Company announcement
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Inside information

Orphazyme A/S
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Company Registration No. 32266355

Orphazyme announces withdrawal of European Marketing Authorisation Application for arimoclomol for the treatment of Niemann-Pick disease type C

- *Decision to withdraw the European Marketing Authorisation Application comes ahead of scheduled final vote on the application later this month*

- *Orphazyme intends to request Type C Meeting with the U.S. Food and Drug Administration to discuss potential pathway for resubmission of the New Drug Application for arimoclomol for Niemann-Pick disease type C*

Copenhagen, Denmark, March 22, 2022 – Orphazyme A/S (ORPHA.CO; ORPH) ("Orphazyme" or the "Company"), a late-stage biopharmaceutical company, announces that, following the receipt of the negative Trend Vote by the Committee for Medicinal Products for Human Use (CHMP) as announced on February 23, 2022 (please see company announcement no. 07/2022), the Company has decided to withdraw its European Marketing Authorisation Application (MAA) for arimoclomol for the treatment of Niemann-Pick disease Type C (NPC) ahead of a final vote and opinion by the CHMP on the MAA scheduled for later this month.

"*Today's decision to withdraw the MAA enables us to consider the best possible path forward for arimoclomol,*" commented Orphazyme Chief Executive Officer, Anders Vadsholt. "*NPC is an ultra-rare neurodegenerative disease with a high unmet medical need, and we want to explore options for arimoclomol in this indication, to deliver on our commitment to patients with this devastating disease.*"

Orphazyme is currently under an in-court restructuring and has reduced its workforce by approximately 50% to reduce costs as it seeks to explore whether a basis can be established for all or part of the Company's operations to continue, including a sale of all or parts of the Company's assets. At this stage it is uncertain whether a solution can be found, and further update will be provided at the appropriate time.

As part of the pursuit of a regulatory pathway in the U.S., the Company continues to work towards resubmission of the Company's New Drug Application for arimoclomol to the U.S. Food and Drug Administration (FDA) and plans to request a Type C Meeting in Q2 2022.

For additional information, please contact

Orphazyme A/S

Anders Vadsholt, Chief Executive Officer and 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 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 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. The Company plans to request a Type C Meeting with the FDA in Q2 2022. On February 23, 2022, the EMA Committee for Medicinal Products for Human Use (CHMP) issued a negative Trend Vote on the Marketing Authorization Application (MAA) for arimoclomol in NPC filed with the European Medicines Agency (EMA). The Company has subsequently decided to withdraw its MAA.

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 forward-looking statements about the U.S. regulatory process for the potential approval of arimoclomol by 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.