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's Phase II/III trial in sporadic Inclusion Body Myositis fully enrolled

- *Study completion expected by end 2020*
- *Results expected in H1 2021*

Copenhagen, April 23, 2019 – Orphazyme A/S (ticker: ORPHA.CO), a biopharmaceutical company dedicated to developing treatments for patients living with rare diseases, today announced that its Phase II/III trial of arimoclomol for the treatment of sporadic Inclusion Body Myositis (sIBM) is fully enrolled. Performance of interim analysis and study completion expected in H1 2020 and by end 2020, respectively. Results are expected in H1 2021.

Anders Hinsby, Chief Executive Officer, said: "We are truly delighted about the strong support for this trial from the patient community and clinicians, which has been paramount to achieving enrollment at an impressive rate. Sporadic Inclusion Body Myositis is a relentlessly progressive and debilitating disease with no current treatment options available and we cannot waste any time in completing this trial in order to evaluate the potential of arimoclomol as a treatment for this disease".

The Phase II/III trial of arimoclomol for sIBM is a 150-patient, 20-month, randomized, double-blinded, placebo-controlled trial at 11 centers of excellence in the US and one in the UK. Orphazyme has initiated an open-label extension trial to which patients from the Phase II/III trial may enroll and continue treatment.

For additional information, please contact

Orphazyme A/S
Anders Hinsby, CEO +45 31 44 31 39

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. 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 sIBM
Sporadic Inclusion Body Myositis (sIBM) is a progressively debilitating muscle-wasting disease. sIBM is characterized by a build-up of protein aggregates and atrophy of muscle cells, which leads to weakness and over time severe disability. The estimated prevalence of sIBM is 24.8-45.6 per million or 17,000-31,000 patients in the USA and Europe. There are no approved treatments for sIBM. Arimoclomol has been granted Orphan Drug Designation (EU and USA) for the treatment of sIBM.

Forward-looking statement

This press release 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 press release 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.