed from the market after obtaining marketing approval. Our product development costs will also increase if we experience delays in testing or in obtaining marketing approvals. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. We may also determine to change the design or protocol of one or more of our clinical trials, including to add additional patients or arms, which could result in increased costs and expenses and / or delays. Significant preclinical study or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates and may harm our business and results of operations. Because we are developing some of our product candidates for the treatment of diseases in which there is limited clinical experience and, in some cases, using new endpoints or methodologies, the FDA or other regulatory authorities may not consider the endpoints of our clinical trials to predict or provide clinically meaningful results. There are currently no therapies approved to treat FSHD, and there may be no therapies approved to treat the underlying causes of diseases that we attempt to address or may address in the future. As a result, the design and conduct of a clinical trial or trials of the product candidates for the treatment of these diseases may take longer, be more costly or be less effective as part of the novelty of development in these diseases. In some cases, we may use new or novel endpoints or

methodologies, such as RWS, which has not been proven for registration to our knowledge. The FDA and other regulatory authorities have indicated support for RWS as a primary endpoint with additional and appropriate supportive data from secondary endpoints. However, such regulatory authorities may not consider the endpoints of our clinical trial (s) to provide clinically meaningful results, even where we believe such results are clinically meaningful. For example, while we have met with regulators to discuss the REACH trial design and registration strategy for losmapimod for FSHD, including our proposed endpoints for REACH, regulators may require additional data to support the RWS functional primary endpoint for approval of losmapimod for FSHD. Even if the FDA does find our primary endpoint to be sufficiently validated and clinically meaningful, we may not achieve the pre- specified endpoint to a magnitude, duration or degree of statistical significance in any pivotal or other clinical trials we may conduct for our product candidates. Even if we do meet the primary endpoint, our trials may produce results that are unpredictable or inconsistent with the results of the other, more traditional efficacy endpoints in the trials. The FDA also could ascribe substantial weight to other efficacy endpoints when interpreting the clinical trial data, such that even if we achieve statistically significant results on our primary endpoint, the FDA may regard the failure to show a statistically significant effect on our secondary efficacy endpoints as raising questions about the efficacy of the drug. The FDA also weighs the benefits of a product against its risks and the FDA may view the efficacy results in the context of safety as not being supportive of approval. Other regulatory authorities in Europe and other countries may make similar findings with respect to these endpoints. If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented. Identifying and qualifying patients to participate in and complete clinical trials for our product candidates is critical to our success. Successful and timely completion of clinical trials will require that we enroll a sufficient number of patients who remain in the trial until its conclusion. For example, in our Phase 1b trial of **pociredir** ~~FTX- 6058 (which is currently on clinical hold)~~ , although we enrolled six subjects in the initial cohort, only three subjects remained evaluable as of the initial data cutoff date. Subsequently, we modified the study protocol to monitor subject adherence. However, if such protocols do not improve adherence and improve compliance ~~once the trial resumes (if at all)~~ , we may not be able to generate meaningful data. Furthermore, we may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside of the United States **. We revised the design of our clinical trial of pociredir in SCD to address the clinical hold imposed by the FDA, and there can be no certainty as to whether we will be successful in completing the clinical trial with its revised design, which include updated inclusion and exclusion criteria and thus a narrower set of eligible patients** . Because of our primary focus on genetically- defined rare diseases, we may have difficulty enrolling a sufficient number of eligible patients. Patient enrollment is affected by a variety of other factors, including: • the prevalence and severity of the disease under investigation; • the eligibility criteria for the trial in question; • the perceived risks and benefits of the product candidate under trial; • the requirements of the trial protocols, including invasive procedures such as muscle biopsies or medical resonance imaging (MRI), which requires the use of specialized equipment; • the availability of existing treatments for the indications for which we are conducting clinical trials; • the ability to recruit clinical trial investigators with the appropriate competencies and experience; • the efforts to facilitate timely enrollment in clinical trials; • the patient referral practices of physicians; • the ability to monitor patients adequately during and after treatment; • the proximity and availability of clinical trial sites for prospective patients; • the conduct of clinical trials by competitors for product candidates that treat the same indications as our product candidates; • the ability to identify specific patient populations for biomarker- defined trial cohort (s); and • the cost to, or lack of adequate compensation for, prospective patients. Our inability to locate and enroll a sufficient number of patients for our clinical trials would result in significant delays, could require us to abandon one or more clinical trials altogether and could delay or prevent our receipt of necessary regulatory approvals. Enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing. If serious adverse events or unacceptable side effects are identified during the development of our product candidates, we may need to abandon or limit our development of some of our product candidates. If our product candidates are associated with serious adverse events or undesirable side effects in clinical trials or have characteristics that are unexpected in clinical trials or preclinical testing, we may need to abandon their development or limit development to more narrow uses or subpopulations in which the serious adverse events, undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk- benefit perspective. For example, ~~on~~ **in** February ~~23,~~ 2023, the FDA placed our IND for **pociredir** ~~FTX- 6058~~ on clinical hold based on hematological malignancies observed in nonclinical toxicology studies. **We** ~~While we intend to address~~ **addressed** the FDA' s ~~concerns~~ **concern** as diligently as possible, including FDA' s request for information about an SCD patient population with an appropriate benefit- risk profile for further clinical development of **pociredir** ~~FTX- 6058~~ , and FDA' s request for information to define the potential risk in any further studies that may be conducted in healthy volunteers **. Although** ~~, there is no guarantee that~~ the FDA **lifted** ~~will allow us to resume clinical development of FTX- 6058. Even if the FDA lifts~~ the clinical hold **in August 2023** ~~and allows clinical studies of FTX- 6058 to resume~~ , we cannot make assurances that patients treated with **pociredir** ~~FTX- 6058~~ will not develop hematological malignancies or other adverse events in the future. We also cannot make assurances that additional observations in preclinical studies of hematological malignancies or other adverse events will not occur. If such additional adverse events were to emerge, further advancement of our clinical studies could be halted or delayed and we may not receive regulatory approval for **pociredir** ~~FTX- 6058~~ . Even if we receive regulatory approval for **pociredir** ~~FTX- 6058~~ , our labeling may be restricted and / or market acceptance for our product may be diminished, and the commercial potential of our **pociredir** ~~FTX- 6058~~ program may be materially and negatively impacted. In pharmaceutical development, many compounds that initially show promise in early- stage or clinical testing are later found to cause side effects that delay or prevent further development of the compound. Additionally, if results of our clinical trials reveal unacceptable side effects, we, the FDA or the IRBs at the institutions in which our studies are conducted could suspend or terminate our clinical trials or the

FDA or comparable foreign regulatory authorities could order us to cease clinical trials or deny approval of our product candidates for any or all targeted indications. Treatment- related side effects could also affect patient recruitment or the ability of enrolled patients to complete any of our clinical trials. If we elect or are forced to suspend or terminate any clinical trial of our product candidates, the commercial prospects of such product candidate will be harmed, and our ability to generate product revenue from such product candidate will be delayed or eliminated. Any of these occurrences could materially harm our business. If any of our product candidates receives marketing approval and we, or others, later discover that the drug is less effective than previously believed or causes undesirable side effects that were not previously identified, our ability to market the drug could be compromised. Clinical trials of our product candidates are conducted in carefully defined subsets of patients who have agreed to enter into clinical trials. Consequently, it is possible that our clinical trials 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 undesirable side effects. If one or more of our product candidates receives regulatory approval, and we, or others, later discover that they are less effective than previously believed, or cause undesirable side effects, a number of potentially significant negative consequences could result, including: • withdrawal or limitation by regulatory authorities of approvals of such product; • seizure of the product by regulatory authorities; • recall of the product; • restrictions on the marketing of the product or the manufacturing process for any component thereof; • requirement by regulatory authorities of additional warnings on the label, such as a " black box " warning or contraindication; • requirement that we implement a REMS or create a medication guide outlining the risks of such side effects for distribution to patients; • commitment to expensive post- marketing studies as a prerequisite of approval by regulatory authorities of such product; • the product may become less competitive; • initiation of regulatory investigations and government enforcement actions; • initiation of legal action against us to hold us liable for harm caused to patients; and • harm to our reputation and resulting harm to physician or patient acceptance of our products. Any of these events could prevent us from achieving or maintaining market acceptance of a particular product candidate, if approved, and could significantly harm our business, financial condition, and results of operations. 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 are focusing our research and development efforts on rare neuromuscular, muscular, hematologic and central nervous system disorders. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later 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 products. 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 such product candidate. Failure to allocate resources or capitalize on strategies in a successful manner will have an adverse impact on our business. We are conducting clinical trials of losmapimod in patients with FSHD in Europe, the United Kingdom, and Canada and currently plan to conduct additional clinical trials for our product candidates at sites outside the United States, and the FDA may not accept data from trials conducted in such locations. We are currently conducting a Phase 3 clinical trial, an open label extension of our Phase 2b clinical trial, and an open label extension of our Phase 2 open label clinical trial of losmapimod in patients with FSHD in Europe, the United Kingdom, and Canada. We may also conduct additional clinical trials outside the United States. Although the FDA may accept data from clinical trials conducted outside the United States, acceptance of these data is subject to conditions imposed by the FDA. For example, the clinical trial must be well designed and conducted and be performed by qualified investigators in accordance with ethical principles. The trial population must also adequately represent the U. S. population, and the data must be applicable to the U. S. population and U. S. medical practice in ways that the FDA deems clinically meaningful. In addition, while these clinical trials are subject to the applicable local laws, FDA acceptance of the data will depend on its determination that the trials also complied with all applicable U. S. laws and regulations, including good clinical practices, and FDA' s ability to validate the data. If the FDA does not accept the data from any trial that we conduct outside the United States, it would likely result in the need for additional trials, which would be costly and time- consuming and could delay or permanently halt our development of the applicable product candidates. Risks Related to the Commercialization of our Product Candidates Even if any of our product candidates receives marketing approval, it 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, and the market opportunity for any of our product candidates, if approved, may be smaller than we estimate. If any of our product candidates receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third- party payors and others in the medical community. Efforts to educate the medical community and third- party payors on the benefits of our product candidates may require significant resources and may not be successful. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including: • the efficacy and potential advantages of our product candidates compared to the advantages and relative risks of alternative treatments; • the effectiveness of sales and marketing efforts; • the cost of treatment in relation to alternative treatments, including any similar generic treatments; • our ability to offer our products, if approved, for sale at competitive prices; • the clinical indications for which the product is approved; • the convenience and ease of administration compared to alternative treatments; • the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies; • the strength of marketing and distribution support; • the timing of market introduction of competitive products; • the availability of third- party coverage and adequate reimbursement, and patients' willingness to pay out of pocket for required co- payments or in the absence of third- party coverage or adequate reimbursement; • the prevalence and severity of any side effects; and • any

