

Company announcement

No. 01/2022

Inside information

Orphazyme A/S

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Company Registration No. 32266355

Orphazyme updates 2021 financial outlook

- *Net revenue range narrowed to DKK 35 – 37 million*
- *Operating expenses lowered to DKK 665 – 675 million, due to strict cost management*
- *Cash position as of December 31, 2021, now anticipated to be no less than DKK 100 million*

Copenhagen – January 18, 2022 – Orphazyme A/S (ORPHA.CO; ORPH or the “Company”), a late-stage biopharmaceutical company, today announces an update to its financial outlook for 2021 based on a review of preliminary unaudited financial results for the year ending December 31, 2021. Audited financial results for the full-year 2021 will be published on March 15, 2022.

The improved financial outlook for 2021 is mainly driven by increased visibility on net revenues from the use of arimoclomol in the Early Access Program in France and lower-than-expected operating expenses due to strict cost management.

	New 2021 guidance	Previous 2021 guidance
Net revenues	DKK 35 – 37 million	DKK 30 – 40 million
Operating expenses	DKK 665 – 675 million	DKK 700 – 720 million
Operating loss	DKK 630 – 640 million	DKK 670 – 700 million
Cash position, December 31, 2021	No less than DKK 100 million	No less than DKK 80 million

“We are pleased with the progress we have made in effectively managing our costs while remaining laser-focused on our mission to gain approval of arimoclomol for Niemann-Pick disease type C. Our interactions with the European regulators are ongoing and we eagerly await an opinion from the CHMP on our Marketing Authorization Application during Q1 2022. Furthermore, we continue to work with the FDA to determine a pathway to resubmission of our New Drug Application for arimoclomol in the United States as well as assessing different possibilities for obtaining additional funding to sustain operations”, said Christophe Bourdon, Chief Executive Officer, Orphazyme A/S.

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

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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 the Company’s anticipated financial results for 2021, the timing of the CHMP opinion on the Company’s MAA, and working with the FDA to determine a pathway to resubmission of the Company’s NDA for arimoclomol in the United States. 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.