Company announcement
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Orphazyme announces intention to launch an Initial Public Offering

Orphazyme, a Danish biotech company with a late stage orphan drug pipeline, announces its intention to launch an Initial Public Offering ("IPO" or "Offering") of its shares and to apply for admission to trading and official listing on Nasdaq Copenhagen.

The intended IPO is expected to consist of an issue of new shares to raise gross proceeds of approximately DKK 600 million to support the Company's strategy by primarily funding on-going and planned clinical trials within four orphan diseases. The total offer size will be announced in connection with the publication of a prospectus by the Company.

Orphazyme is currently owned by Novo Holdings A/S, Coöperative Aescap Venture I U.A., Sunstone Life Science Ventures Fund II K/S, Orpha Pooling B.V. (a joint venture between LSP V Coöperatieve U.A. and ALS Invest 2 B.V.), certain funds managed by Idinvest and Kurma Biofund II and other minority shareholders including members of senior management and employees. Prior to publication of the prospectus, the Company's existing share classes will be merged as part of a reorganisation of the current capital structure. The exact ownership stakes of the current shareholders after the reorganisation of the capital structure will depend on the final offer price in the IPO. None of the current shareholders have expressed an intention to sell shares in connection with the IPO and will continue as shareholders in Orphazyme in addition to undertaking lock-up obligations in connection with the IPO.

Anders Hinsby, CEO of Orphazyme, said:

"Since the foundation in 2009, we have been able to establish a Danish biotech company with a product candidate for the treatment of four severe orphan diseases. We have an extensive news flow coming up and expect to have completed three phase II/III trials by the end of 2020 with the first potential marketing authorisation in 2020. We have, for a longer period of time, had many meetings with domestic and international investors and we are very pleased with the feedback received."

Georges Gemayel, Chairman of Orphazyme, said:

"Today's announcement marks an important step in Orphazyme's strategic journey. We look forward to inviting new shareholders to take part in the future ownership and to join us in our journey to assist patients with a significant unmet medical need."

About Orphazyme

Orphazyme is a Danish biotech company with a late stage orphan drug pipeline, developing new treatment options for orphan protein misfolding diseases. It was founded in 2009 based on early scientific discovery in heat shock proteins ("HSPs"). Since inception, the Company has translated the scientific discovery into a late stage clinical development programme.

The Company focuses on severe and mostly fatal diseases with a high unmet need, and with a particularly strong commitment to neuromuscular diseases and a group of severe genetic diseases called lysosomal storage diseases. The Company plans to pursue development of its lead candidate through to registration in Europe and the United States after which launch and commercialisation is expected to be undertaken by the Company.

The lead candidate arimoclomol is in development as a potential treatment for four orphan diseases; two neuromuscular diseases, sporadic Inclusion Body Myositis ("sIBM") and Amyotrophic Lateral Sclerosis ("ALS"), and two lysosomal storage diseases, Niemann Pick type C ("NPC") and Gaucher disease. Across phase I trials and phase II trials in sIBM and SOD1-ALS (a subtype of ALS), arimoclomol has been well tolerated with no significant safety risks identified to date. The phase II trials have shown consistent trends of treatment benefit. In addition, the clinical trials in NPC and Gaucher disease are pursuant to phase I trials in healthy volunteers and pre-clinical studies showing benefit of arimoclomol.

Arimoclomol stimulates the body's own production of heat shock proteins in cells experiencing stress or toxicity. HSPs form the core of the cells' protein rescue system and guard against the toxicity arising from misfolded proteins by supporting correct folding of mutated proteins and the cell's recycling system.

The Company is headquartered in Copenhagen and has 28 employees.

About orphan drugs

Orphan drugs denote pharmaceutical products that target rare, often severe diseases affecting a small number of people. The diseases are in general serious, debilitating and often fatal if not treated.

Following the Orphan Drug Act in the United States in 1983, implementation of orphan drug regimes have taken place in other key markets as well, for example Japan in 1993 and the EU in 2000. The Orphan Drug Act was set up to financially incentivise the development of pharmaceutical products for severe diseases affecting so few individuals that it would not be considered profitable otherwise.

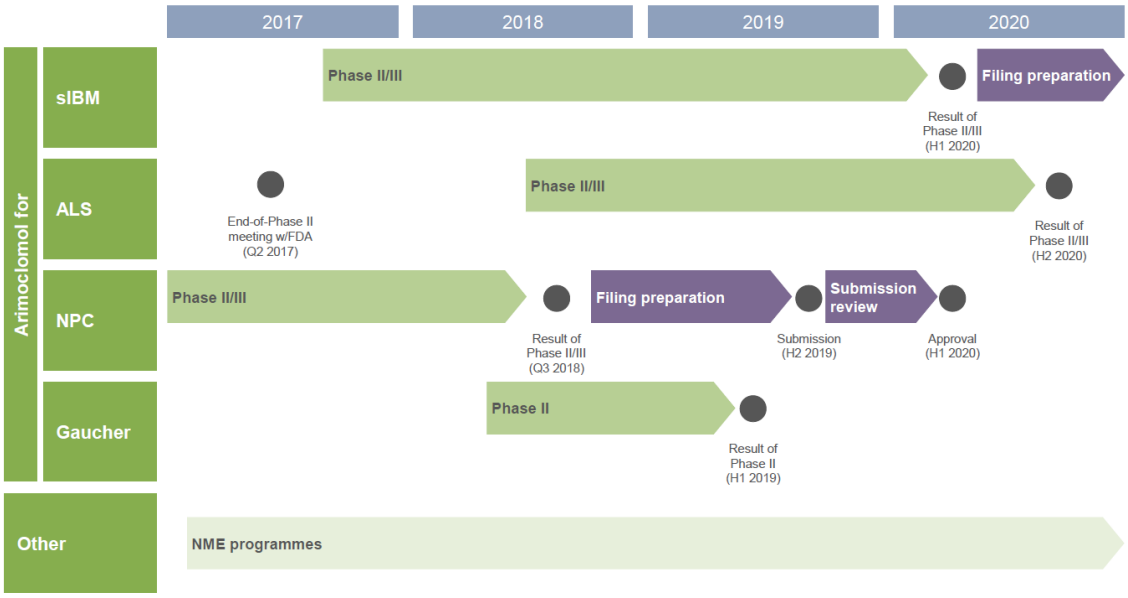
Developing treatments for orphan diseases present a series of advantages for the drug developers during the development and marketing phase, including regulatory support incentives,