restrictions on the use of our products, if approved, together with other medications. Our assessment of the potential market opportunity for our product candidates is based on industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties, one of which we commissioned. Industry publications and third- party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While we believe these industry publications and third- party research, surveys and studies are reliable, we have not independently verified such data. Our estimates of the potential market opportunities for our product candidates include several key assumptions based on our industry knowledge, industry publications and third- party research, surveys and studies, which may be based on a small sample size and fail to accurately reflect market opportunities. While we believe that our internal assumptions are reasonable, no independent source has verified such assumptions. If any of our assumptions or estimates, or these publications, research, surveys or studies prove to be inaccurate, then the actual market for any of our product candidates may be smaller than we expect, and as a result our product revenue may be limited and it may be more difficult for us to achieve or maintain profitability. If we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution agreements with third parties, we may not be successful in commercializing our product candidates if and when they are approved. We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any product for which we have obtained marketing approval, we will need to establish a sales, marketing and distribution organization, either ourselves or through collaborations or other arrangements with third parties. In the future, we expect to build a focused, specialty sales and marketing infrastructure to market some of our product candidates in the United States, if and when they are approved. There are risks involved with establishing our own sales, marketing and distribution capabilities. For example, recruiting and training a sales force is expensive and time- consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. These efforts may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel. Factors that may inhibit our efforts to commercialize our products on our own include: • our inability to recruit, train and retain adequate numbers of effective sales, marketing, coverage or reimbursement, customer service, medical affairs and other support personnel; • the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future products; • the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement and other acceptance by payors; • the inability to price our products at a sufficient price point to ensure an adequate and attractive level of profitability; • restricted or closed distribution channels that make it difficult to distribute our products to segments of the patient population; • 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 product lines; and • unforeseen costs and expenses associated with creating an independent sales and marketing organization. If we are unable to establish our own sales, marketing and distribution capabilities and we enter into arrangements with third parties to perform these services, our product revenues and our profitability, if any, are likely to be lower than if we were to market, sell and distribute any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute our product candidates or may be unable to do so on terms that are acceptable to us. We likely will have little 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 successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates. 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 drug products is highly competitive. We face competition with respect to our current product candidates, and will face competition with respect to any product candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of many of the disease indications for which we are developing our product candidates. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. For example, we are aware of several product candidates in clinical development that could be competitive with product candidates that we may successfully develop and commercialize. See Item 1 “ Business — Competition ” in this Annual Report on Form 10- K. 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 may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. **For example, on December 8, 2023, the FDA approved CASGEVY (exagamglogene autotemcel) and LYFGENIA (lovotibeglogene autotemcel), the first ex vivo cell- based gene therapies for the treatment of SCD. CASGEVY has also been FDA- approved for the treatment of transfusion- dependent beta- thalassemia.** In addition, our ability to compete may be affected in many cases by insurers or other third- party payors seeking to encourage the use of generic products. If our product candidates achieve marketing approval, we expect that they will be priced at a significant premium over competitive generic products. Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory

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 and other early- stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. If the market opportunities for our product candidates are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer. Because certain of the target patient populations of our product candidates are small, and the addressable patient population even smaller, we must be able to successfully identify patients and capture a significant market share to achieve profitability and growth. We primarily focus our research and product development on treatments for genetically- defined rare diseases. Given the small number of patients who have the rare diseases that we are targeting, it is critical to our ability to grow and become profitable that we continue to successfully identify patients with these rare diseases. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, surveys of clinics, patient foundations or market research that we conducted, and may prove to be incorrect or contain errors. New studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. The effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. Additionally, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business. Further, even if we obtain significant market share for our product candidates, because the potential target populations for many of the indications we are evaluating are very small, we may never achieve profitability despite obtaining such significant market share. The target patient populations for some of the indications we are evaluating are relatively small, and there is currently no standard of care treatment directed at some of our target indications, such as FSHD. As a result, the pricing and reimbursement of our product candidates, if approved, is uncertain, but must be adequate to support commercial infrastructure. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our product candidates will be adversely affected. We rely, and expect to continue to rely, on CMOs to manufacture our product candidates. If we are unable to enter into such arrangements as expected or if such organizations do not meet our supply requirements, development and / or commercialization of our product candidates may be delayed. We do not have any manufacturing facilities and rely, and expect to continue to rely, on third parties to manufacture clinical supplies of our product candidates and we expect to rely on third parties to manufacture commercial supplies of our products, if and when approved for marketing by applicable regulatory authorities, as well as for packaging, sterilization, storage, distribution and other production logistics. If we are unable to enter into such arrangements on the terms or timeline we expect, development and / or commercialization of our product candidates may be delayed. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or manufacture our product candidates in accordance with regulatory requirements, if there are disagreements between us and such parties or if such parties are unable to expand capacities to support commercialization of any of our product candidates for which we obtain marketing approval, we may not be able to fulfill, or may be delayed in producing sufficient product candidates to meet, our supply requirements, or we may be forced to manufacture the materials ourselves, for which we may not have the capabilities or resources, or enter into an agreement with a different manufacturer, which we may not be able to do on reasonable terms, if at all. In either scenario, our clinical trial supply could be delayed significantly as we establish alternative supply sources. In some cases, the technical skills required to manufacture our products or product candidates may be unique or proprietary to the original manufacturer and we may have difficulty, or there may be contractual restrictions prohibiting us from, transferring such skills to a back- up or alternate supplier, or we may be unable to transfer such skills at all. In addition, if we are required to change manufacturers for any reason, we will be required to verify that the new manufacturer maintains facilities and procedures that comply with quality standards and with all applicable regulations. We will also need to verify, such as through a manufacturing comparability study, that any new manufacturing process will produce our product candidate according to the specifications previously submitted to the FDA or another regulatory authority. The delays associated with the verification of a new manufacturer could negatively affect our ability to develop product candidates or commercialize our products in a timely manner or within budget. Furthermore, a manufacturer may possess technology related to the manufacture of our product candidate that such manufacturer owns independently. This would increase our reliance on such manufacturer or require us to obtain a license from such manufacturer in order to have another manufacturer manufacture our product candidates. In addition, changes in manufacturers often involve changes in manufacturing procedures and processes, which could require that we conduct bridging studies between our prior clinical supply used in our clinical trials and that of any new manufacturer. We may be unsuccessful in demonstrating the comparability of clinical supplies which could require the conduct of additional clinical trials. These facilities may also be affected by natural disasters, such as floods or fire, as well as public health issues (for example, an outbreak of a contagious disease such as COVID- 19), or such facilities could face manufacturing issues, such as contamination or regulatory concerns following a regulatory inspection of such facility. Our third-party manufacturers will be subject to inspection and approval by the FDA before we can commence the manufacture and sale of any of our product candidates, and thereafter subject to FDA inspection from time to time. Failure by our third- party manufacturers to pass such inspections and otherwise satisfactorily complete the FDA approval regimen with respect to our product candidates may result in regulatory actions such as the issuance of FDA Form 483 notices of observations, warning letters or injunctions or the loss of operating licenses. We or our third- party manufacturers may also encounter shortages in the raw materials or API necessary to produce our product candidates in the quantities needed for our clinical trials or, if our product

candidates are approved, in sufficient quantities for commercialization or to meet an increase in demand, as a result of capacity constraints or delays or disruptions in the market for the raw materials or API, including shortages caused by the purchase of such raw materials or API by our competitors or others. The failure of us or our third- party manufacturers to obtain the raw materials or API necessary to manufacture sufficient quantities of our product candidates, may have a material adverse effect on our business. Our reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or products or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts. We do not have any manufacturing facilities. Although we believe we have obtained sufficient losmapimod tablets from GSK to complete our ongoing clinical trials of losmapimod for the treatment of FSHD, we cannot be sure we have correctly estimated our drug product and API requirements or that such drug product or API will not expire before we want to use it. We have also engaged CMOs to prepare our own API and to manufacture losmapimod tablets. Although we believe we have produced sufficient losmapimod tablets to complete our planned Phase 3 registrational trial, we cannot be sure we have correctly estimated our drug product and API requirements or that such drug product or API will not expire before we want to use it. In addition, although we believe we have obtained sufficient quantities of **pociredir** ~~FTX- 6058~~ from a CMO for the completion of our Phase 1b clinical trial for SCD ~~if we are able to resolve the clinical hold~~, we cannot be sure we have correctly estimated our drug product requirements, which could delay, prevent or impair our development efforts. We expect to rely on third parties for the manufacture of **pociredir** ~~FTX- 6058~~ for any future clinical trials and for the manufacture of any future product candidates for preclinical and clinical testing. We also expect to rely on third- party manufacturers or third- party collaborators for the manufacture of commercial supply of any other product candidates for which we or our collaborators obtain marketing approval. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or products or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts. Our product candidates and any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us. Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. We do not currently have arrangements in place for redundant supply or a source for bulk drug substance. If any of our future contract manufacturers cannot perform as agreed, we may be required to replace such manufacturers. Although we believe that there are several potential alternative manufacturers who could manufacture our product candidates, we may incur added costs and delays in identifying and qualifying any such replacement. Our current and anticipated future dependence upon others for the manufacture of our product candidates or products may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis. Even if we are able to commercialize any product candidates, the products may become subject to unfavorable pricing regulations, third- party coverage or reimbursement practices or healthcare reform initiatives, which could harm our business. 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 might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval. See Item 1 " Business — Government Regulation and Product Approval — Pharmaceutical Insurance Coverage and Health Care Reform " in this Annual Report on Form 10- K. Our ability to commercialize any product candidates successfully also will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third- party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U. S. healthcare industry and elsewhere is cost containment. Government authorities and third- party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third- party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. Coverage and reimbursement may not be available for any product that we commercialize and, even if these are available, the level of reimbursement may not be satisfactory. Reimbursement may affect the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining and maintaining adequate reimbursement for our products may be difficult. We may be required to conduct expensive pharmacoeconomic studies to justify coverage and reimbursement or the level of reimbursement relative to other therapies. If coverage and adequate reimbursement are not available or reimbursement is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval. Coverage and reimbursement by a third- party payor may depend upon a number of factors, including the third- party payor' s determination that use of a product is: • a covered benefit under its health plan; • safe, effective and medically necessary; • appropriate for the specific patient; • cost- effective; and • neither experimental nor investigational. There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or similar regulatory authorities outside of the United States. Moreover, eligibility for coverage and reimbursement does not imply that a drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution expenses. Interim reimbursement levels for new drugs, if applicable, may also not be

sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third- party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to promptly obtain coverage and adequate reimbursement rates from both government- funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition. There can be no assurance that our product candidates, even if they are approved for sale in the United States or in other countries, will be considered medically reasonable and necessary for a specific indication or cost- effective by third- party payors, or that coverage and an adequate level of reimbursement will be available or that third- party payors' reimbursement policies will not adversely affect our ability to sell our product candidates profitably. Our future growth depends, in part, on our ability to penetrate foreign markets, where we would be subject to additional regulatory burdens and other risks and uncertainties that, if they materialize, could harm our business. Our future profitability will depend, in part, on our ability to commercialize our product candidates in markets outside of the United States and the European Union. If we commercialize our product candidates in foreign markets, we will be subject to additional risks and uncertainties, including: • economic weakness, including inflation, or political instability in particular economies and markets, which could include localized disputes that have a broader regional or global impact (such as the Russian invasion of Ukraine **or recent hostilities in Israel and Gaza Strip**); • the burden of complying with complex and changing foreign regulatory, tax, accounting and legal requirements, many of which vary between countries; • different medical practices and customs in foreign countries affecting acceptance in the marketplace; • tariffs and trade barriers, as well as other governmental controls and trade restrictions; • other trade protection measures, import or export licensing requirements or other restrictive actions by U. S. or foreign governments; • longer accounts receivable collection times; • longer lead times for shipping; • compliance with tax, employment, immigration and labor laws for employees living or traveling abroad; • workforce uncertainty in countries where labor unrest is common; • language barriers for technical training; • reduced protection of intellectual property rights in some foreign countries, and related prevalence of generic alternatives to therapeutics; • foreign currency exchange rate fluctuations and currency controls; • differing foreign reimbursement landscapes; • uncertain and potentially inadequate reimbursement of our products; and • the interpretation of contractual provisions governed by foreign laws in the event of a contract dispute. If risks related to any of these uncertainties materializes, it could have a material adverse effect on our business. Clinical trial and product liability lawsuits against us could divert our resources and could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop. We face an inherent risk of clinical trial and product liability exposure related to the testing of our product candidates in human clinical trials, and we will face an even greater risk if we commercially sell any products that we may develop. While we currently have no products that have been approved for commercial sale, the current and future use of product candidates by us in clinical trials, and the sale of any approved products in the future, may expose us to liability claims. These claims might be made by patients that use the product, healthcare providers, pharmaceutical companies or others selling such products. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in: • decreased demand for any product candidates or products that we may develop; • injury to our reputation and significant negative media attention; • withdrawal of clinical trial participants; • significant costs to defend any related 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 commercialize any products that we may develop. We currently hold $ 10 million in clinical trial liability insurance coverage in the aggregate, with a per incident limit of $ 10 million, which may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials or if we commence commercialization of our product candidates. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. If a successful clinical trial or product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired. Risks Related to our Dependence on Third Parties We rely, and expect to continue to rely, on third parties to conduct our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, which may harm our business. We currently rely on third- party contract CROs to conduct our clinical trials. We plan to rely on third- party CROs or third- party research collaboratives to conduct any future clinical trials. We do not plan to independently conduct clinical trials of our other product candidates. We expect to continue to rely on third parties, such as CROs, clinical data management organizations, medical institutions and clinical investigators, to conduct our clinical trials. These agreements might terminate for a variety of reasons, including a failure to perform by the third parties. If we need to enter into alternative arrangements, our product development activities might be delayed. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. 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 will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully develop and commercialize our product candidates. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. We also rely, and expect to continue to rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our

products, producing additional losses and depriving us of potential product revenue. We have entered into, and may in the future enter into, collaborations with third parties for the discovery, development or commercialization of our product candidates. If our collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates and our business could be adversely affected. We have a collaboration and license agreement with MyoKardia (for certain genetically defined cardiomyopathies). See Item 1 " Business — License Agreements and Collaborations " in this Annual Report on Form 10- K. While we have retained all rights to and are developing on our own our current product candidates, we may in the future enter into development, distribution or marketing arrangements with third parties with respect to our other existing or future product candidates. Our likely collaborators for any such sales, marketing, distribution, development, licensing or broader collaboration arrangements include large and mid- size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we enter into any such arrangements with any third parties in the future, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities and efforts to successfully perform the functions assigned to them in these arrangements. Collaborations that we enter into, including our collaboration with MyoKardia, may not be successful, and any success will depend heavily on the efforts and activities of such collaborators. Collaborations pose a number of risks, including the following: • collaborators have significant discretion in determining the amount and timing of efforts and resources that they will apply to these collaborations; • collaborators may not perform their obligations as expected; • collaborators may not pursue development of our product candidates or may elect not to continue or renew development programs based on results of clinical trials or other studies, 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 not pursue commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew commercialization programs based on results of clinical trials or other studies, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that may 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; • we may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such product candidates on a discretionary basis; • collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates and products if the collaborators believe that the competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours; • product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates; • a collaborator may fail to comply with applicable regulatory requirements regarding the development, manufacture, distribution or marketing of a product candidate or product; • a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products; • disagreements with collaborators, including disagreements over intellectual property or proprietary rights, contract interpretation or the preferred course of development, might cause delays or terminations 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 obtain, maintain, enforce, defend or protect our intellectual property or proprietary rights or may use our proprietary information in such a way as to potentially lead to disputes or legal proceedings that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation; • disputes may arise with respect to the ownership of intellectual property developed pursuant to our collaborations; • collaborators may infringe, misappropriate or otherwise violate the intellectual property or proprietary rights of third parties, which may expose us to litigation and potential liability; and • collaborations may be terminated for the convenience of the collaborator (e. g., termination of our collaboration with Acceleron **Pharma, Inc., or Acceleron,** following its acquisition by **Merck & Co., or** Merck), and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates. Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner, or at all. If any collaborations that we enter into do not result in the successful development and commercialization of products or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of our product candidates could be delayed and we may need additional resources to develop our product candidates. All of the risks relating to product development, regulatory approval and commercialization described herein also apply to the activities of our collaborators. Additionally, subject to its contractual obligations to us, if a collaborator of ours is involved in a business combination, the collaborator might deemphasize or terminate the development or commercialization of any product candidate licensed to it by us. For example, in November 2020, subsequent to our entering into the MyoKardia collaboration agreement, MyoKardia was acquired by Bristol- Myers Squibb Company. Bristol- Myers Squibb Company could determine to reprioritize MyoKardia' s development programs such that it ceases to diligently pursue the development of our programs and / or cause the agreement between MyoKardia and us to terminate. If our collaborator terminates its agreement with us, we may find it more difficult to attract new collaborators and our perception in the business and financial communities could be adversely affected. If we are not able to establish or maintain collaborations, we may have to alter our development and commercialization plans and our business could be adversely affected. For some of our product candidates, we may decide to collaborate with pharmaceutical or biotechnology companies for

the development and potential commercialization of those product candidates. For example, in July 2020, we entered into a collaboration and license agreement with MyoKardia to identify and validate potential biological targets for the potential treatment of certain genetically defined cardiomyopathies. We face significant competition in seeking appropriate collaborators, and a number of more established companies may also be pursuing strategies to license or acquire third- party intellectual property rights that we consider attractive. These established companies may have a competitive advantage over us due to their size, financial resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. 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 design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. We may also be restricted under existing or future license agreements from entering into agreements on certain terms with potential collaborators. For example, we are restricted by GSK' s right of first negotiation under our current license agreement with them , and we had restrictions under our collaboration with Acceleron. Under our collaboration with MyoKardia, we are restricted from researching, developing, manufacturing, commercializing, using, or otherwise exploiting any compound or product (a) that is a compound or product under the agreement that is directed against certain targets identified by us in the performance of the research activities for the treatment, prophylaxis, or diagnosis of any indication or (b) for the treatment of any genetically defined cardiomyopathies shown to be related to certain specified genes of interest that are modulated by the targets chosen by MyoKardia under our collaboration, in each case, while we are performing the research activities pursuant to the research plan and for a specified period thereafter. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical and biotechnology companies that have resulted in a reduced number of potential future collaborators. If we are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms or at all, we may have to curtail the development of a product candidate, 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. If we elect to fund and undertake development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates or bring them to market ~~or continue to develop our product engine~~ . Risks Related to our Intellectual Property If we are unable to obtain, maintain, enforce and protect patent protection for our technology and product candidates or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize technology and products similar or identical to ours, and our ability to successfully develop and commercialize our technology and product candidates may be adversely affected. Our success depends in large part on our ability to obtain and maintain protection of the intellectual property we may own solely and jointly with others or may license from others, particularly patents, in the United States and other countries with respect to any proprietary technology and product candidates we develop. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our product candidates that are important to our business and by in- licensing intellectual property related to our technologies and product candidates. If we are unable to obtain or maintain patent protection with respect to any proprietary technology or product candidate, our business, financial condition, results of operations and prospects could be materially harmed. The patent prosecution process is expensive, time- consuming and complex, and we may not be able to file, prosecute, maintain, defend or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, in some circumstances, we do not have the right to control the preparation, filing and prosecution of patent applications, or to maintain, enforce and defend the patents, covering technology that we license from third parties. Therefore, these in- licensed patents and applications may not be prepared, filed, prosecuted, maintained, defended and enforced in a manner consistent with the best interests of our business. The patent position of pharmaceutical and biotechnology companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the scope of patent protection outside of the United States is uncertain and laws of foreign countries may not protect our rights to the same extent as the laws of the United States or vice versa. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. With respect to both owned and in- licensed patent rights, we cannot predict whether the patent applications we and our licensors are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient protection from competitors. Further, we may not be aware of all third- party intellectual property rights potentially relating to our product candidates. In addition, 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 published at all. Therefore, neither we nor our licensors can know with certainty whether either we or our licensors were the first to make the inventions claimed in the patents and patent applications we own or in- license now or in the future, or that either we or our licensors were the first to file for patent protection of such inventions. As a result, the issuance,

