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 announces Interim Report First Half 2019

Copenhagen, Denmark, August 28, 2019 – Orphazyme A/S (ticker: ORPHA.CO), a biopharmaceutical company dedicated to developing treatments for patients living with rare diseases, today announces its interim financial results for the period January 1 – June 30, 2019 and corporate highlights.

"We have made substantial progress in the first half of 2019. We confirmed our filing strategy for arimoclomol in Niemann-Pick disease type C (NPC) with both the European and, more recently, the US regulators, and with this major step forward we will be introducing an Early Access Program for NPC during the fall. We are on track to file for our first marketing authorization during H1 2020 and continue our preparations for launch of arimoclomol in key markets. Arimoclomol is also in clinical development in three other indications of very high unmet need: Amyotrophic Lateral Sclerosis (ALS), sporadic Inclusion Body Myositis (sIBM), and Gaucher disease. We were particularly pleased with the great interest in our ALS phase 3 trial, which is now fully enrolled ahead of the expected timeline. This means that both potential registration trials in ALS and sIBM have now completed enrollment and we look forward to reporting headline results from the trials during the first half of 2021. Furthermore, we have completed enrollment in our phase 2 Gaucher disease trial and results are now expected in H1 2020" said Anders Hinsby, Chief Executive Officer of Orphazyme.

Anders Hinsby continued, *"As previously announced, I will hand over the position of Chief Executive Officer of Orphazyme to Kim Stratton on October 1, 2019. The change in leadership will allow the timely transformation of the company to be able to successfully make arimoclomol available to patients after an approval. It has been a great privilege to work closely with talented scientists, dedicated clinicians, and most of all the awe-inspiring patient organizations. I have always, along with our whole team at Orphazyme, been fueled by these close collaborations and the daunting task of matching the courage, commitment, and perseverance of our close collaborators."*

Business Highlights First Half 2019 and Subsequent Events

- Pivotal trial primary endpoint with arimoclomol for NPC showed 74% reduction in disease progression after 12 months, subgroups showed statistically significant efficacy. Anticipated H1 2020 filing submission in Europe and USA, following positive meetings with regulatory authorities
- Phase 3 trial with arimoclomol for ALS on-going; completed enrollment in July 2019. Topline results from full analysis remain on track for H1 2021
- Phase 2/3 trial with arimoclomol for sIBM on-going; completed enrollment in April 2019. Results from full trial expected H1 2021 (interim analysis expected H1 2020)
- Enrollment of phase 2 Gaucher disease trial completed in August 2019; results expected in H1 2020
- Several leads for new molecular entities that constitute potentially new intellectual property opportunities
- Appointed Kim Stratton as Chief Executive Officer, succeeding Anders Hinsby as of October 1, 2019
- Introduced a new board incentive program, a new share-based incentive program, and a new phantom share program in July 2019
- Strengthened balance sheet in August 2019 with EUR 9 million financing from Kreos Capital

Financial Results First Half 2019

- For the first six months of 2019, Orphazyme reported a net loss of MDKK 164 or DKK 8.21 per share (basic and diluted) compared to a net loss of MDKK 108 or DKK 5.41 per share (basic and diluted) for the same period in 2018
- Research and development expenses for the period totaled MDKK 142 compared to MDKK 94 for the same period in 2018
- General and administrative expenses for the period totaled MDKK 23 compared to MDKK 15 for the same period in 2018
- As of June 30, 2019, Orphazyme held cash totaling MDKK 226 compared to MDKK 513 as of June 30, 2018 and MDKK 395 as of December 31, 2018

Outlook

Orphazyme maintains the 2019 operating loss outlook in the range of DKK 315-345 million and the anticipated cash position at year-end 2019 to be greater than DKK >110 million, as published in the Annual Report of 2018 on March 1, 2019.

Conference Call

Orphazyme will be hosting an investor call at which Chief Executive Officer, Anders Hinsby, and Chief Financial Officer, Anders Vadsholt, will be presenting the Interim Report First Half 2019. The presentation will be followed by a Q&A session.

The call will be held on: **Wednesday, August 28, 2019 at 11.00 AM CEST/5.00 AM EDT.**

Dial-in details:

- Denmark: +45 32 72 80 42
- United Kingdom: +44 (0) 844 571 88 92
- United States: +1 6315 107 495

Event Title: Orphazyme Interim Report First Half 2019

Confirmation code: **8655918**

The presentation will also be available via webcast: https://edge.media-server.com/mmc/p/v2haoix5

After the call, the presentation will be available via the webcast link above.

For additional information, please contact

Orphazyme A/S

Anders Vadsholt, CFO +45 28 98 90 55

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.