(a public limited liability company incorporated in Denmark under registration (CVR) no. 32266355)

This listing prospectus (the "**Prospectus**") has been prepared in connection with an admission of 3,071,673 shares (the "**Listing Shares**") with a nominal value of DKK 1 each in Orphazyme A/S (the "**Company**" or "**Orphazyme**") for trading and official listing on Nasdaq Copenhagen A/S ("**Nasdaq Copenhagen**") (the "**Listing**").

The Listing Shares were issued through VP Securities and registered with the Danish Business Authority on February 11, 2020. The Listing Shares were issued in connection with a Stock Lending and Subscription Agreement entered into on February 6, 2020 among the Company, Danske Bank A/S and Orpha Pooling B.V. and Novo Holdings A/S (the "**Lending Shareholders**")pursuant to which the Company borrowed 3,071,673 existing shares (the "**Lending Shares**") from the Lending Shareholders through Danske Bank A/S as settlement agent in order for the Company to place such Lending Shares in a private placement (the "**Private Placement**"). The Lending Shares were borrowed subject to an obligation for the Company to issue new shares of an equivalent number as the Lending Shares placed in the Private Placement, such new shares being the Listing Shares, and for Danske Bank A/S to use the proceeds from the sale of Lending Shares in the Private Placement to subscribe for the Listing Shares and deliver the Listing Shares to the Lending Shareholders. The Listing Shares were issued in the temporary ISIN code, DK0061274362, and delivered to the Lending Shareholders on February 11, 2020.

The Listing Shares were issued without pre-emptive rights for the existing shareholders and pursuant to an authorization in article 3.4 of the articles of association of the Company (the "**Articles of Association**") granted to the board of directors of the Company (the "**Board of Directors**") at the extraordinary general meeting of the Company on January 25, 2020.

Prior to the Private Placement, the Company had 20,005,449 outstanding shares with a nominal value of DKK 1 each (the "**Existing Shares**"). The capital increase in connection with the issuance of the new shares in the Private Placement and the Listing Shares increased the total nominal share capital by 35.16%, making the total number of outstanding shares 27,038,386.

The Listing Shares are issued in the temporary ISIN code DK0061274362, which will not be admitted to trading and official listing on Nasdaq Copenhagen and such temporary ISIN code will subsequently be merged with the the existing ISIN code for the Existing Shares DK0060910917 in VP Securities. Upon completion of the Listing, the Listing Shares will be admitted to trading and official listing under the ISIN code of the Existing Shares, DK0060910917, which is expected to take place on or around March 17, 2020.

The purpose of this Prospectus is solely to have the Listing Shares admitted to trading and official listing on Nasdaq Copenhagen. No issue or offering of shares is made by the Company or any other person in connection with the publication of the Prospectus.

Any person in whose possession this Prospectus may come, should be aware that an investment in the Shares involves a high degree of risk. See "*Risk factors*" for a description of the factors that should be considered before investing in the Shares.

This Prospectus has been prepared in accordance with Danish legislation and regulations, including the Danish Consolidated Act no. 931 of September 6, 2019 on Capital Markets (the "**Danish Capital Markets Act**"), Regulation (EU) no. 2017/1129 of the European Parliament and of the Council of June 14, 2017, as amended (the "**Prospectus Regulation**"), Commission Delegated Regulation (EU) no. 2019/980 of March 14, 2019 as well as Commission Delegated Regulation (EU) 2019/979 of March 14, 2019, and Nasdaq Copenhagen's Rules for issuers of shares of July 1, 2019 ("**Nasdaq Issuer Rules**").

This Prospectus will not be and may not be distributed or otherwise made available in any jurisdiction (other than any publication of this Prospectus in accordance with Danish law, rules and regulations) and the Shares may not be offered or sold, directly or indirectly, in the United States, Canada, Australia or Japan or in any other jurisdiction. The Company makes no offer or solicitation to any person under any circumstances that may be unlawful. The Shares have not been and will not be registered under the United States Securities Act of 1933, as amended, (the "**U.S. Securities Act**") and are only offered and sold outside the United States or to, or for the account or benefit of, U.S. persons (as defined in Regulation S under the U.S. Securities Act ("**Regulation S**")) in accordance with Regulation S, except in transactions exempt from, or not subject to, the registration requirements of the U.S. Securities Act. See "*Important information*" and "*The Listing–Terms and conditions of the Listing–Terms, expected timetable and restrictions*". *Persons into whose possession this Prospectus comes are required by the Company to inform themselves about and to observe such restrictions.*

Prospectus dated March 16, 2020

IMPORTANT INFORMATION

This Prospectus has been prepared solely for admission to trading and official listing of the Listing Shares on Nasdaq Copenhagen in compliance with Danish legislation and regulations, including the Danish Capital Markets Act, the Prospectus Regulation, Commission Delegated Regulation (EU) no. 2019/980 of March 14, 2019 as well as Commission Delegated Regulation (EU) 2019/979 of March 14, 2019, and Nasdaq Issuer Rules. This Prospectus has been prepared in accordance with Article 14 (Simplified disclosure regime for secondary issuances) of the Prospectus Regulation, Annex 3 (Registration document for secondary issuances of equity securities) and Annex 12 (Securities note for secondary issuances of equity securities or of units issued by collective investment undertakings of the closed-end type) to the Commission Delegated Regulation (EU) no. 2019/980 of March 14, 2019. The Company has elected to apply the aforementioned Annexes, as the proportionate disclosure regime has been specifically implemented to be used in secondary issuances and admissions to trading and official listing of securities from such issuances.

The Listing does not comprise an offer of, an invitation to purchase or subscribe for or a placement of Listing Shares in any jurisdiction and this Prospectus may not be used in connection with any offer of Shares or solicitation by anyone in any jurisdiction. The Company accepts no liability for any violation of any such restrictions by any person. For a more detailed description of certain restrictions in connection with the Listing, see "*The Listing–Terms and Conditions of the Listing*".

This Prospectus will not be and may not be distributed or otherwise be made available in any jurisdiction (other than any publication of this Prospectus in accordance with Danish law, rules and regulations), and the Listing Shares may not be offered or sold, directly or indirectly, in the United States, Canada, Australia or Japan or in any other jurisdiction. Persons into whose possession this Prospectus comes are required by the Company to inform themselves about and to observe such restrictions.

The Company makes no offer or solicitation to any person under any circumstances that may be unlawful.

Notice to Investors in the United States

The Listing Shares have not been approved, disapproved or recommended by the U.S. Securities and Exchange Commission, any state securities commission in the United States or any other U.S. regulatory authority, nor have any of such regulatory authorities passed upon or endorsed the merits of the Listing or the accuracy or adequacy of this Prospectus. Any representation to the contrary is a criminal offence in the United States.

The Listing does not comprise an offer of, an invitation to purchase or subscribe for or a placement of Listing Shares in the United States. The Listing Shares are not, and will not be, registered under the U.S. Securities Act or any applicable state securities laws of the United States. The issuance of the Shares was made in transactions exempt from the registration requirements of the U.S. Securities Act pursuant to Section 4(a)(2) of the U.S. Securities Act, Regulation S, or another available exemption. The Shares may not be offered, pledged, resold, granted, delivered, allotted or otherwise transferred, as applicable, in the United States, except in transactions that are exempt from or not subject to the registration requirements under the U.S. Securities Act and in compliance with any applicable state securities laws.

Any person in the United States that obtains a copy of this Prospectus or any pre-printed issue statement or application form is required to disregard them.

The Listing is subject to Danish legislation and requirements and, therefore, any information contained in this Prospectus may not be comparable with information contained in prospectuses of U.S.-listed companies. For certain restrictions on transfer of the Listing Shares, see "*The Listing–Terms and conditions of the Listing –Terms, expected timetable and restrictions*".

Notice to Investors in the European Economic Area

In relation to each member state of the European Economic Area where the Prospectus Regulation applies (each a "**Relevant Member State**"), no offering of Listing Shares will be made to the public in any Relevant Member State. Notwithstanding the above, if an offering had been made, no offering of Shares could be made to the public in any Relevant Member State prior to the publication of a prospectus concerning the Shares which has been approved by the competent authority in such Relevant Member State or, where relevant, approved in another Relevant Member State and notified to the competent authority in such Relevant Member State, all pursuant to the Prospectus Regulation,

except that an offering of Shares may be made to the public at any time in such Relevant Member State pursuant to the following exemptions from the Prospectus Regulation:

a) to any legal entity which is a qualified investor as defined in the Prospectus Regulation ("**Qualified Investor**");

b) to fewer than 150 natural or legal persons other than Qualified Investors, subject to obtaining the prior written consent of the Company; or

c) in any other circumstances falling within Article 1(4) of the Prospectus Regulation.

In any Relevant Member State, such offering would be only addressed to, and only directed at, investors in such Relevant Member State that fulfil the criteria for exemption from the obligation to publish a prospectus, including Qualified Investors.

For the purposes of the above, the expression an "offer of Shares to the public" in relation to the Listing Shares in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the Listing and the Listing Shares so as to enable an investor to decide whether to acquire or subscribe for the Listing Shares.

Notice to Investors in the UK

This Prospectus is not being distributed in the UK. If it had been distributed, it could only have been distributed to, and directed at, (i) persons outside the UK or (ii) "investment professionals" falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 (the "**Financial Promotion Order**") or (iii) "high net worth companies" and other persons to whom it may lawfully be communicated, falling within the meaning of Article 49(2)(a) to (d) of the Financial Promotion Order (all such persons being "**Relevant Persons**"). Therefore, if a prospectus would have been distributed in the UK, Shares are only available to Relevant Persons and any invitation, offer or agreement to subscribe for, purchase or otherwise acquire such Shares will be engaged in only with Relevant Persons. Any person who is not a Relevant Person should not act on or rely upon such prospectus or any of its contents.

Notice to Investors in Canada, Australia and Japan

The Listing Shares have not been approved, disapproved or recommended by any foreign regulatory authorities, nor have any of such authorities passed upon or endorsed the merits of the Listing or the accuracy or adequacy of this Prospectus.

This Prospectus may not be distributed or otherwise made available, the Listing Shares may not be offered, sold or subscribed for, directly or indirectly, in Canada, Australia or Japan.

TABLE OF CONTENTS

SUMMARY

Section A – Introduction and warnings

Warnings	This summary should be read as an introduction to the Prospectus. Any decision to invest in the Shares should be based on a consideration of the Prospectus as a whole by the investor. Prospective investors in the Shares could lose all or part of the invested capital. Where a claim relating to the information contained in the Prospectus is brought before a court, the plaintiff investor might, under national law, have to bear the costs of translating this Prospectus before the legal proceedings are initiated. Civil liability attaches only to those persons who have tabled the summary, including any translation thereof, but only where the summary is misleading, inaccurate or inconsistent, when read together with the other parts of the Prospectus, or where it does not provide, when read together with the other parts of the Prospectus, key information in order to aid investors when considering whether to invest in the Shares.
Share information	The Nasdaq Copenhagen symbol for the Shares is "ORPHA". The ISIN code for the Existing Shares is DK0060910917. The temporary ISIN code for the Listing Shares is DK0061274362, which will not be admitted to trading and official listing on Nasdaq Copenhagen. The Listing Shares will be admitted to trading and official listing on Nasdaq Copenhagen under the same ISIN code as the Existing Shares.
Issuer information	The issuer of the Listing Shares is Orphazyme A/S (the "**Company**"). The address and other contact details of the Company are Ole Maaløes Vej 3, DK-2200 Copenhagen N, Denmark, telephone number (+45) 39 17 82 72 and email contact@orphazyme.com. The Company has the legal entity identifier (LEI) 54930025OZD2GGSQ7L42 and has registration (CVR) no. 32266355.
Competent authority	This Prospectus has been approved on March 16, 2020 by the Danish Financial Supervisory Authority as competent authority under the Prospectus Regulation. The address and other contact details of the Danish Financial Supervisory Authority are Århusgade 110, DK-2100 Copenhagen OE, Denmark, telephone number +45 33 55 82 82, email finanstilsynet@ftnet.dk and fax +45 33 55 82 00.

Section B – Key information on the issuer

Who is the issuer of the securities?	The Company has its registered office at Ole Maaløes Vej 3, DK-2200 Copenhagen N in the municipality of Copenhagen, Denmark and is incorporated in Denmark as a Danish public limited liability company under the laws of Denmark. The Company has the legal entity identifier (LEI) 54930025OZD2GGSQ7L42 and has registration (CVR) no. 32266355.
Principal activities	The Company is a biopharmaceutical company that pioneers the heat shock protein response ("HSPs") for neurodegenerative orphan disorders. The Company develops innovative therapies for the treatment of orphan diseases with a high unmet medical need which are characterized by protein misfolding, aggregation and lysosomal dysfunction and intends to commercialize such therapies if regulatory approval has been received through an in-house sales force and partnerships with distributors. The Company focusses on rare, neurodegenerative disorders (including neuropathic lysosomal diseases ("LSDs") and progressive neuromuscular disorders) and strives to profoundly impact the lives of patients and their families living with such diseases. As a result of positive data from certain of its clinical trials, the Company has grown from an entrepreneurial R&D company to a company with significant growth aspirations and an emerging late-stage pipeline that is currently moving into the pre-commercial phase. The Company was founded in 2009 based on a scientific discovery (published in Nature1) on the function of HSPs by (among others) Thomas Kirkegaard Jensen, who still serves as Chief Scientific Officer ("CSO"). The Company's pipeline is underpinned by deep expertise in the

[1] Kirkegaard et al., *Nature*, 2010

science of cellular stress, particularly the heat shock response ("HSR"), the body's natural defence to cellular stress. The Company's lead investigational product candidate, arimoclomol, amplifies endogenous HSPs, which are at the core of the HSR and help guard against toxicity caused by protein misfolding, aggregation and lysosomal dysfunction. Arimoclomol is a small molecule that crosses the blood-brain barrier and is presented in oral, nasogastric form, providing for easy administration as an oral capsule, sprinkled in food/beverage or via a feeding tube. At the Prospectus Date, approximately 540 patients and healthy volunteers have been exposed to arimoclomol and no major safety concerns have been observed.

Since inception, the Company has translated certain of its scientific discoveries into a late stage clinical development program. The Company's most progressed clinical trial with arimoclomol is within LSDs for the treatment of Niemann-Pick disease Type C ("NPC"). In January 2019, the Company reported positive results from the full data set of phase II/III arimoclomol trial in NPC and additional positive data from the open-label extension trial was reported in January 2020, which demonstrated a continued positive impact on disease progression over two years. The Company has been granted orphan drug designation by the FDA for arimoclomol as a treatment for NPC in 2015 and orphan designation by the European Medicines Agency (the "EMA") for arimoclomol as a treatment for NPC in 2014. Further, the Company has been granted fast track designation in June 2016, rare pediatric disease designation in January 2018 and breakthrough therapy designation in November 2019 by the FDA for treatment with arimoclomol in NPC. In addition, in January 2020, the Company announced the availability of an early access program ("EAP") in the United States, which permits the Company to make arimoclomol available pre-commercially to U.S. patients. The Company plans to file for an NDA in the United States in H1 2020 and for an MAA in the EU on in H2 2020 for arimoclomol as a treatment for NPC. If regulatory approvals are received, the Company will implement its commercialization plan for NPC to enter the United States and European markets.

In addition, the Company is currently conducting three other clinical trials with arimoclomol, one more for LSDs (Gaucher disease), and two for neuromuscular diseases (Sporadic Inclusion Body Myositis, ("sIBM"), and Amyotrophic Lateral Sclerosis ("ALS"), which are also rare and severe diseases with limited or no current treatment options. Beyond these initial indications, and based on data from the Company's preclinical studies, the Company believes arimoclomol has potential in the treatment of other related diseases, including glucocerebrosidase-deficient (GCase) Parkinson's disease and a range of additional LSDs. The Company plans to pursue development of arimoclomol through to registration in Europe and the United States and has been granted orphan drug designation to arimoclomol by the FDA for (i) sIBM in November 2017 and (ii) ALS was transferred to the Company in January 2012, and orphan designation by the EMA, for (i) sIBM in May 2016 and (ii) ALS was transferred to the Company in September 2011. In addition, in December 2019, the FDA granted fast track designation for arimoclomol as a treatment of sIBM , which further underlines the great potential of the Company's investigational drug.

The below figure provides an overview as of the Prospectus Date of the Company's pipeline and designations granted.

Major Shareholders

At the Prospectus Date, the Company has received notifications of holdings of 5% or more of the share capital or voting rights from the shareholders below

Shareholder	Number of Shares as at latest announcement	Ownership interest as at latest announcement	Voting rights as at latest announcement
Danske Bank A/S(1)	1,244,908	4.60%	6.72%
Novo Holdings A/S(2)	2,021,673	7.5%	7.5%
LSP V Coöperatieve U.A.(3)	2,710,829	10.03%	10.03%
Sunstone Life Science Ventures Fund II K/S	1,804,405	9.10%	9.10%
Coöperative Aescap Venture I U.A.	1,765,605	8.90%	8.90%
Consonance Capman GP LLC	1,900,000	7.03%	7.03%

(1) Danske Bank A/S' shareholding consists of a 3.90% indirect and 0.70% direct ownership through Danica Pension Livsforsikringsaktieselskab, Danica Pension Försäkringsaktiebolag, Investeringsforeningen Danske Invest and Danske Invest SICAV. Danske Bank A/S' control of voting rights in the Company consists of a 6.02% indirect and 0.70% direct control through Danica Pension Livsforsikringsaktieselskab, Danica Pension Försäkringsaktiebolag, Investeringsforeningen Danske Invest, and Danske Invest SICAV.
(2) Novo Holdings A/S is wholly owned by Novo Nordisk Foundation.
(3) This shareholding includes a direct shareholding of 279,157 shares corresponding to 1.03% of the total share capital and voting rights and an indirect shareholding of 2,431,672 shares held through Orpha Pooling B.V. (a joint venture between LSP V Coöperatieve U.A and ALS Invest 2 B.V.) corresponding to 9.00% of the total share capital and voting rights.

The Company is not aware of being owned or controlled, directly or indirectly, by others.

Key managing directors

A the Prospectus Date, the Board of Directors consists of Georges Gemayel (Chairman), Bo Jesper Hansen (Deputy Chairman), Anders Hedegaard, Catherine Moukheibir, Martijn Kleijwegt, Martin Bonde, Rémi Droller and Sten Verland, and the Executive Management consists of Kim Stratton, CEO, and Anders Vadsholt, CFO.

The Executive Management is supported by the Key Employees. The Key Employees consists of Thomas Blaettler, CMO and Thomas Kirkegaard Jensen, CSO.

Statutory auditors

The statutory auditors of the Company is Ernst & Young Godkendt Revisionspartnerselskab. The independent auditors' report included in the consolidated financial statements and the parent company financial statements for the financial year January 1 – December 31, 2019 were signed by State Authorised Public Accountant, Christian Schwenn Johansen and State Authorised Public Accountant, Rasmus Bloch Jespersen."

What is the key financial information regarding the issuer?

The key financial information shown below has been derived from the FY2019 Group Financial Statements prepared in accordance with IFRS as adopted by the EU and additional disclosure requirements of the Danish Financial Statements Act:

Income statement	**Year ended December 31**	
	2019	**2018**
	(DKK thousand)	
Total revenue	-	-
Operating profit/loss	(335,954)	(231,652)
Net profit/loss	(337,497)	(229,600
Total comprehensive income/loss	(337,430)	(229,558)

Balance sheet	**As at December 31**	
	2019	**2018**
	(DKK thousand)	
Total assets	180,754	441,349
Total equity and liabilities	187,754	441,349

Cash flow statement	**Year ended December 31**	
	2019	**2018**
	(DKK thousand)	
Cash flow from operating activities	(326,818)	(234,764)
Cash flow from investing activities	(3,285)	(2,346)
Cash flow from financing activities	58,939	-
Net cash flows	(271,164)	(237,110)
Cash	123,588	394,706

What are the key risks that are specific to the issuer?

The main risks that are specific to the Company's business and industry are:

- Clinical trials being conducted to test the Company's product candidate, arimoclomol, or any future trials for other product candidates may not obtain the desired results or may be delayed or more costly than anticipated.
- The Company is highly dependent on obtaining and maintaining required regulatory approvals and may not receive such approvals.
- The Company has not received approval for any product candidate for commercial sale and, as a result, the Company has never generated any revenue and has incurred significant financial losses, and may continue to incur significant financial losses in the future, which makes it difficult to assess the future viability of the Company.
- The Company's products might not be profitable if the Company is not able to successfully commercialize its products.
- As the Company has focused its efforts on developing applications for arimoclomol, it is currently highly dependent on the potential success of this one product candidate.
- The Company is operating in a field with substantial global competition and swift technological advances which could mean that the competitors of the Company may develop other treatments for similar or the same diseases as those targeted by arimoclomol and may be able to commercialize them more successfully.
- Nearly all aspects of the Company's activities are subject to substantial regulation and compliance and staying up-to-date with such regulation is time-consuming and expensive.
- Even if the Company obtains regulatory approval for arimoclomol, it will remain subject to ongoing regulatory oversight.
- Arimoclomol may be shown to cause undesirable side effects or other properties that could delay or prevent its regulatory approval, limit its commercial profile or result in

significant negative consequences following regulatory approval, if such approval is granted.

- If the Company is unable to obtain or uphold orphan product designation, other designations or marketing exclusivity for its products or product candidates, or if the Company is unable to benefit from the associated marketing exclusivity, it will have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.
- The fact that the Company has received rare pediatric disease designation for arimoclomol as a treatment for NPC is not an indication that it will receive a rare pediatric disease priority review voucher
- The Company may not be able to recruit enough patients for clinical trials to research NMEs or for agreements with investigators and hospitals and this may have a material adverse impact on the Company
- The Company may not be able to recruit enough patients for clinical trials to research NMEs or for agreements with investigators and hospitals and this may have a material adverse impact on the Company

Section C – Key information on the securities

What are the main features of the securities?	The Shares are not divided into share classes. The ISIN code for the Existing Shares is DK0060910917. The temporary ISIN code for the Listing Shares is DK0061274362, which will not be admitted to trading and official listing on Nasdaq Copenhagen. Upon completion of the Listing, expectedly on or around March 17, 2020, the temporary ISIN code of the Listing Shares will be merged with the ISIN code of the Existing Shares, and the Listing Shares will be admitted to trading and official listing on Nasdaq Copenhagen under the permanent ISIN code for the Existing Shares DK0060910917. The Shares are denominated in DKK. At the Prospectus Date, the Company's registered share capital is DKK 27,038,386 divided into 27,038,386 Existing Shares of nominally DKK 1 each or multiples thereof, which are all issued and fully paid up. The Listing comprises up to 3,071,673 Listing Shares for the Listing Shareholders.
Rights attached to the Listing Shares	The Listing Shares are registered with the Danish Business Authority and have the same rights as the Existing Shares. Dividend rights: The Listing Shares have the same rights as the Existing Shares, including with respect to eligibility for any dividends. Any dividends will be paid in DKK to the shareholder's account with VP Securities. No restrictions on dividends or special procedures apply to holders of Listing Shares who are not residing in Denmark. Voting rights and pre-emption rights: All Shares in the Company will rank *pari passu*, including with respect to voting rights and pre-emption rights. All Shares will then carry 1 vote per Share of a nominal value of DKK 1. Liquidation rights: In case of the dissolution or winding-up of the Company, the Listing Shares will be entitled to a proportionate part of the Company's assets after payment of the Company's creditors. The Articles of Association do not contain any provisions on redemption or exchange of Shares.
Restrictions	The Listing Shares are negotiable instruments and no restrictions under the Company's Articles of Association or Danish law apply to the transferability of the Listing Shares. The Shares are subject to transfer and reselling restrictions in certain jurisdictions. Any acquirer of Shares must comply with all applicable laws and regulations in force in any country or region in which it acquires or resells Shares or possesses or distributes this Listing Prospectus and must obtain any consent, approval or permission required for acquiring Shares. This Listing

Prospectus may not be distributed or otherwise made available, and Shares may not be offered or sold, directly or indirectly, in the United States, Canada, Australia or Japan. This Listing Prospectus may not be distributed or otherwise made available, and the Shares may not be offered or sold, directly or indirectly, in any other jurisdiction outside Denmark, unless such distribution, offer, sale or exercise is permitted under applicable laws in the relevant jurisdiction. The Shares have not been approved, disapproved or recommended by any foreign regulatory authorities, nor have any of such authorities passed upon or endorsed the merits of the Listing or the accuracy or adequacy of this Listing Prospectus.

Dividend policy

The Company has not declared or made any dividend payments for the last financial year. Currently, the Company intends to use all available financial resources as well as revenue, if any, for purposes of the Company's current and future business. As of the date hereof, the Company does not expect to make dividend payments within the foreseeable future.

Where will the securities be traded?

The Listing Shares were registered with the Danish Business Authority on February 11, 2020 and issued through VP Securities the same day.

The Listing Shares will not be admitted to trading and official listing on Nasdaq Copenhagen under the temporary ISIN.

The Listing Shares will be admitted to trading and official listing on Nasdaq Copenhagen under the same ISIN code as the Existing Shares, DK0060910917, with the expected first day of trading and official listing being on or around March 17, 2020.

What are the key risks that are specific to the securities?

- It may be difficult or impossible for the Company's shareholders and investors outside Denmark to enforce judgments from their home jurisdictions against the Company
- The market price of the Shares may be highly volatile

Section D – Key information on the offer and admission to trading

Under which conditions and timetable can I invest in this security?

Not applicable as there is no offering of Shares and the purposed of the Prospectus is solely prepared for the purposed of having the Listing Shares admitted to trading and official listing on Nasdaq Copenhagen

The Listing Shares are registered under the temporary ISIN code DK0061274362, which will not be admitted for trading and official listing on Nasdaq Copenhagen and such temporary ISIN code will subsequently be merged with the existing ISIN code for the Existing Shares DK0060910917 in VP Securities. Upon completion of the Listing, the Listing Shares will be admitted to trading and official listing under the ISIN code of the Existing Shares, which is expected to take place on or around March 17, 2020.

Terms and conditions of the offering

The Listing Shares were registered with the Danish Business Authority, issued in VP Securities and delivered to the Lending Shareholders through Danske Bank on February 11, 2020.

The expected timetable is as follows:

Publication of Prospectus	March 16, 2020
Admission of the Listing Shares for trading and official listing under the existing ISIN code	On or around March 17, 2020

Admittance to trading	Nasdaq Copenhagen has approved to admit the Listing Shares for to trading and official listing subject to publication of the Prospectus. The Listing Shares are expected to be admitted for trading and official listing on Nasdaq Copenhagen on or around March 17, 2020 under the existing ISIN code DK0060910917.
Plan for distribution	Not applicable since there is no offering of securities for sale or subscription. The purpose of this Prospectus is solely to have the Listing Shares admitted to trading and official listing on Nasdaq Copenhagen.
Dilution	Not applicable since the Listing of the Listing Shares on NASDAQ OMX Copenhagen will not result in any dilution.
Estimated expenses	The estimated expenses payable by the Company in connection with the Listing are DKK 3-4 million ex. VAT. Expenses include fees to auditors, legal and other advisors as well as other expenses connected to the Listing.
Why is this prospectus being produced?	The Listing Shares were issued through VP Securities and registered with the Danish Business Authority on February 11, 2020. The Listing Shares were issued in connection with a Stock Lending and Subscription Agreement entered into on February 6, 2020 among the Company, Danske Bank and the Lending Shareholders pursuant to which the Company borrowed 3,071,673 existing shares (the "**Lending Shares**") from the Lending Shareholders through Danske Bank as settlement agent in order for the Company to place such Lending Shares in a private placement (the "**Private Placement**"). The Lending Shares were borrowed subject to an obligation for the Company to issue new shares of an equivalent number as the Lending Shares placed in the Private Placement, such new shares being the Listing Shares, and for Danske Bank to use the proceeds from the sale of Lending Shares in the Private Placement to subscribe for the Listing Shares and deliver the Listing Shares to the Lending Shareholders. The Listing Shares were issued in the temporary ISIN code, DK0061274362, and delivered to the Lending Shareholders on February 11, 2020.
Net amounts and use of proceeds	There are no net proceeds since there is no offering of securities for sale or subscription. The purpose of this Listing Prospectus is solely to have the Listing Shares admitted to trading and official listing on NASDAQ OMX Copenhagen.
Underwriting	Not applicable since there is no offering of securities for sale or subscription. The purpose of this Prospectus is solely to have the Listing Shares admitted to trading and official listing on Nasdaq Copenhagen.
Material conflicts of interest	No material conflicts of interest pertaining to the Listing or the admission to trading exists.

RISK FACTORS

Prospective investors should carefully consider all information in this Prospectus (including any information or material incorporated by reference), including the risks described below, before they decide to subscribe to invest in the Shares. This section addresses both general risks associated with the industry and market in which the Company operates and the specific risks associated with its business and its intellectual property rights. If such risks were to materialize, the Company's business, results of operations, cash flows, financial condition, and/or prospects could be materially and adversely affected. With respect to forward-looking statements that involve risks and uncertainties, see "*General Information–Forward-looking statements*".

The risks and uncertainties discussed below are those that the Company's management currently views as material, but these risks and uncertainties are not the only ones that it faces. Additional risks and uncertainties, including risks that are not known to the Company at present or that its management currently deems immaterial, may also arise or become material in the future and could, individually or in the aggregate, have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects. The most material risks, as currently assessed by the Company, taking into account the expected magnitude of their negative impact on the Company and the Company's business and the probability of their occurrence are set out first in each category of risk factors below.

Risks Relating to the Company's Business and Industry

Clinical trials being conducted to test the Company's product candidate, arimoclomol, or any future trials for other product candidates may not obtain the desired results or may be delayed or more costly than anticipated

Prior to launching a pharmaceutical product into the market, its safety and efficacy for treatment of patients must be ascertained through execution of certain pre-clinical studies and clinical trials conducted in accordance with the International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use ("**ICH**"), the U.S. Food and Drug Administration's ("**FDA**"), EMA's and other applicable regulatory authorities' legal requirements, regulations and guidelines, including good laboratory practices ("**GLP**"), an international standard meant to harmonize the conduct and quality of non-clinical studies and the reporting of findings, as well as good clinical practices ("**GCP**"), an international ethical and scientific quality standard for designing, conducting, recording and reporting trials that involve the participation of human subjects. Conducting such trials is complex, costly and time-consuming, and neither the results nor the timing can be predicted with any certainty. Certain of the clinical trials that the Company currently sponsors relate to pediatric diseases for which there are additional regulatory requirements.

The performance of clinical trials is associated with risks, including (but not limited to) risks related to designing the trials in the most appropriate manner, complying with regulatory requirements, entering into agreements with investigators and hospitals and recruiting patients.

There is a risk that the clinical trials that the Company currently sponsors will not confirm previous results or will not demonstrate sufficient evidence of safety and efficacy to receive requisite regulatory approvals. This risk is compounded by the fact that, thus far, the Company has only conducted relatively small phase II clinical trials that were not powered for efficacy. Such clinical trials may not lead to pharmaceutical products that can be effectively commercialized. Adverse or inconclusive results may, despite initially promising results, result in the Company's product candidates not receiving requisite approvals for marketing and sale, and there is a risk that additional clinical trials will be required to obtain such approvals or that the Company's clinical development program will be required to be altered, which would result in increased costs, significant delays to filing with regulatory authorities, filing for a narrower indication than previously anticipated or the abandonment of efforts to commercialization of one or more of the Company's product candidates. In addition, the FDA, EMA or other regulatory authorities may not approve or authorize the labeling that the Company believes is necessary or desirable for the successful commercialization of a product.

All of the Company's current clinical trials are studying the same chemical compound, arimoclomol, but for different indications. There is a risk, therefore, that any unexpected findings, including serious adverse events, in one clinical trial may have a "spill-over" effect on other trials, in particular if the finding is related to the compound's safety and tolerability. An adverse or inconclusive finding in one trial, therefore, may halt or significantly delay the Company's entire clinical development portfolio.

Designing and conducting clinical trials for orphan drugs involves additional risks because, for instance, the relevant indications are not well characterized and experience with treatment of orphan diseases being limited. Such risks may also include delays and increased costs.

As clinical product development can be affected by unforeseen delays, increased costs, unexpected adverse events, unforeseen suspensions and unfavorable results, these circumstances could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

In addition, the Company depends on its ability to enter into agreements with contract research organizations ("**CROs**") conducting clinical trials with respect to arimoclomol. Through the Company's CROs, it is in a close, ongoing dialogue with physicians, who are relevant as investigators. However, if the Company is not able, through its CROs, to enter into the necessary agreements with respect to clinical trials, it may have a material adverse effect on the Company's ability to complete such clinical trials. Further, if the counterparties to the Company-sponsored clinical trial agreements do not carry out their obligations or do so within the agreed deadlines, the clinical trials may be delayed, terminated or deemed unsuccessful, which could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

All the Company's completed clinical trials have been small, each with less than 100 persons, and have advanced through phase I and phase II. The Company's later-phase clinical trials are being conducted with larger patient populations, such as its recently-enrolled phase III clinical trial evaluating arimoclomol for the treatment of ALS, which has enrolled 213 patients. In these trials, additional risks, including previously unidentified low incidence safety risks, safety risks associated with high-dose long-term treatment or lack of efficacy may materialize.

