

ORPHAZZYME

Orphazyme announces topline results from pivotal trial of arimoclomol for Inclusion Body Myositis (IBM)

March 29, 2021

Orphazyme A/S

Company announcement

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Inside information

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Copenhagen – March 29, 2021 – Orphazyme A/S [ORPHA.CO (DK); ORPH (US)], a late-stage biopharmaceutical company pioneering the heat shock protein response for the treatment of neurodegenerative rare diseases, today announced its phase 2/3 trial evaluating arimoclomol for the treatment of inclusion body myositis (IBM), a progressively debilitating muscle-wasting disease, did not meet its primary and secondary endpoints. The primary goal was to evaluate the treatment effect on disease progression as measured by the inclusion body myositis functional rating scale (IBMFRS).

The randomized, placebo-controlled trial was conducted among 150 IBM patients at 12 sites in North America and Europe, in partnership with University College of London and the University of Kansas. Participants were randomized (1:1 ratio) to receive either arimoclomol citrate (400 mg three times daily) or placebo for up to 20 months.

No important safety concerns were detected in the trial. The analysis of the data is continuing and complete findings from the study will be shared in a future scientific forum.

"We recognize these data are disappointing for patients and families who continue to eagerly await a promising option for IBM. We believe the data collected will be useful to the community, since this trial represents one of the largest, long-term studies ever conducted in this disease and will help inform future research in the category," said Thomas Blaettler, MD, Chief Medical Officer, Orphazyme. "We are grateful to the investigators and their sites, and the many patients and families who graciously participated in the trial."

"We continue to believe in the promise of arimoclomol and heat shock protein science and are fully committed to our mission to deliver new therapies to patients," said Anders Vadsholt, interim Chief Executive Officer and Chief Financial Officer, Orphazyme.

Orphazyme expects data from a pivotal Phase 3 trial of arimoclomol in Amyotrophic Lateral Sclerosis (ALS), a neurodegenerative disease, this spring. The company's applications for arimoclomol (to be branded MIPLYFFA ^{TM1}) for Niemann-Pick disease type C (NPC) are under priority review with the U.S. Food and Drug Administration, with an expected action date in June 2021, as well as with the European Medicines Agency, with an opinion from the Committee for Medicinal Products for Human Use (CHMP) expected later this year.

For additional information, please contact:

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About Orphazyme A/S

Orphazyme is a late-stage biopharmaceutical company pioneering the Heat Shock Protein response for the treatment of neurodegenerative orphan 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, including lysosomal storage diseases and neuromuscular degenerative diseases. Arimoclomol, the company's lead candidate, is in clinical development for three orphan diseases: Niemann-Pick disease type C (NPC), for which we have a PDUFA date of June 17, 2021, Amyotrophic Lateral Sclerosis (ALS), and Gaucher disease. Orphazyme is headquartered in Denmark and has operations in the U.S. and Switzerland. Orphazyme's shares are listed on Nasdaq U.S. (ORPH) and 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 ten phase 1,

four phase 2 and two pivotal phase 2/3 trials. Arimoclomol is in clinical development for NPC, Gaucher Disease, IBM, and ALS. Arimoclomol has received orphan drug designation (ODD) for NPC, IBM, and ALS in the US and EU. Arimoclomol has received fast-track designation (FTD) from the U.S. Food and Drug Administration (FDA) for NPC, IBM and ALS. In addition, arimoclomol has received breakthrough therapy designation (BTD) and rare-pediatric disease designation (RPDD) from the FDA for NPC.

About IBM

Inclusion Body Myositis (IBM) is a progressively debilitating muscle-wasting disease. IBM is characterized by a build-up of protein aggregates and atrophy of muscle cells, which leads to weakness and over time severe disability. There are no approved treatments for IBM. Arimoclomol has been granted Orphan Drug Designation (EU and USA) for the treatment of IBM.

Forward-looking statement

This company announcement may contain certain forward-looking statements, including in respect of the anticipated commercialization of arimoclomol. 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 risk that applicable regulatory authorities fail to approve arimoclomol on the anticipated timeline or at all. 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.

¹ MIPLYFFA is a trademark or registered trademark of Orphazyme A/S. The global brand "MIPLYFFA" has received conditional approval from the U.S. Food and Drug Administration; the brand name will be used commercially upon approval for NPC.

Attachment

- [12-2021 Orphazyme announces topline results from pivotal trial of arimoclomol for Inclusion Body Myositis \(IBM\)](#)