

**Company announcement**

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## Orphazyme presents 36-month data supporting durable response to arimoclomol during Parseghian Scientific Conference for NPC Research

**Copenhagen – June 28, 2021** – Orphazyme A/S (ORPHA.CO; ORPH), a late-stage biopharmaceutical company, today announced 24-month interim results of an open-label extension (OLE) trial, providing efficacy and safety data for its investigational treatment arimoclomol in Niemann-Pick disease type C (NPC) for up to 36 months. The data are featured in a presentation as part of the [Parseghian Scientific Conference](#) for Niemann-Pick disease type C Research.

The results demonstrate that arimoclomol provided a sustained benefit to study participants by reducing NPC progression as measured by the 5-domain NPC Clinical Severity Scale (5D-NPCCSS). A slowing of progression from baseline was observed through 36 months in participants who received arimoclomol from the start of the double-blind phase (mean change, 3.5 points). By comparison, disease progression among NPC patients receiving routine clinical care was estimated to be a mean increase of 5.2 points after three years, based on a statistical model combining placebo data from the NPC-002 double-blind study and prospective data from the observational NPC-001 study. The effect was consistent across pre-specified subgroups, including among participants more than four years of age and those treated with miglustat. Also, slowing of progression through 24 months was observed in those participants who initiated arimoclomol treatment upon entering the open-label period (mean change, 0.9 points).

"Following on the outcomes from the 12-month double-blind phase, which indicated a clinically meaningful effect on disease progression, these longer-term data provide an encouraging picture that arimoclomol could deliver a sustained benefit and consistent safety profile over time," said Marc Patterson, MD, Professor of Neurology, Pediatrics and Medical Genetics, Mayo Clinic Children's Center in Rochester, MN.

Arimoclomol demonstrated a consistent safety profile throughout the 36-month treatment period. Adverse events observed during the open label extension phase were similar to those observed in the double-blind phase. A total of 41 patients joined the OLE following the double-blind period; 33 have now completed up to 36 months of treatment.

Data from the 36-month period support the findings from the 12-month double-blind period, which showed a clinically meaningful difference on the 5-domain NPCCSS, with a significant p-value of 0.046 (previously calculated at p=0.0537).

Orphazyme continue to pursue regulatory approval in Europe and evaluate a path forward for arimoclomol in NPC in the US.

*"These data provide further evidence of the clinical profile of arimoclomol to treat this population and may support our efforts to pursue regulatory approval to deliver a much-needed option for the NPC community,"* said CEO Christophe Bourdon. *"We continue to evaluate our path forward in the U.S. following the recent FDA response, and our application remains under active review in the European Union."*

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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 neurodegenerative 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 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.

**About Orphazyme A/S**

Orphazyme is a late-stage biopharmaceutical company pioneering the heat shock protein response for the treatment of rare diseases. The company is harnessing amplification of heat shock proteins (or HSPs) in order to develop and commercialize novel therapeutics for diseases caused by protein misfolding, protein aggregation, and lysosomal dysfunction. Arimoclomol, the company's lead candidate, is in clinical development for orphan 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).

**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 its intention to pursue regulatory approval for arimoclomol in the United States and Europe and the timing of clinical data. 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](http://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.