Investor news
No. 04/2019

Orphazyme A/S
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Orphazyme CMO to speak at FDA-funded rare disease data analytics platform launch event

Copenhagen, Denmark, September 17, 2019 – Orphazyme A/S (ticker: ORPHA.CO), a biopharmaceutical company dedicated to developing treatments for patients living with rare diseases, has been invited to participate in a meeting hosted by the National Organization for Rare Disorders (NORD) and the Critical Path Institute (C-PATH) to launch their joint initiative: the Rare Disease Cures Accelerator Data and Analytics Platform (RDCA-DAP). Funded by the US Food and Drug Administration (FDA), the goal of RDCA-DAP is to accelerate the process of moving therapies from bench to bedside for rare diseases.

Today, Orphazyme's Chief Medical Officer, Thomas Blaettler, MD, will participate in a moderated panel discussion on "Current Challenges in Rare Disease Drug Development," during which he will share Orphazyme's experiences in working to bring new treatments for orphan diseases to market. The event, taking place in Rockville, MD, USA, is open to the public and expected to draw attendance from regulators, patient organizations, clinicians, researchers, and pharmaceutical companies focused on rare disease drug development.

"Orphazyme is proud of the work we have done, in collaboration with leading disease experts and patient organizations, to develop arimoclomol for Niemann-Pick disease Type C (NPC) and other underserved patient communities" said Blaettler. "We are hopeful our experiences can help to smooth the path for others in pursuit of new treatments for those living with rare diseases."

Orphazyme has confirmed its filing strategy for arimoclomol in NPC with both European and US regulators and remains on track to file for its first marketing authorization, in NPC, during H1 2020. Arimoclomol is in clinical development for three additional indications of high unmet need: Amyotrophic Lateral Sclerosis (ALS), sporadic Inclusion Body Myositis (sIBM), and Gaucher disease.

For additional information, please contact
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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.

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.