scope, validity, enforceability and commercial value of our owned and in- licensed patent rights are highly uncertain. Moreover, our owned and in- licensed pending and future patent applications may not result in patents being issued which protect our technology and product candidates, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. 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 and our ability to obtain, protect, maintain, defend and enforce our patent rights, narrow the scope of our patent protection and, more generally, could affect the value or narrow the scope of our patent rights. For information relating to our patent portfolio, see Item 1 " Business — Intellectual Property " in this Annual Report on Form 10- K. Moreover, we or our licensors may be subject to a third- party preissuance submission of prior art to the United States Patent and Trademark Office, or USPTO, or become involved in opposition, derivation, revocation, reexamination, inter partes review, post- grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or product candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize drugs without infringing third- party patent rights. If the breadth or strength of protection provided by our patents and patent applications is threatened, regardless of the outcome, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates. For example, while we believe that the specific and generic claims contained in our U. S. patent provide protection for the method of using losmapimod for the treatment of FSHD and while we also believe that the specific and generic claims contained in our issued and pending U. S. non- provisional and provisional applications provide protection for the pharmaceutical compositions and methods of use for **pociredir** ~~FTX- 6058~~, third parties may nevertheless challenge such claims. If any such claims are invalidated or rendered unenforceable for any reason, we will lose valuable intellectual property rights and our ability to prevent others from competing with us would be impaired. Additionally, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if our owned and in- licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and in- licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate 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 product candidates. Such proceedings also may result in substantial cost and require significant time from our management and employees, even if the eventual outcome is favorable to us. 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, our competitors may be able to circumvent our owned or in- licensed patents by developing similar or alternative technologies or products in a non- infringing manner. As a result, our owned and in- licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing technology and products similar or identical to any of our technology and product candidates. Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time. Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U. S. non- provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics or biosimilars. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. For example, the composition of matter patents covering losmapimod, licensed from GSK have expired and are no longer a barrier to entry for any new uses not covered by our other patents and patent applications. 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 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 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, thereby giving our competitors access to the same technologies licensed to us. If we are unable to obtain rights to required third- party intellectual property rights or maintain the existing intellectual property rights we have, we may be required to expend significant time and resources to redesign our technology, product candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected technology and product candidates, which could harm our business, financial condition, results of operations and prospects significantly. Additionally, 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 candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements, or restrictions on our ability to freely assign or

sublicense our rights under such agreements when it is in the interest of our business to do so, 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, or delay or prohibit the further development or commercialization of one or more product candidates that rely on such agreements. Under our current license agreements, we may not have the final or sole decision on whether we are able to opt out certain of our in- licensed European patents and patent applications from the recently created Unified Patent Court, or the UPC, for the European Union ~~, that is expected to be fully ratified in 2023~~ . ~~Our~~ **While our** licensors ~~may~~ **have** ~~decide~~ **decided** to ~~not~~ opt out ~~the~~ of the UPC, ~~which would subject~~ **we cannot guarantee that** our in- licensed European patents and patent applications ~~to~~ **will be challenged for non- compliance during the opt- out procedure and if successful, brought under** the jurisdiction of the UPC **nor can** ~~. Furthermore, even if our licensors decide to opt out of the UPC,~~ we ~~cannot~~ guarantee that our licensors will **decide to opt back into** ~~comply with the legal formalities and requirements for properly opting out of~~ the UPC **at a later time** . Thus, we cannot be certain that our in- licensed European patents and patent applications will not fall under the jurisdiction of the UPC. Under the UPC, a single European patent would be valid and enforceable in numerous European countries. A challenge to the validity of a European patent under the UPC, if successful, could result in a loss of patent protection in numerous European countries which could have a material adverse impact on our business and our ability to commercialize or license our technology and product candidates. If we do not obtain patent term extension in the United States under the Hatch- Waxman Act and in foreign countries under similar legislation, our business may be materially harmed. In the United States, the patent term of a patent that covers an FDA- approved drug may be eligible for limited patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to, among other factors, the length of time the drug is under regulatory review, but such patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, and only one eligible patent may be extended. Similar provisions are available in Europe and certain other jurisdictions outside the United States. If and when our product candidates receive FDA approval, we expect to apply for patent term extensions where applicable, but there is no guarantee that the applicable governmental authorities will agree with our assessment of whether such extensions should be granted, and even if granted, the length of such extensions. We may not be granted patent term extension either in the United States or in any foreign country 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. If we are unable to obtain any patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following the expiration of our patent rights, and our business, financial condition, results of operations and prospects could be materially harmed. Further, for our licensed patents, we may not have the right to control prosecution, including filing with the USPTO a petition for patent term extension thus if one of our licensed patents is eligible for patent term extension, we may not be able to control whether a petition to obtain a patent term extension is filed, or obtained, from the USPTO. There are detailed rules and requirements regarding the patents that may be submitted to the FDA for listing in the Orange Book. We may be unable to obtain patents covering our product candidates that contain one or more claims that satisfy the requirements for listing in the Orange Book. Even if we submit a patent for listing in the Orange Book, the FDA may decline to list the patent, or a manufacturer of generic drugs may challenge the listing. If one of our product candidates is approved and a patent covering that product candidate is not listed in the Orange Book, an ANDA applicant would not have to provide notice to us with respect to that patent. See Item 1 " Business — Intellectual Property " in this Annual Report on Form 10- K for additional information regarding patent laws and patent protection. **Our** ~~Issued~~ **issued European** patents ~~covering our product candidates~~ could be **subject to** ~~found invalid or unenforceable if challenged in court or~~ the ~~USPTO~~ **jurisdiction of the UPC** . Our European patents and patent applications could be challenged in the UPC ~~that is expected to be fully ratified in 2023~~ . We ~~may decide~~ **decided** to **remove, i. e.,** opt out **,** our European patents and patent applications from **the jurisdiction of** the UPC. However, if certain formalities and requirements are not met, our European patents and patent applications could be challenged for non- compliance and brought under the jurisdiction of the UPC. We cannot be certain that our European patents and patent applications will avoid falling under the jurisdiction ~~of the UPC, if we decide to opt out~~ of the UPC. Under the UPC, a granted European patent would be valid and enforceable in numerous European countries. Although such patent rights would apply to numerous European countries, a successful challenge to a European patent under the UPC could result in loss of patent protection in numerous European countries. Accordingly, a single proceeding under the UPC addressing the validity and infringement of the European patent could result in loss of patent protection in numerous European countries rather than in each validated country separately as such patents always have been adjudicated. Such a loss of patent protection could have a material adverse impact on our business and our ability to commercialize or license our technology and product candidates. Changes to patent laws in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products. Changes in either the patent laws or interpretation of patent laws in the United States, including patent reform legislation such as the Leahy- Smith America Invents Act, or the Leahy- Smith Act, could increase the uncertainties and costs surrounding the prosecution of our owned and in- licensed patent applications and the maintenance, enforcement or defense of our owned and in- licensed issued patents. The Leahy- Smith Act includes a number of significant changes to United States patent law. These changes include provisions that affect the way patent applications are prosecuted, redefine prior art, provide more efficient and cost- effective avenues for competitors to challenge the validity of patents, and enable third- party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent at USPTO- administered post- grant proceedings, including post- grant review, inter partes review, and derivation proceedings. Assuming that other requirements for patentability are met, prior to March 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy- Smith Act, the United States transitioned to a first- to- file system in which,

assuming that the other statutory requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. As such, the Leahy- Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects. In addition, the patent positions of companies in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Recent U. S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. This combination of events has created uncertainty with respect to the validity and enforceability of patents once obtained. Depending on future actions by the U. S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our patent rights and our ability to protect, defend and enforce our patent rights in the future. Although we or our licensors are not currently involved in any litigation ~~, we may become involved in lawsuits~~ to protect or enforce our patent or other intellectual property rights **, we may become involved in such lawsuits** , which could be expensive, time- consuming and unsuccessful. Competitors and other third parties may infringe, misappropriate or otherwise violate our or our licensor' s issued patents or other intellectual property. As a result, we or our licensors may need to file infringement, misappropriation or other intellectual property related claims, which can be expensive and time- consuming. Any claims we assert against perceived infringers could provoke such parties to assert counterclaims against us alleging that we infringe, misappropriate or otherwise violate their intellectual property. In addition, in a patent infringement proceeding, such parties could counterclaim that the patents we or our licensors have asserted are invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, or non- enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. Third parties may institute such claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re- examination, post- grant review, inter partes review, interference proceedings, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e. g., opposition proceedings). The outcome following legal assertions of invalidity and unenforceability is unpredictable. An adverse result in any such proceeding could put one or more of our owned or in- licensed patents at risk of being invalidated or interpreted narrowly, and could put any of our owned or in- licensed patent applications at risk of not yielding an issued patent. A court may also refuse to stop the third party from using the technology at issue in a proceeding on the grounds that our owned or in- licensed patents do not cover such technology. 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 or trade secrets could be compromised by disclosure during this type of litigation. Any of the foregoing could allow such third parties to develop and commercialize competing technologies and products and have a material adverse impact on our business, financial condition, results of operations and prospects. Interference or derivation proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms or at all, or if a non- exclusive license is offered and our competitors gain access to the same technology. Our defense of litigation or interference or derivation proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development partnerships that would help us bring our product candidates to market. 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 this type of 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 have a material adverse effect on the price of our common stock. Third parties may initiate legal proceedings alleging that we are infringing, misappropriating or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business. Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the intellectual property and proprietary rights of third parties. There is considerable patent and other intellectual property litigation in the pharmaceutical and biotechnology industries. We may become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our technology and product candidates, including interference proceedings, post grant review, inter partes review, and derivation proceedings before the USPTO and similar proceedings in foreign jurisdictions such as oppositions before the European Patent Office. Numerous U. S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our technologies or product candidates that we may identify may be subject to claims of infringement of the patent rights of third parties. The legal threshold for initiating litigation or contested proceedings is low, so that even lawsuits or proceedings with a low probability of success might be initiated and require significant resources to defend. Litigation and contested proceedings can also be expensive and time- consuming, and our adversaries in these proceedings may have the ability to dedicate substantially greater resources to prosecuting these legal actions than we can. The risks of being involved in such litigation and proceedings may increase if and as our product candidates near commercialization and as we gain the greater