If the above risks were to materialize, it could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company is highly dependent on obtaining and maintaining required regulatory approvals and may not receive such approvals

Before the Company can start commercializing its products, a number of regulatory registrations and approvals must be obtained. See "*Company information–Regulation*." For instance, approvals from the authorities and ethical committees as well as consents from patients participating in the Company's clinical trials are required before initiating pre-clinical studies and clinical trials, and marketing authorizations must be obtained from the relevant regulatory authorities. Obtaining regulatory registrations and approvals is highly complex and time-consuming. If required regulatory registrations or approvals are delayed, denied or withdrawn or if the regulatory authorities question the efficacy of arimoclomal as a treatment, it is likely to have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company has not received approval for any product candidate for commercial sale and, as a result, the Company has never generated any revenue and has incurred significant financial losses, and may continue to incur significant financial losses in the future, which makes it difficult to assess the future viability of the Company

The Company is a biopharmaceutical company that has not had any product candidates approved for commercial sale. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk, including risks related to the regulatory approval process for the Company's lead product candidate, arimoclomol. To date, the Company has focused on research and development activities and, in particular, on developing arimoclomol, as described in "*Company*

information–Business". Since the inception of the Company in 2009, the Company has incurred significant losses, which have substantially resulted from costs related to its research and development programs and general and administrative activities ("**G&A activities**").

The Company anticipates that its expenses will increase substantially if, and as, the Company, for instance:

- continues the ongoing and planned development of its product candidate, arimoclomol for multiple indications;
- initiates, conducts and completes ongoing, anticipated or future preclinical studies and clinical trials for its current and future product candidates;
- seeks marketing approvals for product candidates that successfully complete clinical trials;
- pays milestone and royalty fees to CytRx and any other third parties from whom the Company has licensed intellectual property in accordance with the terms of the applicable license agreement;
- establishes a sales, marketing and distribution infrastructure to commercialize products for which it may obtain marketing approval;
- continues to build a portfolio of product candidates through the acquisition or in-license of drugs, product candidates or technologies; and
- adds operational, financial and management information systems and personnel, including personnel to support its product development and planned future commercialization efforts.

If annual operating expenses increase significantly over the next several years and the Company has not been able to commercialize its drug candidate, the Company will have less financial resources available for its other business prospects, which could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The commercial success of our drug products depends on the availability and sufficiency of third-party payor coverage and reimbursement

The Company's ability to commercialize any products successfully will also depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government authorities, private health insurers and other organizations. Even if the Company succeed in bringing one or more products to the market, these products may not be considered cost-effective, and the amount reimbursed for any products may be insufficient to allow the Company to sell its products on a competitive basis. Because the Company's product candidates are still under development, the Company is unable at this time to determine their cost effectiveness or the likely level or method of coverage and reimbursement. If the price the Company is able to charge for any products it develops, or the coverage and reimbursement provided for such products, is inadequate in light of the Company's development and other costs, its return on investment could be affected adversely.

The Company's products might not be profitable if the Company is not able to successfully commercialize its products

Whether commercialization will be successful and whether the Company will ultimately be profitable, will depend on factors such as the Company's ability to successfully execute its business strategy and attract and build-up the internal resources necessary to effectively market its products. The Company does not currently have in-house capabilities for sales, marketing and distribution but intends to develop such capabilities in order to market its products, if approved, directly through its own sales and marketing force in selected geographic areas, including the United States and Europe. In order to implement this strategy of commercializing in-house, the Company must develop a sales and marketing organization and establish distribution capabilities. This entails recruiting additional managerial,

operational, financial and other employees, which would be expensive and time-consuming and could delay product launches.

Even if the Company is able to build its in-house capabilities for sales, marketing and distribution, market acceptance by physicians, patients, third-party payors and others in the medical community may be less than estimated. Market acceptance will require the Company to build and maintain strong relationships with key opinion leaders ("**KOLs**") (primarily high-prescribing community physicians, nurses and doctors within treatment of orphan diseases). The number of KOLs within orphan diseases is limited and a failure to build or maintain vital relationships with these KOLs could result in lower market acceptance. The Company's efforts to educate physicians, patients, third-party payors and others in the medical community on the benefits of its product candidate may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of the Company's product candidate, arimoclomol. The degree of market acceptance of, for instance, arimoclomol, if approved for commercial sale, will depend on a number of factors, including, but not limited to:

- the convenience and ease of administration as an oral capsule, sprinkled in food/beverage or via a feeding tube compared to alternative treatments and therapies;
- any restrictions on the use of arimoclomol together with other medications and the prevalence and severity of any side effects;
- the efficacy and potential advantages compared to alternative treatments and therapies;
- the cost-effectiveness of arimoclomol compared to alternative therapies and the ability to offer such drug for sale at competitive prices;
- the effectiveness of sales and marketing efforts and the strength of marketing and distribution support; and
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies.

Even if the Company receives market acceptance for a product candidate, such as arimoclomol, the Company bases its development activities and commercial strategy on estimates of the number of patients who may benefit from and who may be medically eligible for a particular treatment. In addition to the number of patients, the ultimate pricing of the Company's products or product candidate, if and when approved, may be affected by the burden of disease, the extent of unmet need and clinical efficacy. Even if the number of medically eligible patients is correctly estimated, the number of patients who will ultimately receive a particular treatment may be greatly reduced if local governments decide to change reimbursement policies. These estimates are subject to significant uncertainty and may prove to be inaccurate. If they are inaccurate, the Company may not generate significant drug revenue and may not become profitable.

All the above risks, individually or in the aggregate, could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

As the Company has focused its efforts on developing applications for arimoclomol, it is currently highly dependent on the potential success of this one product candidate

To date, the Company has focused substantially all of its efforts on the development of arimoclomol. The Company is currently conducting pre-clinical studies and clinical trials based on the arimoclomol molecule. Although the Company is in the discovery phase with respect to new molecular entities, it remains highly dependent on arimoclomol. Therefore, if arimoclomol does not lead to new treatments for the indications the Company is currently exploring, the Company will have spent substantial time and financial resources without receiving a return on investment. As a result, if arimoclomol does not become a success, this could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company is operating in a field with substantial global competition and swift technological advances which could mean that the competitors of the Company may develop other treatments for similar or the same diseases as those targeted by arimoclomol and may be able to commercialize them more successfully

The life sciences industry is subject to substantial global competition and swift technological advances. Certain companies are currently developing, or may initiate development of, competing product candidates targeting the same diseases as those targeted by the Company. For instance, the Company is aware of several pharmaceutical and biopharmaceutical companies that have successfully commercialized products or have commenced clinical trials of products candidates addressing indications that the Company targets with arimoclomol, including, but not limited to, Edaravone to treat ALS (marketed by Mitsubishi Tanabe Pharma in the United States under the brand name Radicava® and in Japan under the brand name Radicut®) or Zavesca targeting Gaucher's disease Type I in the United States and EU and also approved and marketed in the EU to treat NPC. The Company may also face heightened competition from gene therapy, alternative treatment forms and generics, after expiry of patent protection and loss of any market exclusivity for the Company's products.

Further, these competitors may have greater resources than the Company, develop more effective or affordable product candidates than the Company or develop their product candidates faster or more efficiently than the Company and thereby achieve commercialization of their products earlier or more effectively than the Company.

If the Company is unable to respond effectively to competition, the Company's product candidate, arimoclomol, may be rendered obsolete and the ability of the Company to generate revenue may be limited, which could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

Nearly all aspects of the Company's activities are subject to substantial regulation and compliance and staying up-to-date with such regulation is time-consuming and expensive

The Company's business activities are subject to a wide range of laws as well as regulations, including those promulgated by the FDA and EMA, and other regulatory authorities, regulating matters such as orphan drug designations, clinical trials, use of data, animal testing, approval processes, requirements for production, marketing, sales, pricing, pharmacovigilance and intellectual property rights. Compliance with such laws is time-consuming and expensive. In addition, the FDA, EMA or comparable regulatory authorities may change their policies, adopt additional regulations or revise existing regulations or take other actions, which may prevent or delay approval of the Company's product candidates. Changes to the prevailing legal or regulatory regime, may cause the Company to incur significant costs, revise, delay or discontinue all or part of its development program or adopt new processes and procedures in order to comply with new laws or regulations, which may negatively impact how the Company develops, attests, produces, markets or sells its products, for instance, by making it more costly and demanding to develop or obtain approval for arimoclomol and this may materially and adversely affect the Company's business, results of operations, cash flows, financial condition, and/or prospects.

Even if the Company obtains regulatory approval for arimoclomol, it will remain subject to ongoing regulatory oversight

Even if the Company obtains regulatory approvals for arimoclomol, such approvals will be subject to ongoing regulatory requirements for manufacturing, labelling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information.

Any regulatory approvals that the Company receive for arimoclomol may also be subject to a risk evaluation and mitigation strategy ("**REMS**"), limitations on the approved indicated uses for which the drug may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase IV trials, and surveillance to monitor the quality, safety and

efficacy of the drug. Such regulatory requirements may differ from country to country depending on where the Company has received regulatory approval.

In addition, drug manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the NDA or foreign marketing application. If the Company, or a regulatory authority, discover previously unknown problems with a drug, such as adverse events of unanticipated severity or frequency, or problems with the facility where the drug is manufactured or if a regulatory authority disagrees with the promotion, marketing or labelling of that drug, a regulatory authority may impose restrictions relative to that drug, the manufacturing facility or the Company, including requesting a recall or requiring withdrawal of the drug from the market or suspension of manufacturing.

If the Company fails to comply with applicable regulatory requirements following approval of arimoclomol, a regulatory authority may:

- issue an untitled letter or warning letter asserting that the Company is in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending NDA, MAA or comparable foreign marketing application or any supplements thereto submitted by the Company or its partners;
- restrict the marketing or manufacturing of the drug;
- seize or detain the drug or otherwise require the withdrawal of the drug from the market;
- refuse to permit the import or export of arimoclomol; or
- refuse to allow the Company to enter into supply contracts, including government contracts.

Moreover, the FDA strictly regulates the promotional claims that may be made about drug products. In particular, a product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labelling. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant civil, criminal and administrative penalties. However, physicians may, in their independent medical judgment, prescribe legally available products for off-label uses. The FDA does not regulate the behavior of physicians in their choice of treatments but the FDA does restrict manufacturer's communications on the subject of off-label use of their products.

If the Company is unable to comply with applicable regulatory requirements, it may be subject to fines, withdrawal of regulatory approvals, recall of products, suspension of manufacturing or other operational restrictions, as well as criminal sanctions and damage claims. Any government investigation of alleged violations of law could require the Company to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit the Company's ability to commercialize arimoclomol and harm its business, results of operations, cash flows, financial condition, and/or prospects.

Arimoclomol may be shown to cause undesirable side effects or other properties that could delay or prevent its regulatory approval, limit its commercial profile or result in significant negative consequences following regulatory approval, if such approval is granted

The Company or regulatory authorities may suspend clinical trials at any time if it is believes that patients who participate in such clinical trials are being exposed to unacceptable health risks resulting in an unfavorable risk-benefit assessment or other adverse events. Undesirable side effects caused by arimoclomol could cause the Company or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive use permitted by such regulatory authorities or the delay or denial of approval by such regulatory authorities. In the event that the data from clinical trials suggest an unacceptable severity and prevalence of adverse side effects, such clinical trials could be suspended or terminated, and regulatory authorities could order the Company to cease further development of, or deny approval of, arimoclomol for any or all targeted indications. Treatment-related side effects could affect patient recruitment or the ability of enrolled patients to complete a clinical trial or result in potential product liability claims. In addition, if adverse effects occur during clinical testing or during the Company's EAP, the Company may also have to conduct additional testing, which will cause delays in its development program and result in increased costs, or may ultimately lead to the abandonment of the development of the product in question

Even after receiving approval, the products may later exhibit adverse effects that could prevent their widespread use or necessitate their withdrawal from the market. If adverse events occur once the product is on the market, there is a risk that the Company may have to recall and destroy products, and ultimately there is a risk of fines, suspension or withdrawal of regulatory approvals and potential litigation.

Any of these events could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

If the Company is unable to obtain or uphold orphan product designation, other designations or marketing exclusivity for its product candidates or any future products, or if the Company is unable to benefit from the associated marketing exclusivity, it will have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects

The Company is dependent on retaining and obtaining orphan drug designations for NPC, sIBM and ALS, breakthrough therapy designation for NPC and fast track designation for NPC and sIBM. Further, it is dependent on the conversion of orphan drug designations into orphan drug status for arimoclomol for the treatment of NPC, sIBM and ALS after marketing approval as such status will, subject to certain conditions, grant the Company market exclusivity, which is increasingly important in light of the fact that the Company's general patent protection for the composition-of-matter coverage expired in 2020, as the market exclusivity resulting from a grant of orphan drug status would effectively prolong the period during which the products are protected. The patent protection for treatment of lysosomal diseases expires in 2029.

Even if the Company obtains orphan drug exclusivity for a product, the exclusivity thus created may not effectively protect the product from competition, because exclusivity can be suspended under certain circumstances. In the United States, even after an orphan drug is approved, the FDA can subsequently approve another drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the EU, orphan drug exclusivity will not prevent a marketing authorization being granted for a similar medicinal product in the same indication if the new product is safer, more effective or otherwise clinically superior to the first product or if the marketing authorization holder of the first product is unable to supply sufficient quantities of the product. If the Company's competitors are able to obtain orphan product exclusivity for their products in the same indications for which the Company is developing arimoclomol, the Company may not be able to have its products approved by the applicable regulatory authority within a significant period of time, or at all.

If an orphan drug designation or other designations are revoked or if the market exclusivity granted in connection with the orphan drug designation period is suspended, shortened or revoked, it could have a material adverse impact on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

If the Company is not able to obtain or maintain orphan drug status for NPC, sIBM and ALS or for other diseases or disorders, or if the Company is unable to benefit from the associated marketing exclusivity, it could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The fact that the Company has received rare pediatric disease designation for arimoclomol as a treatment for NPC is not an indication that it will receive a rare pediatric disease priority review voucher

As further described under "*Company information–Business*", the FDA has granted a rare pediatric disease designation to arimoclomol as a treatment for NPC, and the Company may seek rare pediatric disease designations for its other product candidates. Under the FDA's rare pediatric disease priority review voucher program, upon the approval of a new drug application for the treatment of a rare pediatric disease, the sponsor of such application would be eligible for a rare pediatric disease priority review voucher that can be used to obtain priority review for a subsequent new drug application. However, receiving a rare pediatric disease designation for arimiclomol as a treatment for NPC does not automatically mean that the Company will receive a priority review voucher as priority review voucher is only awarded following approval by the FDA of arimoclomol as a treatment for NPC.

If a priority review voucher is granted, the Company may use the voucher for its own FDA approval processes or decide to sell the voucher to other biotech or pharmaceutical companies. There is no established market for priority review vouchers and disclosed sales prices may not be indicative of the current value of vouchers, which may also fluctuate significantly. The term of the rare pediatric disease priority review vouchers program expires after September 2020 and may not be renewed or may be amended or cancelled prior to such expiry. Hence, it may be unavailable to the Company even if it meets all of the requirements. Further, the potential award of a voucher would trigger an obligation to market the relevant rare pediatric disease product within one year from FDA approval or the FDA may revoke the voucher. Finally, a voucher award subjects the Company to post marketing reporting obligations to the FDA.

The Company may not be able to recruit enough patients for clinical trials to research NMEs or for agreements with investigators and hospitals and this may have a material adverse impact on the Company

The diseases for which the Company's product candidates are currently being developed are rare and, consequently, patient groups relevant for testing the Company's product candidate, arimoclomol, are limited in size and located across many jurisdictions, see the relevant descriptions under "*Company information–Business*". Therefore, even though the Company, through its

CROs, cooperates closely with relevant physicians treating such patient groups, finding and recruiting the appropriate number of patients for the clinical trials, as well as patients with a profile appropriate for such clinical trials, may be challenging. Should clinical trials in indications similar to the Company's product candidate be initiated, it could negatively affect the possibility that it will be able to recruit patients. Failure to find and recruit the necessary number of appropriate patients to complete the Company's clinical trials could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

Product liability and other claims or litigation may have material adverse effects on the Company's business

Companies in the life sciences industry, such as the Company, are generally subject to risks related to product liability litigation and other claims or litigation.

Product liability risks are inherent in developing, marketing and sale of pharmaceutical products. Even though the Company is not currently subject to any product liability claims, such claims could arise at a later date. Litigation would be time-consuming for the Company's management and lead to significant costs and losses, which would adversely affect the Company's business, results of operations, cash flows, financial condition, and/or prospects.

In addition, the Company may from time to time become involved in various litigation matters and governmental or regulatory investigations, prosecutions or similar matters arising out of its current or future business. The Company cannot accurately anticipate how the liabilities from any claims asserted against it, regardless of merit or eventual outcome, may harm the Company's reputation. There is no guarantee that the Company will be successful in defending against future litigation or similar matters brought under various laws.

Even though the Company has taken out product liability insurance in respect of all clinical trials it has performed and is performing with respect to its product candidates and insurance coverage appropriate for the commercialization of its products, there can be no assurance that such insurance coverage will continue to be available on reasonable commercial terms or that it will prove adequate. If sufficient insurance coverage is not obtained covering, for instance, product liability, or if such future litigation or investigation exceeds the Company's insurance coverage, the Company could be subject to significant liabilities, which could have material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects

The Company may in the future seek to enter into collaborations with third parties for the development and commercialization of arimoclomol. If such collaborations are not successful, the Company may not be able to capitalize on the market potential of arimoclomol

The Company may in the future enter into collaboration agreements with third-party collaborators, such as by introducing a license right or a distribution agreement, for development and commercialization of existing or other products to address market opportunities that require large development investments and/or special expertise in selected geographic areas, as well as to share the financial risks involved in drug development and commercialization of arimoclomol.

The Company has no significant experience in entering into major collaboration or license agreements. The Company may be unable to attract partners for collaboration agreements or the terms of those collaboration agreements that it chooses to enter into may not be favorable to the Company. This may be a result of factors such as general market demand for particular products or products within specific therapeutic areas, results of clinical trials relating to the product candidates or market competition.

If the Company is not successful in efforts to enter into future partnership agreements, the Company's business, results of operations, cash flows, financial condition, and/or prospects may be negatively affected. Even if the Company is successful in entering into collaboration agreements, such agreements may not lead to development or commercialization of the products in the most efficient manner or at all.

With any future collaboration agreements, the Company expects to have limited control over the amount and timing of resources that such collaborators dedicate to the development or commercialization of the products. The ability to generate revenue from these arrangements will depend on such collaborators' abilities to successfully perform the functions assigned to them in these arrangements. The Company's potential partners may have significant discretion in determining how to pursue planned activities and the Company may have limited control over the quality and nature of the efforts and resources that such a partner applies to the collaboration as well as the branding and marketing of the Company and its products. The Company cannot be certain that any collaborations will be scientifically or commercially successful or that it will receive revenues from any collaboration agreements.

Should any of the risks associated with the entering into collaborations with third parties for the development and commercialization of arimoclomol materialize, these could have a material adverse

effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company may require additional capital in the future, which may not be available to it on commercially favorable terms, or at all

The Company is currently loss-making and it may need to raise additional capital in the future. The Company has so far been financed by funds provided by debt providers or invested by its shareholders. Based on the current operating plan and the existing capital resources together with the net proceeds from the Private Placement, the Company expects to be able to fund its operating plan for at least 12 months. However, the operating plan may change as a result of many factors currently unknown, and it may be necessary to seek additional funds sooner than anticipated. The future funding requirements will depend on many factors, including, but not limited to, the progress, timing, scope, results and costs of the Company's pre-clinical studies and clinical trials, including the ability to enroll patients in a timely manner for clinical trials as well as the time and cost necessary to obtain regulatory approvals for the Company's product candidates. In addition, funding requirements will also depend on the progress in commercialization and promotion of the Company's products and the efforts to develop and commercialize its other existing product candidates as well as the manufacturing, selling and marketing costs associated with the products, including the cost and timing of building sales and marketing capabilities. This extends to the sales price and the availability of adequate third-party coverage and reimbursement for the products of the Company; the number and scope of pre-clinical and discovery programs that the Company may decide to pursue or initiate, the time and cost necessary to respond to technological and market developments and the costs of filing, prosecuting, maintaining, defending and enforcing any patent claims and other intellectual property rights, including litigation costs and the outcome of such litigation.

The Company may seek to raise new capital in the future through public or private debt or equity financings by issuing additional shares, debt or equity securities convertible into shares, or rights to acquire these securities.

Any additional financing that the Company could seek may not be available on favorable terms or at all, which could adversely affect the Company's future plans and its ability to execute its strategy, which in turn could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

Interim, "top-line" and preliminary data from the Company's clinical trials that the Company announces or publishes from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data

From time to time, the Company may publish interim, "top-line" or preliminary data from its clinical trials. Interim data from clinical trials that the Company may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or "top-line" data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data the Company previously published. As a result, interim and preliminary data should be viewed with caution until the final data is available. Differences between preliminary or interim data and final data could significantly harm the Company's business prospects and may cause the trading price of its Shares to fluctuate significantly, which could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company is dependent on third-party vendors to provide certain licenses, products and services and its business and operations, including clinical trials, could be disrupted by any problems with its significant third-party vendors

The Company engages a number of third-party suppliers and service providers to supply critical goods and services, such as contract research services, contract manufacturing services and IT services. Disruptions to the business, financial stability or operations of these suppliers and service providers,

including due to strikes, labor disputes or other disruptions to the workforce, for instance, if, as a result of COVID-19 employees are not able to come to work, or to their willingness and ability to produce or deliver such products or provide such services in a manner that satisfies the requirements put forth by the authorities, or in a manner that satisfies the Company's own requirements, could affect the Company's ability to develop and market its products on a timely basis. If these suppliers and service providers were unable or unwilling to continue to provide their products or services in the manner expected, or at all, the Company could encounter difficulty finding alternative suppliers. Even if the Company is able to secure appropriate alternative suppliers in a timely manner, its costs could increase significantly. Any of these events could adversely affect the Company's business, results of operations, cash flows, financial condition, and/or prospects.

Specifically, the Company depends on agreements with external parties that carry out the clinical trials sponsored by the Company. If these external parties do not carry out their obligations under these agreements, or do not meet expected deadlines, if the parties need to be replaced, or if the quality or accuracy of the clinical data they obtain is compromised, ongoing and planned clinical trials may be extended, delayed or terminated which could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company is currently dependent on third parties for manufacturing its products and, if such manufacturer does not deliver its manufactured products in time, this could have a material adverse effect on the Company's business

Manufacturing includes the production, formulation and stability testing of an active pharmaceutical ingredient and its formulation into pharmaceutical products, such as capsules or tablets. The Company does not have its own manufacturing facility and currently the Company does not intend to develop any such manufacturing capacity. The Company is therefore dependent on third parties for manufacturing its products and if any of those third parties terminates the agreements or moves their facilities to a different location, this could have a material adverse effect on the Company's ability obtain such manufactured products. The Company currently depends on one supplier for arimoclomol and were its relationship with that supplier to deteriorate or the contract with such supplier be terminated then that could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company may be required in the future to enter into agreements with other third parties to manufacture arimoclomol at a larger scale to increase supply for potential marketing and sale of its drug (if arimoclomol as a treatment for any of the indications targeted by the Company receives approval in any jurisdiction). The Company can provide no assurance that it will be able to make the transition from the current scale of production to a larger scale of production of arimoclomol or from laboratory-scale production to development-scale production of new molecules. The Company may need to enter into additional collaborative arrangements with other parties who have established manufacturing capabilities, or have other third parties manufacture its products on a contractual basis. The Company may not have access to the substantial financing on acceptable terms that would be required to scale-up production and develop effective commercial manufacturing processes and technologies. The Company may not be able to enter into collaborative or contracting arrangements on acceptable terms with parties that will meet the Company's requirements for quality, quantity and timeliness.

Any manufacturing of pharmaceutical products is subject to a number of regulatory requirements, for instance quality control and documentation. The Company is dependent on its contract manufacturing partners appropriately handling its product in accordance with good manufacturing practices and the costs of compliance may be high. Manufacturing facilities must be approved by the authorities and will be subject to regular inspections by the authorities. Such inspections may lead to suspension of manufacturing and interfere with product supply and distribution. If the Company's existing or future contract manufacturing partners do not manufacture the Company's products or product candidates properly and otherwise fulfil their contractual and regulatory obligations to deliver agreed quantities of products or product candidates in a timely manner and of sufficient quality., this could adversely affect the Company's business, results of operations, cash flows, financial condition, and/or prospects. In addition, any delays in production would delay the Company's pre-clinical studies and human clinical

trials, which could adversely affect the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company, its employees and third-party contractors are subject to safety requirements and any failure to comply with such requirements could result in liability or reputational damage

Due to the chemical ingredients of pharmaceutical products and the nature of the research and development and manufacturing process, the Company, its employees and third-party contractors are subject to safety reporting requirements, environmental regulations and, going forward, additional requirements following potential receipt of marketing approval. If the Company fails to comply with applicable rules and regulations, it could be subject to criminal sanctions and substantial liability or could be required to suspend or modify its operations. Further, if any of the Company's employees or third party contractors perform acts or omissions that are considered unethical, criminal or otherwise contrary to applicable laws and regulations or internal guidelines, the Company's reputation may be harmed, which could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

Hazardous materials are used by the Company in its research and development, such as Rotenone. The Company cannot completely eliminate the risk of accidental contamination or injury from the use, storage, handling or disposal of these materials. The Company may be held liable for any accident or injury that occurs as a result of this risk, the costs of which may exceed any insurance coverage that the Company currently has, which could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

Although the Company believes that it holds all permits required for its use of hazardous materials, any failure to comply with applicable laws and regulations could result in fines, suspension of permits or authorizations or claims for damages, which could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company's relationships with customers, physicians, and third-party payors may be subject, directly or indirectly, to U.S. federal and state, as well as foreign, healthcare fraud and abuse laws, false claims laws, health information privacy and security laws, and other healthcare laws and regulations. If the Company is unable to comply, or have not fully complied, with such laws, the Company could face substantial penalties

Healthcare providers, physicians and third-party payors in the United States and elsewhere will play a primary role in the recommendation and prescription of any product candidates, including arimoclomol, for which the Company obtains marketing approval. The Company's current and future arrangements with healthcare professionals, principal investigators, consultants, customers and third-party payors may subject it to various U.S. federal and state, as well as foreign, fraud and abuse laws and other healthcare laws, including, without limitation, the U.S. federal Anti-Kickback Statute, the federal civil and criminal false claims laws, transparency laws and regulations, and health information privacy and security laws. In addition, many states have similar laws and regulations that may differ from each other and federal law in significant ways, thus complicating compliance efforts. For example, states have anti-kickback and false claims laws that may be broader in scope than analogous federal laws and may apply regardless of payer. In addition, state data privacy laws that protect the security of health information may differ from each other and may not be preempted by federal law. Moreover, several states have enacted legislation requiring pharmaceutical manufacturers to, among other things, establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales and marketing activities, report information related to drug pricing, require the registration of sales representatives, and prohibit certain other sales and marketing practices. These laws will impact, among other things, the Company's clinical research, proposed sales, marketing and educational programs.

Efforts to ensure that the Company's business arrangements with third parties will comply with applicable healthcare laws and regulations involves substantial costs. Any action against the Company for violation of these laws, even if it successfully defends against it, could cause the Company to incur

significant legal expenses and divert management's attention from the operation of the Company's business operations. If the Company's operations are found to be in violation of any of these laws or any other governmental regulations that may apply to it, the Company may be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, exclusion from participating in government funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if the Company becomes subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, contractual damages, reputational harm and the curtailment or restructuring of the Company's operations.

The Company may not be able to attract, integrate, manage and retain qualified personnel or key employees or its employees may not be able to come to work as a result of COVID-19

The success of the Company's business depends on its ability to successfully develop and commercialize its products. Since the Company's organization currently consists of a limited number of employees with additional personnel hires planned for the years to come, the Company's ability to successfully develop and commercialize its products will depend on recruiting a range of specialist personnel, particularly in the areas of development of new products, planning and managing clinical programs and commercialization of pharmaceutical products, and also requires that the Company retains and develops the necessary qualified personnel who can provide the needed expertise to support its business and operations. The market for qualified personnel is competitive and the Company may not succeed in recruiting personnel to, for instance, commercialize its products as currently envisaged, or it may fail to effectively replace current personnel who depart with qualified or effective successors. The Company's effort to retain and develop personnel may also result in significant additional expenses, which could adversely affect its profitability. The Company can make no assurances that key personnel, including its senior management such as the CEO, the CFO, the CMO, the CCO or the CSO, will continue to be employed or that it will be able to attract and retain qualified personnel in the future. Failure to retain or attract key personnel could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects. In addition, if, as a result of COVID-19, the Company's employees are not able to come to work, then this could also a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company might not be able to identify and develop additional product candidates and, even if it is able to develop additional product candidates, such development might expose the Company to additional and new risks

Efforts to identify, acquire or in-license, and then develop product candidates require substantial technical, financial and human resources, whether or not any product candidates are ultimately identified. The Company's efforts may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development, approved products or commercial revenues for many reasons, including the following:

- the methodology used may not be successful in identifying potential product candidates;
- competitors may develop alternatives that render any product candidates the Company develops obsolete;
- any product candidates the Company develops may nevertheless be covered by third parties' patents or other exclusive rights;
- a product candidate may be shown to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product may not be accepted as safe and effective by physicians, patients, the medical community or third-party payors.

The Company has limited financial and personnel resources and, as a result, the Company may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater market potential. The resource allocation decisions of the Company may cause it to fail to capitalize on viable commercial drugs or profitable market opportunities. If the Company does not accurately evaluate the commercial potential or target market for a particular product, the Company may relinquish valuable rights to that product through collaboration, licensing or other royalty arrangements in circumstances under which it would have been more advantageous for the Company to retain sole development and commercialization rights to such product. If the Company is unsuccessful in identifying and developing additional product candidates or are unable to do so, the Company's business, results of operations, cash flows, financial condition, and/or prospects may be materially and adversely affected.

The Company might be exposed to additional liability as a result of its acquisition of arimoclomol (and certain other molecules) from CytRx Corporation, which could have a material impact on the Company's business, results of operations, cash flows, financial conditions and/or prospects

In 2011, the Company acquired arimoclomol (and certain other molecules), pre-clinical and clinical data, intellectual property rights and other assets, including contractual rights and obligations relating to arimoclomol from the U.S.-based biopharmaceutical company CytRx Corporation ("**CytRx**"). Through the purchase, the Company became party to a number of related research, development and licensing contracts and consequently is responsible for all related obligations under these contracts going forward. There is a risk that, at the time of the acquisition or in the period since, the Company did not manage to properly identify and assess all risks relating to the acquired assets, such as obligations, liabilities, defects or other shortcomings, and that these will materialize at a later stage. If the risks related to the acquisition of arimoclomol (and certain other molecules), as set out above, materialize, they could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects and the Company may have limited recourse against CytRx.

Public health epidemics or outbreaks could adversely impact the Company's business.

In December 2019, a novel strain of coronavirus (COVID-19) emerged in Wuhan, Hubei Province, China. While initially the outbreak was largely concentrated in China and caused significant disruptions to its economy, it has now spread across the world and infections have been reported globally, including the countries where the Company has operations and conduct clinical trials. The extent to which the coronavirus impacts our operations will depend on future developments, which are highly uncertain and inherently unpredictable, including the duration and severity of the outbreak, and the actions to contain the coronavirus or address its impact, among others. In particular, the continued spread of the coronavirus globally could adversely impact our operations, including among others, our manufacturing and supply chain, the Company's potential future sales and marketing, clinical trials, regulatory relations and other activities and could have an adverse impact on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

Global economic uncertainty and other global economic or political and regulatory developments could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects

Growth in the global pharmaceutical market has become increasingly tied to (i) global economic growth as an economic downturn may, for example as the result of COVID-19 paralyzing economic activities, for instance, reduce the amount of funding for the pharmaceutical sector as a whole or certain diseases targeted by the Company and (ii) political conditions, tension and uncertainty which could, for instance, impact the regulation applicable to the Company. The successful commercialization of arimoclomol will depend in part on the extent to which governmental authorities and health insurers are willing or able to establish coverage, and adequate reimbursement levels, as well as pricing policies.

