

Company announcement

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Annual report

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Company Registration No. 32266355

Orphazyme Announces Publication of 2021 Financial Results and Annual Report

Copenhagen, Denmark, June 7, 2022 – Orphazyme A/S (ORPHA.CO) (“Orphazyme” or the “Company”), today reports its financial results for the period from January 1, 2021, to December 31, 2021, and publishes its Annual Report.

In 2021, and until recently, the primary focus of Orphazyme was on seeking marketing authorization in the United States and Europe for its investigational product candidate arimoclomol for the treatment of Niemann-Pick disease type-C (NPC), preparing to commercialize arimoclomol in NPC, if approved, and completing two late-stage clinical studies – a Phase 2/3 trial evaluating arimoclomol in Amyotrophic Lateral Sclerosis (ALS) and a Phase 2/3 trial evaluating arimoclomol in inclusion body myositis (IBM).

A number of unfavorable events occurred during 2021 and early 2022 which significantly impacted Orphazyme’s business.

Significant events during the period

In March 2021, Orphazyme announced that its Phase 2/3 trial of arimoclomol in IBM did not meet its primary and secondary endpoints, and in May 2021 announced that its Phase 3 trial of arimoclomol in ALS did not meet its primary and secondary endpoints. As a result, development was ceased in these indications. In June 2021, the Company received a Complete Response Letter (CRL) from the U.S. Food and Drug Administration (FDA) for its New Drug Application (NDA) for arimoclomol in NPC. This resulted in the company implementing restructuring measures in June 2021 in order to reduce costs and focus on regulatory approval for arimoclomol in Europe and in evaluating the regulatory path forward in the U.S.

Significant events after the period

In January 2022, Christophe Bourdon, Chief Executive Officer (CEO) of Orphazyme, resigned from his position as CEO to take on the role of CEO at another company. Anders Vadsholt was appointed CEO of Orphazyme, effective March 1, 2022, in addition to his position as Chief Financial Officer (CFO).

In February 2022, Orphazyme was notified of a negative trend vote by the European Medicine’s Agency (EMA) Committee for Medicinal Products for Human Use (CHMP) relating to its MAA for arimoclomol in NPC. The MAA was withdrawn in March 2022, ahead of the CHMP’s final vote.

In light of the Company’s financial situation at the time and the negative trend vote from the CHMP, the Board of Directors initiated in-court restructuring proceedings of Orphazyme under the Danish Insolvency Act, which commenced on March 11, 2022. The aim of the in-court restructuring proceedings was to explore whether a basis could be established for all or part of our operations to continue, including a basis for injecting further capital, and/or a basis for a sale of all or parts of our assets.

Following initiation of the in-court restructuring proceedings, Orphazyme voluntarily delisted its American Depositary Shares (ADSs) representing Orphazyme’s ordinary shares from Nasdaq Global Select Market in the U.S. and filed a Form 25 (Notification of Removal from Listing and/or Registration under Section 12(b) of the Securities Exchange Act of 1934) with the Securities and Exchange Commission (SEC) on March 21, 2022. The delisting became effective on March 31, 2022, and deregistration of the ADSs is expected to become effective approximately 90 days after the filing of the Form 25 with the SEC.

In May 2022, Orphazyme announced an agreement to sell substantially all of the Company’s assets and business activities, including those relating to the development and approval of arimoclomol and the full claw back liability

related to the French early access program, to KemPharm Denmark A/S (KemPharm), a wholly owned subsidiary of KemPharm Inc. for a total of USD 12.8 million in cash and assumed liabilities estimated to equal approximately USD 5.2 million (the Sale of Assets). KemPharm is a specialty pharmaceutical company focused on the discovery and development of novel treatments for rare central nervous system diseases. Under the terms of the agreement, KemPharm agreed to acquire substantially all of Orphazyme's assets and business activities, including those relating to the development and approval of arimoclomol, retain a majority of Orphazyme's remaining Danish employees, continue the early access programs with arimoclomol and pursue the potential approval of arimoclomol as a treatment option for NPC.

Subsequently, a restructuring proposal was submitted to the Danish Maritime and Commercial High Court and Orphazyme's known creditors, which amongst other things, included a proposal to complete the Sale of Assets to KemPharm. The restructuring proposal was approved on May 30, 2022, by Orphazyme's known creditors and affirmed by the Danish Maritime and Commercial High Court. Following the approval of the restructuring proposal, the in-court restructuring proceedings were discontinued with immediate effect on May 30, 2022, and the Sale of Assets was completed on May 31, 2022. Completion of the Sale of Assets is expected to provide full coverage to creditors with undisputed claims based on the claims filed during the restructuring. All (undisputed and unconditional) debts related to the time prior to restructuring have been or will be paid in full, including all obligations outstanding under the company's debt facility with Kreos Capital.

As substantially all of Orphazyme's assets and business activities have been sold to KemPharm, the Company has limited ongoing operational business activities and only two employees. Considering this, we previously announced that Stephanie Okey, Carolee Barlow and Martin Bonde stepped down from their positions as members of the Board of Directors as of May 23, 2022, with the view to reduce the number of members on our Board of Directors.

Financial results for the full year 2021

- Net Revenue of DKK 36.2 million (2020: DKK 0 million) from the sale of arimoclomol under the remunerated early access compassionate use program (former nATU) in France.
- Operating expenses of DKK 669.5 million (2020: DKK 608.5 million).
- Operating loss of DKK 633.3 million (2020: DKK 608.5 million).
- Net loss for the year of DKK 626.5 million (2020: DKK 633.2 million).
- Cash and cash equivalents of DKK 102.3 million at year end (2020: DKK 726.9 million).

Financial outlook for 2022

For the full-year 2022 we anticipate an operating profit in the range DKK 10 – 30 million. We expect to end 2022 with more than DKK 30 million in cash and equivalents. Following completion of the sale of substantially all the Company's assets and business activities to KemPharm in May 2022, Orphazyme has limited ongoing operational business activities and only two employees. There are inherent risks and uncertainties in our Outlook for 2022 given the recent closing of the KemPharm transaction, transfer of operating activities from Orphazyme to KemPharm and our future prospects.

For additional information, please contact

Orphazyme A/S

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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 Company's financial performance and its future prospects, payment of debts and other obligations and the deregistration of the Company's ADSs. 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, including pursuant to regulatory or judicial intervention. 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.