

**Company announcement**  
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## **Orphazyme announces US Early Access Program for Niemann-Pick disease Type C**

**Copenhagen, Denmark, January 6, 2020** – Orphazyme A/S (ORPHA.CO), a biopharmaceutical company pioneering Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases, today announces the availability of an Early Access Program (EAP) in the United States (US) for its investigational drug arimoclomol for the treatment of Niemann-Pick disease Type C (NPC).

The EAP provides a pathway for patients with serious, life-threatening diseases or conditions who lack therapeutic alternatives to gain access to investigational drugs before they are approved.

Kim Stratton, Chief Executive Officer, commented, "Orphazyme is deeply committed to providing access to new therapies for people living with rare diseases such as NPC. There are currently no approved products to treat NPC in the US and we are pleased to make arimoclomol, a potential first-in-class innovative oral therapy, available pre-commercially to US patients through this program. We plan to file for approval of arimoclomol in the US in H1 2020".

Orphazyme has partnered with Clinigen Group to administer the arimoclomol EAP and support physicians interested in participation. The EAP is expected to remain open until arimoclomol becomes commercially available in the US.

**Healthcare professionals** can obtain details about the arimoclomol EAP by calling the Clinigen customer service team at +1 877-768-4303 or emailing [usmapoperations@clinigen.com](mailto:usmapoperations@clinigen.com). **Patients** seeking medical information should contact their physician.

Arimoclomol has received fast track, orphan drug, and pediatric disease designations from the US Food and Drug Administration (FDA) and recently received Breakthrough Designation from the FDA.

Orphazyme is currently evaluating how to offer early access in additional countries over time, contingent upon discussions with local authorities and our progress towards filing for regulatory approval or obtaining reimbursement.

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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](http://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 1 and three phase 2 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.

**About Clinigen Group**

Clinigen Group plc (AIM: CLIN) is a global pharmaceutical and services company with a unique combination of businesses focused on providing ethical access to medicines. Its mission is to deliver the right medicine to the right patient at the right time through three areas of global medicine supply; clinical trial, unlicensed and licensed medicines. The Group has sites in North America, Europe, Africa and Asia Pacific. In October 2018, the Group acquired CSM, a specialist provider of packaging, labelling, warehousing and distribution services, with sites in the US and Europe, and iQone, a specialist pharmaceutical company in Switzerland. For more information, please visit [www.clinigengroup.com](http://www.clinigengroup.com)

**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.