

Investor News
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Orphazyme A/S
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Orphazyme to present additional data on arimoclochol's mechanism of action during 2022 Annual WORLDSymposium™

Copenhagen, Denmark, February 7, 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 announced it will present new non-clinical data on its investigational drug arimoclochol during the 18th Annual [WORLDSymposium™](#) Scientific Meeting, to be held in San Diego, CA and virtually on February 7-11, 2022.

The details of the presentations are:

- **Persistent effect of arimoclochol in patients with Niemann-Pick disease type C: 24-month results from an open-label extension of a pivotal Phase 2/3 study**
 - Presented by Marc Patterson, M.D.
 - Wednesday, February 9, 2022, Poster 229

- **Arimoclochol increases the transcription of lysosomal genes, including NPC1, to facilitate lysosomal function**
 - Presented by Nikolaj Petersen, Ph.D.
 - Thursday, February 10, 2022, Poster 236

For additional information, please contact

Orphazyme A/S

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About Orphazyme A/S

Orphazyme is a late-stage biopharmaceutical company developing arimoclochol 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 arimoclochol

Arimoclochol 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. Arimoclochol is administered orally, and has now been studied in 10 Phase 1, four Phase 2, and three pivotal Phase 2/3 trials. Arimoclochol has received Orphan Drug Designation (ODD) for NPC in the US and EU. Arimoclochol 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 arimoclochol for the treatment of NPC. A marketing authorization application (MAA) for arimoclochol 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 arimoclochol. 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.