Uncertain political and geopolitical conditions currently exist in various parts of the world, including, but not limited to, barriers to free trade and free movement of people in the EU following the United Kingdom's exit from the EU on January 31, 2020 and transition period that is set to end on December 31, 2020. The full effects of the United Kingdom's exit from the EU are impossible to predict but may result in significant market volatility and dislocation, and adversely affect the UK, European and global economy. In addition, as part of Brexit, EMA has formally relocated to Amsterdam on March 30, 2019. This relocation might interrupt current administrative routines and occupy resources, which may cause delays in EMA's handling of the Company's applications or otherwise adversely affect its dealings with EMA. In addition, the UK will no longer be covered by the centralized procedures for obtaining EU-wide marketing authorization from EMA and, unless a specific agreement is entered into, a separate process for authorization of medical products, including the Company's product candidate, arimoclomol, will be required in the UK, the potential process for which is currently unclear. Brexit may, therefore, adversely affect and delay the Company's ability to commercialize, market and sell arimoclomol in the UK. Brexit may also result in a reduction of funding to EMA if the UK no longer makes financial contributions to European institutions, such as EMA. If UK funding is reduced, it could create delays in EMA issuing regulatory approvals for arimoclomol.

Future legal or regulatory changes in jurisdictions where the Company currently operates, or in such jurisdictions in which it may choose to operate in the future, could materially and adversely affect the Company's business, results of operations, cash flows, financial condition, and/or prospects, including by imposing regulatory and operational restrictions and compliance obligations on its business, reducing its revenue or increasing its expenses. For instance, changes in applicable laws in the following areas may have an impact on the Company's operations: orphan drugs; clinical trials; use of data; animal testing; regulatory approval processes; requirements to production; marketing, sales and pricing of pharmaceutical products; pharmacovigilance and other regulatory requirements; and intellectual property rights.

In the United States, in particular, and in the other principal markets in which the Company may in the future sell arimoclomol, if and when approved, there is continued economic, regulatory and political pressure to promote changes in healthcare systems that would limit healthcare costs and expand access to healthcare. This uncertainty is further heightened in light of the impeding 2020 presidential elections. Legislation that has been enacted in the United States, at both the federal and state levels, has introduced cost-reduction measures and other provisions that could decrease the coverage and compensation that the Company may receive for arimoclomol, if and when, approved. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. In March 2010, the Patient Protection and Affordable Care Act of 2010 ("**ACA**"), as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "**PPACA**"), was passed, which is a sweeping law intended to broaden access to health insurance, improve quality, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. In the years since enactment of the PPACA, there have been, and continue to be, significant developments in, and continued judicial and legislative activity around, attempts to repeal or repeal and replace the ACA. On December 14, 2018, a Texas U.S. District Court Judge ruled that the PPACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Cuts and Jobs Act of 2017. Additionally, on December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the PPACA are invalid as well. It is unclear how this decision, future decisions, subsequent appeals, and other efforts to repeal and replace the PPACA will impact the PPACA and our business. Due to these efforts, there is significant uncertainty regarding the future of the PPACA, and its impact on the Company's business and operations. . Additionally, there has been heightened governmental scrutiny in the United States of pharmaceutical pricing practices in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several recent congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. In addition, changes to the political landscape in the United States

(including as a result of the 2020 presidential elections) may impact the market sentiment surrounding the pharmaceutical industry.

In the EU, changes to healthcare systems, including the establishment and operation of health services and the pricing and reimbursement of medicinal products, are almost exclusively a matter for national, and not EU, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most EU member states have resulted in restrictions on the pricing and reimbursement of medicines, and such measures are expected to continue, which could affect the Company's ability to commercialize any product candidate for which it obtains marketing approval.

The above circumstances, individually or in the aggregate, could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company is subject to risks related to data privacy concerns, cyber security breaches and failure to comply with privacy regulations and security requirements relating to data

The Company is subject to data protection laws, privacy requirements and other regulatory restrictions in the various jurisdictions in which the Company operates. During the course of its business, the Company comes in the possession of sensitive personal data, including information from clinical trials, and health data obtained in connection with reporting of adverse events. This information needs to be handled by the Company in compliance with such laws.

The Company's failure to keep apprised of, and comply with, privacy, data use and security laws, standards and regulations, including, for instance, unauthorized disclosure of, or access to, data, could result in the suspension or revocation of the Company's approvals or registrations, the limitation, suspension or termination of services or the imposition of administrative, civil or criminal penalties, including fines which may be as high as 20 million Euros or 4% of the annual worldwide revenue for serious infringements of the EU General Data Protection Regulation that entered into force in May 25, 2018.

In addition, the Company may obtain health information from third parties in the United States (including research institutions from which the Company may obtain clinical trial data) that are subject to privacy and security requirements under the Health Insurance Portability and Accountability Act of 1996 ("**HIPAA**"), as amended by the Health Information for Economic and Clinical Health Act of 2009 ("**HITECH**"). Additionally, HITECH created four new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in U.S. federal courts to enforce HIPAA and seek attorneys' fees and costs associated with pursuing federal civil actions. Depending on the facts and circumstances, the Company could be subject to criminal penalties, including if it knowingly obtains, uses, or discloses individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA. In addition, such failure or non-compliance may cause existing or potential partners, including hospitals, physicians and patients to cease interacting with the Company, and could damage its reputation and brand. In addition, to the extent more restrictive laws, rules or security requirements relating to business and personal data are adopted in the future in the various jurisdictions in which the Company operates, such changes could have an adverse impact on the Company's business by increasing its costs or imposing restrictions on its business processes. Accordingly, the Company's failure to keep apprised of, and comply with, privacy, data use and security laws, standards and regulations could have a material adverse effect on its reputation and negative affect the Company's business, results of operations, cash flows, financial condition, and/or prospects.

Cyber security attacks on the Company's servers, information systems and databases, or the third-party servers, information systems and databases on which the Company's information is stored, could compromise the security of its data or could cause interruptions in the operations of its business. Notwithstanding safeguards, cyber security breaches, internal security breaches, physical security

breaches or other unauthorized or accidental access to the Company's servers, other information systems or databases could result in tampering with, or the theft or publication of, sensitive information or the deletion or modification of data, or could otherwise cause interruptions in the Company's operations, which could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The tampering with, disruption to, or the theft or publication of, sensitive information or the deletion or modification of records held either in the Company's systems or the systems of others to which it has access, could subject the Company to increased costs and exposure to litigation. The loss of confidential information could result in the payment of damages and reputational harm and could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The financial exposure from the items referenced above could either not be insured against or not fully covered through any insurance that the Company maintains and could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The intended tax effects of the Company's corporate structure depend on the application of the tax laws of various jurisdictions and on how the Company operates its business

During the ordinary course of business, there are many transactions and calculations for which the ultimate tax determination is uncertain. For example, the Company's effective tax rates could be adversely affected by changes in foreign currency exchange rates or by changes in the relevant tax, accounting, and other laws, regulations, principles and interpretations. As the Company intends to operate in numerous countries and tax jurisdictions, the application of tax laws can be subject to diverging and sometimes conflicting interpretations by tax authorities of these jurisdictions. It is not uncommon for tax authorities in different countries to have conflicting views, for instance, with respect to, among other things, the manner in which the arm's length standard is applied for transfer pricing purposes, or with respect to the valuation of intellectual property. Therefore, it is uncertain whether the Company will be able to fully utilize the Company's net operating losses as an income tax benefit for future periods. To the extent that the Company's ability to use its net operating losses is restricted, this may result in the Company paying more tax and could therefore reduce the Company's post-tax profits. In addition, tax laws are subject to change as new laws are passed and new interpretations of the law.

The Company is exposed to changes in foreign currency exchange rates and interest rates

Substantially all of the Company's income is expected to be in U.S. dollars and Euros, while part of its operating costs are currently denominated in DKK, although in the future such DKK denominated operating costs are likely to constitute a smaller percentage of the total operating costs. The Company does not currently have in place hedging contracts to cover its currency risks and, accordingly, fluctuations in DKK against, in particular U.S. dollars, could have an adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company's interest rate risk mainly derives from the fact that the Company holds a large cash position. Significant negative changes in interest rates could affect the value of its funds and any placement thereof and may thereby adversely affect the Company's business, results of operations, cash flows, financial condition, and/or prospects.

Risks Relating to Intellectual Property Rights

If the Company is unable to obtain and maintain protection for relevant intellectual property rights, the value of its products will be significantly and adversely affected

To a large extent, the Company's success is dependent on its ability to obtain and maintain patents and other intellectual property rights for its products. The Company's ability to obtain and maintain such rights may be influenced by a number of factors. For instance, patents issued or licensed may be challenged and/or be invalid or unenforceable or circumvented. Other risks include patents not being issued to the Company based on applications that are currently pending or the scope of the claims being narrowed during the examination process; future products not being patentable; the scope of any patent protection not being sufficiently broad to exclude other competitors; and that others may claim rights to patents and other proprietary rights which the Company holds or licenses.

The patent position of biotech and pharmaceutical companies, including the Company, is generally uncertain and comprises complex legal and factual issues. If the Company fails to obtain and maintain patent protection for its products, the Company could lose its competitive advantage, and the competition it faces would increase, which would have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company may face infringement claims and other challenges by third parties

The Company may be subject to time-consuming infringement actions and could incur significant costs if third parties believe that its products, or the methods used to manufacture or use them, infringe patents or other proprietary rights held by such third parties. The Company and its patent advisers, when performing freedom to operate searches as part of the development of a patent strategy or preparing and processing patent applications, may fail to identify relevant prior art. Should the Company be met with infringement claims or other challenges of its intellectual property rights by third parties, an adverse outcome could be costly, time-consuming and may subject the Company to significant liabilities, and force the Company to curtail or cease the development, marketing and sale of some or all of its products or lead to significant costs for developing non-infringing products or licensing technology or products from the party claiming infringement (which license may not be available on commercially reasonable terms or at all) which could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company may not have enough financial resources to successfully enforce and defend its intellectual property rights

The enforcement and defense of the Company's intellectual property rights, including patent rights, through legal or administrative proceedings may be costly and time-consuming, may divert its personnel from their usual responsibilities and may provide its competitors and others with insights into its proprietary rights. Moreover, there can be no assurance that the Company will have sufficient financial or other resources to conduct such enforcement or defense actions. An adverse determination in any litigation or other proceeding could put one or more of the Company's patents at risk of being invalidated or interpreted narrowly and could put the Company's pending patent applications at risk of not being issued. The occurrence of any of the above could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The previous employers of the Company's employees and consultants may attempt to assert rights over the Company's intellectual property

The vast majority of the Company's employees and consultants were previously employed at universities or biopharmaceutical or pharmaceutical companies, including competitors and potential competitors to the Company. The Company may be subject to claims that it or these employees have, inadvertently or otherwise, used or disclosed intellectual property, trade secrets or other proprietary information of their former employers. Such claims may lead to material costs for the Company, or an inability to protect or use its intellectual property rights, which could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

The Company will not seek to protect its intellectual property rights in all jurisdictions throughout the world and may not be able to adequately enforce its intellectual property rights even in the jurisdictions where protection is sought

Filing, prosecuting and defending patents on the products in all countries and jurisdictions throughout the world would be prohibitively expensive, and the intellectual property rights in some countries could be less extensive than those in the EU or the United States, assuming that rights are obtained in the EU and the United States. Competitors may use the Company's technologies in such jurisdictions to develop their own products and, further, may export otherwise infringing products to territories where the Company have patent protection, but enforcement is not as strong as that in the EU or the United States.

In addition, the laws of some countries do not protect intellectual property rights to the same extent as in the EU and the United States. Many companies have encountered significant problems in protecting and defending intellectual property rights in certain jurisdictions. The legal systems of some countries, particularly developing countries, do not favor the enforcement of patents and other intellectual property protection, especially those relating to biopharmaceuticals or biotechnologies. This could make it difficult to stop the infringement of the Company's patents, if obtained, or the misappropriation of other intellectual property rights. For example, many countries have compulsory licensing laws under which a patent owner under certain conditions must grant licenses to third parties. In addition, many countries limit the enforceability of patents against third parties, including government agencies or government contractors. In these countries, patents may provide limited or no benefit. Patent protection must ultimately be sought on a country-by-country basis, which is an expensive and time-consuming process with uncertain outcomes.

Such lack of patent protection may lead to material costs for the Company, or the Company may be unable to protect or use its intellectual property rights, which could have a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

If the Company is unable to protect the confidentiality of certain information, the value of its products and technology could be materially adversely affected

The Company's know-how, trade secrets and other intellectual property, including the ability to protect its intellectual property, are significant to the Company. In addition to patented products and technology, the Company relies upon unpatented proprietary technology processes, know-how and data that it regards as trade secrets. The Company seeks to protect its trade secrets in part through confidentiality agreements with employees, consultants and third parties. These agreements may be breached, and the Company may not have adequate remedies for any such breach. In addition, the Company's trade secrets may otherwise become known or be independently developed by competitors in a manner providing the Company with no practical recourse against the competing parties. If any such events were to occur, there could be a material adverse effect on the Company's business, results of operations, cash flows, financial condition, and/or prospects.

Risks Related to the Listing and the Shares

It may be difficult or impossible for the Company's shareholders and investors outside Denmark to enforce judgments from their home jurisdictions against the Company

The Company is incorporated, and a majority of its assets and operations are held and conducted in, Denmark. As such, it may be difficult or impossible for shareholders and investors outside of Denmark to enforce judgments obtained in courts of such shareholder's and investor's home jurisdictions against the Company.

The market price of the Shares may be highly volatile

The market price of the Shares has been and may in the future continue to be highly volatile, subject to significant fluctuations in response to various factors, many of which are beyond the Company's control and which may be unrelated to the Company's business, operations or prospects.

RESPONSIBILITY STATEMENT

The Company's Responsibility

The Company is responsible for this Prospectus in accordance with Danish law.

The Company's Statement

We hereby declare that we, as the persons responsible for this Prospectus on behalf of the Company, have taken all reasonable care to ensure that, to the best of our knowledge, the information contained in this Prospectus is in accordance with the facts and makes no omission likely to affect its import.

We furthermore declare that this Prospectus has been approved by the Danish Financial Supervisory Authority as competent authority under the Prospectus Regulation. The Danish Financial Supervisory Authority only approves this Prospectus as meeting the standards of completeness, comprehensibility and consistency imposed by the Prospectus Regulation. Such approval should not be considered as an endorsement of the Company that is the subject of this Prospectus. The Prospectus has been drawn up as part of a simplified prospectus in accordance with Article 14 of the Prospectus Regulation.

Copenhagen, March 16, 2020

Orphazyme A/S

Board of Directors

Georges Gemayel *Chairman*	Bo Jesper Hansen *Deputy Chairman*
Martijn Kleijwegt *Board Member*	Martin Bonde *Board Member*
Sten Verland *Board Member*	Anders Hedegaard *Board Member*
Catherine Moukheibir *Board Member*	Rémi Droller *Board Member*

Georges Gemayel: Professional board member
Bo Jesper Hansen: Professional board member
Martijn Kleijwegt: Founder and Managing Partner at LSP
Martin Bonde: Professional board member
Sten Verland: Partner and co-founder at Sunstone Life Science Ventures
Anders Hedegaard: Chief Executive Officer of Rodenstock GmbH
Catherine Moukheibir: Professional board member
Rémi Droller: Managing Partner at Kurma Partners

Executive Management

Kim Stratton *CEO*	Anders Vadsholt *CFO*

GENERAL INFORMATION

This Prospectus has been prepared in compliance with Danish law, including the Danish Capital Markets Act, the Prospectus Regulation, Commission Delegated Regulation (EU) no. 2019/980 of March 14, 2019 as well as Commission Delegated Regulation (EU) 2019/979 of March 14, 2019, and Nasdaq Issuer Rules. This Prospectus is governed by Danish law.

References in this Prospectus to the "Company" are references to Orphazyme A/S, and references to the "Group" are references to Orphazyme A/S together with its subsidiaries unless the context requires otherwise. See "*Glossary*" for a list of terms and definitions frequently used in this Prospectus.

This Prospectus is not intended to provide the basis of any credit or any other evaluation and should not be considered as a recommendation or invitation by the Company that any recipient of this Prospectus should acquire any Shares. Any person who comes into the possession of this Prospectus should determine for itself the relevance of the information contained in this Prospectus, and any acquisition of Shares should be based upon such information as it deems necessary.

The information contained in this Prospectus has been provided by the Company and by other sources identified herein

The information contained herein is as at the Prospectus Date, unless stated otherwise. Any material changes in connection with the information in this Prospectus which may affect the evaluation of the Shares, which occurs or is ascertained between the time of approval of this Prospectus and the commencement of trading on Nasdaq Copenhagen of the Listing Shares, will be published as a supplement to this Prospectus pursuant to applicable rules and legislation in Denmark.

Further, no person has been authorized to give any information or to make any representation concerning the Group or the Shares other than contained in this Prospectus, and, if given or made, any such information or representation should not be relied upon as having been authorized by the Company. The Company accepts no liability for any such information or representation.

The Prospectus may not be forwarded, reproduced or otherwise redistributed, in whole or in part, by anyone but the Company. Investors may not disclose any of the contents of this Prospectus or use any information herein.

The Company or any of its representatives will not make any representation to any subsequent purchaser of the Shares.

Furthermore, the Listing Shares are subject to transfer and selling restrictions in certain jurisdictions. See "*The Listing–Terms and conditions of the Listing–Terms, expected timetable and restrictions*". Prospective investors of Shares must comply with all applicable rules and legislation in countries or territories in which they offer or sell Shares or possess or distribute this Prospectus and must obtain consent, approval or permission, as required, for the acquisition of Shares. Any person into whose possession this Prospectus may come are required by the Company to inform themselves about such restrictions and to observe such restrictions. Neither the Company nor the Company's auditors accepts any liability for any violation of these restrictions by any person, irrespective of whether such person is an Existing Shareholder or a potential purchaser of Shares.

Enforceability of judgments

The Company is a public limited liability company organized under Danish law. Some of the members of Management are residents of Denmark, and all or a substantial share of assets of the Company and such persons are located in Denmark. As a result, it may not be possible for investors to effect service of process upon such persons or the Company outside Denmark or to enforce judgments obtained in courts outside Denmark based on applicable legislation in jurisdictions outside Denmark against such persons or the Company.

Third party information

This Prospectus contains statistics, data and other information relating to markets, market sizes, market shares, market positions and other industry data pertaining to the Group's business and markets. Unless otherwise indicated, such information is based on the Company's analysis of multiple sources, including market studies that the Company commissioned from Defined Health, a Cello Health business and Medical Marketing Economics, as well as the U.S. Food and Drug Administration, the European Medicines Agency and Evaluate Pharma.

While the Company can confirm that information from external sources has been accurately reproduced, the Company has not independently verified and cannot give any assurances as to the accuracy of market data as presented in this Prospectus that was extracted or derived from these external sources. As far as the Company is aware and able to ascertain from this information, no facts have been omitted which would render the information provided inaccurate or misleading.

Industry publications or reports generally state that the information they contain has been obtained from sources believed to be reliable, but the accuracy and completeness of such information is not guaranteed. Market data and statistics are inherently predictive and subject to uncertainty and not necessarily reflective of actual market conditions. Such statistics are based on market research, which itself is based on sampling and subjective judgements by both the researchers and the respondents.

The Company makes no representation as to the accuracy of such information that was extracted or derived from these external sources. Thus, any development in the Group's activities may deviate from the market developments stated in the Prospectus. The Company does not assume any obligation to update such information. If information has been obtained from third parties, the Company confirms that such information has been accurately reproduced and that, to the best of the Company's knowledge and belief and in so far as can be ascertained from the information published by such third party, no facts have been omitted which would render the information reproduced inaccurate or misleading.

As a result, prospective investors should be aware that statistics, data, statements and other information relating to markets, market sizes, market shares, market positions and other industry data in this Prospectus (and projections, assumptions and estimates based on such information) may not be reliable indicators of the Company's future performance and the future performance of the industry in which it operates. Such indicators are necessarily subject to a high degree of uncertainty and risk due to the limitations described above and to a variety of other factors, including those described under "*Risk Factors*" included elsewhere in this Prospectus.

Presentation of financial statements and other information

Certain accounting and statistical figures in this Prospectus have been subject to rounding adjustments. Accordingly, the sum of these figures is not necessarily equivalent to the total amounts stated. In addition, certain percentage figures reflect calculations based on the underlying information prior to rounding up and, accordingly, the percentage figures may not necessarily be exactly equivalent to the figures that would be derived if the relevant calculations were based upon the rounded numbers.

References to "DKK" are references to Danish kroner. References to "EUR" are references to the common European currency, and references to "U.S. dollar", "USD" or "$" are references to US Dollar, the lawful currency of the United States.

The audited consolidated financial statements of the Group for the financial year 2019 are included in the Prospectus by reference. The historical financial information for the financial year 2019 has been prepared in accordance with IFRS as adopted by the EU and additional Danish disclosure requirements for annual reports for listed companies. The Company publishes its consolidated financial statements in DKK.

Forward-looking statements

Certain statements in this Prospectus, including, but not limited to, certain statements in "*Summary*", "*Risk Factors*", "*Company information–Information on assets and liabilities, financial position, results and dividend policy–Dividend policy*", "*Company information–Information on assets and liabilities, financial position, results and dividend policy–Financial statement*", "*Company information–Business*" and "*Company information–Prospective Financial Information–Prospective Financial Information*" are based on views of Management, as well as on assumptions made by and information currently available to Management, and such statements may constitute forward-looking statements within the meaning of securities laws of certain jurisdictions. Such forward-looking statements (other than statements of historical fact) regarding the Group's future results of operations, financial position, cash flows, business strategy, plans and objectives of Management for future operations can generally be identified by terminology such as "targets", "believes", "estimates", "expects", "aims", "intends", "plans", "seeks", "will", "may", "anticipates", "would", "could", "continues" or similar expressions or the negative forms thereof. Other forward-looking statements can be identified in the context in which the statements are made.

Such forward-looking statements are subject to known and unknown risks, uncertainties related to investments in the Company and other factors because they relate to events and depend on circumstances that may or may not occur in the future. The Company's actual results may differ significantly from the results discussed or implied in the forward-looking statements. Factors that may cause such difference include, but are not limited to, those discussed in "*Risk Factors*", "*Company information–Business*" and "*Company information–Prospective Financial Information*" herein. The forward-looking statements are made as at the Prospectus Date and, except as required by law or rules and regulations (including, but not limited to the rules of Nasdaq Copenhagen), the Company and the Group undertake no obligation to publicly update or publicly revise any forward-looking statements, whether as a result of new information, future events or otherwise. If one or more of the risk factors described in this Prospectus materializes, it may have an adverse effect on the Company's business, position, results of operations or objectives. In addition, other risks that have not yet been identified or which the Company has not considered to be material may have an adverse effect, and investors may lose all or part of their investments. See "*Risk factors*". In addition, even if its result of operations, financial position and cash flows, and the development of the industry in which it operates, are consistent with the forward-looking statements contained in this Prospectus, those results or developments may not be indicative of results or developments in subsequent periods.

All subsequent written or oral forward-looking statements attributable to the Group or to persons acting on the Company's behalf are expressly qualified in their entirety by the cautionary statements referred to above and contained in this Prospectus, including those set forth under "*Risk Factors*".

COMPANY INFORMATION

1 PERSONS RESPONSIBLE, THIRD PARTY INFORMATION, EXPERTS' REPORTS AND COMPETENT AUTHORITY APPROVAL

1.1 Persons responsible and approval from competent authority

See "*Responsibility statement*" for more details.

1.2 Experts' reports and third party information

This Prospectus does not contain any expert statements or expert reports.

For details on information sourced from third parties, see "*General information–Third party information"*.

2 AUDITORS

The Company's independent auditors are:

ERNST & YOUNG Godkendt Revisionspartnerselskab

CVR no. 30700228

c/o Postboks 250

Dirch Passers Allé 36

Denmark

ERNST & YOUNG Godkendt Revisionspartnerselskab is represented by Christian Schwenn Johansen, State Authorized Public Accountant and Rasmus Bloch Jespersen, State Authorized Public Accountant.

The consolidated financial statements and the parent company financial statements for the financial year January 1 – December 31, 2019 were audited by Christian Schwenn Johansen and Rasmus Bloch Jespersen.

ERNST & YOUNG Godkendt Revisionspartnerselskab has issued reports in this Prospectus.

The auditors in charge are members of FSR – Danish Auditors, the Danish association for state-authorized public accountants, (FSR – Danske Revisorer).

3 RISK FACTORS

See "*Risk Factors*" for more details.

4 COMPANY INFORMATION

4.1 Name and registered office

Orphazyme A/S
CVR no. 32 26 63 55
Ole Maaløes Vej 3
DK-2200 Copenhagen N
Denmark

Legal Entity Identifier (LEI): 54930025OZD2GGSQ7L42

Telephone: (+45) 39 17 82 72
E-mail: contact@orphazyme.com
Website: www.orphazyme.com

The information on the website does not form part of the Prospectus unless that information is incorporated by reference into the Prospectus.

4.2 Country of incorporation and governing law

The Company is a limited liability company incorporated in Denmark and is subject to Danish law.

5 REGULATION

Government authorities in the United States, within the EU or EEA and in other countries, extensively regulate, among other things, the research and development, testing, marketing and import and export of pharmaceutical products. The processes for obtaining regulatory approvals in the United States, the EU and other countries, along with subsequent compliance with applicable statutes and regulations, require the expenditure of substantial time, human and financial resources. Below is a brief overview of how such approval for pharmaceutical product can be obtained, focusing on the market in the United States and in Europe. This high-level summary is in not intended to be exhaustive.

United States

Marketing approval in the United States

Assuming successful completion of the required clinical testing, the results of the nonclinical and clinical studies, together with detailed information relating to the product's pharmaceutical aspects and proposed labeling, among other things, are submitted to the FDA for review as part of an NDA requesting approval to market the product for one or more indications. The review by the FDA typically takes ten months (measured from the "filing" date rather than the receipt date for NDAs, which typically adds approximately two months to the timeline for review and decision from the date of submission). This review can be accelerated through special FDA expedited review and approval programs. See "-Special FDA expedited review and approval programs."

The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity. The FDA may refer an application for a novel drug to an advisory committee, but is not bound by the recommendations of that advisory committee. After evaluating the NDA and all related information, including the advisory committee recommendation, if any, and inspection reports regarding the manufacturing facilities and clinical trial sites, the FDA may issue an approval letter, or, in some cases, a complete response letter. A complete response letter generally contains a statement of specific conditions that must be met in order to secure final approval of the NDA and may require additional clinical or preclinical testing in order for the FDA to reconsider the application. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA will typically issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications and patient populations.

Even if the FDA approves a product, it may, among other things, limit the approved indications for use of the product, require that contraindications, warning or precautions are included, require that post-approval studies be conducted to further assess a drug's safety or effectiveness after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, which can materially affect the potential market and profitability of the product. In addition, after approval, some types of changes to the approved product, such as adding new indications, manufacturing changes, and additional labeling claims, are subject to further testing requirements and FDA review and approval.

U.S. Orphan drug designation

Under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug intended to treat a rare disease or condition, which is a disease or condition that affects fewer than 200,000 individuals in the United States, or if it affects more than 200,000, there is no reasonable expectation that sales of the drug in the United States will be sufficient to offset the costs of developing and making the drug available in the United States. Orphan drug designation must be requested before submitting an NDA. Orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If the FDA approves a sponsor's marketing application for a designated orphan drug for use in the rare disease or condition for which it was designated, the sponsor is eligible for a seven-year period of marketing exclusivity upon NDA/BLA approval, during which the FDA may not approve another sponsor's marketing application for a drug with the same active moiety and intended for the same use or indication as the approved orphan drug, except in limited circumstances, such as if a subsequent sponsor demonstrates its product is clinically superior. During a sponsor's orphan drug exclusivity period, competitors, however, may receive approval for drugs with different active moieties for the same indication as the approved orphan drug, or for drugs with the same active moiety as the approved orphan drug, but for different indications. Orphan drug exclusivity could block the approval of one of the Company's products for seven years if a competitor obtains approval for a drug with the same active moiety intended for the same indication before the Company does, unless the Company is able to demonstrate that grounds for withdrawal of the orphan drug exclusivity exist, or that the Company's product is clinically superior. Further, if a designated orphan drug receives marketing approval for an indication broader than the rare disease or condition for which it received orphan drug designation, it may not be entitled to exclusivity.

Special FDA expedited review and approval programs

The FDA has various programs, including fast track designation, accelerated approval, priority review, and breakthrough therapy designation, which are intended to expedite or simplify the process for the development and FDA review of drugs that are intended for the treatment of serious or life-threatening diseases or conditions and demonstrate the potential to address unmet medical needs. The purpose of these programs is to provide important new drugs to patients earlier than under standard FDA review procedures and reduces the review period to six months (measured from the "filing" date rather than the receipt date for NDAs, which typically adds approximately two months to the timeline for review and decision from the date of submission). Most products that are eligible for fast track and breakthrough therapy designation are also likely to be considered eligible to receive a priority review.

To be eligible for a fast track designation, the FDA must determine, based on the request of a sponsor, that a product is intended to treat a serious or life-threatening disease or condition and demonstrates the potential to address an unmet medical need. The FDA will determine that a product will fill an unmet medical need if it will provide a therapy where none exists or provide a therapy that may be potentially superior to existing therapy based on efficacy or safety factors. As a condition of approval, the FDA may require a sponsor of a drug receiving accelerated approval to perform post-marketing studies to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical endpoint, and the drug may be subject to accelerated withdrawal procedures.

Breakthrough therapy designation is a program intended to expedite the development and review of drugs to treat serious or life-threatening diseases in cases where preliminary clinical evidence shows that the drug may provide substantial improvements over available therapy.

Rare Pediatric Disease ("**RPD**"), is defined by the FDA as a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and the disease affects fewer than 200,000 individuals in the United States or affects more than 200,000 in the United States and for which there is no reasonable expectation that the cost of developing and marketing a drug in the United States for such disease or condition will be received from sales in the United States of such drug. The RPD designation by the FDA enables priority review voucher ("**PRV**"), eligibility upon U.S. market approval of a designated drug for rare pediatric diseases. The

voucher, which is awarded upon NDA or BLA approval to the sponsor of a designated RPD can be sold or transferred to another entity and used by the holder to receive priority review for a future NDA or BLA submission.

Other U.S. Healthcare Laws

The Group is currently, or will in the future be, subject to federal and state healthcare regulation and enforcement by the U.S. federal government and the states in which the Group will conduct its business once its product candidates are approved by the FDA and commercialized in the United States. In addition to the FDA's restrictions on marketing of pharmaceutical products, the U.S. federal and state healthcare laws and regulations that may affect the Group's ability to operate include: fraud and abuse laws, including federal and state anti-kickback and false claims laws, data privacy and security laws, and transparency laws. These laws may adversely affect the Group's sales, marketing and other activities with respect to any product candidate for which the Group receives marketing approval in the United States by imposing administrative and compliance burdens on the Group. If the Group's operations are found to be in violation of any of the federal and state laws described above or any other governmental regulations that apply to the Group, the Group may be subject to significant penalties and sanctions.

In the United States, there has been, and continues to be, several legislative and regulatory changes and proposed reforms of the healthcare system in an effort to contain costs, improve quality, and expand access to care, including the proposed modification to some of the aforementioned laws. These reform initiatives may, among other things, result in modifications to the aforementioned laws and/or the implementation of new laws affecting the healthcare industry. Similarly, a significant trend in the healthcare industry is cost containment. Third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. The Group's ability to commercialize any of its products successfully will depend in part on the extent to which coverage and adequate reimbursement for these products and will be available from third-party payors. As such, cost containment reform efforts may result in an adverse effect on the Group's operations.

Europe

Pharmaceutical Approval in the European Economic Area

In the European Economic Area ("**EEA**", which is comprised of EU, plus Norway, Iceland and Liechtenstein) medicinal products can only be commercialized after obtaining a marketing authorization ("**MA**"). There are three types of MAs:

- The Community MA, which is issued by the European Commission through the Centralized Procedure, based on the opinion of the Committee for Medicinal Products for Human Use ("**CHMP**") of the EMA, and which is valid throughout the entire territory of the EEA. The Centralized Procedure is mandatory for certain types of products, such as biotechnology medicinal products, orphan medicinal products, and medicinal products containing a new active substance indicated for the treatment of AIDS, cancer, neurodegenerative disorders, diabetes, autoimmune and viral diseases. The Centralized Procedure is optional for products containing a new active substance not yet authorized in the EEA, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the EU.

- Decentralized Procedure ("**DCP**"), MAs are available for medical products not falling within the mandatory scope of the Centralized Procedure. An identical dossier, including a draft summary of the product characteristics ("**SmPC**"), and draft labeling and package leaflet, is submitted to the competent authorities of each of the member of the EEA in which the MA is sought, one of which is selected by the applicant as the reference member state ("**RMS**"). The competent authority of the RMS prepares a draft assessment report including proposed revisions to the draft SmPC, draft labeling and package leaflet, which is sent to the other member states (referred to as the concerned member states ("**CMS**")) for their approval. If the CMSs raise no objections, based on a potential serious risk to public health, to the assessment, SmPC, labeling, or package leaflet proposed by the RMS, the product is subsequently granted a national MA in each of the involved member states (i.e., in the RMS and the selected CMS). Where a product has already been

authorized for marketing in a member state of the EEA, a Mutual Recognition Procedure ("**MRP**"), is conducted to enable recognition of the national MA in other selected EEA member states (CMSs). Similarly to the DCP, if the CMSs raise no objections, based on a potential serious risk to public health, to the assessment of the RMS, the product is subsequently granted a national MA in each of the involved CMSs.

