

Investor news
No. 02/2022

Orphazyme A/S
Ole Maaløes Vej 3
DK-2200 Copenhagen N

www.orphazyme.com

Company Registration No. 32266355

Orphazyme provides update for planned NDA resubmission for arimoclomol for the treatment of Niemann-Pick disease type C in the United States

- *Orphazyme has made progress towards resubmission of the NDA for arimoclomol to the FDA and plans to request a Type C Meeting in Q2 2022*
- *Subject to these discussions, the Company aims to resubmit the NDA during H2 2022*

Copenhagen, Denmark, February 11, 2022 – Orphazyme A/S (ORPHA.CO; ORPH) (“Orphazyme” or the “Company”), a late-stage biopharmaceutical company pioneering the Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases, today provides an update on the process and anticipated timelines for resubmission of its New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for investigational product candidate, arimoclomol, for the treatment of Niemann-Pick disease type C (NPC).

In line with recommendations from the FDA during a Type A Meeting held in October 2021, the Company intends to request a Type C Meeting to discuss the additional data, information, and analyses addressing certain topics in the Complete Response Letter (CRL) to align on a path to resubmission for arimoclomol in NPC with the FDA. The Company expects to request the Type C Meeting in Q2 2022. Subject to these discussions, the Company aims to resubmit the NDA during H2 2022.

Anders Vadsholt, Chief Financial Officer of Orphazyme said, “*Requesting a Type C Meeting is the next step in establishing a potential path to resubmission of our NDA to the FDA. NPC is a rare neurodegenerative disease for which there are no approved treatments in the United States, and we look forward to continuing our interactions with the Agency as we seek to gain approval of arimoclomol in the United States for this devastating disease.*”

The EU Marketing Authorisation Application (MAA) for arimoclomol for the treatment of NPC was filed with the European Medicines Agency (EMA) in November 2020. As previously communicated, an opinion from the Committee for Medicinal Products for Human Use (CHMP) on this application is expected in Q1 2022.

Christophe Bourdon, Chief Executive Officer of Orphazyme said, “*Our team has been working at pace and we are looking forward to be interacting with an ad-hoc expert group in the coming weeks for the European submission.*”

For additional information, please contact

Orphazyme A/S

Anders Vadsholt, Chief Financial Officer +45 2898 9055

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).

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.

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 the U.S. and EU regulatory processes for the potential approval of arimoclomol and the resubmission of the NDA to the FDA. 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.