

Company announcement

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Inside information

Orphazyme A/S

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Orphazyme announces update on regulatory review of arimoclomol in the European Union

Copenhagen, Denmark, February 23, 2022 – Orphazyme A/S (ORPHA.CO (DK); ORPH (US)) (the “Company”), a late-stage biopharmaceutical company, today announced an update on the ongoing review of the Marketing Authorisation Application (MAA) for its investigational product candidate, arimoclomol, for the treatment of Niemann-Pick disease type C (NPC) by the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA).

While Orphazyme was encouraged by the positive feedback of the ad-hoc expert group meeting held on February 17, 2022, Orphazyme has today been notified by the CHMP of a negative trend vote on the MAA for arimoclomol in NPC following an Oral Explanation. The trend vote indicates that the CHMP’s current orientation is to not approve arimoclomol when it convenes by the end of March 2022. Orphazyme considers it unlikely that this position will change before the formal vote is undertaken next month.

In the light of the recent development and the Company’s financial situation, the Company will now assess the Company’s strategic options and provide an update to the market at the applicable time.

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

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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 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 Company’s assessment of its strategic options. 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.