- National Procedure MAs, which are issued by a single competent authority of the member states of the EEA and only covers their respective territory, are also available for products not falling within the mandatory scope of the Centralized Procedure. Once a product has been authorized for marketing in a Member State of the EEA through the National Procedure, this National Procedure MA can also be recognized in other member states through the MRP.

EU Orphan Drug Designation

In the EU, Orphan Drug Legislation (2000) was introduced to stimulate the development of orphan drugs in the EU. In the EU, the Committee for Orphan Medicinal Products is responsible for the scientific examination of applications leading to the designation of an Orphan Medicinal Product. Such designation is reassessed at the time a marketing authorization is granted. In the EU, a medicinal product may be designated as an orphan medicinal product if its sponsor can establish that the prevalence of the condition in the EU is not more than five in 10,000 or that it is unlikely that marketing the medicinal product in the EU, without incentives, would generate sufficient return to justify the necessary investment. In the EU there is a further requirement that the drug is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition for which there exists no satisfactory method of diagnosis, prevention or treatment or, if such method exists, that the drug will be of significant benefit as compared to existing treatments to those affected by the condition. If a product is granted Orphan Medical Product status, it is eligible for a 10-year exclusive marketing period in the EU. The period can be reduced to six years in the EU if a product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. In the EU, orphan exclusivity may also be extended for an additional two years (for a maximum of 12 years of orphan exclusivity), if the product is approved on the basis of a dossier that includes pediatric clinical trial data generated in accordance with an approved pediatric investigation plan.

6 BUSINESS

This business review contains a number of observations, judgments and estimates, especially in relation to market sizes, market share and market trends, which are based on Management's estimates and publicly available information. Management's estimates are generally based on the Group's knowledge of the market and various external research and industry reports. External sources were used only to a limited extent in the preparation of this business and market review. However, there can be no assurance that other sources may not express a different opinion of the market, etc. than the one on which Management has based its views. The information regarding market conditions is based on Management's estimates. The forward-looking estimates are subject to substantial uncertainty.

6.1 Overview

The Company is a biopharmaceutical company that pioneers the heat shock protein response ("**HSPs**") for neurodegenerative orphan disorders. The Company develops innovative therapies for the treatment of orphan diseases with a high unmet medical need which are characterized by protein misfolding, aggregation and lysosomal dysfunction and intends to commercialize such therapies if regulatory approval has been received through an in-house sales force and partnerships with distributors. The Company focusses on rare, neurodegenerative disorders (including neuropathic lysosomal diseases ("**LSDs**") and progressive neuromuscular disorders) and strives to profoundly impact the lives of patients and their families living with such diseases. As a result of positive data from certain of its clinical trials, the Company has grown from an entrepreneurial R&D company to a company with significant growth aspirations and an emerging late-stage pipeline that is currently moving into the pre-commercial phase.

The Company was founded in 2009 based on a scientific discovery (published in *Nature*[2]) on the function of HSPs by (among others) Thomas Kirkegaard Jensen, who still serves as Chief Scientific Officer ("**CSO**"). The Company's pipeline is underpinned by deep expertise in the science of cellular stress, particularly the heat shock response ("**HSR**"), the body's natural defence to cellular stress. The Company's lead investigational product candidate, arimoclomol, amplifies endogenous HSPs, which are at the core of the HSR and help guard against toxicity caused by protein misfolding, aggregation and lysosomal dysfunction. Arimoclomol is a small molecule that crosses the blood-brain barrier and is presented in oral, nasogastric form, providing for easy administration as an oral capsule, sprinkled in food/beverage or via a feeding tube. At the Prospectus Date, approximately 540 patients and healthy volunteers have been exposed to arimoclomol and no major safety concerns have been observed.

Since inception, the Company has translated certain of its scientific discoveries into a late stage clinical development program. The Company's most progressed clinical trial with arimoclomol is within LSDs for the treatment of Niemann-Pick disease Type C ("**NPC**"). In January 2019, the Company reported positive results from the full data set of phase II/III arimoclomol trial in NPC and additional positive data from the open-label extension trial was reported in January 2020, which demonstrated a continued positive impact on disease progression over two years. The Company has been granted orphan drug designation by the FDA for arimoclomol as a treatment for NPC in 2015 and orphan designation by the European Medicines Agency (the "**EMA**") for arimoclomol as a treatment for NPC in 2014. Further, the Company has been granted fast track designation in June 2016, rare pediatric disease designation in January 2018 and breakthrough therapy designation in November 2019 by the FDA for treatment with arimoclomol in NPC. In addition, in January 2020, the Company announced the availability of an early access program ("**EAP**") in the United States, which permits the Company to make arimoclomol available pre-commercially to U.S. patients. The Company plans to file for an NDA in the United States in H1 2020 and for an MAA in the EU on in H2 2020 for arimoclomol as a treatment for NPC. If regulatory approvals are received, the Company will implement its commercialization plan for NPC to enter the United States and European markets.

In addition, the Company is currently conducting three other clinical trials with arimoclomol, one more for LSDs (Gaucher disease), and two for neuromuscular diseases (Sporadic Inclusion Body Myositis, ("s**IBM**"), and Amyotrophic Lateral Sclerosis ("**ALS**"), which are also rare and severe diseases with limited or no current treatment options. Beyond these initial indications, and based on data from the Company's preclinical studies, the Company believes arimoclomol has potential in the treatment of other related diseases, including glucocerebrosidase-deficient (GCase) Parkinson's disease and a range of additional LSDs. The Company plans to pursue development of arimoclomol through to registration in Europe and the United States and has been granted orphan drug designation to arimoclomol by the FDA for (i) sIBM in November 2017 and (ii) ALS was transferred to the Company in January 2012, and orphan designation by the EMA, for (i) sIBM in May 2016 and (ii) ALS was transferred to the Company in September 2011. In addition, in December 2019, the FDA granted fast track designation for arimoclomol as a treatment of sIBM , which further underlines the great potential of the Company's investigational drug.

The below figure provides an overview as of the Prospectus Date of the Company's pipeline and designations granted.

[2] Kirkegaard et al., *Nature*, 2010

6.2 History and development of the Company

The Company was founded in 2009 for the purpose of developing new therapies for patients suffering from protein misfolding diseases with no or limited treatment options available. Since the Company's incorporation, the Company has grown to a company that employs approximately 100 people with relevant experience and expertise in the research and clinical development of orphan drugs.

A brief historical overview of the key milestones in the Company's development is presented below:

2009	• The Company was founded to pursue the opportunity of developing new therapies based on the cell protective function of HSPs
2010	• The Company's current CSO and co-authors publish scientific foundation of the Company in Nature
	• The Company raised DKK 22 million in a seed financing round (share issue). Novo Holdings and Sunstone Capital became shareholders
2011	• The Company acquired arimoclomol and a portfolio of other molecules from the U.S.based biopharmaceutical company CytRx
	• The Company completed a series A financing round with cash proceeds of DKK 104 million. Aescap Venture became shareholder
2013	• The EMA granted orphan designation to HSP70 for the treatment of NPC
2014	• The EMA granted orphan designation to arimoclomol for the treatment of NPC
	• The Company was awarded the Wellcome Trust Pathfinder Award in collaboration with Oxford University for the project "Regulation of the Heat Shock Response as a Treatment for Niemann-Pick Type C disease"
	• The Company received the EY Entrepreneur of the Year award in the Life Science category
2015	• The Company raised DKK 150 million in a series B financing round (share issue). Kurma Partners and Idinvest Partners became shareholders

- The FDA granted orphan drug designation to arimoclomol for the treatment of NPC
- The Company initiated observational trial in NPC

2016

- University College London and University of Kansas invested DKK 1.3 million as part of the sIBM collaboration
- Preclinical and phase II clinical data with arimoclomol as a potential for the treatment for sIBM was published[3]
- The EMA granted orphan designation to arimoclomol for the treatment of sIBM
- The Company and University of Miami announced successful phase II trial of arimoclomol in SOD1-ALS patients.[4] Data presented at the 27th International Symposium on ALS/MND in Dublin
- The Company's current CSO and co-authors published preclinical data demonstrating the potential of HSP70 and arimoclomol as a treatment for multiple LSDs, including NPC and Gaucher disease[5], in Science Translational Medicine
- The FDA granted a fast track designation for the phase II/III clinical trial with arimoclomol for the treatment of NPC
- Dosing began in the Company's phase II/III clinical trial with arimoclomol for the treatment of NPC

2017

- The Company raised DKK 109 million as an extension to the series B financing round (share issue). LSP and the ALS Investment Fund (through ALS Invest 2 B.V.) became shareholders
- The Company completed enrollment into the clinical phase II/III trial with arimoclomol for the treatment of NPC
- The FDA granted orphan drug designation to arimoclomol for the treatment of sIBM
- Dosing began in the Company's phase II/III clinical trial with arimoclomol for the treatment of sIBM
- End of phase II meeting with the FDA regarding arimoclomol for ALS
- The Company assumed sponsorship of the phase II/III arimoclomol trial for sIBM from University of Kansas Medical Center and UCL
- The Company completed its initial public offering and listing on Nasdaq Copenhagen, raising gross proceeds of DKK 600 million

2018

- The Company was granted rare pediatric disease designation from the FDA for arimoclomol for the treatment of NPC, enabling eligibility for a Priority Review Voucher

---

[3] Ahmed M et al, *Science Translational Medicine*, 2016
[4] Company announcement "University of Miami and Orphazyme ApS Announce Successful Phase II Trial of Arimoclomol in ALS Patients", December 9, 2016
[5] Kirkegaard et al, *Nature*, 2017

	• The Company established its U.S. subsidiary with offices in Newton, Massachusetts
	• The Company enrolled the first patients in a phase II clinical trial for arimoclomol for the treatment of Gaucher disease and phase III clinical trial for arimoclomol the treatment of for ALS
	• The Company's current CSO and co-authors published preclinical proof-of-concept for arimoclomol in Gaucher disease[6]
2019	• The Company announced top-line data from its clinical phase II/III trial in NPC
	• The Company confirmed preparation of filings for arimoclomol in NPC with the FDA and the EMA
	• The Company completed enrollment of clinical phase II/III trial in sIBM
	• Kim Stratton joined the Company as Chief Executive Officer, succeeding Anders Hinsby
	• The Company completed enrollment of clinical phase III trial in ALS
	• The Company has completed enrollment of clinical Phase II in Gaucher.
	• The Company strengthened its balance sheet with EUR 9 million financing from Kreos Capital
	• Arimoclomol for the treatment of NPC received Breakthrough Therapy Designation from the FDA
	• Arimoclomol for the treatment of sIBM receives fast track designation in the United States
2020	• The Company reported positive data from its open-label Phase 2/3 extension in NPC
	• U.S. EAP for arimoclomol as a treatment for NPC
	• The Company incorporated a subsidiary in Switzerland
	• The Company raised DKK 745,000,000 through the Private Placement

6.3 The Company's key strengths

The Company believes that it has a number of key strengths, which will contribute to the successful implementation of its strategy outlined below.

The Company is close to making arimoclomol available to NPC patients and moving into the (pre-)commercial phase

NPC is a rare and severe disease for which there are currently only limited treatment options. The Company's positive data from its Phase II/III trial in NPC (further strengthened by the positive results from the open-label phase II/III extension) has shown that arimoclomol has a clinically meaningful effect on disease progression in NPC and has a favorable safety and tolerability profile. As a result of this

[6] Fog et al, Ebiomedicine 2018.

and prior data, the Company received several beneficial regulatory designations, including orphan drug designation, fast track designation and rare pediatric disease designation (with eligibility for a Priority Review Voucher). In addition, in November 2019, the Company received breakthrough therapy designation from the FDA. As a result of this breakthrough therapy designation as well as its fast track designation and rare pediatric disease designation, the Company will receive expedited review of a future marketing application for arimoclomol in the United States. The Company understands the urgency of making arimoclomol available to NPC patients and is therefore in discussion with the relevant regulatory authorities in the United States and the EU to enable the planned submissions of a New Drug Application ("NDA") in H1 2020 in the United States and a Marketing Authorisation Application ("MAA") with the EMA in H2 2020 with potential approval in H2 2020. In anticipation of these potential approvals, the Company is preparing for the commercial activities for its lead program in NPC by identifying and engaging with physician experts and centers of excellence hospitals to create awareness and interest through direct contact by field-based rare disease scientific liaisons (who are the U.S. field medical face of the Company and essential to communicating with external thought leaders, treatment team members, and payers) in the United States, medical education programs and strategic medical communications. The Company has established a Patient Advocacy Relations function in 2018 and has been working closely with the Niemann-Pick patient groups globally to create relationships, increase awareness of the Company's research and support NPC education for the patient community. In January 2020, the Company announced the availability of an EAP in the United States, which allows the Company to make arimoclomol available pre-commercially to U.S. patients before the drug is approved by the FDA. The Company has partnered with Clinigen Group to administer the arimoclomol EAP and support physicians interested in participation. The EAP is expected to remain open until arimoclomol becomes commercially available in the United States. The Company is currently evaluating how to offer early access in additional countries over time, contingent upon discussions with local authorities and the Company's progress towards filing for regulatory approval or obtaining reimbursement.

The Company has an emerging late-stage project pipeline with several ongoing registration studies and actively evaluating further supplemental indications

The positive results from the Phase II/III trial in NPC further strengthened by the positive results from the open-label phase II/III extension) and the expertise developed in connection therewith and other clinical trials further strengthens the Company's confidence in heat-shock protein amplification as a potential treatment for a range of other protein-misfolding diseases and diseases characterized by lysosomal dysfunction. Treatment with arimoclomol is already in development for three other indications, including one other LSDs (Gaucher disease) and two neuromuscular diseases (sIBM and ALS). Like NPC, these are rare and severe diseases with limited or no current treatment options. Results from the Phase II trial in Gaucher disease are expected in H1 2020, and the results from the Phase II/III trial in sIBM and the Phase III trial in ALS are expected in 2021. The Company benefits from orphan drug designation in sIBM and ALS, which grants market exclusivity for seven years in the United States and ten years in Europe. In addition, in December 2019, the Company received fast track designation for arimoclomol as a treatment of sIBM, which further underlines the great potential of the Company's investigational drug.

Furthermore, based on its published preclinical data, the Company believes there is broad potential for arimoclomol in other lysosomal, neurodegenerative disorders such as other sphingolipidoses and e.g. GCase deficient Parkinsons disease and are actively evaluating these opportunities.[7]

The Company has an experienced management team and board of directors with global and broad-based scientific, medical and commercial expertise

The Company's senior management team and international board of directors have valuable global and diversified expertise in the biotech and pharmaceutical industries and orphan disease space both from

[7] Kirkegaard et al., Science Transl. Med. 2016; Fog et al., Ebiomedicine 2018.

a scientific perspective and a commercial perspective. Members of the Company's management team have experience advancing pharmaceutical products from discovery stage through to market.

As the Company has grown from an entrepreneurial R&D company to a pre-commercial stage company, Kim Stratton was appointed as CEO of the Company on July 15, 2019. She has more than 25 years' global commercial experience from biopharmaceuticals and brings significant general management experience across multiple geographies, including the UK, the United States, Europe, and emerging markets to the team.

As scientific expertise is a cornerstone to the Company's business model, the Company's Board of Directors is comprised of industry experts with experience from companies focused on rare diseases, including Genzyme and Swedish Orphan Biovitrum. In addition, the Company's CSO is the founder of the company and leading expert within the discovery and translation of the heat shock protein responses and lysosomal function and authored the articles, which forms the basis of the Company's research.

The Company could receive market exclusivity for certain indications as a result of orphan drug designations in combination with strong intellectual property protection through active patenting strategy

Arimoclomol has been granted orphan drug designation by the FDA for NPC, sIBM and ALS, and orphan designation by the EMA for NPC, sIBM and ALS. Among other advantages, orphan drug designation provides that if a drug candidate with orphan drug designation is approved by the regulatory authorities upon completion of clinical trials, it may receive orphan drug status, which provides for market exclusivity for seven years in the United States and ten years in Europe, once approved. In addition, the Company strategically and actively pursues patent protection of its inventions. The Company holds the rights to 9 patent families, each with a number of issued patents (around 109 in total) and pending patent applications (more than 44 in total). The Company continues to actively pursue further patent protection and exclusivity opportunities. The Company focuses on protecting small molecule amplifiers of HSPs, including arimoclomol as well as NMEs. Protection is sought for a relevant scope to obtain commercial exclusivity in the main areas, and covering geographically at least the United States and Europe (via the European Patent Convention), as well as further geographic areas of interest. Patents directed to arimoclomol and its use in treating NPC, Gaucher disease and ALS have been issued to us in the United States and a range of European countries.

The Company has a scientific development platform identifying new NMEs and future product candidates

As a result of its experience with arimoclomol as a treatment of NPC, Gaucher disease, sIBM and ALS and building on the scientific expertise of its management team, the Company has created a scientific development platform, based on new research in the field of HSPs, with the aim of identifying additional diseases and further characterizing pathologies that may be targeted with HSP-based treatments. Through the Company's platform, the Company has developed assays and expertise to assess new leads and has identified a number of new molecular entities ("**NMEs**"), which may generate future clinical candidates beyond arimoclomol. To support these efforts, the Company has a research team with relevant experience and expertise in the research and clinical development of orphan drugs.

6.4 The Company's strategy

The Company's goal is to pioneer the HSPs for the treatment of neurodegenerative orphan diseases. Important elements of the Company's strategy to achieve this goal are the following:

The Company is focused on rapidly advancing clinical development of arimoclomol for the treatment of NPC, Gaucher disease, sIBM and ALS by continuously collaborating with the regulatory authorities

The Company's objective is to successfully conduct and complete the planned and ongoing trials of arimoclomol for the treatment of the lysosomal storage diseases, NPC and Gaucher disease and the neuromuscular diseases, sIBM and ALS. The Company develops new therapies for orphan diseases

where few products, if any, have made it through to regulatory approval and the Company maintains frequent interactions with the regulatory bodies in the United States and Europe to advance its program toward potential approval in the most expedient manner.

For example, in the process of getting the necessary approvals for arimoclomol as a treatment for NPC, the Company has had frequent collaborative interactions with the FDA and the EMA, which provided the Company with thorough guidance on data presentation in the NDA and MAA, respectively. The valuable advice and encouraging feedback received from health authorities in both the United States and Europe increases the Company's optimism that it may soon be able to provide a treatment option for NPC.

The Company aims to maximize the commercial value of the Company's product candidates by designing a commercialization strategy attune to the specific market being targeted

As the clinical development program for arimoclomol progresses, in particular with respect to NPC, the Company intends to finalize its commercialization strategy and further build its commercial structure and operations. As there are a limited number of specialists and highly-specialized centers, the Company believes it is most efficient to build its own commercial organization that can collaborate with leading disease experts and patient organizations to develop arimoclomol. Therefore, in 2018, the Company established a commercial organization in Boston to further develop partnerships and build relationships with the key stakeholders of researchers, healthcare providers, and patient organizations within the largest healthcare market in the world.

If one or more of the Company's product candidates is approved by the FDA and/or the EMA, the Company intends to pursue the commercialization of such product candidates by (i) building a small in-house sales force in Europe and the United States; (ii) hiring medical and market access field teams specialized in rare diseases to work towards market acceptance by physicians and patients and obtaining reimbursement coverage from payors; (iii) promoting its product candidates by communicating and discussing unmet medical need and arimoclomol data with experts in the field, speaking at conferences while entering into partnership agreements with specialist pediatric neurologists, specialist metabolic centers and patient organizations and (iv) building the appropriate commercial and medical core operational capabilities while outsourcing non-core activities. The Company believes sales, medical and market access field teams can be supported by core rare disease commercial and medical operational functions based in the United States and Denmark, and a small commercial satellite office elsewhere in Europe.

Although the Company is currently focused on potentially entering the United States and European markets, the Company may also subsequently choose to pursue the approval and commercialization of its product candidates in collaboration with strategic partners, particularly in China, Japan, Taiwan, S. Korea and other geographies which are more effectively managed by companies with local expertise.

In case of successful outcomes within all four indications, the Company sees notable synergy potential and will consider expanding and organizing two separate sales, market access and medical field teams based on the designated therapeutic areas of LSDs and neuromuscular diseases, supported by a central commercial and medical operations function in each country under global leadership and strategy.

The Company is working on expanding the potential opportunities for arimoclomol beyond initial indications and developing NMEs for other protein misfolding diseases based on expertise and current technology platform

The Company believes there is broad potential for arimoclomol in other lysosomal, protein misfolding and aggregation disorders and the Company is actively evaluating opportunities in disorders such as GCase deficient Parkinson's disease and other LSD's.

Protein misfolding is the hallmark of a broad range of neurodegenerative diseases. The Company's strategy is to use its expertise, including proprietary know-how to select and develop new leads for suitable diseases. Orphazyme intends to select diseases suitable for the NMEs based on genetic and

mechanistic insights into selected protein misfolding diseases. In line with this, the Company is developing a proprietary suite of NMEs with improved, disease-tailored characteristics.

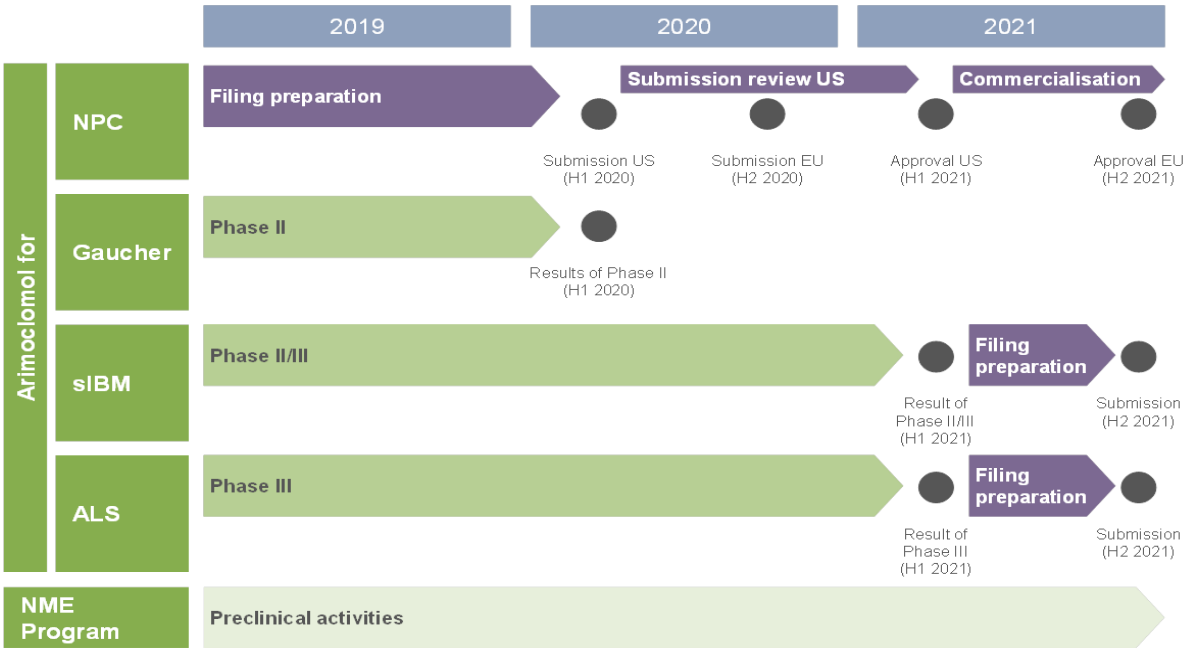
The Company has entered into, and will continue to enter into, strategic collaborations in orphan diseases

The Company strives to further develop its expertise within its therapeutic areas of interest through close collaborations with academic experts and patient organizations. Through these partnerships, the Company supports the advancement of molecular and clinical understandings and performs preclinical evaluations in biological models of relevant diseases. The Company's academic partners include academic professors and clinicians from institutions such as, for instance, the University of Oxford, University College London and the University of Kansas. *See* Material Contracts. Through these collaborations, the Company has conducted a number of preclinical studies that have provided insights into the potential of HSP amplifying therapeutic strategies for LSDs and neuromuscular diseases. Further, partnerships with the patient community has ensured that patient-relevant outcomes have been assessed in scientific models and subsequently published in peer-reviewed scientific journals.

6.5 The Company's clinical pipeline

The Company is developing a broad pipeline of drugs targeted at amplifying the body's own production of cytoprotective HSPs, spearheaded by arimoclomol, which the Company is investigating as a treatment for four indications: the neurodegenerative and -muscular diseases sIBM and ALS, and in the LSDs, NPC and Gaucher disease.

The figure below provides and overview of the expected news flow until 2021 and includes tentative dates on which the Company could learn whether or not its product candidate for the treatment of NPC has been approved.



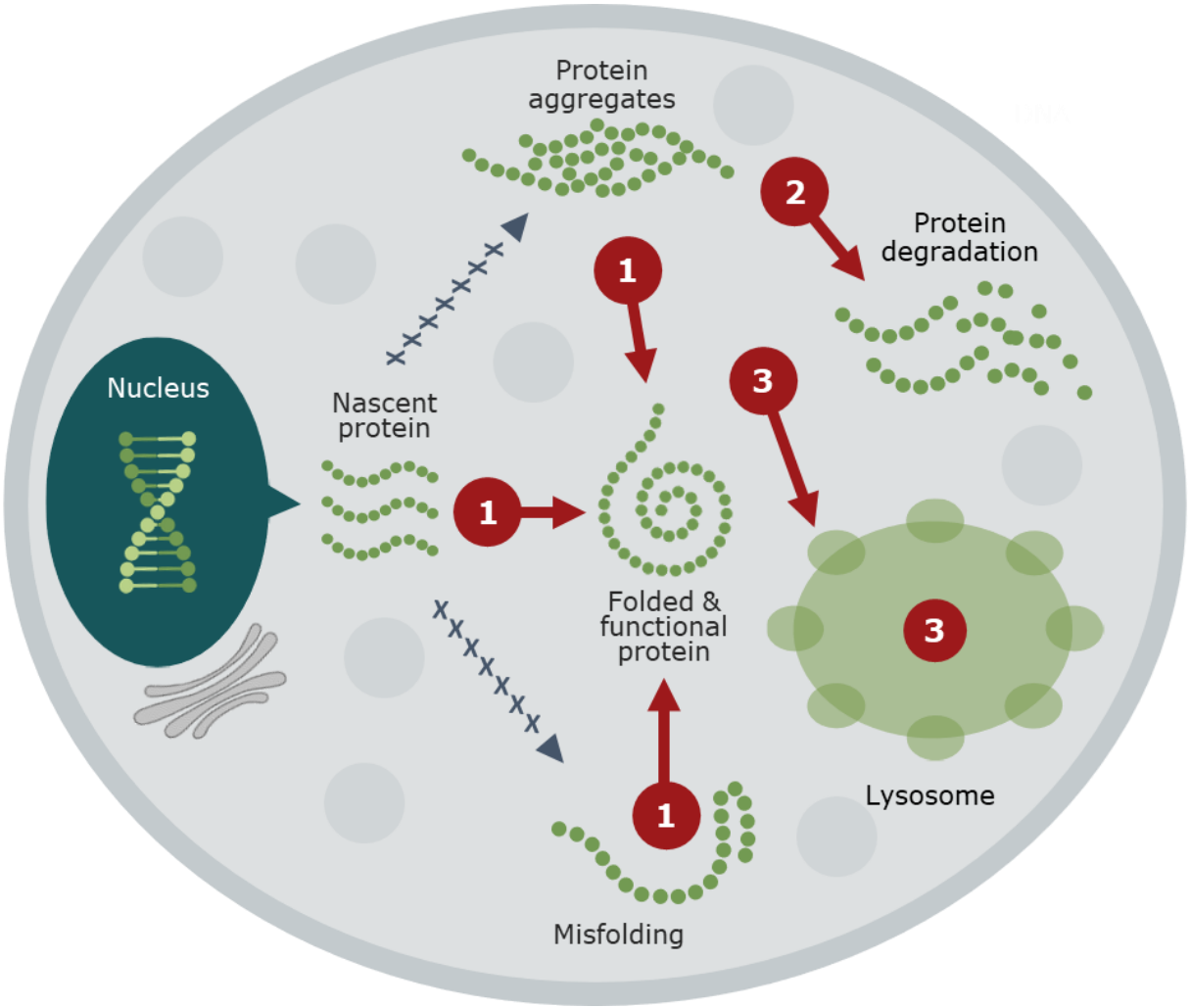
Note: Timeline subject to success of trials and receipt of approval from the FDA and EMA.

6.5.1 *Arimoclomol mechanism of action*

Arimoclomol works by increasing the production of HSPs inside the cells and thereby enhancing the natural biological mechanisms that reduce protein misfolding and aggregation and improve lysosomal function (the cells recycling system), uniquely targeting cells that are under stress.

Proteins are large biomolecules that have a vast array of functions in cells. To do their job, proteins must attain the correct shape. In many diseases, protein misfolding causes toxicity, either as a consequence of protein aggregation or the loss of protein function. Protein aggregation, a hallmark of neuromuscular diseases such as sIBM and ALS, leads to toxicity as aggregation interrupts the cell functions. Loss of protein function, a classical feature of LSDs such as NPC and Gaucher disease, leads to an accumulation of toxic substances as a result of the absence of functional proteins that would otherwise have helped eliminate such waste products. HSPs constitute an evolutionary highly conserved natural defense system, which makes other proteins work correctly and guards against the toxicity arising from misfolded proteins and dysfunctional lysosomes. In particular, HSPs are endogenous molecular chaperones that promote the survival of stressed cells by re-folding misfolded proteins into their correct conformation, or by directing 'terminally' misfolded proteins to be broken down. They also protect cells by augmenting lysosomal function hereby inhibiting lysosomal membrane permeabilisation and preventing cell death, allowing cells to clear away waste and return to their healthy status.

The below figure shows in (1) how HSPs ensure correct folding and function of proteins, (2) how HSPs can degrade protein aggregates by facilitating removal through degradation and (3) that HSPs are biological chaperones promoting lysosomal function by increasing lipid and protein metabolism and removal, thereby stabilizing lysosomal membranes and preventing cell death.



There are several different types of HSPs which work in conjunction. A prominent regulator HSP is HSP70, which the Company use as a key parameter to measure activity of its product candidates. HSP70 has been shown to protect against the formation of protein aggregates which are the defining characteristic of a number of neurodegenerative diseases including ALS and sIBM. In addition, HSP70 is important for lysosomal function and has been identified as a co-factor for lysosomal sphingolipid breakdown, a necessary step in the metabolism of stored lipids which cause toxicity if accumulated in

the lysosome. In both NPC and Gaucher disease, as well as other LSDs, mutations lead to misfolding and loss of enzyme functions involved in the breakdown and recycling of these sphingolipids, leading to a toxic build-up of these and other molecules within the lysosomes. By amplifying the production of HSPs, this pathological cascade can be addressed by rescuing the function of the recycling enzymes and improving the function of lysosomes.

The production of HSPs is regulated by a transcription factor, heat shock factor 1, or HSF1. A transcription factor is a protein that regulates production of other proteins in the cell. In the case of HSF1, the proteins being regulated are HSPs. Activation of HSF1 starts the production of the major stress-inducible HSP70-chaperone along with other HSP-chaperones, which help reshape the cells' misfolded proteins and take care of the recycling systems. Under normal cellular conditions, HSF1 is inactive. However, the transcription factor can be activated by an initial cellular stress, such as protein misfolding, and becomes fully activated under a sustained stress signal.

Arimoclomol amplifies and prolongs the activated, HSP-producing state of HSF1. This leads to an amplification in the production of cell protective HSPs, but only in physiologically stressed cells.

6.5.2 *Clinical profile of arimoclomol*

The Company believes that the clinical experience with arimoclomol strongly supports its continued development and commercialization. Highlights from clinical trials performed to date are summarized below.

Safety. No major safety concerns have been identified during clinical trials with arimoclomol to date and arimoclomol has been well-tolerated in eight phase I trials and five phase II/III trials.

A comprehensive nonclinical program covering pharmacology, pharmacokinetics, drug metabolism, safety pharmacology, single- and repeat-dose toxicity, genotoxicity, reproductive and developmental toxicity, juvenile toxicity, local tolerance and interaction studies has been conducted. Carcinogenicity studies are ongoing.

In summary, the nonclinical safety studies revealed adverse findings after oral administration of arimoclomol which included mortality and clinical signs (primarily neuro-behavioural) at high doses, slight effects on the CNS (increased activity), kidney toxicity (of no human relevance or of uncertain toxicological significance), changes in the liver (mainly adaptive changes; sporadic adverse changes at high doses), gastrointestinal tract effects (short term, but no long term effects), increased density of the lens of the eye (at high doses in a single study), and reduced male and female fertility, embryo-foetal survival and offspring body weight.

