

Company announcement

No. 20/2020

Orphazyme A/S

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Company Registration No. 32266355

Orphazyme announces Annual Report 2019

Copenhagen, Denmark, February 28, 2020 – Orphazyme A/S (ORPHA.CO), a biopharmaceutical company pioneering Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases, today announces its Annual Report for the period January 1–December 31, 2019.

Kim Stratton, Chief Executive Officer of Orphazyme, said *"2019 was an exceptional year for Orphazyme. We delivered on key priorities with our late-stage programs in Niemann-Pick disease Type C, sporadic Inclusion Body Myositis and Amyotrophic Lateral Sclerosis and continued to advance our proof-of-concept phase 2 study in Gaucher disease. We believe our investigational drug, arimoclomol, holds significant potential across a range of neurodegenerative diseases and we are excited to be on the cusp of bringing this first-in-class Heat-Shock Protein amplifier to patients. We are on track to submit marketing applications in the US in H1 2020 and in EU in H2 2020 and, pending the outcome and timing of regulatory reviews, plan to reach the market in 2021"*.

Business Highlights in 2019

- Reported positive phase 2/3 results for arimoclomol in Niemann-Pick disease Type C (NPC) in January 2019
- Announced preparations for filing in EU and US. Plan to submit a New Drug Application with the US Food and Drug Administration (FDA) in H1 2020 and to submit a Marketing Authorization Application (MAA) to the European Medicines Agency in H2 2020
- Completed enrollment in phase 2/3 trial with arimoclomol in sporadic Inclusion Body Myositis (sIBM) in April 2019; results expected H1 2021
- In July, announced appointment of Kim Stratton as Chief Executive Officer, effective October 1, 2019
- Completed enrollment in phase 3 trial with arimoclomol in Amyotrophic Lateral Sclerosis (ALS) earlier than anticipated (July 2019); on track to report results in H1 2021
- Introduced a new board incentive program, a new share-based incentive program, and a new phantom share program in July 2019
- Strengthened balance sheet in August 2019 with EUR 9 million financing from Kreos Capital
- Completed enrollment in phase 2 Gaucher disease trial in August 2019; results expected in H1 2020
- Received Breakthrough Therapy Designation from FDA in November 2019 for arimoclomol in NPC
- Granted Fast-Track designation in December 2019 by the FDA for arimoclomol in sIBM

Financial Results 2019

For the period January 1–December 31, 2019

- Net loss amounted to MDKK 337.5 or DKK 16.87 per share (basic and diluted) compared to a net loss of MDKK 229.6 or DKK 11.49 per share (basic and diluted) for the same period in 2018
- Research and development expenses for the period totaled MDKK 285.4 compared to MDKK 196.5 for the same period in 2018
- General and administrative expenses for the period totaled MDKK 50.5 compared to MDKK 35.1 for the same period in 2018
- As of December 31, 2019, Orphazyme held cash totaling MDKK 124 compared to MDKK 226 as of June 30, 2019 and MDKK 395 as of December 31, 2018

Subsequent events

- In January 2020, Orphazyme reported positive data from the open-label phase 2/3 extension study in NPC showing sustained effect of arimoclomol in reducing disease progression over two years and new genetic sub-group analysis recommended by US FDA supporting robustness of results
- In January 2020, Orphazyme announced US Early Access Program for NPC
- In February 2020, Orphazyme announced completion of an offering of 7,032,937 shares in a directed issue and private placement raising approximately DKK 745 million (EUR 100 million/USD 110 million)

Outlook 2020

For 2020, Orphazyme anticipates an operating loss in the range of DKK 500 - 550 million and a cash position at year-end 2020 of more than DKK 300 million, as outlined in the Annual Report 2019, published February 28, 2020.

The full report is attached as a PDF file and can be found on the Company's website www.orphazyme.com.

Conference Call

Orphazyme will host an investor call at which Chief Executive Officer, Kim Stratton, and Chief Financial Officer, Anders Vadsholt, will provide a company update and discuss results for the year ending December 31, 2019. The presentation will be followed by a Q&A session.

The call will be held on Friday, February 28, 2020 at 11.00 AM CET/5.00 AM EDT.

Dial-in details:

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Event Title: Orphazyme Annual Report 2019

Confirmation code: 4153337

The presentation will also be available via webcast: <https://edge.media-server.com/mmc/p/f2rrgte8>

After the call, the presentation will be available via the above link.

For additional information, please contact

Orphazyme A/S

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About Orphazyme A/S

Orphazyme is a biopharmaceutical company focused on bringing novel treatments to patients living with life-threatening or debilitating rare diseases. Our research focuses on developing therapies for diseases caused by misfolding of proteins, including lysosomal storage diseases. Arimoclomol, the company's lead candidate, is in clinical development for four orphan diseases: Niemann-Pick disease Type C, Gaucher disease, sporadic Inclusion Body Myositis, and Amyotrophic Lateral Sclerosis. The Denmark-based company is listed on Nasdaq Copenhagen (ORPHA.CO). For more information, please visit www.orphazyme.com.

Forward-looking statement

This company announcement may contain certain forward-looking statements. Although the Company believes its expectations are based on reasonable assumptions, all statements other than statements of historical fact included in this company announcement about future events are subject to (i) change without notice and (ii) factors beyond the Company's control. These statements may include, without limitation, any statements preceded by, followed by, or including words such as "target," "believe," "expect," "aim," "intend," "may," "anticipate," "estimate," "plan," "project," "will," "can have," "likely," "should," "would," "could", and other words and terms of similar meaning or the negative thereof. Forward-looking statements are subject to inherent risks and uncertainties beyond the Company's control that could cause the Company's actual results, performance, or achievements to be materially different from the expected results, performance, or achievements expressed or implied by such forward-looking statements. Except as required by law, the Company assumes no obligation to update these forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in the forward-looking statements, even if new information becomes available in the future.