visibility associated with being a public company. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of merit. We may not be aware of all such intellectual property rights potentially relating to our technology and product candidates and their uses, or we may incorrectly conclude that third party intellectual property is invalid or that our activities and product candidates do not infringe such intellectual property. Thus, we do not know with certainty that our technology and product candidates, or our development and commercialization thereof, do not and will not infringe, misappropriate or otherwise violate any third party' s intellectual property. Third parties may assert that we are employing their proprietary technology without authorization. There may be third- party patents or patent applications with claims to materials, formulations or methods, such as methods of manufacture or methods for treatment, related to the discovery, use or manufacture of the product candidates that we may identify or related to our technologies. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that the product candidates that we may identify may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Moreover, as noted above, there may be existing patents that we are not aware of or that we have incorrectly concluded are invalid or not infringed by our activities. If any third- party patents were held by a court of competent jurisdiction to cover, for example, the manufacturing process of the product candidates that we may identify, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize the product candidates that we may identify. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure. We may choose to take a license or, if we are found to infringe, misappropriate or otherwise violate a third party' s intellectual property rights, we could also be required to obtain a license from such third party to continue developing, manufacturing and marketing our technology and product candidates. 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 and other third parties access to the same technologies licensed to us and could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or product. In addition, we could be found liable for significant monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right and could be forced to indemnify our customers or collaborators. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. In addition, we may be forced to redesign our product candidates, seek new regulatory approvals and indemnify third parties pursuant to contractual agreements. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar material adverse effect on our business, financial condition, results of operations and prospects. Intellectual property litigation or other legal proceedings relating to intellectual property could cause us to spend substantial resources and distract our personnel from their normal responsibilities. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and may also have an advantage in such proceedings due to their more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of intellectual property litigation or other proceedings could compromise our ability to compete in the marketplace. 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, renewal and annuity fees and various other government fees on any issued patent and pending patent application must be paid to the USPTO and foreign patent agencies in several stages or annually over the lifetime of our owned and in- licensed patents and patent applications. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In certain circumstances, we rely on our licensing partners to pay these fees to, or comply with the procedural and documentary rules of, the relevant patent agency. With respect to our patents, we rely on an annuity service, outside firms and outside counsel to remind us of the due dates and to make payment after we instruct them to do so. 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 noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include failure to respond to official actions within prescribed time limits, non- payment of fees and failure to properly legalize and submit formal documents. In such an event, potential competitors might be able to enter the market with similar or identical products or technology. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, it would

have a material adverse effect on our business, financial condition, results of operations and prospects. If we fail to comply with our obligations in our intellectual property licenses and funding arrangements with third parties, or otherwise experience disruptions to our business relationships with our licensors, we could lose intellectual property rights that are important to our business. We are party to license and funding agreements, such as our agreement with GSK **and our recent license agreement with CAMP4** , and we may enter into additional licensing and funding arrangements with third parties that impose or may impose diligence, development and commercialization timelines, milestone payment, royalty, insurance and other obligations on us. Under our existing licensing and funding agreements, we are obligated to pay royalties on net product sales of product candidates or related technologies to the extent they are covered by the agreements. If we fail to comply with such obligations under current or future license and funding agreements, our counterparties may have the right to terminate these agreements or require us to grant them certain rights. Such an occurrence could materially adversely affect the value of any 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, or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology, which would have a material adverse effect on our business, financial condition, results of operations and prospects. We also have licenses and agreements to certain technologies **that we** ~~used~~ **use** in our ~~product engine~~ **discovery efforts** , all of which are non- exclusive. While we still face all of the risks described herein with respect to those agreements, we cannot prevent third parties from also accessing those technologies. In addition, our licenses may place restrictions on our future business opportunities. For example, under our license with GSK, GSK has certain rights of first negotiation if we wish to sublicense any of the patent or data rights licensed by GSK to us to a third party for use outside the United States. This may prevent or delay certain transactions, which could have an adverse effect on the development and commercialization of losmapimod and on our business. Disputes may arise regarding intellectual property subject to a licensing agreement, including: • the scope of rights granted under the license agreement and other interpretation related issues; • the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement; • the sublicensing of patent and other rights under our collaborative development relationships; • our diligence obligations under the license agreement and what activities satisfy those diligence obligations; • the inventorship and ownership of inventions and know- how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and • the priority of invention of patented technology. In addition, the agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected technology and product candidates, which could have a material adverse effect on our business, financial conditions, results of operations and prospects. Our current or future licensors may have relied on third- party consultants or collaborators or on funds from third parties such that our licensors are not the sole and exclusive owners of the patents and patent applications we in- license. If other third parties have ownership rights to patents or patent applications we in- license, they may be able to license such patents to our competitors, and our competitors could market competing products and technology. This could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects. In spite of our best efforts, our licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements, thereby removing our ability to develop and commercialize product candidates and technology covered by these license agreements. If these in- licenses are terminated, or if the underlying intellectual property fails to provide the intended exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products and technologies identical to ours. This could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects. We may not be able to protect our intellectual property and proprietary rights throughout the world. Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States, and even where such protection is nominally available, judicial and governmental enforcement of such intellectual property rights may be lacking. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection or licenses but enforcement is not as strong as that in the United States. These products may compete with our products, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing. Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries and in Russia, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our intellectual property and proprietary rights generally. In addition, certain jurisdictions do not protect to the same extent or at all inventions that constitute new methods of treatment. Proceedings to enforce our intellectual property and proprietary rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted

narrowly, could put 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. Accordingly, our efforts to enforce our intellectual property and proprietary rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license. Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected. We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property. We or our licensors may be subject to claims that former employees, collaborators or other third parties have an interest in our owned or in-licensed patents, trade secrets or other intellectual property as an inventor or co- inventor. For example, we or our licensors may have inventorship disputes arise from conflicting obligations of employees, consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or our or our licensors' ownership of our owned or in- licensed patents, trade secrets or other intellectual property. If we or our licensors fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, intellectual property that is important to our product candidates. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects. We may be subject to claims by third parties asserting that our employees, consultants or contractors have wrongfully used or disclosed confidential information of third parties, or we have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting we have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property. Many of our employees, consultants and contractors were previously employed at universities or other pharmaceutical or biotechnology companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and contractors do not use the proprietary information or know- how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual' s current or former employer. Litigation may be necessary to defend against these claims. In addition, while it is our policy to 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. Our intellectual property assignment agreements with them may not be self- executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property. Such claims could have a material adverse effect on our business, financial conditions, results of operations and prospects. If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could have a material adverse effect on our competitive business position and prospects. 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, which license may not be available on commercially reasonable terms, or at all, or such license may be non- exclusive. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to our management and employees. If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed. In addition to seeking patents for our product candidates, we also rely on trade secrets and confidentiality agreements to protect our unpatented know- how, technology and other proprietary information, to maintain our competitive position, including certain aspects of our **discovery technology** ~~proprietary product engine~~ . We seek to protect our trade secrets and other proprietary technology, in part, by entering into non- disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, CROs, CMOs, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants, but we cannot guarantee that we have entered into such agreements with each party that may have or has had access to our trade secrets or proprietary technology. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Detecting the disclosure or misappropriation of a trade secret and enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside of the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them, or those to whom they communicate it, 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 or other third party, our competitive position would be materially and adversely harmed. Intellectual property rights do not necessarily address all potential threats. The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example: • ~~portions of our product engine are protected by trade secrets, but much of our product engine is not protected by intellectual property, including patents, trade secrets and know- how, and we may not be able to develop, acquire or in- license any patentable technologies or other intellectual property related to the unprotected portions of our product engine; •~~ others may be able to make product candidates that are similar to ours but that are not covered by the claims of the patents that we own; • we, or our license partners or current or future collaborators, might not have been the