The existing safety pharmacology and toxicology data demonstrate an acceptable safety profile for arimoclomol with adequate safety margins to the human exposure and support oral administration of arimoclomol at the anticipated daily human doses of up to 400 mg t.i.d.

As of August 1, 2019, a total of 112 healthy subjects, 82 patients with ALS, and 35 patients with NPC (34 patients on randomized treatment and 1 patient who only received a single-dose for PK evaluation) have been exposed to arimoclomol in completed clinical trials (or a completed trial phase). In addition, 19 patients with SOD1 ALS and 16 patients with IBM have been exposed to arimoclomol in investigator-initiated clinical trials. In the ongoing trials/trial phases, 474 patients with NPC, GD, ALS, and IBM have been exposed to blinded investigational medicinal product ("**IMP**") or open label arimoclomol as of August 1, 2019.

Based on data from the clinical pharmacology trials in healthy subjects and from clinical trials in patients with ALS (AALS-001) and NPC (CT-ORZY-NPC-002), arimoclomol may lead to an increase in serum creatinine and/or a decrease in mean creatinine clearance without effects on glomerular function or renal haemodynamics. This suggests an inhibitory effect of arimoclomol on tubular secretion of creatinine and is supported by the finding that arimoclomol is an inhibitor of OCT-2 transporter responsible for creatinine secretion in the kidneys.

In the ongoing (blinded) placebo-controlled clinical trials in ALS and sIBM, increased transaminases (>3x upper limit of normal) have been observed in a minority of patients. For the majority of these affected patients, the elevations were asymptomatic although for some patients a concomitant rash was observed. The elevations have most commonly been observed within the first three months of treatment and values have normalized either after withdrawal or during continued treatment. The transaminase elevations were not accompanied by elevations of alkaline phosphatase.

In the ongoing trial in sIBM, IBM4809, one patient in the arimoclomol group (1200mg/day) experienced severe tubulointerstitial nephritis with acute tubular injury and tubular necrosis. Due to the strong temporal relationship with IMP initiation without other more likely explanations for the event, a possible causal relation of the event to IMP cannot be excluded.

The final evaluation of clinical significance of the potential risks and the impact on the safety profile of arimoclomol will await completion of the Company's current ongoing clinical trials.

Pharmacokinetics. Arimoclomol is orally administered and has a bioavailability of 80% to 90%. High bioavailability reduces the amount of drug administered while achieving the desired pharmacological effect.

In addition, arimoclomol readily crosses the blood-brain barrier. The blood-brain barrier is a selectively permeable membrane which prevents large compounds from entering the brain. It has been demonstrated in mice that arimoclomol reaches the brain tissue. Furthermore, arimoclomol has been shown in ALS patients to reach the cerebrospinal fluid ("**CSF**") in a dose-dependent manner. CSF is a liquid surrounding the brain and spinal column. Good brain penetrance is key to treatment of many central neurological diseases.

Efficacy. Arimoclomol has achieved a clinical proof-of-concept in ALS[8] and sIBM[9], with Phase II trials in both indications having shown consistent trends in pre-defined efficacy analyses. See *"– Overview of neuromuscular disorders–Clinical trials of arimoclomol for sIBM"* and *"– Overview of neuromuscular disorders–Clinical trials of arimoclomol for the treatment of ALS"*

6.5.3 *Overview of lysosomal storage diseases*[10]

LSDs are inherited metabolic disorders where enzyme deficiencies result in an accumulation of toxic materials in the lysosomes, our cells' recycling centers. This leads to lysosomal dysfunction and cell death and consequently organ dysfunction. The enzyme deficiencies are often caused by mutations leading to misfolding and degradation of the enzymes. Lysosomes are membrane-bound compartments located in the body's cells, used to break down fats, proteins and other large molecules into the respective building blocks. Loss of lysosomal enzyme activity due to the enzymes misfolding and dysfunction

[8] Clinical proof-of-concept in ALS achieved by demonstrating benefit across efficacy endpoints and assessments as compiled evidence, also in absence of statistical significance.

[9] Clinical proof-of-concept in sIBM achieved by demonstrating benefit across efficacy endpoints and assessments as compiled evidence, also in absence of statistical significance.

[10] The information in this section is principally derived from management information based on research and market reports

prohibits the lysosomes from performing their normal function and results in accumulation of metabolites in the lysosomes, why these diseases are referred to as lysosomal storage diseases.

LSDs are comprised of more than 50 different diseases, that may affect different parts of the body, including the brain, central nervous system, spleen, liver, skeleton, skin and heart, and new disorders continue to be discovered. Although clinical trials are in progress, only a few approved treatments are currently available for a few LSDs, such as Gaucher Type I, Pompe disease, Fabry disease, MPS I, MPS II, MPS IV, MPS VI, LAL-D, etc.

The two LSDs that the Company is currently targeting are NPC and neuropathic Gaucher disease.

I Arimoclomol for the treatment of Niemann-Pick disease

a. Overview of NPC

NPC is a rare, genetic and relentlessly progressive disease that impairs the ability of the body to recycle cholesterol and other types of lipids, inside the cells. NPC is fatal. Although symptom onset and disease progression are highly variable, most patients do not expect to live beyond their late teens.[11]

As many cases of NPC go misdiagnosed or undiagnosed. According to Aptis Partners[12], an estimated 40-70% of all patients are diagnosed for NPC depending on the country. It is therefore difficult to determine true frequency of the disease. According to the National Niemann-Pick Disease Foundation, or NNPDF, NPC has an estimated 500 new cases diagnosed annually worldwide. NPC often appears in childhood but can appear at any age. The incidence of the disease is estimated to be 1 in 100,000 livebirths. Based on the incidence rates described above, the prevalent number of potential NPC patients in the United States and in the EU is conservatively estimated to be approximately 2,000 individuals in total.[13]

b. Treatment options for NPC and unmet need

The majority of current treatment options aim at alleviating pain and are only directed towards the specific symptoms apparent in each individual.

Only one drug, Zavesca (miglustat) marketed by Actelion Pharmaceuticals and also now available as a generic product in several countries, is currently approved for the treatment of NPC. It is approved only in Europe, Canada, Australia, New Zealand, and several countries in Asia and South America as Zavesca and in Japan as Brazaves.[14] In Europe, miglustat is indicated for the treatment of progressive neurological manifestations in adult patients and pediatric patients with NPC disease. A range of safety and tolerability problems are known to be associated with miglustat, including weight loss, decreased appetite, diarrhea, nausea and thrombocytopenia. Miglustat has not been approved by the FDA for treatment of NPC, but it is approved for the treatment of Gaucher type I disease in the United States.

Studies are currently being performed to test the safety and efficacy of other treatment options. In addition to the Company's phase II/III trial with arimoclomol, four treatment options have been identified by the Company as being in development:

1. Adrabetadex (VTS-270), which has been evaluated in a phase IIb/III (by Mallinckrodt Pharmaceuticals). Mallinckrodt Pharmaceuticals has communicated that the Phase II/III top-line results did not meet statistical significance (p=0.55). This agent has orphan drug designation by both the FDA and EMA.

[11] Vanier MT., Orphanet Journal of Rare Diseases, 2010.
12 Aptis Partners Market Assessment and Target Product Profile Analysis for Orphazyme, 2019.
[13] Medical Marketing Economics for Orphazyme, 2016.
[14] Medical Marketing Economics for Orphazyme, 2016.

2. Trappsol in phase I/IIa by Cyclo Therapeutics (former CTD Holding). This agent has orphan drug designation by both the FDA and EMA.

3. IB1001 in phase II by IntraBio. This agent has orphan drug designation by both the FDA and EMA.

4. ESB1609 in Phase I by E-scape Bio. It is unknown if this agent has any orphan drug designation

Due to the limited availability and efficacy and side effects of the existing treatment option, the Company believes that a significant unmet need for treatment of NPC continues to exist.

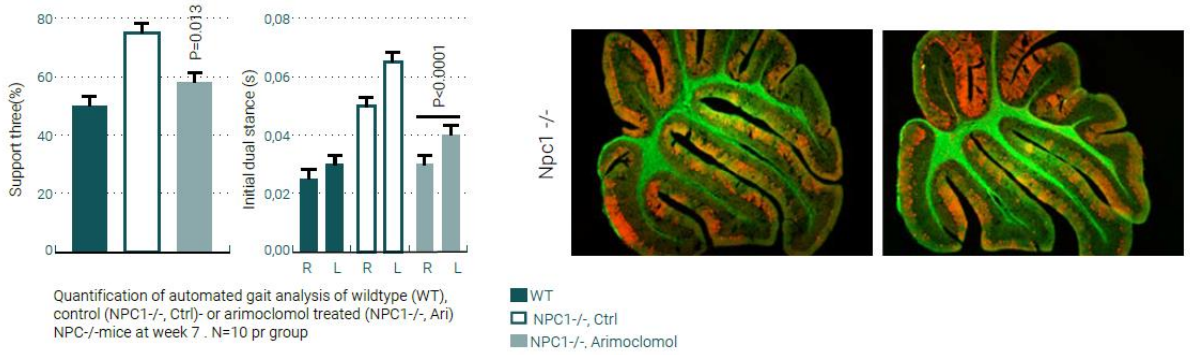
c. Preclinical studies of arimoclomol in NPC

The Company published preclinical studies in 2016, which indicated that arimoclomol may correct the underlying pathology of NPC.

Arimoclomol has been extensively tested in preclinical models of NPC. Recent scientific publications underscore that the induction of HSPs is an attractive therapeutic target in LSDs.[15] This is particularly the case for NPC, where HSP70 has been demonstrated to be critical for the proper folding and activity of the NPC1 protein.[16] In addition, it has been demonstrated that the majority of NPC patients have mutations that are responsive to therapies aimed at rescuing and improving the folding of the NPC1 protein.[17]

The preclinical studies published in 2016 demonstrated a clear potential therapeutic benefit of arimoclomol in NPC.[18] These studies demonstrated that arimoclomol increased the activation of HSF1 and production of HSP70 in the brains of the NPC mouse model. The studies further demonstrated that HSP70 reduces CNS white matter (myelin) loss and cerebellar atrophy in the NPC mouse model.

Importantly, treatment with arimoclomol improved all measurable manifestations of loss of motor coordination/ataxia in the animal model and extended life. This is important as loss of coordination (ambulation) is identified to be the most important factor for quality of life in patients. [19]



Rescue of brain white matter and quantification of improved coordination. The figure on the right illustrates a brain cross section of NPC mice without (left) and following (right) treatment to increase brain levels of HSP70. The increased green area demonstrates the rescue of white matter. The figure on the left illustrates the loss of coordination quantitatively measured by gait analyses. Treatment with arimoclomol demonstrated a response on all (13 out of 13) quantitative ataxic manifestations of the disease, two of the most important which are depicted here.

---

[15] Ingemann & Kirkegaard, J Lipid Research, 2014
[16] Nakasone et al., J Biol Chem, 2014
[17] Macias-Vidal et al., FEBS J, 2014
[18] Kirkegaard, Gray et al., Science Translational Medicine, 2016
[19] Cortina-Borja et al, Orphanet Journal of Rare Diseases 2018, vol 13(1), p. 143, PMID: 30115089.

d. Clinical trials of arimoclomol for the treatment of NPC

The Company initiated a phase II/III, randomized placebo-controlled trial in July 2016, after receiving regulatory advice from the EMA and the FDA. Interaction with the European Pediatric Committee took place in 2015 and resulted in an agreed pediatric investigation plan. A scientific advice meeting was held with the EMA on refined clinical endpoints. The FDA granted the Company a fast track designation for the investigation of arimoclomol intended for the treatment of NPC in June 2016. The aim of the phase II/III trial is to investigate the safety and efficacy of arimoclomol. Prior to the interventional trial, some of the patients were enrolled in a prospective observational trial allowing for the assessment of natural progression of disease. Information obtained from this observational trial offered the opportunity to adjust the phase II/III statistical analysis plan. The multi-center trial completed enrollment in May 2017, with patients recruited at sites across Europe and the United States, and was completed in the second half of 2018. The Company received rare pediatric disease designation in January 2018. In January 2019, the Company announced clinical trial results of its phase II/III trial of arimoclomol for the treatment of NPC and, in November 2019, the Company received break through therapy designation for arimoclomol in NPC from the FDA.

Following positive results of the Companys Phase II/III trial in early 2019, the Company announced further positive arimoclomol data from its open-label phase II/III extension in NPC in January 2020. This 12-month interim data from the open-label extension trial shows sustained effect in reducing disease progression over two years and further evidence of the efficacy and safety profile of arimoclomol. Collectively, the Company believes this new data strengthens the regulatory marketing applications for arimoclomol in the United States and Europe.

e. Clinical development plan

While the phase II/III trial was a small trial including 50 patients and the open-label extension trial included 41 patients , the Company believes that, based on its discussions with the FDA and the EMA, it will be sufficient to form the basis for NDA and MAA submission and, possibly, an approval from the FDA or EMA. The Company expects to submit an NDA to the FDA in H1 2020 and an MAA with the EMA in H2 2020.

e. Commercialization plans

In anticipation of potentially receiving approval for the use of arimoclomol as a treatment for NPC, the Company has been establishing (and maintaining) good relationships with the few NPC specialists and specialized NPC medical centers in the world to create awareness and acceptance of its product candidate. The Company has been actively collaborating with leading disease experts and patient organizations to develop arimoclomol. Therefore, in 2018, the Company established a commercial organization in Boston to further develop partnerships and build relationships with the key stakeholders of researchers, healthcare providers, and patient organizations within the largest healthcare market in the world. In addition, in January 2020, the Company announced the availability of the EAP in the United States. The EAP provides a pathway for patients with serious, life-threatening diseases or conditions who lack therapeutic alternatives to gain access to investigational drugs before they are approved. As a result, the Company can make arimoclomol pre-commercially available to U.S. NPC patients prior to the drug being approved by the FDA as a treatment for NPC and has partnered with Clinigen Group to administer the arimoclomol EAP and support physicians interested in participating. The EAP is expected to remain open until arimoclomol becomes commercially available in the United States. The Company is currently evaluating how to offer early access in other countries over time, contingent on discussions with local authorities and its progress towards filing for regulatory approval or obtaining reimbursement. If NPC is approved by the FDA and/or the EMA, the Company intends to pursue the commercialization of such product by (i) building a small in-house sales force and partnering with established local/regional distributors with a focus on rare diseases in other markets such as Middle East, Latin America, Asia-Pacific; (ii) hiring medical and market access field teams specialized in rare diseases to work towards market acceptance by physicians and patients and obtaining reimbursement coverage from payors; (iii) promoting its drug by communicating and discussing unmet medical need and arimoclomol data with experts in the field, speaking at conferences while entering into partnership agreements with specialist pediatric neurologists, specialist metabolic centers and patient organizations

and (iv) building the appropriate commercial and medical core operational capabilities while outsourcing non-core activities. The Company believes sales, medical and market access field teams can be supported by core rare disease commercial and medical operational functions based in the United States and Denmark, and a small commercial satellite office elsewhere in Europe.

II Arimoclomol for the treatment of Gaucher disease

a. Overview of Gaucher disease

Gaucher disease is a rare, inherited metabolic disorder causing certain sugar (glucose) containing fat (lipids and especially glycolipids) to abnormally accumulate in the lysosomes of cells, especially within cells of the blood system and nerve cells, hereby affecting organs such as the brain, bone marrow, spleen and liver, due to the lack of a certain enzyme (glucocerebrosidase). Like NPC, Gaucher disease is an autosomal recessive disorder where the lack of glucocerebrosidase activity is caused by mutations in the gene (named GBA) encoding the enzyme.

Three distinct forms of Gaucher disease have been identified to date based on the absence or presence of neurological complications and the extent of such.

- Gaucher type 1 is at the outset characterized by a lack of neurological complications and usually result in a low level of blood clotting cells (thrombocytopenia) and a low level of red blood cells in circulation (anemia) causing easy bruising and chronic fatigue, respectively. In addition, affected individuals may experience an abnormally enlarged liver and/or spleen (hepatosplenomegaly) and skeletal anomalies. A number of patients diagnosed with Gaucher type I develop neurological symptoms (up to 50%, including 5-7% with parkinsonism) later in life.

- Gaucher type 2 (so-called acute neuronopathic Gaucher disease) is characterized by onset in the early months of life and entails neurological complications arising from the accumulation of a certain lipid (glucocerebroside) in the brain. The symptoms include enlargement of the spleen (splenomegaly), loss of motor skills, involuntary muscle spasms (spasticity), decreased muscle tone (hypotonia) and dysphagia. Gaucher type 2 usually results in fatality during the first to third year of life as a result of respiratory distress of suffocation from food entering the respiratory passage.

- Patients with Gaucher type 3 (so-called chronic neuronopathic Gaucher disease) may experience the same blood and bone anomalies as Gaucher type 2, however, with the neurological complications usually progressing at a slower rate. Gaucher type 3 usually onsets during the first decade of life and patients may live into their teens, early 20s and some even longer.

As the presence of neurologic symptoms are not confined to a single type of Gaucher disease, and with no approved treatments for the neurological manifestations of the disease, the Company's focus is on all Gaucher disease patients with neurologic symptoms.

Gaucher disease is the most common LSD affecting a conservatively estimated patient population of up to 15,000 in Europe and the United States combined. The prevalence of all Gaucher disease types is about 1 in 100,000 individuals or higher in the general population[20] with an exceptionally high prevalence in the Ashkenazi Jewish population, up to 1 per 800 individuals.[21] Neuronopathic -or neurologic- Gaucher disease, which is the Company's focus, is estimated to account for at least 10% to 30%[22] of all patients with Gaucher disease in the Western world. There are regions with high prevalence; neuronopathic Gaucher disease may affect a third of the Gaucher patients in India.[23] The Company also focuses on type 1 patients with neurological symptoms as the treatment needs of these groups are not met by current treatment options. A number of reference points exist for the market for treatment

[20] Orphanet https://www.orpha.net/consor/cgi-bin/OC_Exp.php?Expert=355&lng=EN.
[21] Stirnemann et al. Int J Mol Sci. 2017 Feb 17;18(2). pii: E441; Nalysnyk et al. Hematology. 2017 Mar;22(2):65-73.
[22] Stirnemann et al. Int J Mol Sci. 2017 Feb 17;18(2). pii: E441; Nalysnyk et al. Hematology. 2017 Mar;22(2):65-73.
[23] A. Nagral J Clin Exp Hepatol. 2014 Mar;4(1):37-50.

options for Gaucher type 1. According to Global Data, in 2018, global sales of products for treatment of Gaucher disease was €1.23 billion.

b. Treatment options for Gaucher disease and unmet need

Two types of treatment are currently available for patients with Gaucher disease, (i) enzyme replacement therapy, or ERT, such as Cerezyme (Sanofi), Elelyso (Pfizer) and Vpriv (Shire), and (ii) substrate reduction therapy using Zavesca or Cerdelga (Sanofi) that reduce the production of substrates. These treatments were approved based on their ability to improve the non-systemic peripheral features of Gaucher disease,[24] but not the neurological manifestations of the disease, and none of them are approved for treatment of Gaucher disease type 2 and 3.

In addition to ERT and substrate reduction therapies, and the Company's double-blinded, randomized placebo-controlled Phase II trial of Arimoclomol, there are few other advanced programs.

- Genzyme, a Sanofi subsidiary, is currently evaluating the combined use of two agents in an ongoing open label phase II trial with a target enrolment of 10 patients. The patients receive combination treatment with GZ/SAR 402671 (venglustat, Sanofi), a substrate reduction therapy in combination with the marketed ERT Cerezyme (imiglucerase, Genzyme). The clinical efficacy in this study is unknown to the Company.

- Tottori University Hospital and Shire (a Takeda subsidiary) have registered an open-label, Phase II/III study in the Japan clinical trial registry, which evaluates the efficacy and safety of ambroxol hydrochloride (an over-the-counter antitussive first registered in 1978) in patients with neuronopathic Gaucher's disease. The clinical efficacy in this study is unknown to the Company.

- Finally, AVROBIO Inc is sponsoring an ongoing open-label, non-randomised Phase I/II trial Lentiviral Vector Gene Therapy AVR-RD-02 for subjects with type 1 Gaucher Disease. A total of 16 subjects are planned. The clinical efficacy in this study is unknown to the Company.

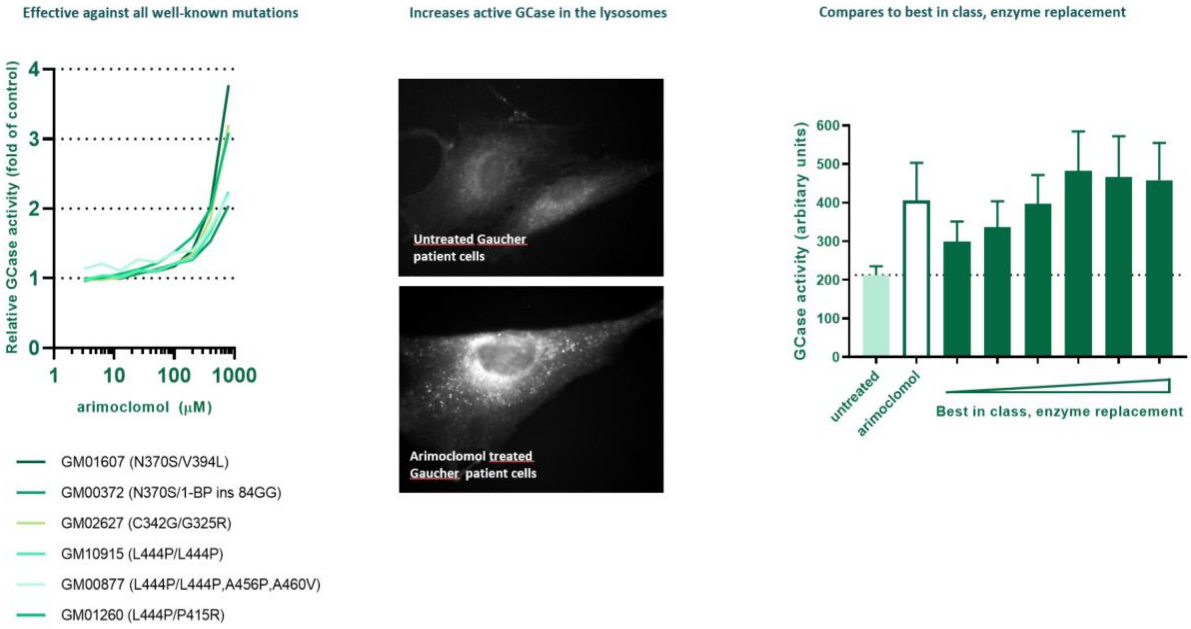
As currently available therapies do not treat the pathology of the central nervous system, a significant unmet need for treatment of Gaucher disease continues to exist, especially for Gaucher type 2 and type 3 and Gaucher type 1 patients developing neurological symptoms at a later stage.

c. Preclinical studies of arimoclomol for the treatment of Gaucher disease

In preclinical studies arimoclomol has been demonstrated to amplify HSP70 production leading to an increase in refolding, maturation and correct localization of glucocerebrosidase ("**GBA**"), meaning that the enzyme was correctly built and located in the part of the cell where it is needed. The increase in GBA activity was confirmed in a complimentary neurological Gaucher disease model system.

The potential efficacy of arimoclomol for the treatment of Gaucher disease has been extensively tested in preclinical in vitro assays. In accordance with its mechanism of action, arimoclomol induced HSP70 and facilitated the proper folding, maturation and lysosomal localization of GBA leading to a marked effect on GBA activity across a number of different mutations, including the most abundant L444P and N370S mutations, as illustrated in the figures below. The results in primary patient fibroblasts were corroborated in a neurological model system, employing neuron-like cells (so-called neuronally differentiated multipotent adult stem cells, or MASCs) from neuronopathic Gaucher disease patients. Consistent with the studies on primary fibroblasts, neuronal differentiated MASCs treated with arimoclomol had increased GBA activity across genotypes.

[24] Bennett et al, Ann Pharmacother, 2013



Highlight of preclinical data on arimoclomol for the treatment of Gaucher disease. The left chart illustrates that arimoclomol increases the activity of GBA across several different mutations in cells from Gaucher disease patients. The microscopy pictures in the middle illustrate arimoclomol's ability to increase the GBA enzyme activity and its correct cellular localization. Increase in active GBA and correct localization (brighter fluorescence, cellular distribution) were visible following treatment with arimoclomol. The chart to the right illustrates that treatment with arimoclomol increases the GBA enzyme activity in Gaucher disease patient cells to levels comparable to treatment with the current, best in class ERT therapy.[25]

d. Clinical trials of arimoclomol for the treatment of Gaucher disease

The Company initiated a randomized, double-blinded, dose-ranging phase II trial in June 2018 and fully enrolled by August 2019. The trial includes approximately 40 patients, who are naive to any treatment for Gaucher disease. The trial is taking place at clinical sites in India where access to ERT is limited. This allows for direct assessment of the effect of arimoclomol treatment on pharmacodynamic biomarkers in blood and other tissues, as ERT treatment may obscure the effect of arimoclomol on peripheral symptoms and biomarkers. Peripheral markers of effect are validated biomarkers for treatment effectiveness in Gaucher disease. In addition, performing the trial in India provides patients (aged

[25] Fog et al, ebiomedicine 2018

between 4 and 60 years old) suffering from this rare disease with the opportunity to receive a potential treatment.

In November 2018, the Company announced preclinical proof-of-concept for arimoclomol in Gaucher disease.

e. Clinical development plan

The Company initiated a phase II trial in arimoclomol for Gaucher disease in June 2018 with top-line Phase II results expected in H1 2020. The Company expect to start a phase III registration study in 2021, if the phase II trial demonstrates clinical proof-of-concept (based on trend effect across independent efficacy endpoints).

6.5.4 *Overview of neuromuscular disorders*[26]

Neuromuscular disorders encompass a range of conditions affecting a part of the neuromuscular system and impairing the functioning of the muscles, thereby causing muscle weakness and fatigue. In particular, the disorders affect the nerve cells (so-called neurons) that send messages to control the voluntary muscles, *i.e.* muscles one can control, such as arms and legs) and the muscles themselves. As the disorders manifest, the neurons become unhealthy or die, resulting in a loss of communication between the nervous system and the muscles. Weakening of the muscles may lead to aches, pains, movement problems and may affect the heart function and ability to breathe. The causes of neuromuscular disorders vary by type, but the diseases targeted by us are characterized by protein misfolding and aggregation, prohibiting proper recycling. Protein aggregation can cause cell stress and eventually cell death.

The two neuromuscular disorders that the Company is currently targeting are sIBM and ALS.

I Arimoclomol for the treatment of sporadic Inclusion Body Myositis

a. Overview of sIBM

sIBM is an acquired, rare and a relentlessly progressing muscle disorder, the onset of symptoms of muscle weakness typically occurs between 45 and 70 years of age, and progression is insidious. Among individuals older than 50 years it is the most common muscle wasting disorder.

The cause of sIBM is unknown. It is possible that multiple immunological, genetic and environmental factors and age-related factors (degenerative factors) are all involved in the development of the disorder. The significant degenerative component suggests that sIBM is primarily a degenerative muscle disorder, making protein misfolding and aggregation a prominent target for therapy.

The exact prevalence of sIBM is not fully elucidated, but the size of the patient population in Europe and the United States has been conservatively estimated to be up to 40,000 individuals.[27] Based on a few epidemiological studies, the prevalence of sIBM appears to vary considerably between different populations and racial groups, which suggest that genetic factors may play a role in determining susceptibility to the disease. Even though awareness of the sIBM disorder is growing, many expert clinicians believe it remains underdiagnosed, and thus, the number of patients is likely higher than current estimates. The Company believes it is likely that awareness of the disease would increase and patients may be diagnosed at an earlier stage of disease progression. This may in turn lead to an increase in the prevalence of sIBM through the identification of patients who are currently not diagnosed or misdiagnosed.

[26] The information in this section is principally derived from management information based on research and market reports
[27] Callan et al. Journal of Neuromuscular Diseases 4 (2017) 127–137, assuming approximately 850 million inhabitants in USA + EU

b. Treatment options for sIBM and unmet need

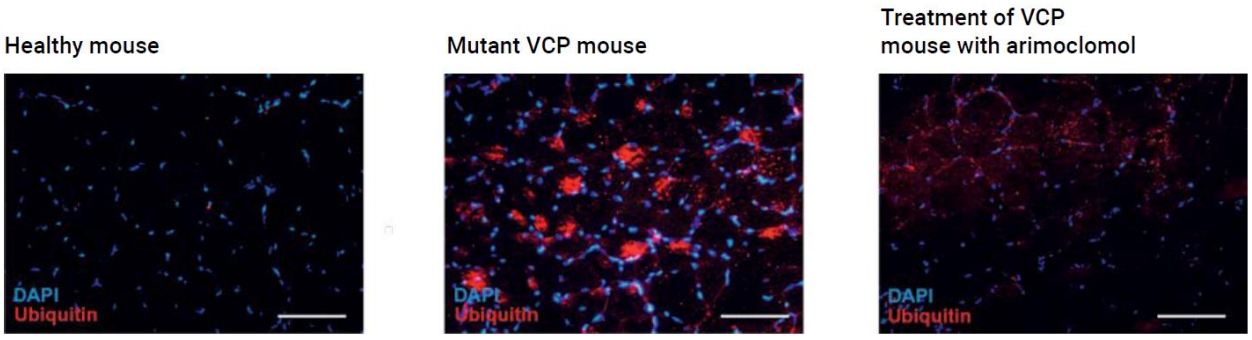
There are currently no effective treatments for sIBM. No therapeutic agent has shown efficacy in preventing, halting or reversing the progression of sIBM and therefore, no drugs have been approved for sIBM. In particular, the disorder has not shown to respond to conventional therapies for autoimmune disorders, such as corticosteroids or immunosuppressive drugs (drugs suppressing the immune system, such as azathioprine, methotrexate, cyclosporine, and cyclophosphamide). Therefore, the standard treatment option for sIBM consists only of supportive therapy such as physical, speech and occupational therapy. Only a few investigational therapies are being studied for patients with sIBM.

As there are currently no effective treatments for sIBM, there is a significant unmet need for safe and efficacious treatment options.

c. Preclinical studies of arimoclomol for the treatment of sIBM

Arimoclomol has been tested in both in vitro and in vivo models of sIBM. In in vitro models of sIBM in primary rat myocyte cultures, administration of arimoclomol was found to induce HSP70 and provided improvement on the molecular pathological hallmarks of the disease; the inclusion bodies and the processes leading to their formation. This was demonstrated by the reduction of the accumulation of ubiquitinated inclusion bodies and other molecular markers of protein aggregation.

The cellular effects of arimoclomol were recapitulated in a mouse model of the disease, where arimoclomol provided the same benefit in the mouse muscles, which was accompanied by a significant functional improvement in clinically relevant manifestations of the disease such as muscle force. In these studies, treatment with arimoclomol was initiated after the onset of clinical symptoms in the mice.[28] The figure below illustrates the test of arimoclomol's effect on Inclusion Body Myositis-like pathology in a mouse model of the disease, illustrating a consistent reduction of disease pathology upon treatment with arimoclomol.



Mutated mice with Inclusion Body Myositis hallmarks were treated with arimoclomol from disease onset (four months) plus 10 months. A cross-section of muscle cells from a healthy mouse before the

[28] Ahmed et al, Translational Medicine, 2016

disease is illustrated to the left. No inclusion bodies are present (ubiquitin, red). In the middle picture, inclusion bodies (red) are evident in a mutant VCP mouse throughout the muscle cells. The mouse has a significant decrease in muscle force. In the picture to the right, treatment of the VCP mouse with arimoclomol results in upregulated HSP70, inclusion bodies are reduced and the loss of muscle force is prevented. Source: Ahmed et. al. Targeting protein homeostasis in sporadic inclusion body myositis, Science Translational Medicine, page 7, March 23, 2016, vol. 8, issue 331.

d. Clinical trials of arimoclomol for sIBM

Results from a randomized, double blinded, placebo-controlled phase II clinical trial in sIBM was published in 2016.[29] The primary endpoint was safety, but trends in improved clinically relevant efficacy endpoints at all assessment time points were demonstrated. Post-hoc responder analysis indicated a very consistent treatment response.

