Company announcement
No. 20/2021
Inside information

Orphazyme A/S
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Orphazyme announces restructuring to focus resources on supporting a path forward for arimoclomol in NPC

-Significant headcount reduction of global workforce to free resources-

-Changes to the Board of Directors-

-Outlook for 2021 reiterated-

Copenhagen – June 28, 2021 – Orphazyme A/S (ORPHA.CO; ORPH), a late-stage biopharmaceutical company, today announced a restructuring intended to enable the company to advance its corporate strategy and the development of arimoclomol for Niemann-Pick disease type C (NPC). The resulting cost savings include an approximate two thirds reduction in our global workforce. Orphazyme remains committed to pursuing regulatory approval in Europe and assessing a path forward for arimoclomol in the U.S. following receipt of a Complete Response Letter from the U.S. Food and Drug Administration (FDA) on June 17, 2021.

Orphazyme CEO Christophe Bourdon said: "*As a result of the restructuring of the company and our rigorous cost saving program, we will have to part ways with many of our most valued and talented colleagues. I thank each of them for their strong commitment to Orphazyme and dedication to showing up for patients in need. The immediate actions we are taking are necessary to protect and support the ongoing approval process in Europe and the evaluation of a path forward in the U.S.*"

As part of the restructuring, Orphazyme will significantly scale back its global organization, including teams based in the U.S. and Europe, with the purpose of reducing the number of employees to those who will support essential activities moving forward. This includes pursuing regulatory approval in Europe, assessing the path forward in partnership with the FDA in the U.S., and supporting the existing global Expanded Access Program (EAP). In Denmark, Orphazyme will immediately initiate negotiations under the Danish Act on Collective redundancies and the Act on Information and Consultation.

Further, Rémi Droller, Martijn Kleijwegt, and Anders Hedegaard will resign from the Board of Directors effective June 30, 2021. The Board of Directors will thereafter consist of Georges Gemayel, Chairman, Bo Jesper Hansen, Deputy Chairman, Carrolee Barlow, Martin Bonde, Catherine Moukheibir, and Stephanie Smith Okey.

Georges Gemayel, Chairman of the Board of Directors of Orphazyme, stated: "*I would like to express our gratitude to Rémi Droller, Martijn Kleijwegt and Anders Hedegaard for their valuable contributions to Orphazyme over the years. In line with the restructuring of the company, the Board of Directors will not replace Rémi, Martijn and Anders. The Board is appropriately sized to support the path forward for Orphazyme.*"

Reiterate outlook for 2021
Orphazyme's financial outlook remains unchanged for 2021, as announced in company announcement no. 16/2021 on June 18, 2021.

Orphazyme intends to provide an update and further information in connection with the publication of its interim report for the first half of 2021, due for release August 24, 2021.

For additional information, please contact

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About Niemann-Pick disease type C
Niemann-Pick disease type C (NPC) is a rare, genetic, progressively debilitating, and often fatal neurovisceral disease. It belongs to a family known as lysosomal storage diseases and is caused by mutations leading to defective NPC protein. As a consequence, lipids that are normally cleared by the lysosome accumulate in tissues and organs, including the brain, and drive the disease pathology. We estimate the incidence of NPC to be one in 100,000 live births and the number of NPC patients in the United States and in Europe to be approximately 1,800 individuals. There are no approved treatments for NPC in the U.S.

About Orphazyme A/S
Orphazyme is a late-stage biopharmaceutical company. Arimoclomol, the company's lead candidate, is in clinical development for rare diseases including Niemann-Pick disease type C (NPC) and Gaucher disease. 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.CO).

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.

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 in respect of the scope, cost and implication of the restructuring announced today, its intention to pursue regulatory approval for arimoclomol in the United States and Europe, its anticipated operating expenses and operating loss for any future period and anticipated cash position at any future date. 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. 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.