first to make the inventions covered by the issued patent or pending patent applications that we license or may own in the future; • we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions; • others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or in- licensed intellectual property rights; • it is possible that our owned and in- licensed pending patent applications or those we may own or in- license in the future will not lead to issued patents; • issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors; • our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets; • we cannot ensure that any of our patents, or any of our pending patent applications, if issued, or those of our licensors, will include claims having a scope sufficient to protect our product candidates; • we cannot ensure that any patents issued to us or our licensors will provide a basis for an exclusive market for our commercially viable product candidates or will provide us with any competitive advantages; • we cannot ensure that our commercial activities or product candidates will not infringe upon the patents of others; • we cannot ensure that we will be able to successfully commercialize our product candidates on a substantial scale, if approved, before the relevant patents that we own or license expire; • **portions of our discovery technology are protected by trade secrets, but much is not protected by intellectual property, including patents, trade secrets and know- how, and we may not be able to develop, acquire or in- license any patentable technologies or other intellectual property related to the unprotected portions of our discovery portfolio; •** we may not develop additional proprietary technologies that are patentable; • the patents of others may harm our business; and • we may choose not to file a patent in order to maintain certain trade secrets or know- how, and a third party may subsequently file a patent covering such intellectual property. Should any of these events occur, they could have a material adverse effect on our business, financial condition, results of operations and prospects. Risks Related to Regulatory Approval of our Product Candidates and Other Legal Compliance Matters Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time- consuming and uncertain, and we may not obtain approvals for the commercialization of some or all of our product candidates. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize our product candidates, and our ability to generate revenue will be materially impaired. Marketing approval of drugs in the United States requires the submission of a new drug application, or NDA, to the FDA and we are not permitted to market any drug candidate in the United States until we obtain approval of the NDA. An NDA must be supported by extensive clinical and preclinical data, as well as extensive information regarding pharmacology, chemistry, manufacturing and controls. We have not submitted an application for or received marketing approval for any of our product candidates in the United States or in any other jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third- party clinical research organizations or other third- party consultants or vendors to assist us in this process. 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. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use. If any of our product candidates receives marketing approval, the accompanying label may limit the approved use of our drug, which could limit sales of the product. The process of obtaining marketing approvals, both in the United States and abroad, is expensive, 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. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is 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 ultimately obtain may be limited or subject to restrictions or post- approval commitments that render the approved product not commercially viable. Disruptions at the FDA and other agencies may prolong the time necessary for regulatory submissions to be reviewed and / or new drugs to be 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, have had to furlough critical employees and stop critical activities. If a prolonged government shutdown were to occur, 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. ~~In addition, since March 2020 when foreign and domestic inspections of facilities were largely placed on hold due to the COVID- 19 pandemic, the FDA has been working to resume routine surveillance, bioresearch monitoring and pre- approval inspections. Should the FDA determine that an inspection is necessary for approval and an inspection cannot be completed during the review cycle due to restrictions on travel, and the FDA does not determine a remote interactive evaluation to be adequate, the agency has stated that it generally intends to issue, depending on the circumstances, a complete response letter or defer action on the application until an inspection can be completed. During the COVID- 19 public health emergency, a number of companies announced receipt of complete response letters due to the FDA' s inability to complete required inspections for their applications. Regulatory authorities outside the U. S. may adopt similar restrictions or other policy measures in response to the ongoing COVID- 19 pandemic and may experience delays in their regulatory activities.~~ If we experience delays in obtaining approval or if we fail to obtain approval of our product candidates, the commercial prospects for our product candidates may be harmed and our ability to generate revenues will be materially impaired. We may not be able to obtain or maintain orphan drug designation or 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~~ **European Union** , may designate drugs for relatively small patient populations as orphan drugs. The FDA and EMA have granted orphan drug designation to losmapimod for the treatment of FSHD. We may seek orphan drug designation for our other current and future product candidates. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of ~~marketing~~ --- **market** exclusivity, which precludes the FDA or the EMA from approving another marketing authorization application for the same drug for a certain time period. The applicable period is seven years in the United States and ten years in the European Union. The exclusivity period in the European Union can be reduced to six years at the end of the fifth year **if** it is determined that a drug no longer meets the criteria for orphan drug designation, including if the drug is sufficiently profitable so that market exclusivity is no longer justified. **Proposed amendments to European Union regulations regarding orphan medicines are under consideration that could reduce the ten- year marketing exclusivity period to eight to nine years (or even as little as three to five years for well- established medicines). The European Union' s April 2023 draft legislative proposal is under review, including by the European Parliament and European Council but, if implemented in due course, may mean that orphan medicines have reduced marketing exclusivity.** Orphan drug exclusivity may be lost if the FDA or 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 obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because competing drugs containing a different active ingredient can be approved for the same condition. In addition, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is clinically superior to the first drug to obtain orphan drug exclusivity because it is shown to be safer, more effective or makes a major contribution to patient care. Moreover, if we pursue and obtain approval for the same product for another indication for which we are not entitled to or do not have orphan drug exclusivity, our period of orphan exclusivity will not prevent third parties from obtaining approval for a competing drug containing the same active ingredient for use in this other, non- orphan indication. If that were to occur, the protection we derive from orphan exclusivity may be adversely affected. ~~Special designation~~ **Designation** by the FDA, such as fast track or breakthrough therapy, may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval. The FDA granted fast track designation to losmapimod for the treatment of FSHD and to **pociredir** ~~FTX- 6058~~ for the treatment of SCD, and we may seek fast track designation for some of our other product candidates as well as breakthrough therapy designation, including for losmapimod. If a drug is intended for the treatment of a serious or life- threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply for FDA fast track designation. The FDA has broad discretion whether or not to grant this designation, so even if we believe a particular product candidate is eligible for this designation, we cannot assure stockholders that the FDA would decide to grant it. Even with fast track designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life- threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. Even if we receive breakthrough therapy designation, the receipt of such designation for a product candidate may not result in a faster development process, review or approval compared to drugs 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 qualify as breakthrough therapies, the FDA may later decide that the products no longer meet the conditions for qualification or decide that the time period for FDA review or approval will not be shortened. Even if the FDA agrees that we may pursue an accelerated approval NDA submission, approval of the NDA is not assured, nor does submission of an accelerated approval NDA ensure that the product candidate will have a faster development or regulatory review process. We may seek approval, as applicable, of our product candidates using the FDA' s accelerated approval pathway. A product may be eligible for accelerated approval if it treats a serious condition, generally provides a meaningful advantage over available therapies, and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit (i. e., an intermediate clinical endpoint). Prior to seeking such accelerated approval, we will seek feedback from the FDA and otherwise evaluate our ability to seek and receive such accelerated approval. There can be no assurance that, after feedback from FDA, 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, there can be no assurance that such submission or application will be accepted or that any expedited review or approval will be granted on a timely basis, or at all. Moreover, as a condition of accelerated approval, the FDA likely would require that we perform adequate and well- controlled post- marketing clinical trials to confirm the product' s clinical benefit. These confirmatory trials must be completed with due diligence. Under the Food and Drug Omnibus Reform Act of 2022, or FDORA, the FDA is permitted to require, as appropriate, that a post- approval confirmatory study or studies be underway prior to approval or within a specified time period after the date of approval for a product granted accelerated approval. FDORA also requires sponsors to send updates to the FDA every 180 days on the status of such studies, including progress toward enrollment targets, and the FDA must promptly post this

information publicly. FDORA also gives the FDA increased authority to withdraw approval of a drug or biologic granted accelerated approval on an expedited basis if the sponsor fails to conduct such studies in a timely manner, send the necessary updates to the FDA, or if such post- approval studies fail to verify the drug' s predicted clinical benefit. Under FDORA, the FDA is empowered to take action, such as issuing fines, against companies that fail to conduct with due diligence any post-approval confirmatory study or submit timely reports to the agency on their progress. In addition, the FDA generally requires pre- approval of promotional materials for products under consideration for accelerated approval, which could adversely impact the timing of the commercial launch of the product. Thus, even if we seek to utilize the accelerated approval pathway for a product candidate, we may not experience a faster development or regulatory review or approval process for that product. In addition, receiving accelerated approval does not assure that the product' s accelerated approval will ultimately be converted to a traditional approval. Failure to obtain marketing approval in foreign jurisdictions would prevent our product candidates from being marketed abroad. In order to market and sell our products in the European Union and many other foreign jurisdictions, we or our potential third- party collaborators 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 regulatory approval process outside of the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside of the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We or our potential third- party collaborators may not obtain approvals, including conditional authorization, from regulatory authorities outside of 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 of the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. However, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in other countries. We may not be able to file for marketing approvals and may not receive **the** necessary approvals to commercialize our products in any market. Additionally, now that the United Kingdom is no longer part of the European Union, separate applications and procedures will be required to obtain regulatory approval for our products in the United Kingdom and the European Union. Any delay in obtaining, or an inability to obtain, any marketing approvals could prevent us from commercializing any product candidates in the United Kingdom and / or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. Any product candidate for which we obtain marketing approval could be subject to post- marketing restrictions or withdrawal from the market and we may be subject to substantial penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved. Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and other regulatory authorities. Even if marketing approval of a product candidate is granted, 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 REMS. If any of our product candidates receives marketing approval, the accompanying label may limit the approved use of our drug, which could limit sales of the product. The FDA may also impose requirements for costly post- marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product, including the adoption and implementation of REMS. The FDA and other agencies, including the Department of Justice, or the DOJ, closely regulate and monitor the post- approval marketing and promotion of drugs to ensure they are marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. Violations of the FDCA and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription drugs may lead to investigations and enforcement actions alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws. In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may have various consequences, including: • suspension of or restrictions on such products, manufacturers or manufacturing processes; • restrictions and warnings 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; • fines, restitution or disgorgement of profits or revenues; • suspension of any ongoing clinical trials; • suspension or withdrawal of marketing approvals; • damage to relationships with any potential collaborators; • unfavorable press coverage and damage to our reputation; • refusal to permit the import or export of our products; • product seizure or detention; • injunctions or the imposition of civil or criminal penalties; or • litigation involving patients using our products. Non- compliance with European Union requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the European Union' s or United Kingdom' s requirements regarding the protection of personal information can also lead to significant penalties and sanctions. 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 applicable to drug manufacturers. Additionally, under FDORA, sponsors of approved drugs and biologics must provide six months' notice to the FDA of any changes in marketing status, such as the withdrawal of a drug, and failure to do so could result in the FDA placing the product on a list of discontinued products, which would revoke the product' s ability to be marketed. We will also be subject to other regulatory requirements, including submissions of safety and other post- marketing information and reports, registration and listing requirements, requirements regarding the distribution of samples to clinicians, and recordkeeping. Our relationships with healthcare providers, physicians and third- party payors will be subject to applicable anti- kickback, fraud and abuse, false claims, transparency, health information privacy and security, and