The arimoclomol phase II trial in sIBM indicated consistent trends of benefit in favor of arimoclomol across pre-defined independent efficacy endpoints and assessment time points. Following the in vitro and in vivo preclinical studies, arimoclomol was assessed in a phase II investigator initiated trial. The trial results demonstrated that 100 mg arimoclomol administered three times per day for four months was well tolerated and associated with clinically meaningful benefits when comparing with placebo treatment and these benefits persisted for several months beyond treatment period. The trial was not powered to show efficacy. However, a responder analysis revealed consistent positive trends in favor of the arimoclomol-treated group.

e. Clinical development plan

The Company is currently conducting a phase II/III trial in the United States and Europe. The trial was initiated in August 2017 and fully enrolled in April 2019. The trial is intended to form the basis for registration. The Company is conducting the trial in collaboration with the University of Kansas and the University College London. The trial is a randomized, double blinded, placebo-controlled phase II/III trial assessing efficacy and safety of arimoclomol 400 mg three times per day in patients with sIBM. The primary endpoint analysis is after 20 months. This trial started as an investigator-initiated trial led by the University of Kansas, and the sponsorship of the trial was transferred to the Company in December 2017. Top-line results from the phase II/III trial are expected to be available in the first half of 2021. If successful, the trial has the potential to form the basis for regulatory approval. In November 2017, the FDA granted an orphan drug designation to arimoclomol for the treatment of sIBM. In addition, in December 2020, the Company received fast track designation from the FDA for arimoclomol as a treatment of sIBM, which further underlines the great potential of the Company's investigational drug.

II Arimoclomol for the treatment of Amyotrophic Lateral Sclerosis (ALS)

a. Overview of ALS

ALS, also called Lou Gehrig's disease, is a rapidly progressive and invariably fatal neurological disease with onset typically around 40 to 60 years of age. It attacks neurons responsible for controlling voluntary

[29] Ahmed et al, *Science Translational Medicine*, 2016

muscles. ALS results in muscle weakness, progressive disability and eventually death, typically from respiratory failure. The cause of damage to the neurons is unknown, but several theories have been proposed, including glutamate toxicity, protein misfolding and oxidative stress.

Despite being classified as a rare disease, ALS is considered one of the more common neurodegenerative diseases worldwide. The patient population in Europe and the United States is estimated to be up to 50,000 patients.[30] The CDC (Centers for Disease Control and Prevention) estimates that there are approximately 16,000 cases of ALS in the United States and that approximately 5,000 new cases are diagnosed each year. The number of total cases for the largest five markets in the EU (Germany, France, Italy, Spain and the United Kingdom) is estimated to be slightly higher and for the broader European geography the estimate is approximately 40,000 cases. Hence, the conservatively estimated market for the United States and Europe is up to 50,000 cases. In addition, Japan is a large market for ALS and is estimated to have between 8,000 and 14,000 cases. [31]

b. Treatment options for ALS and unmet need

There are currently a limited number of available treatments for ALS and these only impart a modest effect. The focus of medical care is to give symptomatic management of patients with mild to moderate disease and easing (palliative) intervention in patients with severe or terminal disease.

Until recently, the only pharmaceutical product used for modifying ALS was Rilutek (riluzole) developed by Sanofi, which was the first drug to be approved by the FDA for the treatment of the disease more than 20 years ago and is now available in oral generic tablets as well as branded liquid and film formulations. In May 2017, the FDA approved Radicava (edaravone), which is administered through chronic cycles of 14-days of intravenous infusions. Data from the study demonstrated that patients receiving Radicava for six months experienced significantly less decline in physical function compared to placebo. The Company believes that the approval of Radicava, based on a study with relatively few people, provides evidence of the support from the FDA for development of drugs for the treatment of ALS, which could also be relevant for the Company's product candidates. Mitsubishi Tanabe, the manufacturer of Radicava, withdrew its EMA MAA from the EMA in May 2019.[32] A clinical trial of oral Radicava has just been started. The newly FDA-approved drug for the treatment of ALS, Radicava (edaravone) developed by Mitsubishi Tanabe Pharma Corporation, has an indicated cost of $1,000 for each infusion. The ALS Association has estimated an annual treatment cost of $146,000.

In addition to the current treatment options for ALS, a number of pharmaceutical products are being developed to treat ALS, including (i) levosimendan (Orion Pharma) a calcium channel sensitizer approved in many countries except for the United States for treatment of acutely decompensated heart failure, (ii) NurOwn (Brainstorm therapeutic) a mesemchymal stem cell treatment and (iii) BIIB067/tofersen (Biogen) an antisense oligonucleotide therapy of SOD1-ALS. Each of these studies are

---

[30] Defined Health for Orphazyme, Mehta, P.; Kaye, W.; Bryan, L.; Larson, T.; Copeland, T.; Wu, J.; Muravov, O.; Horton, K. Prevalence of Amyotrophic Lateral Sclerosis - United States, 2012 - 2013 . MMWR Sur veill Summ, 2016; 65 (8): 1 – 12 and Chiò, A.; Logroscino, G.; Traynor, BJ.; Collins, J.; Simeone, JC.; Goldstein, LA.; White, LA. Global epidemiology of amyotrophic lateral sclerosis: a systematic review of the published literature. Neuroepidemiology. 2013; 41(2): 118–130. DOI:10.1159/000351153.

[31] Mehta et al, MMWR Surveill Summ 2016, Chio et al., Neuroepidemiology, 2013 and Defined Health for Orphazyme, 2017.

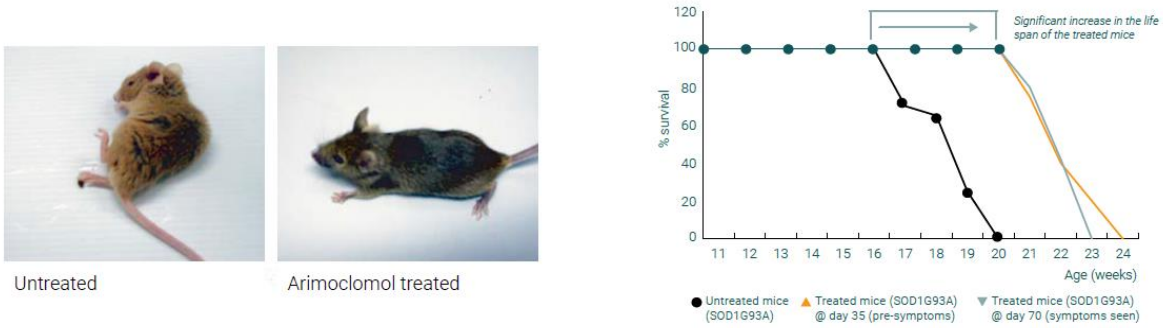
[32] ENCALS invites MT Pharma to conduct a trial in Europe, European Network to Cure ALS, https://www.encals.eu/encals-statement-edaravone/

currently undergoing phase 3 clinical development by pharmaceutical companies. In addition, there are phase 3 plans for (i) Imatinib (AB Science) a tyrokinase inhibitor used for mast cell tumors in animals and (ii) reldesemtiv (Cytokinetics) a fast skeletal muscle troponin activator.

With the current treatment options there is still a major need for new effective treatments for patients with ALS in order to improve the clinical course of the disease and to further extend survival.

c. Preclinical studies of arimoclomol for the treatment of ALS

Arimoclomol has been extensively tested in the best characterized preclinical model of ALS – a transgenic mouse model overexpressing human mutant SOD1. This model has a phenotype and pathology similar to the human disease, including loss of motor neurons in the spinal cord and resulting loss of muscle function. In this model, arimoclomol treatment led to amplified production of HSPs with concomitant reduction of protein aggregates in affected motor neurons in the spinal cord. The arimoclomol-treated mice also showed improvement in motor neuron survival, functional benefit and extension of life by up to 22%, even when arimoclomol was administered after symptom onset[33] as illustrated in the figure below. An expert review when the findings were published stated that more than 70 drugs have been tested in the mouse model, but only few compounds prolong survival by more than 10%, even when started pre-symptomatically.[34]



Results of the SOD1-ALS mouse study. The pictures of mice to the left depict untreated and arimoclomol-treated mice. The untreated SOD1 mouse shows significant signs of hind limb muscle wasting, no toe-spreading reflex, marked kyphosis and is unable to right itself. This mouse was judged to have reached the disease endpoint. The arimoclomol treated, age-matched SOD1 littermate mouse shows definite toe-spreading reflex, no signs of hind limb muscle wasting or kyphosis, and is able to perform a righting reflex test with no delay. The panel on the right depicts Kaplan-Meier survival plots of SOD1 mice over time. A decline in the curve represents a death, so the higher the line, the more extended the survival. Mice treated with arimoclomol (red and green triangles) survived significantly longer than untreated mice (black circles), even when treatment was started after disease onset (at 70 days, green triangles). Source: Kieran et al. Treatment with arimoclomol, a coinducer of heat shock proteins, delays disease progression in ALS mice, Nature Medicine, page 404, April 2004, vol. 10, no. 4.

d. Clinical trials of arimoclomol for the treatment of ALS

Arimoclomol has so far been tested in two Phase II ALS trials, one dose-ranging trial in sporadic ALS and one trial in ALS caused by SOD1 mutations. A randomized, double-blinded, placebo-controlled phase II clinical trial in SOD1 ALS was concluded in 2016. Consistent trends were observed across all efficacy endpoints and arimoclomol was observed to be well-tolerated. The primary endpoints of safety and tolerability were met. While not powered to show statistically significant therapeutic effect, the trial results indicated a consistent benefit of arimoclomol over placebo on all pre-defined clinical endpoints as exemplified by a hazard ratio of death 0.67 in favor of arimoclomol. Patients with the common A4V mutation were balanced between the treatment and placebo group. Other mutations were either only in

[33] Kieran et al., Nature Medicine, 2004; Kalmar et al., J Neurochemistry, 2008
[34] 58 Robert H Brown Jr, News and Views, Nature Medicine, 2004

the treated group or in the placebo group. An efficacy analysis of the A4V subpopulation was therefore predefined in the protocol.

In the A4V patient population, the trial demonstrated a clinically meaningful reduction of the progression rate on the ALSFRS-R scale by up to 39%. The treatment difference increased when correcting for Riluzole use and baseline pulmonary function. On the survival endpoint, hazard ratios of 0.59 were in favor of arimoclomol. In line with a functional and survival benefit there was also a reduction in the decline of pulmonary function by up to 33%. CAFS, a score combining functionality and mortality, also indicated that arimoclomol was superior to placebo (20.5 for arimoclomol versus 14.5 for placebo, where a lower rank score indicates a worse outcome). Sensitivity analyses correcting for baseline imbalances confirmed the effects observed in the primary analyses.

In conclusion, the trial results demonstrated that 200 mg of arimoclomol administered three times per day for 12 months was well tolerated. The trial also demonstrated that patients treated with arimoclomol showed positive trends across all clinical endpoints when compared to placebo treatment. The trial was not powered to show efficacy. However, pre-defined analyses revealed consistent trends in favor of the arimoclomol-treated group across all pre-defined clinical endpoints.

e. Clinical development plan

In August 2018, the Company initiated a phase III clinical trial which was fully enrolled by July 2019. The trial was intended to support a marketing authorization in the broad ALS population. The trial design includes clinical enrichment strategies to ensure homogeneous disease progression in the trial. The trial is a randomized, double blinded, placebo-controlled phase III trial assessing efficacy and safety of arimoclomol 400 mg three times per day. The trial has enrolled 237 (245 including 8 patients on edavarone) patients. Top-line results from the trial are expected to be announced in the first half of 2021. If the outcome is positive, the Company intends to file for registration for ALS in the second half of 2021.

6.5.5 *New molecular entity programs*

The Company is developing a new series of potential drugs based on its expertise and know-how about the heat shock response and HSPs, protein aggregation and cellular recycling systems, and how these can be targeted for therapeutic benefit for relevant protein misfolding diseases. These molecules are currently being vetted and will be prioritized based on their suitability and potential for specific diseases.

6.6 Intellectual property

The Company's patent strategy is to continuously ensure patent protection covering any inventions that support its development programs. To date, this has resulted in a wide patent portfolio with a strategic scope of protection and geographic coverage. The Company's lead product candidate, arimoclomol, is covered by a number of second medical use patents and pending patent applications. These cover the medical use of arimoclomol – as well as other inducers of HSPs - in the treatment of relevant medical indications. These include LSDs, specifically NPC and Gaucher disease, as well as neuromuscular/neurodegenerative disorders, such as ALS and Parkinson's disease (PD), including GBA-associated PD, and frontotemporal disorders such as frontotemporal dementia (FTD) and ALS-FTD. The Company continuously explores the opportunity for additional patent coverage for the use of arimoclomol in the treatment of new indications including specific protein aggregation diseases. Furthermore, arimoclomol in an extended-release formulation is covered by the current patent portfolio.

The Company strives to further identify and develop novel small molecule inducers of HSPs through its NME program. The Company continuously evaluates potential compounds with the ultimate aim to obtain patent protection for the NME compounds, preferably as composition-of-matter.

As of the Prospectus Date, the Company holds the rights to 9 patent families, each with a number of granted patents (109 in total) and pending patent applications (44 in total). While patents generally expire 20 years from the filing date, the Company is highly observant of the possibility to extend the exclusivity period for pharmaceutical products up to five years via Supplementary Protection Certificate

("**SPC**"), available in most European countries, and via the United States analogue Patent Term Extension ("**PTE**"). The Company considers it realistic that at least one SPC/PTE can be obtained per product.

Arimoclomol for treatment of the LDSs, NPC and Gaucher disease, is covered by basic patent coverage until June 2029, with potential for up to three and five year extensions in the United States and Europe, respectively. Other countries offer similar extensions, including Japan and Canada.

Patent family	**Type**	**Expiration**[35]	**Regions**	**Status**
Use of HSP70 as a regulator of enzymatic activity (PCT/DK2009/050151)	Second medical use – directed to hydroxylamine derivative type small molecule inducers of the HSP, including arimoclomol, and HSP70 protein, for treatment of LSDs, including NPC and Gaucher disease	Projected patent expiry date: 26JUN 2029	AU, BR, CA, CN, EP, HK, IL, JP, RU, US	Granted and maintained in: AU, CA, CN, EP (AT, BE, CH/LI, CZ, DE, DK, ES, FI, FR, GB, GR, HU, IE, IT, NL, NO, PL, PT, RO, SE, TR), HK, IL, JP, RU, US Pending in: BR, CA, EP, US
Methods for increasing intracellular activity of HSP70 (PCT/DK2011/050444)	Second medical use – directed to hydroxylamine derivative type small molecule inducers of the HSP, including arimoclomol, and HSP70 protein, for treatment of additional LSDs, such as NCL	Projected patent expiry date: 22 NOV2031	EP, US	Granted and maintained in: EP (AT, BE, CH/LI, CZ, DE, DK, ES, FI, FR, GB, GR, HU, IE, IT, NL, NO, PL, PT, RO, SE, TR), US Pending in: EP, US
Arimoclomol formulation (PCT/DK2015/050275)	Formulation: Extended-release formulation of arimoclomol	Projected patent expiry date: 15 SEP2035	AU, BR, CA, CN, EP, IL, IN, JP, KR, RU, US	Pending in: AU, BR, CA, CN, EP, IL, IN, JP, KR, RU, US
Heat Shock Proteins and Cholesterol Homeostasis (PCT/DK2017/050114)	Second medical use – directed to hydroxylamine derivative type small molecule inducers of the HSPs, including arimoclomol, and HSP70 protein, for treatment of diseases associated with dysregulation of cholesterol homeostasis	Projected patent expiry date: 10 APR2037	EP, US	Pending in: EP, US
Arimoclomol for treating glucocerebrosidase associated disorders (PCT/EP2017/060205)	Second medical use – directed to arimoclomol for treatment of glucocerebrosidase (GBA)-associated disorders, including GBA-associated Parkinson's disease	Projected patent expiry date: 28 APR2037	AU, BR, CA, CN, EP, IL, JP, KR, RU, US	Pending in: AU, BR, CA, CN, EP, IL, JP, KR, RU, US

[35] Not including potential patent term extensions and patent term adjustments

Patent family	Type	Expiration[35]	Regions	Status
Heat shock protein inducers and frontotemporal disorders[36] (PCT/EP2018/063662)	Second medical use – directed to hydroxylamine derivative type small molecule inducers of the heat shock proteins, including arimoclomol, and HSP70 protein, for treatment of frontotemporal disorders including frontotemporal dementia FTD and ALS-FTD	Projected patent expiry date: 24 MAY2038	AU, BR, CA, CN, EP, IL, JP, MX, RU, US	Pending in: AU, BR, CA, CN, EP, IL, JP, MX, RU, US
HSP70 as a biomarker (PCT/EP2019/063854)	Biomarker - directed to the correlation of reduced HSP70 protein levels observed in peripheral blood mononuclear cell (PBMC) samples and certain diseases; such as LSDs and neurodegenerative diseases, neuromuscular disorders.	Projected patent expiry date: 28 MAY 2039	PCT	Pending - PCT
A pyridine-1-oxide derivative, and process for its transformation into pharmaceutically effective compounds PCT/HU01/00046)	Manufacture - directed to an arimoclomol intermediate compound and its use in the preparation of arimoclomol	Projected patent expiry date: 17 APR2021	AU, BR, CA, CN, EP, IL, JP, US	Granted and maintained in: AU, BR, CA, CN, EP (DE, DK, FR, GB), IL, JP, US
Use of a hydroximic acid halide derivative in the treatment of neurodegenerative diseases (PCT/HU04/00098)	Second medical use – directed to arimoclomol for treatment of neurodegenerative diseases, including ALS and Parkinson's disease	Projected patent expiry date: 25 OCT2024	AU, BR, CA, EP, JP, RU, US, ZA	Granted and maintained in: AU, CA, EP (AT, BE, DE, DK, ES, FR, GB, GR, HU, IT, NL, PL, PT, SE, TR, UA), JP, RU, US, ZA Under appeal in:

[36] Filed in the name of Orphazyme ApS and UCL Business PLC (University College London); governed by a Collaborative Research Agreement and Material Transfer Agreement signed in April 2015.

Patent family	Type	Expiration[35]	Regions	Status
				BR, US

The Company has registered "**ORPHAZYME**" as word mark in relevant trademark classes and jurisdictions (EU and International Madrid Protocol designating US, CN, IL, IN, JP and RU).

Significant change in the operations and principal activities

There has been no significant change to the operations and principal activities of the Group since the end of the period covered by the FY2019 Group Financial Statements.

6.7 Investments

Since the end of the period covered by the FY2019 Group Financial Statements, the Company has not made any material investments, is not in progress of making any material investments and/or has no firm commitments to make any material investments.

7 TREND INFORMATION

Most significant recent trends

There has been no significant trends in production, sales and inventory, and costs and selling prices since the end of the period covered by the FY2019 Group Financial Statements.

Significant change in the financial performance

There has been no significant change to the financial performance of the Group since the end of the period covered by the FY2019 Group Financial Statements.

8 PROSPECTIVE FINANCIAL INFORMATION

8.1 Statement by Management on prospective financial information for the Group for the financial year 2020

Management's prospective consolidated financial information for the financial year 2020 is presented below (the "**Prospective Financial Information**").

We have prepared and presented the Prospective Financial Information, including the key assumptions set out in "*Company information–Prospective Financial Information–Prospective Financial Information–Methodology and assumptions*". The Prospective Financial Information has been compiled and prepared on a basis which is both comparable with the financial information in the FY2019 Annual Report and consistent with the accounting policies applied in the consolidated financial statements for year ended December 31, 2019 ("**FY2019 Group Financial Statements**").

The Prospective Financial Information has been prepared for the purpose of this Prospectus.

The Prospective Financial Information is based on a number of factors, including certain estimates and assumptions. The material assumptions on which the Prospective Financial Information is based are described in "*Company information–Prospective Financial Information–Prospective Financial Information–Methodology and assumptions*".

The Prospective Financial Information represents the best estimates of Management at the Prospectus Date. Actual results are likely to be different from the Prospective Financial Information since anticipated events may not occur as expected, or may materially differ from the forecast provided. The Prospective Financial Information in this section should be read in conjunction with "*Risk factors*" and "*General Information–Forward-looking statements*" included elsewhere in this Prospectus.

Board of Directors

Georges Gemayel *Chairman*	Bo Jesper Hansen *Deputy Chairman*
Martijn Kleijwegt *Board Member*	Martin Bonde *Board Member*
Sten Verland *Board Member*	Anders Hedegaard *Board Member*
Catherine Moukheibir *Board Member*	Rémi Droller *Board Member*

Executive Management

Kim Stratton *CEO*	Anders Vadsholt *CFO*

8.2 Prospective Financial Information

Introduction

The Company prepared the Prospective Financial Information for the FY2019 Group Financial Statements, which is included in this Prospectus, in accordance with applicable laws, rules and regulations.

The Prospective Financial Information was not prepared with a view towards compliance with published guidelines of the U.S. Securities and Exchange Commission and the American Institute of Certified Public Accountants (the "**AICPA**"), for preparation and presentation of prospective financial information. Accordingly, this information does not include disclosure of all information required by the AICPA guidelines on prospective financial information.

While this Prospective Financial Information is presented with numerical specificity, this information is based upon a number of assumptions and estimates, which the Company considers reasonable. As a result, this Prospective Financial Information is inherently subject to significant business, operational, economic and competitive uncertainties and contingencies, and based upon future business decisions that are subject to change.

Therefore, the Company's expectations presented in the Prospective Financial Information as to future developments may deviate substantially from actual developments, and the Group's actual results of operations are likely to be different from the Prospective Financial Information since anticipated events may not occur as expected, or may materially differ from the forecast provided. Accordingly, potential investors should treat this information with caution and not place undue reliance on the expectations set forth below.

Management's Responsibility

The Management is responsible for the proper compilation of the Prospective Financial Information on the basis stated and for accounting policies used for the Prospective Financial Information and their consistency with the accounting policies of the Company and for such internal control as the Management determines is necessary to enable the preparation of Prospective Financial Information on the basis stated.

Furthermore, the Management is responsible for the assumptions underlying the Prospective Financial Information.

Methodology and assumptions

The Prospective Financial Information has been prepared in accordance with the accounting policies presented in the FY2019 Group Financial Statements, which have been prepared in accordance with IFRS as issued by the IASB and in accordance with IFRS as adopted by the EU.

The Prospective Financial Information is prepared for the purpose of this Prospectus.

The Prospective Financial Information has been based on Management's updated budget for 2020 prepared in accordance with the Company's forecasting and budgeting procedures and on a basis comparable to the FY2019 Group Financial Statements.

The Prospective Financial Information is based on a number of factors, including certain estimates and assumptions. The key assumptions concerning the future, and other key sources of estimation uncertainty at the date of the Prospective Financial Information that have a significant risk of causing a material adjustment to the prospective amounts of expenses, and change to assets and liabilities during the year ending December 31, 2020, are listed below. The Company based its assumptions and estimates on information available when the Prospective Financial Information was prepared.

Certain assumptions, uncertainties and contingencies relating to the Prospective Financial Information are wholly or partly within the control of the Company, while others are outside or substantially outside the control of the Company.

While the Company has presented the key assumptions on which the prospective financial information is based below, it is likely that one or more of the assumptions that the Company has relied upon will not prove to be accurate in whole or in part.

The Group's results of operations could deviate materially from its forecasts as a result of other factors, including but not limited to those described in "*General Information–Forward-looking statements*" and "*Risk Factors*".

For the purpose of preparing the Prospective Financial Information, the Company has applied the key assumptions below:

Currency

The Prospective Financial Information is presented in the Company's reporting currency DKK. Many of the expected costs for the year are denominated in foreign currencies. While many of the large cost drivers within research and development costs are denominated in EUR, which is pegged to the DKK within a narrow fluctuation band, many of the pre-commercial and launch costs anticipated will be incurred in the U.S. in USD. Accordingly, future changes in the exchange rates between the DKK and the USD will impact the Company's actual expenses and expose the Company to currency gains or losses that will impact the expected amounts of assets and liabilities, income and expenses and the impact could be material. The currency assumptions applied for purposes of the Prospective Financial Information are outside the control of the Company.

For the time being, the Company has decided not to utilize foreign currency forward contracts or other derivative instruments to mitigate cash flow or market value risks associated with foreign-currency-denominated transactions.

Research and development expenses

Research and development expenses include costs arising from research and clinical development activities, including employee costs for research and development personnel (i.e. salaries, bonuses, employer contributions to pension schemes, share-based compensation) legal expenses related to the protection, defense and enforcement of the Company's intellectual property, as well as depreciation on right-of-use assets associated with facilities and equipment used for research and development purposes.

The Company's research and development expenses vary from period to period depending on the phase of development of its product candidates. For the financial year ending December 31, 2019, research and development expenses were mainly affected by the full costs of the s-IBM phase 2/3 trial and the ALS phase 3 trial, as they both completed recruitment during the year. In addition, open label extensions of both trials were initiated during 2019 and pre-clinical activities increased in preparation for the regulatory filings.

For the financial year ending December 31 2020, the Company expects to continue to incur substantial costs associated with clinical trials. Clinical trial costs are a significant component of research and development expenses. The Company's clinical trials activities are performed by third-party Clinical Research Organizations (CROs) and in order to estimate the amount of costs to charge to expense, Management has developed expense models for each clinical trial based on specific estimates and assumptions related to that particular clinical trial (see additional information in note 2.1 of the 2019 Group Financial Statements). The majority of the assumptions associated with estimating research and development costs are considered substantially outside the Company's control.

Another significant component of research and development activities are costs related to the manufacturing of our drug candidate, arimoclomol, which the Company expects to increase in 2020. In addition, costs to prepare for the filing of the NDA in H1-2020 and the MAA in H2-2020 are expected to significantly increase in 2020.

General and administrative expenses

General and administrative expenses include salaries for administrative employees and Executive Management, remuneration to the Board of Directors, share-based compensation costs, depreciation on right-of-use assets associated with facilities not used for research and development purposes, and investor relations. In addition, the Company includes pre-commercial activities in general and administrative expenses, such as the costs associated with the Early Access Program for NPC, tradename costs, market and pricing studies, and launch preparation activities. The majority of the assumptions associated with estimating general and administrative expenses are substantially within the Company's control.

The Company's general and administrative expenses are expected to significantly increase in 2020, both in number of FTEs as well as costs of activities, as it prepares for commercial launch by building up the U.S. organization and prepares to expand the global reach. In addition, the support functions such as finance, legal and IT are also expected to increase both in FTEs and costs in order to effectively support the organization. Lastly, the Company also anticipates increased investor relations activities.

Income tax benefit

Consistent with prior years, the Company has included an estimated income tax benefit in the Prospective Financial Information resulting from the anticipated tax credit for research and development expenses at the applicable tax rate under the Danish Corporate Income Tax Act. No capitalization of deferred tax assets is included.

Expectations for the net result for the financial year ending December 31, 2020

Based on the assumptions and methodology as set out above, the Company confirms the expectations for the net result for the financial year ending December 31, 2020, as set out in the FY2019 Group Financial Statements, and accordingly expects a net loss for the financial year 2020 in the range of DKK 500 million - DKK 550 million. At December 31, 2020 the Company anticipates a cash position greater than DKK 300 million.

The Company's result of operations for the financial year 2020 could deviate materially from this forecast as a result of other factors, including, but not limited to, those described in "*General Information–Forward-looking statements*" and "*Risk Factors*".

9 BOARD OF DIRECTORS, EXECUTIVE MANAGEMENT AND KEY EMPLOYEES

9.1 Overview

The Company has a two-tier governance structure consisting of the Board of Directors and the Executive Management. The two bodies are separate and have no overlapping members. The Executive Management is supported by the Company's key employees (the "**Key Employees**").

The business address of the Board of Directors, Executive Management and the Key Employees is Ole Maaløes Vej 3, DK-2200 Copenhagen N, Denmark.

For a description of the remuneration of the Board of Directors, Executive Management and Key Employees, including ownership of Shares and holding of Share related instruments granted under the Company's current incentive schemes, see the FY2019 Group Financial Statements.

Board of Directors

The Board of Directors is responsible for the overall and strategic management and proper organization of the Company's business and operations and supervises the Company's activities, management and organization. The Board of Directors appoints and dismisses the members of the Executive Management, who are responsible for the day-to-day management of the Company.

In accordance with article 8.1 of the Articles of Association, the general meeting of the Company shall elect not less than six and not more than nine members to the Board of Directors. The Board of Directors elects a chairman (the "**Chairman**") and, if so decided, a deputy chairman ("**Deputy Chairman**") of the Board of Directors among its members. See article 8.3 of the Articles of Association.

The members of the Board of Directors elected by the general meeting are elected for a term of one year. Members of the Board of Directors may be re-elected. See article 8.2 of the Articles of Association.

At the date of this Prospectus, the Board of Directors comprises of eight members elected by the general meeting comprising the Chairman, the Deputy Chairman and six board members.

The following table presents an overview of the current composition of the Board of Directors:

Name	Position	Independent[1]	Year of first appointment	Expiration of term
Georges Gemayel	Chairman	Independent	2012	2020
Bo Jesper Hansen	Deputy Chairman	Independent	2010	2020
Anders Hedegaard	Member	Independent	2017	2020
Catherine Moukheibir	Member	Independent	2017	2020
Martijn Kleijwegt	Member	Independent	2017	2020
Martin Bonde	Member	Independent	2010	2020
Rémi Droller	Member	Independent	2015	2020
Sten Verland	Member	Independent	2010	2020

(1) The Company has based its assessment of independence on the basis of the criteria set out in the current Corporate Governance Recommendations.

All members of the Board of Directors are considered by the Company to be independent under the current Corporate Governance Recommendations.

Biographies

Other than as presented below, none of the members of the Board of Directors have been a member of the administrative, management or supervisory bodies of a company or a partnership or been a partner in a partnership outside the Company within the past five years.

Georges Gemayel (born 1960, American nationality) has been a member of the Board of Directors since November 2012 and Chairman since September 2014. Georges Gemayel is currently chairman of the board of directors of Dynacure SAS, Enterome SA and OxThera AB and a member of the board of directors of Momenta Pharmaceuticals Inc. (publ) and Supernus Pharmaceuticals Inc. (publ). Georges Gemayel is also a partner in Gemayel Investment LLC as well as a director of the non-governmental

organization, St. Andrew's School in Ngong Inc. and a trustee of the Gemayel Family Foundation. In the past five years, Georges Gemayel has previously been chairman of the board of directors of Dimension Therapeutics Inc. (publ), Epitherapeutics ApS and Vascular Magnetics Inc., a member of the board of directors of NPS Pharmaceuticals Inc. (publ) and Raptor Pharmaceuticals Corp. (publ), a consultant for Novo Ventures 1 A/S, Fidelity Ventures and Noveome Biotherapeutics Inc. as well as a director of the non-governmental organization, International Institute of New England. Georges Gemayel holds a Master's degree and a PhD degree in Pharmacology from Paris-Sud University and a Docteur d' Exercice en Pharmacie from the St. Joseph University.

Bo Jesper Hansen (born 1958, Danish nationality) has been a member of the Board of Directors since December 2010 and Deputy Chairman since October 2017. Bo Jesper Hansen is currently chairman of the board of directors of Laborie Inc., Innoventa Medica ApS and Karo Pharma AB as well as a member of the board of directors of Azanta A/S and Ascelia Pharma AB. Bo Jesper Hansen is also a venture partner at Wellington Partners Life Science Fund LP and an advisory consultant for Aescap 2.0, Nordic Capital, EQT AB and Broad Street Principal Investments Europe Ltd. and senior business advisor for HBM Ventures Ltd. In the past five years, Bo Jesper Hansen has previously been chairman of the board of directors and a member of the executive management of Swedish Orphan Biovitrum AB (publ), chairman of the board of directors of Ablynx NV, Karolinska Development AB (publ) and a member of the board of directors of Newron Pharmaceuticals SpA, CMC Sweden AB, Hyperion Therapeutics Inc. (publ) and Inspyr Inc. (publ). Bo Jesper Hansen holds a MD and PhD degree in Medicine from the University of Copenhagen.

Anders Hedegaard (born 1960, Danish nationality) has been a member of the Board of Directors since November 2017. Anders Hedegaard is currently chief executive officer of Rodenstock Group and chairman of the board of directors Rodenstock Danmark A/S. Anders Hedegaard is also recommended as the chairman of the board of directors of ALK-Abelló A/S (publ) from March 2020. In the past five years, Anders Hedegaard has previously been chairman of the board of directors of Natus Medical Denmark ApS, a chief executive officer of GN Store Nord A/S (publ) and GN Hearing A/S, a member of the executive management and a member of the board of directors of GN Advanced Hearing Protection A/S as well as a member of the board of directors of the Confederation of Danish Enterprise, Hearing Instrument Manufacturers Software Association A/S and HIMSA II A/S. Anders Hedegaard holds a Master of Science in Chemical Engineering and Biochemistry from the Technical University of Denmark.

Catherine Moukheibir (born 1959, American, Lebanese and British nationality) has been a member of the Board of Directors since November 2017. Catherine Moukheibir is currently chairman of the board of directors and chief executive officer of MedDay Pharmaceuticals SA as well as member of the board of directors of Genkyotex SA (publ), Ironwood Pharmaceuticals, Inc. and Kymab Ltd. Catherine Moukheibir is also currently a member of the advisory board of Imperial College Business School. In the past five years, Catherine Moukheibir has previously been a member of the executive management and a consultant for Innate Pharma Inc. (publ), chairman of the board of directors of Creabilis Therapeutics SRL, a member of the board of directors of Zealand Pharma A/S (publ), Ablynx NV (publ) and Cerenis Therapeutics SA (publ) and a member of the international advisory board of the Yale School of Management. Catherine Moukheibir holds a Master in Economics and an MBA degree, both from Yale University.

