

Company announcement
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Orphazyme A/S
Ole Maaløes Vej 3
DK-2200 Copenhagen N

www.orphazyme.com
Company Registration No. 32266355

Orphazyme receives Breakthrough Therapy Designation for arimocloamol in Niemann-Pick Disease Type C (NPC)

- *There are currently no US FDA-approved products for NPC*
- *Orphazyme plans to file arimocloamol in the US in H1 2020*

Copenhagen, Denmark, November 19, 2019 – Orphazyme A/S (ticker: ORPHA.CO), a biopharmaceutical company dedicated to developing treatments for patients living with rare diseases, today announces that the US Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for arimocloamol, an investigational drug for the treatment of Niemann-Pick Disease Type C (NPC).

Kim Stratton, Chief Executive Officer at Orphazyme, commented, *"We are delighted with FDA's decision to grant Breakthrough Therapy Designation to arimocloamol for NPC. Arimocloamol has been shown to have a clinically meaningful effect on disease progression in NPC that is further supported by a biomarker effect indicating an effect on the biological underpinnings of the disease and a favorable safety and tolerability profile. We are committed to bringing this product to patients as soon as possible. Breakthrough Therapy Designation is designed to expedite the development and review of products for serious diseases with the direct involvement of senior staff and we look forward to working closely with the FDA to further advance arimocloamol. Our preparations for filing in the US are underway and we are on track to submit a New Drug Application in H1 2020."*

About Breakthrough Therapy Designation

Breakthrough Therapy Designation is a program intended to expedite the development and review of drugs to treat serious or life-threatening diseases in cases where preliminary clinical evidence shows that the drug may provide substantial improvements over available therapy.

For additional information, please contact

Orphazyme A/S

Kim Stratton, CEO +45 31 44 31 39
Anders Vadsholt, CFO +45 28 98 90 55

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 and lysosomal dysfunction. Arimocloamol, 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 arimocloamol

Arimocloamol 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. Arimocloamol is administered orally, crosses the blood brain barrier, and has been studied in seven Phase I and three Phase II trials. Arimocloamol 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 1,000-2,000. There are no approved treatments for NPC in the USA and only one approved product in Europe. Arimocloamol 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 statement

This company announcement may contain certain forward-looking statements. 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.