other healthcare laws and regulations, which, in the event of a violation, could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings. If we obtain regulatory approval and commercialize any products, healthcare providers, physicians and third- party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with healthcare providers, physicians and third- party payors may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any products for which we obtain marketing approval. In addition, we may be subject to transparency laws and patient privacy regulation by U. S. federal and state governments and by governments in foreign jurisdictions in which we conduct our business. See Item 1 " Business — Government Regulation and Product Approvals — Health Care Law and Regulation " in this Annual Report on Form 10- K. The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record- keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products. The scope and enforcement of each of these laws is uncertain and subject to rapid change in the current environment of healthcare reform, especially in light of the lack of applicable precedent and regulations. Efforts to ensure that our business arrangements with third parties 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, including anticipated activities that would be conducted by our sales team, 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, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. 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 participation in government funded healthcare programs. 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. The legislative and regulatory framework for the collection, use, safeguarding, sharing, transfer and other processing of information worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. Globally, virtually every jurisdiction in which we operate has established its own data security and privacy frameworks with which we must comply. For example, the collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the European ~~Union~~ **Economic Area, or EEA** , including personal health data, is subject to the European Union' s General Data Protection Regulation, or EU GDPR ~~, which took effect across all Member States of the European Economic Area, or EEA, in May 2018~~ . Following the withdrawal of the United Kingdom from the European Union, or Brexit, the EU GDPR has been incorporated into United Kingdom' s laws, or UK GDPR **, alongside the UK Data Protection Act 2018,** and together with the EU GDPR, **is referred to as** GDPR. Despite Brexit, the EU and UK GDPR remain largely aligned. Currently, the most impactful point of divergence relates to transfer mechanisms (i. e., the ability for companies in the European Union or the United Kingdom to transfer personal information to third countries, including the United States), because it requires us to implement a variety of different contractual clauses approved by European Union' s or United Kingdom' s regulators **, and carry out transfer impact assessments to establish whether the third country can ensure essential equivalency** . This complexity and the additional contractual burden increases our overall risk exposure **, and may result in us needing to make strategic considerations around where EEA and UK personal data is stored and which service providers we can utilize for the processing of EEA and UK personal data** . There may be further divergence in the future, including with regard to administrative burdens. The ~~United Kingdom~~ **UK Government** has ~~announced plans~~ **also now introduced a Data Protection and Digital Information Bill, or the UK Bill, into the UK legislative process. The aim of the UK Bill is** to reform the ~~country'~~ **UK'** s data protection ~~legal framework in its Data Reform~~ **regime following Brexit. If passed, the final version of the UK** Bill **may have** ~~, which will introduce significant changes from~~ the ~~EU GDPR~~ **effect of further altering the similarities between the UK and EEA data protection regime** . This may lead to additional compliance costs and could increase our overall risk exposure as we may no longer be able to take a unified approach across the European Union and the United Kingdom, and we will need to amend our processes and procedures to align with the new framework. Similar data protection laws are either in place or under way in the United States. There are a broad variety of **privacy and** data ~~protection and~~ security laws and regulations that may be applicable to our activities governing the collection, use, disclosure, and protection of health- related and other personal information (including ~~, for example~~ , state data breach notification laws, health information and / or genetic privacy laws and federal and state consumer protection laws including Section 5 of the FTC Act, HIPAA, and the California Consumer ~~Protection~~ **Privacy** Act **,** or CCPA). ~~A wide range of enforcement agencies at both the state and federal levels that can review companies for privacy and data security concerns based on general consumer protection laws. The Federal Trade Commission and state Attorneys General all are aggressive in reviewing privacy and data security protections for consumers. New laws also are being considered at both the state and federal levels.~~ For example, ~~in~~ **the CCPA as amended by the** California **Privacy Rights Act** ~~, the CCPA, which went into effect in January 2020~~ , has created certain requirements for data use, sharing and transparency, and provides California residents certain rights concerning their personal information, **such as access, correction, deletion** and ~~many~~ **opt out of or selling or sharing such data. Several** other states have ~~introduced~~ **implemented privacy legislation similar to the CCPA** or are ~~considering similar legislation. For more information regarding~~ **preparing to implement** ~~the GDPR, the CCPA and other~~ **their own** ~~regulations~~ **regulatory frameworks. A wide range of enforcement agencies at both the state and federal levels** , ~~see~~ **such as the Federal Trade Commission and state Attorneys General have been increasingly aggressive in reviewing and enforcing privacy and data**

security- related consumer protection laws. See Item 1 " Business – Government Regulation and Product Approvals " in this Annual Report on Form 10- K. Given the breadth and depth of changes in privacy, data protection and consumer protection obligations, preparing for and complying with these requirements is rigorous and time intensive and requires significant resources and ~~a~~ **ongoing** review of our technologies, systems and practices, as well as those of any third- party collaborators, service providers, contractors or consultants that store, process or transfer personal data on our behalf ~~. Many of these laws differ from each other in significant ways and may be interpreted and applied in a manner that is inconsistent from one jurisdiction to another, thus complicating compliance efforts~~ . Compliance with the GDPR and other similar laws or regulations associated with the enhanced protection of certain types of sensitive data, such as healthcare data or other personal information from our clinical trials, could require us to change our business practices and put in place additional compliance mechanisms, may interrupt or delay our development, regulatory and commercialization activities and increase our cost of doing business. Any failure or perceived failure by us to comply with such laws and regulations could lead to government enforcement actions, private litigation and significant fines and penalties against us and could have a material adverse effect on our business, financial condition or results of operations. There is also the threat of consumer class actions related to these laws and the overall protection of personal data. 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 reputation and our business. Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and affect the prices we may obtain for any products that are approved in the United States or foreign jurisdictions. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post- approval activities and affect our ability to profitably sell any product candidates for which we obtain marketing approval. The pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by legislative initiatives. 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 receive for any FDA approved product. If reimbursement of our products is unavailable or limited in scope, our business could be materially harmed. See Item 1 " Business — Government Regulation and Product Approval — Pharmaceutical Insurance Coverage and Health Care Reform " in this Annual Report on Form 10- K. In August 2022 the Inflation Reduction Act of 2022 was passed, which among other things, allows for Centers for Medicare & Medicaid Services to negotiate prices for certain single- source drugs and biologics reimbursed under Medicare Part B and Part D, beginning with select high- cost drugs in 2026. The legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for offering a price that is not equal to or less than the price negotiated 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. Further, the legislation caps Medicare beneficiaries' annual out- of- pocket drug expenses at $ 2, 000 **. The implementation of the IRA is currently subject to ongoing litigation challenging the constitutionality of the IRA' s Medicare drug price negotiation program** . The effect of Inflation Reduction Act of 2022 on our business and the healthcare industry in general is not yet known. We expect that these healthcare reforms, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product and / or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. Governments outside of the United States tend to impose strict price controls, which may adversely affect our revenues, if any. In countries outside of the United States, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. 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 candidate to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially. If we or any third- party manufacturers we engage now or in the future fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs or liabilities that could harm our business. We and third- party manufacturers we engage now are, and any third- party manufacturers we may engage in the future will be, 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. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Liability under certain environmental laws governing the release and cleanup of hazardous materials is joint and several and could be imposed without regard to fault. We also could incur significant costs associated with civil or criminal fines and penalties or become subject to injunctions limiting or prohibiting our activities for failure to comply with such laws and regulations. Although we maintain general liability insurance as well as 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, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials. In addition, we

may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions. Further, with respect to the operations of our current and any future third- party contract manufacturers, it is possible that if they fail to operate in compliance with applicable environmental, health and safety laws and regulations or properly dispose of wastes associated with our products, we could be held liable for any resulting damages, suffer reputational harm or experience a disruption in the manufacture and supply of our product candidates or products. In addition, our supply chain may be adversely impacted if any of our third- party contract manufacturers become subject to injunctions or other sanctions as a result of their non- compliance with environmental, health and safety laws and regulations. We are subject to anti- corruption laws, as well as export control laws, customs laws, sanctions laws and other laws governing our operations. If we fail to comply with these laws, we could be subject to civil or criminal penalties, other remedial measures and legal expenses, be precluded from developing manufacturing and selling certain products outside the United States or be required to develop and implement costly compliance programs, which could adversely affect our business, results of operations and financial condition. Our operations are subject to anti- corruption laws, including the U. K. Bribery Act 2010, or Bribery Act, the U. S. Foreign Corrupt Practices Act, or FCPA, and other anti- corruption laws that apply in countries where we do business and may do business in the future. The Bribery Act, FCPA and these other laws generally prohibit us, our officers, and our employees and intermediaries from bribing, being bribed or making other prohibited payments to government officials or other persons to obtain or retain business or gain some other business advantage. Compliance with the FCPA, in particular, 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, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions. We may in the future operate in jurisdictions that pose a high risk of potential Bribery Act or FCPA violations, and we may participate in collaborations and relationships with third parties whose actions could potentially subject us to liability under the Bribery Act, FCPA or local anti- corruption laws. In addition, we cannot predict the nature, scope or effect of future regulatory requirements to which our international operations might be subject or the manner in which existing laws might be administered or interpreted. If we expand our operations outside of the United States, we will need to dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. We are also subject to other laws and regulations governing our international operations, including regulations administered by the governments of the United Kingdom and the United States, and authorities in the European Union, including applicable export control regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, collectively referred to as the Trade Control laws. In addition, 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. If 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. There is no assurance that we will be completely effective in ensuring our compliance with all applicable anti- corruption laws, including the Bribery Act, the FCPA or other legal requirements, including Trade Control laws. If we are not in compliance with the Bribery Act, the FCPA and other anti- corruption laws or Trade Control laws, we may be subject to criminal and civil penalties, disgorgement and other sanctions and remedial measures, and legal expenses, which could have an adverse impact on our business, financial condition, results of operations and liquidity. The SEC also may suspend or bar issuers from trading securities on U. S. exchanges for violations of the FCPA' s accounting provisions. Any investigation of any potential violations of the Bribery Act, the FCPA, other anti- corruption laws or Trade Control laws by United Kingdom, **United States** ~~U. S.~~ or other authorities could also have an adverse impact on our reputation, our business, results of operations and financial condition. Our employees, independent contractors, consultants and vendors may engage in misconduct or other improper activities, including non- compliance with regulatory standards and requirements and insider trading, which could cause significant liability for us and harm our reputation. We are exposed to the risk of fraud or other misconduct by our employees, independent contractors, consultants and vendors. Misconduct by these partners could include 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 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 EU GDPR. We are also exposed to risks in connection with any insider trading violations by employees or others affiliated with us. It is not always possible to identify and deter employee 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. Our internal computer and information technology systems and infrastructure, or those of our collaborators or other contractors or consultants, may fail or suffer security compromises or breaches, which could result in a material disruption of our product