Martijn Kleijwegt (born 1955, Dutch nationality) has been a member of the Board of Directors since January 2017. Martijn Kleijwegt is currently founder and managing partner at LSP Management Group BV and a member of the board of directors of AM Pharma BV, Kiadis Pharma N.V. (publ), OxThera AB, Eloxx Pharmaceuticals Ltd. and Pharvaris BV. In the past five years, Martijn Kleijwegt has previously been a member of the board of directors of Prosensa N.V. (publ). Martijn Kleijwegt holds a Master's degree in Economics from the University of Amsterdam.

Martin Bonde (born 1963, Danish nationality) has been a member of the Board of Directors since June 2010 and was chairman of the Board of Directors until September 2014. Martin is currently Entrepreneur-in-Residence at BiOrigin ApS, a Novo Seeds company. Martin Bonde is also a member of board of directors of Visiopharm A/S, chief executive officer of Bohrs Tower ApS as well as a member of the board of directors and the executive management of Biotopix ApS. In the past five years, Martin Bonde has been chairman of the board of directors of the trade organization Dansk Biotek, chief

executive officer of Vaccibody AS and Epitherapeutics ApS. Martin Bonde holds a Graduate Diploma in Business Administration (HD i Udenrigshandel) from Copenhagen Business School, a Master of Science and a PhD in Chemical Engineering from the Technical University of Denmark.

Rémi Droller (full name: Rémi Pascal Louis Droller, born 1975, French nationality) has been a member of the Board of Directors since January 2015. Rémi Droller is currently managing partner of Kurma Partners SA and member of the board of directors of Dyncaure SAS, ImCheck Therapeutics SAS, OxThera AB, AM Pharma BV, Flamingo Therapeutics BV, Vico Therapeutics BV and Pharvaris BV. In the past five years, Rémi Droller has previously been chairman of the board of directors of Step Pharma SAS and a member of the board of directors of STAT Dx (sold to Qiagen). Rémi Droller holds a Master's degree in Molecular Biology from Pierre and Marie Curie University in Paris and a Master's degree in Finance and Management of Innovation from AgroParisTech.

Sten Verland (born 1957, Danish nationality) has been a member of the Board of Directors since December 2010. Sten Verland is currently co-founder and member of the board of directors of Sunstone Capital A/S, member of the board of directors and general partner at Sunstone Life Science Ventures A/S, a member of the board of directors of STipe Therapeutics ApS, Anergis SA, MinervaX ApS, OxThera AB, the Danish Venture Capital and Private Equity Association (DVCA) as well as a member of the board of directors and executive management in certain companies in, or associated with, the Sunstone group. Sten Verland is also currently a member of the executive management of Verland Capital ApS, Verland Holding ApS, Verland Holding II ApS and Genobiotix ApS. In the past five years, Sten Verland has previously been a member of the board of directors of F2G Ltd., Vaximm AG, Rigontec GmbH, Selskabet af 9. september 2015 A/S and Selskabet af 23. september 2015 ApS, a member of the board of directors and chief executive officer of VetVerland ApS as well as a general partner and a member of the board of directors and executive management in certain companies in or associated with the Sunstone group. Sten Verland holds a Master's degree in Biology and Mathematics and a PhD in Immunology, both from the University of Copenhagen.

Executive Management

According to article 9.1 of the Articles of Association, the Board of Directors appoints an Executive Management consisting of one to three members. The primary task of the Executive Management is to carry out the day-to-day management of the Company with the support of the Key Employees.

The following table presents an overview of the current members of the Executive Management:

Name	Position	Year of first appointment	Year of appointment to current position
Kim Stratton	Chief Executive Officer	2019	2019
Anders Vadsholt	Chief Financial Officer	2016	2016

Biographies

Other than as presented below, none of the members of the Executive Management have been members of the administrative, management or supervisory bodies of a company or a partnership or a partner in a partnership outside the Company within the past five years.

Kim Stratton (full name: Kim Narelle Stratton, born 1962, Australian nationality) has been Chief Executive Officer since she joined the Company in October 2019. Kim Stratton is currently a member of the board of directors of Novozymes A/S (publ) and Vifor Pharma AG. In the past five years, Kim Stratton has previously been a member of the board of directors of the European Federation of Pharmaceutical Industries and Associations (EFPIA) and Head of International Commercial for all ex-U.S. Business across Specialty and Rare Diseases at Shire. Kim Stratton is a Registered Nurse and received her certification at Royal North Shore Hospital (Australia).

Anders Vadsholt (full name: Anders Fink Vadsholt, born 1969, Danish nationality) has been Chief Financial Officer since he joined the Company in May 2016. Anders Vadsholt is currently owner and a member of the executive management of Alpha Healthcare Investments ApS as well as a partner at

Obton Solenergi Sinope Komplementaranpartsselskab. In the past five years, Anders Vadsholt has previously been a member of the executive management of Lakeside Invest ApS and Copenhagen Innovation Capital Management ApS. Anders Vadsholt holds a Bachelor of Science in Corporate Law from the University of Aalborg, an MBA in Finance and Strategy from the University of Melbourne, a Master of Science in Corporate Law and Economics from Copenhagen Business School as well as a Diploma in Basic Pharmaceutical Medicine, Pharmacology and Pathology from LIF.

Key Employees

The Key Employees are employed by the Company with responsibility for their functional areas.

The following table presents an overview of the Company's current Key Employees:

Name	Position	Year of first appointment	Year of appointment to current position
Thomas Blaettler	Chief Medical Officer	2016	2016
Thomas Kirkegaard Jensen	Chief Scientific Officer	2009(1)	2010

(1) Thomas Kirkegaard Jensen joined the Company as CEO in 2009 and was made CSO in 2010.

Biographies

Other than as presented below, none of the Key Employees have been members of the administrative, management or supervisory bodies of a company or a partnership or a partner in a partnership outside the Company within the past five years.

Thomas Blaettler (born 1967, Danish and Swiss nationalities) has been Chief Medical Officer, since he joined the Company in November 2016. Thomas Blaettler has previously been PD Neuroscience Group Medical Director at F. Hoffmann-La Roche Ltd. Thomas Blaettler holds a Doctorate in Medicine from the University of Zürich and a Medical School Certificate Swiss State Examination from the Medical School of the University of Zürich and is a board certified neurologist by the Swiss Medical Association (the Foederation Medicorum Helveticorum).

Thomas Kirkegaard Jensen (born 1977, Danish nationality) joined the Company as Chief Executive Officer in 2009 and was made Chief Scientific Officer in March 2010. Thomas Kirkegaard Jensen is currently a member of the executive management of Dare to Dream ApS, an expert reviewer for the European Research Council and a member of the advisory board for the Rare Disease Report. In the past five years, Thomas Kirkegaard Jensen has been a member of the board of directors and executive management of OZ Holding ApS and vice-chairman of the national Orphan Disease Council. Thomas Kirkegaard holds a Bachelor of Science in Biochemistry, a Master of Science in Human Biology and a PhD in Medicine from the University of Copenhagen.

Statement of kinship

There are no family ties among the members of the Board of Directors, the Executive Management or any of the Key Employees.

Statement on past records

During the past five years, none of the members of the Board of Directors, the Executive Management or any of the Key Employees have been (i) convicted of fraudulent offenses; (ii) directors or officers of companies that have entered into bankruptcy, receivership, liquidation or companies put into administration, except as set out immediately below; or (iii) subject to any official public incrimination and/or sanctions by statutory or regulatory authorities (including designated professional bodies), and have not been disqualified by a court from acting as a member of an issuer's board of directors, executive

management or supervisory body or from acting in the management or conduct of the affairs of any issuer.

Georges Gemayel was chairman of Epitherapeutics ApS until 2015, when the company went into voluntary liquidation (which is currently still ongoing). Georges Gemayel was a member of the board of directors of NPS Pharmaceuticals Inc. until 2015, when the company was dissolved following merger; Prosensa N.V. until 2015, when it was dissolved following merger; and Raptor Pharmaceuticals Corp. until 2016, when the company was dissolved following merger.

Bo Jesper Hansen was a member of the board of directors of Hyperion Therapeutics Inc. until 2015, when the company was dissolved following acquisition.

Anders Hedegaard was chairman of the board of directors of Scanning Technology A/S until 2015, when the company was dissolved following merger.

Martijn Kleijwegt was a member of the board of directors of Prosensa N.V. until 2015, when the company was dissolved following merger.

Martin Bonde was a member of the executive management of Epitherapeutics ApS, when it was dissolved following merger in 2015.

Sten Verland was a member of the board of directors of P/S Sunstone Biomedicinsk Venture III, which is currently undergoing voluntary liquidation and NsGene A/S, when it was dissolved by voluntary liquidation in May 2017. Sten Verland was a member of the executive management of Sunstone LSV Partners Holding III ApS, when it was dissolved following declaration of payments in January 2015 and Sunstone LSV Partners & Co. Holding III ApS, when it was dissolved following declaration of payments in January 2015.

Anders Vadsholt was a member of the executive management of Lakeside Invest ApS, when it was dissolved by voluntary liquidation in 2018 and Copenhagen Innovation Capital Management ApS, when it was dissolved by voluntary liquidation in 2018.

9.2 Conflict of interest

Statement on conflicts of interest

No actual or potential conflicts of interest exist between any of the duties of the members of the Board of Directors, the Executive Management and the Key Employees and their private interests or other duties.

None of the members of the Board of Directors, or the Executive Management or any other Key Employees have conflicts of interest with respect to their duties as members of the Board of Directors, or the Executive Management or as Key Employees except for the members of the Board of Directors, Martin Kleijwegt, Rémi Droller and Sten Verland, for the reasons set out in the paragraph above.

None of the members of the Board of Directors, the Executive Management or the Key Employees have positions in other companies which could result in a conflict of interest vis-à-vis such companies, either because the Company has an equity interest in such company or because the Company and the company concerned have an ongoing business relationship. However, the Company may do business in the ordinary course with companies in which members of the Board of Directors, or the Executive Management, or the Key Employees may hold positions as directors or officers.

With the exception of the members of the Board of Directors, Martin Kleijwegt, Rémi Droller and Sten Verland, the Company is not aware of any member of the Board of Directors, or the Executive Management or any of the Key Employees having been appointed to their current position pursuant to an agreement or understanding with Major Shareholders, customers, suppliers or others.

It follows from the Rules of Procedure of the Company's Board of Directors and the Danish Companies

Act that a member of the Board of Directors or the Executive Management shall not participate in the preparation, discussions or the decision-making process concerning an agreement between the Company and the member in question or concerning legal proceedings between the member in question and the Company or an agreement between the Company and any third party or legal proceedings brought against any third party if the member in question has a significant interest therein that may conflict with its interests.

Restrictions on securities trading

The Board of Directors, the Executive Management and the Key Employees are subject to lock-up restrictions provided in connection with the Private Placement preventing them from disposing of or otherwise transferring shares of the Company for a period of 90 days from the date of closing of the Private Placement on February 11, 2020, subject to certain customary exemptions. In addition, certain restrictions on securities trading apply in respect of the Board of Directors, the Executive Management and the Key Employees as provided by law and the Company's internal rules.

10 MAJOR SHAREHOLDERS

Pursuant to section 38 of the Danish Capital Markets Act and section 55 of the Danish Companies Act, the Company has received notifications of holdings of 5% or more of the share capital or voting rights from the shareholders below.

The following table presents an overview of Major Shareholders as at the Prospectus Date:

Shareholder	Number of Shares as at latest announcement	Ownership interest as at latest announcement	Voting rights as at latest announcement
Danske Bank A/S[1]	1,244,908	4.60%	6.72%
Novo Holdings A/S[2]	2,021,673	7.5%	7.5%
LSP V Coöperatieve U.A.[3]	2,710,829	10.03%	10.03%
Sunstone Life Science Ventures Fund II K/S	1,804,405	9.10%	9.10%
Coöperative Aescap Venture I U.A.	1,765,605	8.90%	8.90%
Consonance Capman GP LLC	1,900,000	7.03%	7.03%

[1] Danske Bank A/S' shareholding consists of a 3.90% indirect and 0.70% direct ownership through Danica Pension Livsforsikringsaktieselskab, Danica Pension Försäkringsaktiebolag, Investeringsforeningen Danske Invest and Danske Invest SICAV. Danske Bank A/S' control of voting rights in the Company consists of a 6.02% indirect and 0.70% direct control through Danica Pension Livsforsikringsaktieselskab, Danica Pension Försäkringsaktiebolag, Investeringsforeningen Danske Invest, and Danske Invest SICAV.
[2] Novo Holdings A/S is wholly owned by Novo Nordisk Foundation.
[3] This shareholding includes a direct shareholding of 279,157 shares corresponding to 1.03% of the total share capital and voting rights and an indirect shareholding of 2,431,672 shares held through Orpha Pooling B.V. (a joint venture between LSP V Coöperatieve U.A and ALS Invest 2 B.V.) corresponding to 9.00% of the total share capital and voting rights.

The percentage of voting rights described above is based on the entire registered share capital of the Company as at the Prospectus Date.

The Company is not authorized to issue company announcements regarding major shareholdings unless the Company has received a prior notice to that effect from a shareholder. Thus, the number of shares and voting rights of major shareholders stated in the specification above may have changed.

The Major Shareholders do not have different voting rights. All Shares in the Company will rank *pari passu*, including with respect to voting rights. All Shares will carry 1 vote per share of a nominal value of DKK 1.

The Company is not aware of being owned or controlled, directly or indirectly, by others, and the Company is not aware of any agreements that could later result in others taking over the control of the Company.

11 RELATED PARTY TRANSACTIONS

Since the FY2019 Group Financial Statements, the Company has entered into a related party transaction with the Lending Shareholders by entering into a stock lending and subscription agreement as further described in "*The Listing–Key Information on Capitalization and Background of the Listing–Bankground for the Listing*."

12 INFORMATION ON ASSETS AND LIABILITIES, FINANCIAL POSITION, RESULTS AND DIVIDENDS

12.1 Financial Statement

The information explicitly listed in the table below has been incorporated by reference into this Prospectus pursuant to Article 19 of the Prospectus Regulation. Non-incorporated parts of the documents incorporated by reference are either not relevant for the investor or covered elsewhere in this Prospectus. Direct and indirect references in the documents included in the table below to other documents or websites are not incorporated by reference and do not form part of this Prospectus. The documents speak only for the period in which they are in effect and have not been updated for purposes of this Prospectus. Potential investors should assume that the information in this Prospectus as well as the information incorporated by reference herein is accurate only in the period in which they are in effect.

The information incorporated by reference into this Prospectus is exclusively set out in the cross reference table below, and is available on the Group's website www.Orphazyme.com.

Document/information:

FY2019 Group Financial Statements
Published at February 28, 2020
Management statement, page 88
Independent auditor's report, pages 89-92
Consolidated financial statement including notes, pages 41-87

The tables set out below comprise key financial information pertaining to the Group and has been derived from the FY2019 Group Financial Statements prepared in accordance with IFRS as adopted by the EU and additional disclosure requirements of the Danish Financial Statements Act:

Income statement	**Year ended December 31**	
	2019	**2018**
	(DKK thousand)	
Total revenue	-	-
Operating profit/loss	(335,954)	(231,652)
Net profit/loss	(337,497)	(229,600)
Total comprehensive income/loss	(337,430	(229,558)

Balance sheet	**As at December 31**	
	2019	**2018**
	(DKK thousand)	
Total assets	180,754	441,349
Total equity and liabilities	180,754	441,349

Cash flow statement	**Year ended December 31**

	2019	2018
	(DKK thousand)	
Cash flow from operating activities	(326,818)	(234,764)
Cash flow from investing activities	(3,285)	(2,346)
Cash flow from financing activities	58,939	-
Net cash flows	(271,164)	(237,110)
Cash	123,588	394,706

12.2 Auditing of financial statement

The audit report for the FY2019 Group Financial Statements is included in this Prospectus by reference.

See "*Company information–Information on assets and liabilities, financial position, results and dividends–Financial statement*".

12.3 Legal and arbitration proceedings

As at the Prospectus Date, the Company is not involved in any governmental, legal or arbitration proceedings, and Management is not aware of any such proceedings being threatened that could have a significant effect on the Company's or the Group's financial position or profitability, nor has the Company or the Group been involved in any such governmental, legal or arbitration proceedings during the previous 12 months as at the Prospectus Date.

12.4 Significant change to the Group's financial

Since the end of the period covered by the FY2019 Group Financial Statements, no significant change to the financial position of the Group has occurred.

12.5 Pro forma financial information

No pro forma financial information has been included in this Prospectus.

12.6 Dividend policy

The Company has not declared or made any dividend payments for the last financial year. Currently, the Company intends to use all available financial resources as well as revenue, if any, for purposes of the Company's current and future business. As of the date hereof, the Company does not expect to make dividend payments within the foreseeable future.

Any future determination related to our dividend policy and the declaration of any dividends will be made at the discretion of the Board of Directors and will depend on a number of factors, including the Company's results of operations, financial condition, future prospects, contractual restrictions, restrictions imposed by applicable law and other factors the Board of Directors deems relevant. There can be no assurances that the Group's performance will facilitate dividend payments, and, in particular, the Company's ability to pay dividends may be impaired if any of the risks described in this Prospectus were to occur. See "*Risk factors*".

The information on the Company's policies relating to dividend constitutes forward-looking statements. Forward-looking statements are not guarantees of future financial performance, and the Company's actual dividends could differ materially from those expressed or implied by such forward-looking statements as a result of many factors, including those described under "*General information–Forward-looking statements*" and "*Risk factors*".

The Board of Directors is not authorized to distribute extraordinary dividends.

13 ADDITIONAL INFORMATION

13.1 Share capital before and after the Listing

Prior to the Private Placement, the Company had 20,005,449 Existing Shares with a nominal value of DKK 1 each. As at the Prospectus Date, the Company's share capital had a nominal value of DKK 27,038,386 divided into 27,038,386 Shares with a nominal value of DKK 1 each or multiples thereof as determined by the Board of Directors. All Shares are issued and fully paid up.

Under the Company's LTIP, the Company has issued Matching Shares and Performance Shares, of which a total of up to 31,250 Matching Share and up to 211,700 Performance Shares are outstanding and unvested at the date of this Prospectus, entitling the holders of such Matching Shares and Performance Shares of the LTIP to subscribe for or acquire one shares per Matching Share and/or Performance Share at a price per share of DKK 1, subject to certain conditions for vesting. The Company may deliver Matching Shares and Performance Shares under its LTIP by a variety of means, including by way of delivering treasury shares or directed issues of shares and/or bonus shares. The Company expects to meet its obligations under the LTIP by delivering Matching Shares and Performance Shares to the participants of the LTIP through directed share issuances.

14 REGULATORY DISCLOSURES

During the last 12 months, the Company has announced the following inside information in accordance with Regulation (EU) No 596/2014 on market abuse ("**Market Abuse Regulation**"):

- Its directed issue and private placement of shares (See company announcement no. 08/2020 and no. 12/2020, dated February 6, 2020 and February 7, 2020)

- Preliminary results for 2019 and financial outlook for 2020 (See company announcement no. 07/2020, dated February 4, 2020)

- its receipt of Fast Track designation for arimoclomol in sporadic Inclusion Body Myositis (sIBM) (See company announcement no. 31/2019, dated December 18, 2019);

- (a) its positive results from (i) full data set of Phase II/III arimoclomol trial in Niemann-Pick disease Type C ("**NPC**") (See company announcement no. 01/2019, January 30, 2019) and (ii) open-label Phase II/III extension in NPC (See company announcement no. 01/2020, dated January 3, 2020); (b) receipt of Breakthrough Therapy Designation for arimoclomol in NPC (See company announcement no. 29/2019, dated November 19, 2019) and (c) the availability of an Early Access Program in the United States for its investigational drug arimoclomol for the treatment of NPC (See company announcement no. 03/2020, dated January 6, 2020);

- preparation for filing of the drug, arimoclomol as a treatment for NPC in Europe (See company announcement no. 15 dated June 7, 2019) and in the United States (See company announcement no. 18/2019, dated July 21, 2019);

- its full enrollment in Phase II/III trial in sIBM (See company announcement no. 13/2019, dated April 23, 2019) and its completion of enrollment of phase 3 trial evaluating arimoclomol in ALS (See company announcement no.17/2019, dated July 18, 2019);

- its appointment of Kim Stratton as Chief Executive Officer (See company announcement no. 16/2019, dated July 15, 2019); and

- its financing from Kreos Capital, which strengthens its balance sheet with EUR 9 million (See company announcement no. 25/2019, dated August 27, 2019).

In addition, the Company disclosed certain transactions with persons discharging managerial responsibilities in the Company in accordance with Article 19 of Market Abuse Regulation, including (i) grants of restricted share-units and vesting of Matching Shares under the Company's LTIP and Board Incentive Program, (ii) acquisitions of shares by a closely associated person to a person discharging

managerial responsibilities in the Company and (iii) acquisitions of shares by a person discharging managerial responsibility.

15 MATERIAL CONTRACTS

Material agreements

This section contains brief summaries of (i) material agreements, other than agreements entered into in the ordinary course of business, to which the Company or a company of the Group is a party, for the two years immediately preceding publication of this Prospectus and of (ii) other agreements (not being agreements entered into in the ordinary course of business) entered into by a company of the Group which contain provisions under which a company of the Group has an obligation or entitlement which is material to the Group as at the Prospectus Date.

Asset purchase agreement with CytRx

In May 2011, the Company entered into an asset purchase agreement with the U.S. biopharmaceutical company CytRx. Pursuant to this agreement, CytRx irrevocably sold and transferred certain preclinical and clinical data, intellectual property rights and other assets, including contractual rights and obligations relating to a portfolio of chemical compounds, including arimoclomol, to the Company.

Under the terms of the asset purchase agreement, the Company made an up-front cash payment of USD 150,000 and further agreed to make future payments to CytRx contingent upon the achievement of specified clinical/regulatory and sales milestones as well as royalty payments based on a specified percentage of any eventual net sales of products containing one of the compounds purchased as summarized further below.

Clinical/regulatory milestone payment obligations (non-ALS or stroke products)

The Company has agreed to pay CytRx clinical and regulatory milestone payments for the first two products being developed for indications other than for the treatment or prevention of amyotrophic lateral sclerosis or stroke (non-ALS or stroke products). Payments are triggered upon achieving certain key clinical or regulatory milestones. The aggregate amount of milestone payments that may be triggered is USD 12.1 million for the first non-ALS or stroke product and USD 10.3 million for the second non-ALS or stroke product developed assuming (for both products) approval in the EU (or a major European market), the United States and Japan. A second non-ALS or stroke product is not calculated as a second product (and hence does not trigger milestone payments) unless it contains a different compound than the first non-ALS or stroke product. In 2016, the Company paid CytRx USD 0.1 million for achievement of a clinical milestone for the first product.

Clinical/regulatory milestone payment obligations (ALS or stroke products)

The Company has also agreed to pay CytRx clinical and regulatory milestone payments (payable one time only) for each product developed that is being developed or labelled for the treatment or prevention of amyotrophic lateral sclerosis or stroke (ALS or stroke products). Payments are triggered upon achieving certain key clinical or regulatory milestones. The aggregate amount of milestone payments that may be triggered per ALS or stroke product is USD 23.8 million assuming approval in the EU (or a major European market), the United States and Japan. The milestone obligations are payable only once per ALS or stroke product. A subsequent ALS or stroke product may only achieve a milestone and trigger a payment obligation, if it contains a different compound than an ALS or stroke product previously achieving the same milestone or if it contains the same compound as another ALS or stroke product previously achieving same milestone but is for a different indication. Under the terms of the asset purchase agreement, the Company was assigned and hence became the party to a royalty agreement with the ALS Charitable Remainder Trust pursuant to which the Company is obliged to pay a 1% royalty to the ALS Charitable Remainder Trust on global net sales of products to treat ALS.

Sales milestones. The Company also agreed to pay CytRx milestone payments upon reaching certain aggregated global net sales of all products developed by the Company containing any of the compounds purchased from CytRx. The first milestone payment is triggered on aggregate net sales exceeding USD 100 million. The aggregate milestone payment obligations may be up to USD 50 million assuming global net sales in excess of USD 1 billion.

Royalties. The Company has agreed to pay CytRx a low teens double-digit royalty on net sales of ALS or stroke products and a mid-single digit royalty on net sales of all other products developed by the Company or its licensees containing any of the compounds purchased from CytRx. Royalties accrue on a country-by-country and product-by-product basis until the latest of expiration of relevant patent claims in the country, expiry of regulatory exclusivity in the country or ten years from the date of the approval of the product in the country; provided, however, that the royalty rate may be reduced on a country-by-country and product-by-product basis by 20% for the remainder of the royalty term on expiration of the relevant patent claims and the expiration of regulatory exclusivity; and by 40% in the event that there are no valid patent claims or any regulatory exclusivity at the time of first commercial sale in the country.

The Company has no contractual obligations to CytRx to develop or commercialize any products under the terms of the asset purchase agreement and the Company cannot be held liable towards CytRx for the Company's failure to do so.

Exclusive license agreement with University of Miami

In September 2019, the Company entered into an exclusive license agreement with the University of Miami. Pursuant to the exclusive license agreement, the Company have been granted a global royalty bearing, exclusive license to data, know-how and patent rights generated by the University of Miami in a phase II clinical trial of arimoclomol for the treatment of ALS with the a4V SOD1 mutation to use or apply the study data. The Company has also been granted internal development use rights to the data, know-how and patent rights.

Under the terms of the exclusive license agreement, the Company made an up-front cash payment of USD 75,000 and further agreed to make future payments of certain license fees, a development milestone payment upon receiving regulatory approval for ALS and annual fees as well as a royalty of 0.75% of net sales of products sold within ALS linked to mutations in the SOD1 gene. Any annual fees will be creditable against royalty and milestone payments.

License agreement with University of Kansas and UCL Business PLC

In October 2017, the Company entered into a license agreement with the University of Kansas and UCL Business PLC (a wholly-owned subsidiary of University College London). The license agreement grants the Company the global, royalty bearing exclusive license to develop and commercialize products under all data generated in the course of the ongoing phase II/III clinical trial on arimoclomol for the treatment of sIBM. The Company's license includes any inventions and know-how included in such data. The trial was initiated in August 2017 with the University of Kansas as sponsor and supported by an FDA grant. Sponsorship of the trial was transferred to the Company in December 2017.

Under the terms of the license agreement, the Company is obliged to pay an aggregate royalty of 2% of net sales of products sold for the treatment of sIBM. The Company is required to use commercially diligent efforts to develop and commercialize such products. The license agreement also provides that, in consideration of the license, the Company is obliged to issue bonus shares in favor of the University of Kansas and UCL Business PLC, for up to an aggregate value of USD 2.5 million (around DKK 15.8 million) depending on the size of the grants awarded to the universities under the trial (with a price per Share calculated based on the average closing price of the Shares on Nasdaq Copenhagen for the 30 days immediately prior to the date of issuance). The Shares are required to be issued or delivered on a yearly basis subject to certain reporting requirements. To date, 58,090 bonus shares have been issued.

Agreements related to manufacture and clinical studies of arimoclomol

In order to support a cost-effective development model that allows internalization of the relevant expertise at the appropriate time during the development process, the Company engages with third party CDMOs to manufacture, store and distribute the Company's products for clinical trials. These third-party CDMOs include, in particular, a cGMP manufacturer of the active ingredient of arimoclomol and on a quote-by-quote basis, a provider of packaging, storage and distribution services in relation to the Company's clinical trials. The manufacturing is done at an FDA inspected U.S. facility. The cGMP manufacturer also manages quality control, release and warehousing. The Company also engages with third-party CROs to facilitate and assist in its clinical trials.

In 2013, the Company entered into a master service agreement with a UK-based CRO. Under the terms of the master service agreement, the CRO agreed to provide various services to support the Company's clinical testing of arimoclomol for the treatment of NPC. Contracted services comprise trial set-up and monitoring, trial and data management, statistical analytical work as well as preparation of final study report. As part of the study set-up, the Company is co-signing as sponsor on the clinical trial agreements entered into with the individual trial sites. Under the terms of the master service agreement, all intellectual property rights pertaining to arimoclomol, including possible patents based on data provided from inventions made during the trial, are owned by the Company. The Company has agreed to indemnify the CRO for third-party claims arising from the performance of the trial or from the use of arimoclomol.

The Company has subsequently entered into similar master service agreements for clinical trial services with three additional CROs, to support the Company's dose-ranging phase II trial of arimoclomol for the treatment of Gaucher disease, our phase III clinical trial of arimoclomol in ALS and our phase II/III clinical trial of arimoclomol for the treatment of sIBM.

Loan facility agreement with Kreos Capital VI (UK) Limited

On August 27, 2019, the Company entered into an up to EUR 18,000,000 loan facility agreement with Kreos Capital VI (UK) Limited as lender (the "**Facility Agreement**") for the purpose of financing the Company's general working capital requirements. The Company has utilized the first tranche of the loan in an amount of EUR 9,000,000. Utilization of the second tranche of the loan is subject to the Company having received a minimum of USD 20,000,000 (or its equivalent in DKK) by way of new equity financing. The term of the Facility Agreement is 42 months. Interest accrues on the loans utilized under the Facility Agreement at an annual fixed rate of 9.75%. Usual representations and warranties are included in the Facility Agreement, and the Group is subject to general undertakings such as restrictions on distributions of dividends, financial indebtedness, security and disposals outside its ordinary course of business. Subject to those general undertakings, no financial covenants are included in the Facility Agreement. Certain security interests are granted by the Group in favor of Kreos Capital VI (UK) Limited as security for the obligations secured under the Facility Agreement. The Company has agreed to pay fees to Kreos Capital VI (UK) Limited in connection with this transaction, which includes an end of loan payment fee and a facilitation fee in an amount equal to the greater of (i) 10% of the aggregate amount of the amount borrowed and (ii) the percentage increase in the Company's share price between the 30-day volume-weighted average share price on the date of the Facility Agreement and the closing share price on the day immediately preceding the date of the notification applied to 10% of the aggregate amount of amounts borrowed.

Other agreements

Save as disclosed above, there are no agreements (other than entered into in the ordinary course of business) to which the Company is a party which (i) are, or may be, material to the Company and which have been entered into in the two years immediately preceding the date of this Prospectus; or (ii) contain any obligations or entitlements which are, or may be, material to the Company as of the date of this Prospectus.

16 DOCUMENTS AVAILABLE

For the term of this Prospectus, the following documents are available for inspection at the Company's head office:

- The Company's Memorandum of Association and Articles of Association.
- The FY2019 Group Financial Statements.
- The Prospectus related to the Listing.

Any request for copies of the Prospectus may be made to:

Orphazyme A/S, CVR-no. 32 26 63 55

Ole Maaløes Vej 3

DK-2200 Copenhagen

Tel: (+45) 39 17 82 72 contact@orphazyme.com

E-mail: contact@orphazyme.com

Subject to certain exceptions, the Memorandum of Association, Articles of Association, the FY2019 Group Financial Statements and the Prospectus can also be downloaded from the Company's website: www.orphazyme.com. Except for the information incorporated herein by reference, the contents of the website do not form part of the Prospectus.

The purpose of this Prospectus is solely to have the Listing Shares admitted to trading and official listing on Nasdaq Copenhagen. No issue or offering of Shares is made by the Company in connection with the publication of the Prospectus. This Prospectus will not be and may not be distributed or otherwise made available in any jurisdiction (other than any publication of this Prospectus in accordance with Danish law, rules and regulations) and the Shares may not be offered or sold, directly or indirectly, in the United States, Canada, Australia or Japan or in any other jurisdiction. The Company makes no offer or solicitation to any person under any circumstances that may be unlawful. Persons into whose possession this Prospectus may come are required to inform themselves about and to observe such restrictions.

THE LISTING

1 PERSONS RESPONSIBLE, THIRD PARTY INFORMATION, EXPERTS' REPORTS AND COMPETENT AUTHORITY APPROVAL

1.1 Persons responsible and approval from competent authority

See "*Responsibility statement*" for more details.

1.2 Experts' reports and third party information

This Prospectus does not contain any expert statements or expert reports.

For details on information sourced from third parties, see "*General information–Third party information*".

2 RISK FACTORS RELATED TO THE LISTING

See "*Risk Factors*" for more details.

3 KEY INFORMATION ON CAPITALIZATION AND BACKGROUND OF THE LISTING

3.1 Interest of natural and legal persons involved in the Listing

Management is not aware of any potential conflicts of interest in relation to the Listing that would be material to the Company.

