

Investor news
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Orphazyme A/S
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Orphazyme announces presentations at **WORLDSymposium™**

Copenhagen, Denmark, February 11, 2020 – Orphazyme A/S (ORPHA.CO), a biopharmaceutical company pioneering Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases, today announces two presentations at the 16th Annual **WORLDSymposium™**, Orlando, Florida, February 10-13, 2020. These presentations highlight data from the Company’s randomized double-blind, placebo-controlled phase 2/3 trial with investigational drug candidate, arimoclomol, in Niemann-Pick disease Type C (NPC), and include previously reported primary endpoint data, pre-specified and post-hoc subgroup analyses, safety, and supportive biomarker data.

Presentation details:

Poster	Platform (oral) presentation	Poster presentation
<i>Efficacy and safety of arimoclomol in patients with Niemann-Pick type C: Results from a double-blind, randomized placebo-controlled trial with a novel treatment [Poster # 322]</i>	Wednesday, February 12, 2020 at 4:15PM EST	Tuesday, February 11, 2020, 4:30 – 6:30PM EST
<i>Rescue of NPC1 protein and effect on biomarkers by arimoclomol treatment in Niemann-Pick disease type C [Poster # 181]</i>	Thursday, February 13, 2020 at 3:45PM EST	Tuesday, February 11, 2020, 4:30 – 6:30PM EST

WORLDSymposium attendees who would like further information may speak with an Orphazyme representative by visiting Exhibit Hall booth #215.

For additional information, please contact Orphazyme A/S

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About Orphazyme A/S

Orphazyme is a biopharmaceutical company focused on bringing novel treatments to patients living with life-threatening or debilitating rare diseases. Our research focuses on developing therapies for diseases caused by misfolding of proteins, including lysosomal storage diseases. Arimoclomol, the company’s lead candidate, is in clinical development for four orphan diseases: Niemann-Pick disease Type C, Gaucher disease, sporadic Inclusion Body Myositis, and Amyotrophic Lateral Sclerosis. The Denmark-based company is listed on Nasdaq Copenhagen (ORPHA.CO). For more information, please visit www.orphazyme.com.

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, crosses the blood brain barrier, and has been studied in seven Phase I and three Phase II trials. Arimoclomol is in clinical development for NPC, Gaucher disease, sIBM, and ALS.

About NPC

Niemann-Pick disease Type C (NPC) is a 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 build-up in tissues and organs, including the brain, and drive the disease pathology. The estimated prevalence of NPC in the USA and Europe combined is 1,000. There are no approved treatments for NPC in the USA and only one approved product in Europe. Arimoclomol has been granted Orphan Drug Designation (EU and USA), Rare Pediatric Disease Designation (USA), and Fast Track designation (USA) for the treatment of NPC.

Forward-looking statements

This company announcement may contain certain forward-looking statements, including with respect to the timing of the Company's clinical trials and the expected submission of application for approval of arimoclomol for the treatment of NPC in the U.S and the EU. 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.