Company announcement
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Orphazyme provides regulatory and financial updates

Copenhagen – October 5, 2021 – Orphazyme A/S (ORPHA.CO; ORPH), a late-stage biopharmaceutical company, today announces regulatory updates for its investigational drug candidate, arimoclomol, for which it is seeking marketing authorization in both Europe and the United States for the treatment of Niemann-Pick disease Type C (NPC). The company also announces that it now expects to end 2021 with a cash position of no less than DKK 80 million compared to prior guidance of approximately DKK 50 million.

The review process under the centralized procedure in Europe is underway. Following dialogue with the Rapporteurs and the anticipated timeline to respond to the Day 180 List of Outstanding Issues, the company now anticipates a CHMP opinion in Q1 2022 compared to previous Q4 2021.

In the United States, Orphazyme requested and has been granted a Post-Action Type A meeting with the U.S. Food and Drug Administration (FDA), which has been scheduled for mid-October. At this meeting, the company plans to engage in a collaborative dialogue with the FDA to better understand its decision in the Complete Response Letter issued in June 2021, as well as to discuss proposals to address key topics raised in the letter. The company expects to communicate an update to the market once it has received and assessed formal minutes from the FDA Type A meeting.

The improved cash guidance relates to the ongoing restructuring plan, which has enabled the company to deliver more than anticipated savings in terms of headcount and other costs. The company continues to assess different possibilities for obtaining additional funding to sustain operations in the longer-term.

For additional information, please contact:

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About Niemann-Pick disease type C
Niemann-Pick disease type C (NPC) is a rare, genetic, progressively debilitating, and often fatal neurodegenerative disease. It belongs to a family known as lysosomal storage diseases and is caused by mutations leading to defective NPC protein. As a consequence, lipids that are normally cleared by the lysosome accumulate in tissues and organs, including the brain, and drive the disease pathology. We estimate the incidence of NPC to be one in 100,000 live births and the number of NPC patients in the United States and in Europe to be approximately 1,800 individuals. There are no approved treatments for NPC in the U.S.

About arimoclomol
Arimoclomol is an investigational drug candidate that amplifies the production of heat shock proteins (HSPs). HSPs can rescue defective misfolded proteins, clear protein aggregates, and improve the function of lysosomes. Arimoclomol is administered orally, and has now been studied in 10 Phase 1, four Phase 2, and three pivotal Phase 2/3 trials. Arimoclomol has received Orphan Drug Designation (ODD) for NPC in the US and EU. Arimoclomol has received Fast-Track Designation (FTD), Breakthrough Therapy Designation (BTD), and Rare Pediatric Disease Designation (RPDD) from the U.S. Food and Drug Administration (FDA) for NPC. On June 17, 2021, Orphazyme received a Complete Response Letter from the FDA regarding its New Drug Application for arimoclomol for the treatment of NPC. A marketing authorization application (MAA) for arimoclomol in NPC has been filed with the European Medicines Agency and is under review.

About Orphazyme A/S
Orphazyme is a late-stage biopharmaceutical company developing arimoclomol for Niemann-Pick disease type C (NPC). Orphazyme is headquartered in Denmark and has operations in the U.S. and Switzerland. ADSs representing Orphazyme's shares are listed on Nasdaq U.S. (ORPH) and its shares are listed on Nasdaq Copenhagen (ORPHA).

Forward-looking statement
This company announcement may contain certain forward-looking statements under the U.S. Private Securities Litigation Reform Act of 1995 and otherwise, including as to its net cash position at year-end 2021, and the timing of the CHMP opinion and FDA meeting. 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, including the risks and uncertainties that are described in the Risk Factors section of the Company's Annual Report on Form 20-F for the year ended December 31, 2020 filed with the U.S. Securities and Exchange Commission (SEC) on March 2, 2021, the Company's Report on Form 6-K filed with the SEC on June 11, 2021, and other filings Orphazyme makes with the SEC from time to time. These documents are available on the "Investors & Media" section of Orphazyme's website at www.orphazyme.com. 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.