3.2 Background for the Listing

The Listing Shares were issued through VP Securities and registered with the Danish Business Authority on February 11, 2020. The Listing Shares were issued in connection with a Stock Lending and Subscription Agreement entered into on February 6, 2020 among the Company, Danske Bank A/S and the Lending Shareholders pursuant to which the Company borrowed 3,071,673 existing shares (the "**Lending Shares**") from the Lending Shareholders through Danske Bank A/S as settlement agent in order for the Company to place such Lending Shares in a private placement (the "**Private Placement**"). The Lending Shares were borrowed subject to an obligation for the Company to issue new shares of an equivalent number as the Lending Shares placed in the Private Placement, such new shares being the Listing Shares, and for Danske Bank A/S to use the proceeds from the sale of Lending Shares in the Private Placement to subscribe for the Listing Shares and deliver the Listing Shares to the Lending Shareholders. The Listing Shares were issued in the temporary ISIN code, DK0061274362, and delivered to the Lending Shareholders on February 11, 2020.

The Listing Shares issued in connection with the Private Placement are initially issued under the temporary ISIN code DK0061274362, which is not listed on Nasdaq Copenhagen.

The purpose of this Prospectus is to have the Listing Shares admitted to trading and official listing on Nasdaq Copenhagen.

The Listing Shares are expected to be admitted for trading and official listing on Nasdaq Copenhagen on or around March 17, 2020under the existing ISIN code of the Existing Shares DK0060910917.

3.3 Working capital statement

In the opinion of the Company, the working capital available as of the date of this Prospectus is sufficient for its present working capital needs for the 12 months following the date of this Prospectus.

3.4 Capitalization and indebtedness

The following table sets forth the capitalization, indebtedness (distinguishing between guaranteed and unguaranteed, secured and unsecured) and cash, cash equivalents, and securities of the Company as of February 29, 2020 on an actual basis reflecting the carrying amounts on the balance sheet of the Company.

See "*Information concerning the Listing Shares*" for information relating to the Company's issued share capital and number of outstanding Shares. You should read this table in conjunction with the Company's FY2019 Group Financial Statements.

	As of February 29, 2020
	DKK million
Cash	768.1
Total cash	**768.1**
Bank debt	12.8
Lease liabilities	2.9
Trade payables	45.5
Other payables	18.4
Current financial debt	**79.7**
Of which is secured	12.8
Of which is guaranteed	0
Of which is unsecured/unguaranteed	66.9
Bank debt	51.6
Lease liabilities	9.8
Other non-current financial debt	0.4
Non-current financial debt	**61.8**
Of which is secured	51.6
Of which is guaranteed	0
Of which is unsecured/unguaranteed	10.2
Total financial indebtedness	**141.5**
Net financial indebtedness	**(626.6)**
Share capital	27.0
Share premium	1,613.4
Accumulated deficit	(952.8)
Total equity	**687.6**

Other than as specifically set out above, all of the Company's interest-bearing liabilities are unsecured and unguaranteed.

The Company may need additional capital in the future and may seek to obtain further financing through raising new equity capital or debt financing.

The Company has no reason to believe that there has been any material change to its actual capitalization since February 29, 2020, other than changes resulting from the ordinary course of business.

4 INFORMATION CONCERNING THE LISTING SHARES

4.1 Type of security and ISIN codes

The Company's Existing Shares are admitted to trading and official listing on Nasdaq Copenhagen under ISIN code DK0060910917.

The Listing Shares are issued under the temporary ISIN code DK0061274362. The Listing Shares will not be admitted to trading and official listing on Nasdaq Copenhagen under the temporary ISIN code. The Listing Shares under the temporary ISIN code will solely be registered with VP Securities.

Upon completion of the Listing, expectedly on or around March 17, 2020, the temporary ISIN code of the Listing Shares will be merged with the ISIN code of the Existing Shares, and the Listing Shares will be admitted to trading and official listing on Nasdaq Copenhagen under the permanent ISIN code for the Existing Shares DK0060910917.

4.2 Applicable law and jurisdiction

This Prospectus has been prepared in accordance with Danish legislation and regulations applicable in Denmark, including the Danish Capital Markets Act, the Prospectus Regulation, Commission Delegated Regulation (EU) no. 2019/980 of March 14, 2019 as well as Commission Delegated Regulation (EU) 2019/979 of March 14, 2019, and the Nasdaq Issuer Rules. Any dispute which may arise as a result of the Listing shall be brought before the Danish courts of law.

4.3 Registration

The Listing Shares were registered with the Danish Business Authority, issued in VP Securities and delivered to the Lending Shareholders through Danske Bank A/S on February 11, 2020.

4.4 Currency

The Listing Shares are denominated in DKK.

4.5 Rights attached to the Listing Shares

The Listing Shares are registered with the Danish Business Authority and have the same rights as the Existing Shares.

4.6 Resolutions, authorizations and approvals

The issuance of the Listing Shares took place pursuant to an authorization granted to the Board of Directors at the Company's general meeting on January 25, 2020. At the meeting, the Board of Directors was authorized to increase the Company's share capital in one or more issues of new shares without pre-emption rights for the Company's existing shareholders by up to a nominal amount of DKK 8,000,000 from the date of the meeting until January 25, 2025. The capital increase took place at market price as determined by the Board of Directors through a book-building process and shall be effected by cash payment. The authorization was adopted as an amendment to Article 3.4 of the articles of association.

The Listing Shares issued in connection with the completion of the Private Placement is expected to be admitted for trading and official listing on Nasdaq Copenhagen under the existing ISIN code on or around March 17, 2020.

4.7 Issue Date of the Listing Shares

The Listing Shares were registered with the Danish Business Authority on February 11, 2020 and issued through VP Securities the same day.

4.8 Negotiability and transferability of Listing Shares

The Listing Shares are negotiable instruments under Danish law and there are no restrictions with respect to the transferability of the Listing Shares. General transfer restrictions in connection with the Listing Shares are described in *"-Terms and conditions of the Listing"*. The articles of association of the Company do not contain obligations on shareholders to have their shares redeemed.

4.9 Taxation

Introduction

Please find below a summary of certain Danish key tax aspects for Danish and foreign investors in relation to acquisition, ownership and disposal of Shares in the Company. The purpose and focus of this summary is solely to outline general information and, as such, the summary should not in any way serve as – and does not purport to constitute – tax or legal advice.

The summary is not an exhaustive description of all tax matters and aspects that may be relevant in relation to the acquisition, ownership or disposal of Shares in the Company.

The summary only includes a description of the Danish taxation of dividends and gains and losses on shares admitted to trading on a regulated market. It is assumed that the relevant shareholder is the beneficial owner of the Shares, and in respect of non-Danish shareholders that the Shares are not attributed to a Danish permanent establishment of the non-Danish shareholder.

The tax legislation applicable to investors may impact the income received from the Shares, investors should take advice from their own tax advisers in relation to identifying the tax consequences of a potential acquisition, ownership or disposal of Shares in the Company based on their specific conditions, including the consequences of state, local or other national tax legislation, if any.

This summary does not include a description of the tax consequences for investors who qualify as traders in securities for tax purposes or for professional investors, such as pension companies etc. Further, the summary does not address situations where Shares are acquired on the basis of subscription rights granted as part of an employment. This summary is based on current legislation, judicial decisions and rulings in Denmark as at the Prospectus Date, all of which may be amended, in some cases with retroactive effect.

Taxation of shareholders resident in Denmark for tax purposes

Individuals who are residents of Denmark, or who stays in Denmark for at least six months only interrupted by short stays abroad due to holidays etc., and companies etc. registered in Denmark, or whose effective seat of management is in Denmark, are normally fully liable to pay tax in Denmark. Further, the income of foreign companies controlled from Denmark having income mainly of a financial nature may be taxable in Denmark. The income of foreign companies will generally also be subject to Danish tax if a Danish affiliated company has opted for international joint taxation under Danish tax rules. In case the individual or company is also fully liable to pay tax in another country, specific rules not mentioned in this summary may apply.

Taxation of dividends

Individuals, in general

For individuals, dividends are taxed as share income. In the income year 2020, a tax rate of 27% must be paid on the annual share income up to DKK 55,300 (DKK 110,600 for married couples cohabiting at the end of the income year) and 42% of the annual share income exceeding DKK 55,300 (DKK 110,600 for married couples cohabiting at the end of the income year).

The thresholds are adjusted annually and include all share income of the individual / couple concerned during the year.

In case of dividend payments, 27% dividend tax is normally withheld by the company.

Special rules apply to individuals' investment of pension savings. See "*–Capital gains taxation*" for a description of the tax treatment of investment of pension savings.

Companies etc.

In general, a company holding shares in another company admitted to trading on a regulated market is liable for tax on dividends received on the shares. The dividends are taxable at a tax rate of 22%, which is withheld by the company distributing the dividends in connection with the payment of dividends.

Regardless of ownership period, companies may receive tax-exempt dividends in case the shares are subsidiary shares or group company shares. See "*–Capital gains taxation*" regarding the definition of subsidiary shares and group company shares.

Capital gains taxation

Individuals, in general

Capital gains realized on shares are taxed as share income. In the income year 2020, 27% tax must be paid on the annual share income up to DKK 55,300 (DKK 110,600 for married couples cohabiting at the end of the income year) and 42% of the annual share income exceeding DKK 55,300 (DKK 110,600 for married couples cohabiting at the end of the income year). The maximum amounts allowed are adjusted annually and include all share income of the individual/couple concerned during the year.

In case of loss on shares admitted to trading on a regulated market, the loss may be offset against taxable income (capital gains and dividends) from other shares admitted to trading on a regulated market. If the individual is married and the total loss on shares admitted to trading on a regulated market exceeds the individual's capital gains and dividends realized on other shares admitted to trading on a regulated market, the remaining loss is offset against the spouse's share income pursuant to similar rules provided that the spouses are cohabiting at the end of the income year. In case there are still unutilized losses, these may be carried forward indefinitely to be offset against future income from similar shares.

It is a condition for offsetting losses on shares admitted to trading on a regulated market that the Danish tax authorities have received information on the identity of the shares, the quantity, the acquisition date, and the acquisition price before expiry of the deadline for filing the tax return for the income year in which the shares were acquired. The information is generally provided to the Danish tax authorities automatically when the shares are placed in a custody account with a Danish financial institution.

Capital gains and losses are calculated pursuant to the average cost formula according to which the acquisition price of each specific share is calculated as a proportionate part of the total acquisition price for the shareholder's total number of shares in the issuing company.

Individuals, investment of pension savings

Within certain limits, investors have the possibility of placing pension savings in shares having the effect that the net profit will be subject to the Danish Pension Returns Tax Act. The net profit is calculated as the annual realized and unrealized capital gains and losses and added any other profits (such as dividend etc.). The annual net profit is taxed at a rate of 15.3%. Pension return tax is normally settled by the pension company. A transfer from a pension savings custody account to the individual's ordinary custody account is considered a disposal and must be made at market value.

Companies etc.

Irrespective of the period of ownership, companies are liable for tax on capital gains and losses on shares admitted to trading on a regulated market except in case of subsidiary shares and group company shares. The annual realized and unrealized capital gains are taxed pursuant to the mark-to-market principle and is included in the statement of taxable income. Losses calculated pursuant to the mark-to-market principle may be deducted in the statement of taxable income, including in other corporate income. The taxable income is taxed at a rate of 22%.

Capital gains and losses incurred in connection with the sale of group company shares and subsidiary shares are not included in the statement of taxable income of companies. "Subsidiary shares" is generally

defined as shares owned by a company holding at least 10% of the share capital of the company issuing the shares. "Group company shares" is generally defined as shares owned by a company, which is jointly taxed (pursuant to section 31 of the Danish Corporation Tax Act) with the company in which shares are owned or which may be internationally jointly taxed (pursuant to section 31 of the Danish Corporation Tax Act) with the company in which shares are owned.

For tax purposes, the transition from subsidiary share status and group company share status to portfolio share status and vice versa is treated as a disposal of shares and acquisition at market value at the time of the transition of status.

Special anti-avoidance rules may apply to prevent, e.g., that shareholdings are pooled in an intermediary holding company in order to avoid taxation of dividends and capital gains. These rules are not further described in this summary.

Danish taxation of investors not fully liable to pay tax in Denmark

Taxation of dividends

Individuals

As a main rule, individuals who are not Danish tax residents are subject to a 27% withholding tax on dividends from Danish companies.

However, it is possible to apply for partial reimbursement of Danish withholding tax if the individual (i) is entitled to a reduction of the tax under a double taxation treaty concluded between Denmark and the tax jurisdiction in which the shareholder is resident; or (ii) holds less than 10% of the Danish company and the competent authority in the state, or in Greenland or in the Faroe Islands, where the person is resident is required to exchange information with the Danish tax authorities according to a double taxation treaty, another international agreement or an administrative agreement of assistance in tax issues. If the shareholder is resident in a country outside the EU, it is also a condition that the shareholder, together with related parties, holds less than 10% of the Danish company. The amount of the reimbursement in question (i) depends on the provisions of the specific double taxation treaty whereas the final withholding tax rate (which also determines the amount of reimbursement) and in situation (ii) constitutes 15%.

Regardless of whether the (final) taxation is reduced as described above, the Danish dividend-distributing company is, as a main rule, obliged to withhold 27% dividend tax. Consequently, the said foreign shareholders subject to a reduced taxation need to file an online application to the Danish tax authorities for the repayment of the excess amount of withholding tax.

Companies etc.

As a main rule, companies who are not Danish tax residents are subject to a 27% withholding tax on dividends from Danish companies.

In general, a foreign company may, however, always apply for partial reimbursement of Danish withholding tax down to 22%.

Moreover, companies may apply for reimbursement if the shareholder (i) is entitled to a reduction of tax under the double taxation treaty concluded between Denmark and the tax jurisdiction in which the shareholder is resident; or (ii) holds less than 10% of the Danish company and the competent authority in the state, or in Greenland or in the Faroe Islands, where the person is resident is required to exchange information with the Danish tax authorities according to a double taxation treaty, another international agreement or an administrative agreement of assistance in tax issues. If the shareholder is resident in a country outside the EU, it is also a condition that the shareholder, together with related parties, holds less than 10% of the Danish company. The amount of the reimbursement in question (i) depends on the provisions of the specific double taxation treaty whereas the final withholding tax rate (which also determines the amount of reimbursement) and in situation (ii) constitutes 15%.

Regardless of whether the (final) taxation is reduced as described above, the Danish dividend-distributing company is, as a main rule, obliged to withhold 27% dividend tax. Consequently, the said foreign shareholders subject to reduced taxation need to file an online application with the Danish tax authorities for the repayment of the excess amount of withholding tax.

A foreign company is exempt from withholding tax on dividends received from a Danish company if the foreign company:

a) receives dividends on shares in subsidiaries and may rely on either reduction or elimination of Danish dividend tax according to the EU Parent-Subsidiary Directive (Directive 2011/96/EU as amended) or according to a double taxation convention between the foreign company's tax jurisdiction and Denmark; or

b) receives dividends on shares in group companies, which are not shares in subsidiaries, when (i) the company receiving the dividends is resident in an EU/EEA member state; and (ii) the taxation of dividends should be waived or reduced according to the provisions of the EU Parent-Subsidiary Directive or a double taxation convention between the foreign company's tax jurisdiction and Denmark if the shares had qualified as shares in subsidiaries.

Accordingly, dividend tax will not be withheld in the two above cases.

Dividends (announced alterations)

The Danish Government has announced that it is contemplating to introduce a new model for taxation of dividends distributed on shares admitted to trading on a regulated market. This has the consequence that dividends will be taxed on the distribution date at a final tax rate based on each shareholder's specific conditions. Therefore, information on the shareholders (beneficial owner pursuant to Danish legislation) must be provided before the actual distribution in order for the dividend-distributing companies to be able to calculate and withhold the correct amount of dividend tax from each shareholder. The purpose of this new model is to eliminate fraud and facilitate the process for the tax authorities of verifying that dividend tax is not reimbursed wrongfully.

The details of the proposed new model have not yet been introduced or announced. Neither has the expected date of implementation of this new model been announced.

Capital gains taxation

Individuals

As a main rule, individuals who are not Danish tax residents are not liable to pay tax in Denmark on capital gains on the sale of shares in Danish companies.

However, capital gains and losses on shares in Danish companies are taxable in Denmark pursuant to the same rules that apply to individuals resident in Denmark in case the shares are attributable to a permanent establishment in Denmark.

Special rules apply to distributions in connection with capital reductions or the resale of shares to the issuing company.

Companies etc.

As a main rule, companies who are not Danish tax residents are not liable to pay tax in Denmark on capital gains on the sale of shares in Danish companies.

Capital gains and losses on shares in Danish companies are taxable in Denmark pursuant to the same rules that apply to corporate investors resident in Denmark in case the shares are attributable to a permanent establishment in Denmark.

Special rules apply to distributions in connection with capital reductions or the resale of shares to the issuing company as well as sale of shares to a group company.

Share transfer duty

There is no share transfer duty in Denmark.

4.10 Rights attaching to the Listing Shares

Dividend rights

The Listing Shares have the same rights as the Existing Shares, including with respect to eligibility for any dividends.

Any dividends will be paid in DKK to the shareholder's account with VP Securities. No restrictions on dividends or special procedures apply to holders of Listing Shares who are not residing in Denmark. See "*The Listing–Information regarding the Listing Shares–Taxation*" for a description of the treatment of dividends under Danish tax law.

Dividends which have not been claimed by shareholders within three years from the time they are payable will be forfeited and will accrue to the Company.

Voting rights and pre-emption rights

All Shares in the Company will rank *pari passu*, including with respect to voting rights and pre-emption rights. All Shares will then carry 1 vote per Share of a nominal value of DKK 1.

Liquidation rights

In case of the dissolution or winding-up of the Company, the Listing Shares will be entitled to a proportionate part of the Company's assets after payment of the Company's creditors. The Articles of Association do not contain any provisions on redemption or exchange of Shares.

4.11 Danish legislation concerning tender offers, redemption of shares and disclosure of shareholdings

Mandatory takeover bids

Applicable rules on mandatory takeover bids as set out in part 8 of the Danish Capital Markets Act and the executive order issued pursuant thereto.

If a shareholding is transferred, directly or indirectly, to an acquirer or to persons acting in concert with such acquirer, the acquirer must enable all shareholders of the company to dispose of their shares on identical terms if such transfer involves the acquirer obtaining control of the company.

An acquirer has control of the company when the acquirer or persons acting in concert with such acquirer directly or indirectly holds at least one-third of the voting rights in a company, unless it can be proven – under special circumstances – that such ownership does not constitute control. An acquirer who does not hold more than one-third of the voting rights in a company will, nevertheless, have control if the acquirer or person acting in concert with such acquirer has the right to control at least one-third of the voting rights of a company by virtue of an agreement or has the right to appoint or dismiss the majority of the members of a company's central governing body.

If special conditions apply, the Danish Financial Supervisory Authority may grant an exemption from the obligation to make a mandatory offer.

Squeeze-out

Pursuant to section 70 of the Danish Companies Act, shares in a company may be redeemed in whole or in part by a shareholder holding more than nine-tenths of the shares and the corresponding voting rights in the company.

Further, pursuant to section 73 of the Danish Companies Act, a minority shareholder may require that a majority shareholder holding more than nine-tenths of the shares and the corresponding voting rights redeem the minority shareholder's shares.

Major shareholdings

Pursuant to section 38 of the Danish Capital Markets Act, a shareholder of a company whose shares or financial instruments are admitted to trading on a regulated market within the EU is required to notify the listed company and the Danish Financial Supervisory Authority as soon as possible if the shareholder's shareholding directly or indirectly represents 5% or more of the voting rights or the share capital, and if the shareholders' shareholding directly or indirectly entails that the 5%, 10%, 15%, 20%, 25%, 50% or 90% thresholds and the thresholds of one-third or two-thirds of the voting rights or the share capital are reached or no longer reached.

The notification must comply with the requirements for the contents thereof set out in sections 15 and 16 of the Danish Executive Order on Major Shareholders, including the identity of the shareholder and the date when the threshold is reached or no longer reached. Failure to comply with the disclosure requirements is punishable by a fine. When the Company has received such notification, it must publish the contents of such notification as soon as possible.

Further, the general duty of notification under the Danish Capital Markets Act applies as well as special duties of notification in respect of the Company's insider group pursuant to Regulation (EU) No. 596/2014 on market abuse.

4.12 Public takeover bids for the Company

No takeover bids have been made by any third party in respect of the Shares during the past or the current financial years.

5 TERMS AND CONDITIONS OF THE LISTING

5.1 Terms, expected timetable and restrictions

The Listing Shares are registered under the temporary ISIN code DK0061274362 which will not be admitted for trading and official listing on Nasdaq Copenhagen and such temporary ISIN code will subsequently be merged with the existing ISIN code for the Existing Shares DK0060910917 in VP Securities. Upon completion of the Listing, the Listing Shares will be admitted to trading and official listing under the ISIN code of the Existing Shares, which is expected to take place on or around March 17, 2020.

Expected timetable of principal events:

Publication of Prospectus	March 16, 2020
Admission of the Listing Shares for trading and official listing under the existing ISIN code	On or around March 17, 2020

Restrictions applicable to the Listing

General restrictions

The Listing consists of an admission to trading and official listing of the Listing Shares on Nasdaq Copenhagen. The Listing does not comprise an offer or placement of Shares in any jurisdiction.

The Listing is made pursuant to Danish law, and the Company has not taken any action or will not take any action in any jurisdiction, which may result in a public offering of the Listing Shares.

The Listing does not comprise an offer of, an invitation to purchase or subscribe for or a placement of Listing Shares in any jurisdiction and this Prospectus may not be used in connection with any offer of Shares or solicitation by anyone in any jurisdiction. Persons into whose possession this Prospectus may come must inform themselves of and observe all such restrictions. The Company accepts no liability for any violation of any such restrictions by any person. For a more detailed description of certain restrictions in connection with the Listing see selling restrictions outlined below.

Further, the Listing Shares are subject to transfer and selling restrictions in certain jurisdictions. Prospective investors of Shares must comply with all applicable rules and legislation in countries or territories in which they acquire, subscribe for, offer or sell Shares or possess or distribute this Prospectus and must obtain consent, approval or permission, as required, for the acquisition of Shares. Any person into whose possession this Prospectus may come is required by the Company to inform themselves about such restrictions and to observe such restrictions. Neither the Company nor the Company's auditors accepts any liability for any violation of these restrictions by any person, irrespective of whether such person is an Existing Shareholder or a potential purchaser of Shares.

This Prospectus will not be and may not be distributed or otherwise be made available in any jurisdiction (other than any publication of this Prospectus in accordance with Danish law, rules and regulations), and the Listing Shares may not be offered or sold, directly or indirectly, in the United States, Canada, Australia or Japan or in any other jurisdiction.

Selling restrictions in the United States

The Listing Shares have not been approved, disapproved or recommended by the U.S. Securities and Exchange Commission, any state securities commission in the United States or any other U.S. regulatory authority, nor have any of such regulatory authorities passed upon or endorsed the merits of the Listing or the accuracy or adequacy of this Prospectus. Any representation to the contrary is a criminal offence in the United States.

The Listing does not comprise an offer of, an invitation to purchase or subscribe for or a placement of Listing Shares in the United States. The Listing Shares are not, and will not be, registered under the U.S. Securities Act or any applicable state securities laws of the United States. The issuance of the Shares was made in transactions exempt from the registration requirements of the U.S. Securities Act pursuant to Section 4(a)(2) of the U.S. Securities Act, Regulation S, or another available exemption. The Shares may not be offered, pledged, resold, granted, delivered, allotted or otherwise transferred, as applicable, in the United States, except in transactions that are exempt from or not subject to the registration requirements under the U.S. Securities Act and in compliance with any applicable state securities laws.

Any person in the United States that obtains a copy of this Prospectus or any pre-printed issue statement or application form is required to disregard them.

Restrictions on sales in the European Economic Area

In relation to each member state of the European Economic Area where the Prospectus Regulation applies (each a "**Relevant Member State**"), no offering of Listing Shares will be made to the public in any Relevant Member State. Notwithstanding the above, if an offering had been made, no offering of Shares could be made to the public in any Relevant Member State prior to the publication of a prospectus concerning the Shares which has been approved by the competent authority in such Relevant Member State or, where relevant, approved in another Relevant Member State and notified to the competent authority in such Relevant Member State, all pursuant to the Prospectus Regulation, except that an

offering of Shares may be made to the public at any time in such Relevant Member State pursuant to the following exemptions from the Prospectus Regulation:

d) to any legal entity which is a qualified investor as defined in the Prospectus Regulation ("**Qualified Investor**");

e) to fewer than 150 natural or legal persons other than Qualified Investors, subject to obtaining the prior written consent of the Company; or

f) in any other circumstances falling within Article 1(4) of the Prospectus Regulation.

In any Relevant Member State, such offering would be only addressed to, and only directed at, investors in such Relevant Member State that fulfil the criteria for exemption from the obligation to publish a prospectus, including Qualified Investors.

For the purposes of the above, the expression an "offer of Shares to the public" in relation to the Listing Shares in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the Listing and the Listing Shares so as to enable an investor to decide whether to acquire or subscribe for the Listing Shares.

Notice to Investors in the UK

This Prospectus is not being distributed in the UK. If it had been distributed, it could only have been distributed to, and directed at, (i) persons outside the UK or (ii) "investment professionals" falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 (the "**Financial Promotion Order**") or (iii) "high net worth companies" and other persons to whom it may lawfully be communicated, falling within the meaning of Article 49(2)(a) to (d) of the Financial Promotion Order (all such persons being "**Relevant Persons**"). Therefore, if a prospectus would have been distributed in the UK, Shares are only available to Relevant Persons and any invitation, offer or agreement to subscribe for, purchase or otherwise acquire such Shares will be engaged in only with Relevant Persons. Any person who is not a Relevant Person should not act on or rely upon such prospectus or any of its contents.

Restrictions on sales in Canada, Australia and Japan and any other jurisdictions outside Denmark

The Listing Shares have not been approved, disapproved or recommended by any foreign regulatory authorities, nor have any of such authorities passed upon or endorsed the merits of the Listing or the accuracy or adequacy of this Prospectus.

This Prospectus may not be distributed or otherwise made available, the Listing Shares may not be offered, sold or subscribed for, directly or indirectly, in Canada, Australia or Japan.

5.2 Subscription ratio and allocation

Not applicable since there is no offering of securities for sale or subscription. The purpose of this Prospectus is solely to have the Listing Shares admitted to trading and official listing on Nasdaq Copenhagen.

5.3 Listing and proceeds

Not applicable since there is no offering of securities for sale or subscription. The purpose of this Prospectus is solely to have the Listing Shares admitted to trading and official listing on Nasdaq Copenhagen.

5.4 Plan of distribution

Not applicable since there is no offering of securities for sale or subscription. The purpose of this Prospectus is solely to have the Listing Shares admitted to trading and official listing on Nasdaq Copenhagen.

6 ADMISSION TO TRADING AND OFFICIAL LISTING

6.1 Admission to trading and official listing

Nasdaq Copenhagen has approved to admit the Listing Shares to trading and official listing subject to publication of the Prospectus. The Listing Shares are expected to be admitted for trading and official listing on Nasdaq Copenhagen on or around March 17, 2020 under the existing ISIN code DK0060910917.

6.2 Market making

The Company has not entered into any market making agreement.

6.3 Stabilization

No stabilization measures will be taken in connection with the Listing.

7 SELLING SHAREHOLDERS AND LOCK-UP

7.1 Shareholders who have indicated that they expect to sell their Shares

The Company has not received any indications from shareholders that they intend to sell their Shares. There is no offering of Shares for sale or subscription since the purpose of this Prospectus solely is to have the Listing Shares admitted to trading and official listing on Nasdaq Copenhagen.

7.2 Lock-up agreements with the Company

In connection with the Private Placement, the Company and each member of the Board of Directors and the executive management entered into certain lock-up agreements in relation to new shares, subject to customary exceptions. Pursuant to such lock-up agreement, the Company has agreed that it will not for a period of 90 days following the date of the closing of the Private Placement, which occurred on February 11, 2020, dispose of or othersise transfer any Shares or certain other types of securities, subject to certain customary exceptions. See also "*Company Information- Board of Directors, Executive management and key employees-Conflict of Interest-Restrictions on Securities Trading"*.

8 COSTS OF THE LISTING

The estimated expenses payable by the Company in connection with the Listing are approximately DKK 3 to 4 million ex. VAT. Expenses include fees to auditors, legal and other advisors as well as other expenses connected to the Listing.

9 DILUTION

The Listing of the Listing Shares on Nasdaq Copenhagen will not result in any dilution.

10 ADDITIONAL INFORMATION

10.1 Advisers

Legal adviser to the Company in connection with the Listing:

Gorrissen Federspiel Advokatpartnerselskab
Axeltorv 2
DK-1609 Copenhagen V
Denmark

Auditors to the Company:

ERNST & YOUNG Godkendt Revisionspartnerselskab
Osvald Helmuths Vej 4
DK-2000 Frederiksberg
Denmark

GLOSSARY

In the Prospectus, the following words and expressions have the meanings stated below, unless the context requires otherwise.

Articles of Association	The Company's Articles of Association of February 6, 2020.
Board Incentive Program	The board incentive program of the Company.
Board of Directors	The board of directors of the Company.
Chairman	The chairman of the Board of Directors.
Company	Orphazyme A/S, CVR no. 32 26 63 55.
Corporate Governance Recommendations	The recommendations on corporate governance published by the Committee on Corporate Governance in November 2017 (as updated in August 2019).
CVR no.	The Danish Central Business Register number.
Danish Capital Markets Act	The Danish Consolidated Act no. 931 of September 6, 2019 on Capital Markets (in Danish: "*kapitalmarkedsloven*"), as amended.
Danish Companies Act	The Danish Consolidated Act no. 763 of July 23, 2019 on public and private limited companies (in Danish: "*selskabsloven*"), as amended.
Danish Business Authority	The Danish Business Authority (in Danish: "*Erhvervsstyrelsen*").
Danish Corporation Tax Act	The Danish Consolidated Act no. 1164 of September 6, 2016 on corporate income tax (in Danish: "*selskabsskatteloven*"), as amended.
Danish Pension Returns Tax Act	The Danish Consolidated Act no. 1126 of October 10, 2014 on pension returns tax (in Danish: "*pensionsafkastbeskatningsloven*"), as amended.
Deputy Chairman	The deputy chairman of the Board of Directors.

DKK	The official currency of the Kingdom of Denmark.
Executive Management	The Executive Management of the Company as registered with the Danish Business Authority at the Prospectus Date.
Existing Shares	The Company's share capital before the Private Placement consisting of 20,005,449 Shares with a nominal value of DKK 1 each.
Lending Shareholders	Orpha Pooling B.V. and Novo Holdings A/S.
FY2019 Group Financial Statements	The consolidated financial statement of the Company for the financial year ended December 31, 2019.
Group	The Company and its subsidiaries.
IFRS	International Financial Reporting Standards as adopted by the EU.
Key Employees	Thomas Kirkegaard Jensen and Thomas Blaettler.
Listing	The admission for trading and official listing of the Listing Shares on Nasdaq Copenhagen.
Listing Shares	3,071,673 shares with a nominal value of DKK 1 each in the Company
LTIP	The long-term incentive program of the Company.
Major Shareholders	Shareholders who have notified the Company that they hold more than 5% of the Company's registered share capital.
Management	The Board of Directors and the Executive Management.
Market Abuse Regulation	Regulation (EU) No. 596/2014 of April 16, 2014 on market abuse (market abuse regulation) and repealing Directive 2003/6/EC of the European Parliament and of the Council and Commission

	Directives 2003/124/EC, 2003/125/EC and 2004/72/EC.
Nasdaq Copenhagen	Nasdaq Copenhagen A/S.
Nasdaq Issuer Rules	The rules for issuers of shares on Nasdaq Copenhagen of July 1, 2019.
Orphazyme	Orphazyme A/S, CVR-no. 32 26 63 55, Ole Maaløes Vej 3, DK-2200 Copenhagen.
Prospective Financial Information	The prospective consolidated financial information for the financial year ended December 31, 2020.
Prospectus	This Prospectus covering the listing of the Listing Shares.
Prospectus Date	March 16, 2020.
Prospectus Regulation	Regulation (EU) No. 2017/1129 of June 14, 2017 on the prospectus to be published when securities are offered to the public or admitted to trading on a regulated market, and repealing Directive 2003/71/ECText with EEA relevance.
Qualified Investors	As defined in the Prospectus Regulation.
Regulation S	Regulation S under the U.S. Securities Act.
Relevant Member State	Each member state of the European Economic Area, where the Prospectus Regulation apply.
Relevant Persons	Persons who: (i) are investment professionals falling within Article 19(5); or (ii) fall within Article 49(2)(a) to (d) ("high net worth companies; unincorporated associations, etc."), of the UK Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 or other persons to whom such investment or investment activity may lawfully be made available.
Shares	Existing Shares and the Listing Shares.
VP Securities	VP Securities A/S, CVR. no. 21599336.

United States	The United States of America.
U.S. Securities Act	Securities Act of 1933, enacted by the 73rd United States Congress, as amended.