likely less competition and high unmet medical need, higher clinical development success rate, generally lower development and commercialisation costs as patient groups are small, potential market exclusivity and usually higher pricing of products.

Project Pipeline

Orphazyme has potential registration studies in several indications ongoing or in the planning. The first indication for arimoclomol is expected to be the treatment of NPC. The results of the already initiated clinical trial in NPC are expected in Q3 2018 with potential marketing authorisation in H1 2020. In Q3 2017 a sIBM phase II/III clinical trial was initiated. Additionally, Orphazyme expects to have initiated clinical studies in the remaining indications, ALS and Gaucher disease, by the end of 2018. Further, the Company has developed expertise to assess new leads and have identified a number of new molecular entities ("NMEs") which may generate clinical candidates.

The figure below provides an overview of the expected news flow until 2020, subject to completion of the Offering in order to fund the clinical trials currently ongoing and in planning.



The above timeline is subject to success of trials.

Strategy

Orphazyme's strategy is to develop treatments for orphan diseases with protein misfolding where it can apply its specialised know-how in HSPs. Important elements of Orphazyme's strategy are the following:

- Advance the development of arimoclomol for the treatment of sIBM, ALS, NPC and Gaucher disease by completion of clinical development programmes
- Design the commercialisation and go-to-market strategy for arimoclomol
- Develop new molecular entities for other protein misfolding diseases based on current technology platform

Orphazyme's key strengths

Late stage project pipeline with several potential registration studies ongoing or in planning

Orphazyme is developing arimoclomol in four indications. Orphazyme has fully recruited a phase II/III trial in NPC (trial results expected Q3 2018), initiated a phase II/III trial in sIBM in August 2017 and plans to start a phase II/III trial in ALS in H2 2018. In addition, a phase II proof-of-concept trial in Gaucher disease will be initiated in Q2 2018[1]. If positive, all three phase II/III trials are intended to form the basis for a single study filing in each respective indication. The Company expects to have completed all three phase II/III trials by the end of 2020 with the first potential marketing authorisation in 2020.

Careful choice of target indications, based on insight into the function of HSPs and optimisation for clinical success by focusing on diseases with well-defined patient populations, disease pathology and/or genetics

Orphazyme aims to enhance development success rates by choosing target patient populations with well-defined pathology and/or genetics that are expected to respond more uniformly to treatment and by using clinical and biochemical parameters to select and stratify patients for the clinical trials. A tightly defined patient population increases the likelihood of robust signal detection in a clinical trial and to optimise chances of clinical success. Orphazyme therefore specifically pursues rare disease indications instead of more common diseases.

Technology platform shows potential with clinical proof-of-concept[1]

Orphazyme's lead program, arimoclomol, has been studied in a number of pre-clinical experiments allowing it to be characterised in a variety of different disease models and pathologies, many of which are published in leading peer-reviewed scientific journals including Nature Medicine and Science Translational Medicine. The ability of arimoclomol to mobilise HSPs and provide benefit have been validated across several protein misfolding diseases including relevant animal models for NPC, ALS and sIBM. Moreover, the benefit of arimoclomol has been established and characterised in cells from patients diagnosed with NPC and Gaucher disease across a range of mutations.

Technology protection with active patenting strategy aimed at maintaining Orphazyme's technology position

Since its founding, Orphazyme has strategically and actively pursued patent protection of its inventions. Orphazyme now holds the rights to a comprehensive patent portfolio and continues to actively pursue further patent protection and exclusivity opportunities. Orphazyme focuses on protecting small molecule inducers of HSPs, including the main candidate arimoclomol, as well as new molecular entities.

Experienced management team with relevant and complementary skill set

Orphazyme's senior management team has experience from the biotech and pharmaceutical industries with a broad range of skills to succeed in the industry, including experience with research and development, finance and intellectual property rights. The Company's CEO and CSO have been with the Company since its inception.

[1] The Company considers "proof-of-concept" to be benefit across efficacy endpoints and assessments as compiled evidence that constitutes proof-of-concept, also in absence of statistical significance

Bank Syndicate

Carnegie and Danske Bank are acting as Joint Global Coordinators and Joint Bookrunners, and Oddo BHF SCA is acting as Co-Lead Manager for the proposed IPO.

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