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**Company announcement** No. 07/2020 Orphazyme A/S Ole Maaløes Vej 3 DK-2200 Copenhagen N

www.orphazyme.com Company Registration No. 32266355

# Preliminary results for 2019 and financial outlook for 2020

**Copenhagen, Denmark, February 4, 2020** – Orphazyme A/S (ORPHA.CO), a biopharmaceutical company pioneering Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases, today releases preliminary results for 2019 as well as the financial outlook for 2020.

Based on unaudited financial numbers, operating loss for the full year 2019 is expected to be approximately DKK 335 million and the cash position at the end of 2019 is expected to be DKK 123 million, both in line with the earlier communicated outlook from our Annual Report 2018, published on March 1, 2019. Further details will be provided in the Annual Report for 2019, including management report and audited financial numbers, which is expected to be released on February 28, 2020.

In 2020, Orphazyme expects to continue its development based on the positive arimoclomol data package made available to the market in 2019. Orphazyme currently expects to submit an application for approval of arimoclomol for the treatment of Niemann-Pick disease Type C (NPC) in H1 2020 in the US, for which the company has been granted Fast Track approval status. Furthermore, Orphazyme expects to release results from the on-going phase 2 trial for Gaucher disease in H1 2020 and continue its on-going phase 3 trial in Amyotrophic Lateral Sclerosis (ALS) and phase 2/3 trial in sporadic Inclusion Body Myositis (sIBM). Based on this continued high activity level, Orphazyme expects an operating loss in the range of DKK 500-540 million for the financial year 2020.

## 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 "ban," "project," "will," "can have," "likely," "should," "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. 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.