Orphazyme Capital Markets Day 2018 – from Biology to Bedside

Copenhagen, May 29, 2018 – Orphazyme A/S, a biopharmaceutical company dedicated to developing treatments for patients living with rare diseases, announces that a Capital Markets Day will take place today.

The Capital Markets Day will be hosted by Chief Executive Officer, Anders Hinsby, who will be joined by Professor Frances Platt, University of Oxford, Dr Pedro Machado, University College London, and key members of Orphazyme’s Management Team.

At today’s Capital Markets Day, updates will be given on Orphazyme’s four clinical trials as follows:

- **Niemann-Pick disease Type C (NPC)**
  - Data presented from the completed observational trial NPC-001
    - Disease progression rate confirms assumption used to design the Phase II/III trial
    - Candidate biomarkers have been analyzed and confirmed as disease biomarkers
  - Status of arimoclomol Phase II/III trial (AIDNPC)
    - Last Patient Last Visit of randomized trial (NPC-002) accomplished; database lock and results expected in Q3 2018
    - Based on blinded data, no safety concerns were identified in the NPC-002 interventional trial
    - Open-label extension on-going

- **Gaucher disease**
  - On schedule to enroll first patient in Q2 2018

- **Sporadic Inclusion Body Myositis (sIBM)**
  - Update from Phase II/III trial
    - Investigator meeting held in the USA, including investigators from all 12 participating sites

- **Amyotrophic Lateral Sclerosis (ALS)**
  - New preclinical data supporting the potential of arimoclomol in ALS
  - Phase III trial
    - Phase III ALS trial design agreed upon with regulatory authorities
    - Trial design: 18-month, placebo-controlled trial including 212 patients. Interim analysis at 70% completion in H2 2020 and full analysis in H1 2021. Primary endpoint: Combined assessment of function and survival
    - Trial design and trial patient baseline characteristics were determined based on a systematic analysis of data from the largest publicly available repository of ALS clinical trial data (PRO-ACT) in conjunction with arimoclomol ALS trial data. Based on this analysis, an 18-month trial duration was chosen to increase the likelihood of demonstrating an effect on survival

- **Financials**
  - Orphazyme maintains the company’s ability to complete the four clinical trials in respectively NPC, Gaucher diseases, sIBM, and ALS with the company’s current cash position.

The full presentation, excluding slides from external speakers, will become available today on [www.orphazyme.com](http://www.orphazyme.com).
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.