

**Company announcement**

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**Orphazyme accelerates arimoclomol pre-launch activities and updates financial outlook for 2020**

**Copenhagen, Denmark, October 23, 2020** – Orphazyme A/S (ORPHA.CO), a late-stage biopharmaceutical company pioneering the Heat-Shock Protein (HSP) response for the treatment of neurodegenerative orphan diseases, today announces an update to its financial outlook for 2020. The update is primarily driven by an acceleration in commercial and other pre-launch activities during the fourth quarter of 2020 in preparation for potential approval of its investigational product candidate arimoclomol in Niemann-Pick disease Type C (NPC), which is currently under Priority Review by the U.S. Food and Drug Administration (FDA) with a target action date of March 17, 2021.

The increased expenditure is based on Orphazyme's reinforced financial position following a successful global offering, as well as the FDA acceptance of its New Drug Application (NDA) for arimoclomol for NPC and granting of Priority Review. The Company also plans additional investments to further develop its pipeline.

Kim Stratton, Chief Executive Officer of Orphazyme, said, "There is real momentum here at Orphazyme as we move closer to potential approval of arimoclomol in the U.S. in its first indication of NPC and accelerating our preparatory efforts now will help ensure a smooth launch. There are currently no approved products for NPC in the US and arimoclomol has the potential to make a significant difference to patients with this devastating disease, so our team is working expeditiously to ensure we are optimally positioned for a successful launch if approved."

| <b>Full-year 2020 anticipated operating loss</b> | <b>DKKm</b>   | <b>USDm*</b> |
|--|---------------|--------------|
| Previous guidance                                | (500) – (550) | (80) – (88)  |
| New guidance                                     | (625) – (650) | (96) – (104) |

| <b>Cash position at December 31, 2020</b> | <b>DKKm</b> | <b>USDm*</b> |
|---|-------------|--------------|
| Previous guidance                         | >300        | >48          |
| New guidance                              | >700        | >111         |

\*USD figures are for reference only; FX rate 1 DKK / 0.16 USD.

Year to date, operating expenses have tracked in line with the Company's previous guidance of an operating loss of DKK 500 – 550M. Following the successful global offering, the company has decided to accelerate its pre-launch activities during the fourth quarter in preparation for potential approval of arimoclomol in the U.S. and will further invest in its Early Access Programs, additional API manufacturing, regulatory affairs, and clinical safety activities. Orphazyme also expects continued costs through year-end for the on-going trials in sporadic Inclusion Body Myositis and Amyotrophic Lateral Sclerosis in order to provide home nursing and direct to patient distribution due to the COVID-19 pandemic. In addition, the Company intends to increase investment in its NME program of next generation HSP amplifiers and will incur additional operational costs in Q4 2020 associated with the U.S. listing. As a result, the operating loss is expected to be in the range of DKK 625M to DKK 650M (USD 96M to USD 104M), for the fiscal year ending December 31, 2020.

Orphazyme expects to finish 2020 with cash of DKK >700M (USD >111M). This compares to previous guidance of DKK >300M (USD >48M) and includes net proceeds to date from the Company's recent global offering.

In September 2020, the FDA accepted Orphazyme's New Drug Application for arimoclomol for NPC, with Priority Review. The FDA set a target action date of March 17, 2021 under the Prescription Drug User Fee Act, or PDUFA, for completion of its review of the NDA. Orphazyme continues to expect to submit a Marketing Authorization Application, or MAA, to the European Medicines Agency in the second half of 2020.

**For additional information, please contact****Orphazyme A/S**

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**About Orphazyme A/S**

Orphazyme is a late-stage biopharmaceutical company pioneering the Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases. The company is harnessing amplification of Heat-Shock Proteins (or HSPs) in order to develop and commercialize novel therapeutics for diseases caused by protein misfolding, protein aggregation, and lysosomal dysfunction, including lysosomal storage diseases and neuromuscular degenerative diseases. Arimoclomol, the company's lead candidate, is in clinical development for four orphan diseases: Niemann-Pick disease Type C (NPC), Amyotrophic Lateral Sclerosis (ALS), sporadic Inclusion Body Myositis (sIBM) and Gaucher disease. Orphazyme is headquartered in Denmark and has operations in the U.S. and Switzerland. Orphazyme's shares are listed on Nasdaq Copenhagen (ORPHA.CO).

**Forward-looking statement**

This company announcement may contain certain forward-looking statements, including with respect to Orphazyme's full year 2020 anticipated operating loss and cash position at December 31, 2020. Although Orphazyme 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 Orphazyme'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 Orphazyme's control that could cause Orphazyme'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, including in respect of unanticipated expenditures during the fourth quarter of 2020. Except as required by law, Orphazyme 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.