development programs. Our internal computer and information technology systems and infrastructure and those of our CROs, collaborators, and other contractors or consultants upon which our business relies, are vulnerable to breakdown or damage or interruption or otherwise may sustain damage from computer viruses, unauthorized access, data breaches, phishing attacks, cybercriminals, system malfunction, natural disasters (including hurricanes and earthquakes), terrorism, war and telecommunication and electrical failures. Such systems and infrastructure are also vulnerable to service interruptions or to security compromises or breaches from inadvertent or intentional actions by our employees, CROs or other third- party vendors, contractors, consultants and / or business partners or other third parties, or from cyber- attacks by malicious third parties. Cyber-attacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cyber-attacks could include wrongful conduct by insider employees or vendors, hostile foreign governments, industrial espionage, wire fraud and other forms of cyber fraud or cyber- attacks, including the deployment of harmful malware, ransomware, denial-of- service attacks, unauthorized access to or deletion of files, phishing attacks and social engineering, business email compromise, and other means to affect service reliability and threaten the confidentiality, integrity and availability of information. ~~Accordingly~~ **We have experienced cyber incidents in the past** , ~~if~~ **and we cannot guarantee that the measures we take to prevent, detect, and respond to cyber- attacks will be effective to prevent or remediate future incidents. If** our cybersecurity measures or those of our service providers fail to protect against unauthorized access, attacks, compromise or the mishandling of data by our employees or contractors, then our reputation, customer trust, business, results of operations and financial condition could be adversely affected. Because the techniques used by threat actors who may attempt to penetrate and sabotage our computer systems or those of our collaborators or other contractors or consultants change frequently and may not be recognized until launched against a target, we may be unable to anticipate these techniques ~~. For example, we make extensive use of cloud- based storage systems, and in October 2018, we experienced a breach of one such system. While this breach did not result in the permanent loss or theft of any of our critical information or any other material consequences, it could have, and while we took steps to remediate this breach, such as establishing multi- factor authentication and implementing improvements to our data securities protocols, we cannot guarantee that the measures we have taken to date, and actions we may take in the future, will be sufficient to remediate any future breaches~~ . While we have not experienced any 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 and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security compromise or breach were to result in a loss of, damage to, unauthorized access, or misuse of our data, systems, infrastructure or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability (including in connection with or resulting from litigation or governmental investigations and enforcement actions), our competitive position could be harmed and the further development and commercialization of our product candidates could be delayed and our business could be otherwise adversely affected. Risks Related to Employee Matters and Managing Growth Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel. We are highly dependent on the research and development, clinical, financial, operational and other business expertise of our executive officers, as well as the other principal members of our management, scientific and clinical teams. Although we have entered into employment offer letters with our executive officers, each of them may terminate their employment with us at any time. We do not maintain " key person " insurance for any of our executives or other employees. Recruiting and retaining qualified scientific, clinical, manufacturing, accounting, legal and sales and marketing personnel will also be critical to our success. We have had recent executive transitions, including of our chief executive officer, **chief financial officer,** president of research and development, chief scientific officer, and chief medical officer. We cannot predict the likelihood, timing or effect of future transitions among our executive leadership. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy **. For example, our employees have taken on increased responsibilities in light of this turnover, which could divert attention from key business areas. Additionally, the number of recent departures has created some uncertainty** . Furthermore, replacing executive officers and 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 successfully develop, gain regulatory approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel 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. **Even if we are successful in our efforts to replace our executive leadership, we cannot guarantee that we will not face similar turnover in the future.** In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. In August 2022, we announced a workforce reduction in our research and development function, which may make us a less attractive employer to future candidates. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. Our success as a public company also depends on implementing and maintaining internal controls and the accuracy and timeliness of our financial reporting. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited. We expect to expand our development and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations. We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, clinical, regulatory affairs and, if any of our product candidates receives marketing approval, sales,

marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations. Risks Related to our Common Stock Our executive officers, directors and principal stockholders, if they choose to act together, have the ability to control or significantly influence all matters submitted to stockholders for approval. As of ~~March 2~~ **February 20**, 2023, our executive officers and directors and our stockholders who owned more than 5 % of our outstanding common stock in the aggregate beneficially owned shares representing approximately ~~57~~ **48**. ~~1~~ **6** % of our capital stock. As a result, if these stockholders were to choose to act together, they would be able to control or 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, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of ownership control may: • delay, defer or prevent a change in control; • entrench our management and board of directors; or • delay or prevent a merger, consolidation, takeover or other business combination involving us that other stockholders may desire. Provisions in our corporate charter documents and under Delaware law could make an acquisition of our company, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current directors and members of management. Provisions in our certificate of incorporation and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of our company that stockholders may consider favorable, including transactions in which 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 only one of three classes of directors is elected each year; • 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 our board of directors; • establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors; • 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 stockholder meetings; • 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 specified provisions of our certificate of incorporation or bylaws. Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, or the DGCL, 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. If securities analysts do not publish or cease publishing research or reports or publish misleading, inaccurate or unfavorable research about our business or if they publish negative evaluations of our stock, the price and trading volume of our stock could decline. The trading market for our common stock relies, in part, on the research and reports that industry or financial analysts publish about us or our business. There can be no assurance that existing analysts will continue to cover us or that new analysts will begin to cover us. There is also no assurance that any covering analyst will provide favorable coverage. Although we have obtained analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock or publish inaccurate or unfavorable research about our business, or provides more favorable relative recommendations about our competitors, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price and trading volume to decline. The price of our common stock may be volatile and fluctuate substantially, 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 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 biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including: • results of or developments in preclinical studies and clinical trials of our product candidates or those of our competitors or potential collaborators ~~, including the recent clinical hold on FTX- 6058 (and whether or not we are able to resolve such hold)~~ ; • our success in commercializing our product candidates, if and when approved; • the success of competitive products or technologies; • regulatory or legal developments in the United States and other countries; • developments or disputes concerning patent applications, issued patents or other intellectual property or 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 discover, develop, acquire or in- license products, product candidates, technologies or data referencing rights, the costs of commercializing any such products and the costs of development of any such product candidates or technologies; • actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts; • variations in our financial results or the financial results of companies that are perceived to be similar to us; • 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. In the past, following periods of volatility in the market price of a company' s securities, securities class- action litigation has often been instituted against that company. Any lawsuit to which we are a party, with or without merit, may result in an unfavorable judgment. We also may decide to settle lawsuits on unfavorable terms. Any such negative outcome could result in payments of substantial damages or fines, damage to our reputation or adverse changes to our offerings or business practices. Such litigation may also cause us to incur other substantial costs to defend such claims and divert management' s attention and resources. Furthermore, negative public announcements of the results of hearings, motions or other interim proceedings or developments could have a negative effect on the market price of our common stock. A significant portion of our total outstanding shares are eligible to be sold into the market, which could cause the market price of our common stock to drop significantly, even if our business is doing well. Sales of a substantial number of shares of our common stock in the public market, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. Persons who were our stockholders prior to our initial public offering continue to hold a substantial number of shares of our common stock. If such persons sell, or indicate an intention to sell, substantial amounts of our common stock in the public market, the trading price of our common stock could decline. In addition, we have filed or intend to file universal shelf registration statements (which allows us to offer and sell securities from time to time pursuant to one or more offerings at prices and terms to be determined at the time of sale) subject to an aggregate offering amount stated therein, as well as registration statements registering all shares of common stock that we may issue under our equity compensation plans or pursuant to equity awards made to newly hired employees outside of equity compensation plans. Such registered shares can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates. We are an " emerging growth company, " and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors. We are an " emerging growth company, " or EGC, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We may remain an EGC until December 31, 2024, although if the market value of our common stock that is held by non- affiliates exceeds $ 700 million as of any June 30 before that time or if we have annual gross revenues of $ 1. 235 billion or more in any fiscal year, we would cease to be an EGC as of December 31 of the applicable year. We also would cease to be an EGC if we issue more than $ 1 billion of non- convertible debt over a three- year period. For so long as we remain an EGC, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not EGCs. These exemptions include: • not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting; • not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor' s report providing additional information about the audit and the financial statements; • reduced disclosure obligations regarding executive compensation; and • exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We may choose to take advantage of some or all of the available exemptions. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. In addition, the JOBS Act permits an EGC to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected not to " opt out " of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either (i) irrevocably elect to " opt out " of such extended transition period or (ii) no longer qualify as an EGC. We have incurred and will continue to incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices. As a public company we have incurred, and particularly after we are no longer an EGC, we will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes- Oxley Act of 2002, the Dodd- Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The Nasdaq Global 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 will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs, particularly as we hire additional financial and accounting employees to meet public company internal control and financial reporting requirements, and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance, which in turn could make it more difficult for us to attract and retain qualified members of our board of directors. We are evaluating these rules and regulations, and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices. Pursuant to Section 404 of the Sarbanes- Oxley Act of 2002, or Section 404, we are required to furnish a report by our management on our internal control over financial reporting. However, while we remain an EGC or a smaller reporting company with less than $ 100 million in revenue, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we conduct a process each year to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, including through hiring additional financial

and accounting personnel, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses in our internal control over financial reporting, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements. Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be our stockholders' sole source of gain. We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. As a result, capital appreciation, if any, of our common stock will be our stockholders' sole source of gain for the foreseeable future. Our certificate of incorporation designates the state courts in the State 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 the company and our directors, officers and employees. Our 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 of the State of Delaware does not have jurisdiction, the federal district court for the District of Delaware) will be the sole and exclusive forum for (1) any derivative action or proceeding brought on our behalf, (2) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or stockholders to our company or our stockholders, (3) any action asserting a claim arising pursuant to any provision of the DGCL or as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware or (4) any action asserting a claim arising pursuant to any provision of our certificate of incorporation or bylaws (in each case, as they may be amended from time to time) or governed by the internal affairs doctrine. This exclusive forum provision will not apply to actions arising under the Securities Act 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. Alternatively, if a court were to find the choice of forum provision contained in our certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could materially adversely affect our business, financial condition and operating results. ~~90~~