Company announcement
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Orphazyme A/S
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Company Registration No. 32266355

Orphazyme announces Annual Report 2018

Copenhagen, Denmark, March 1, 2019 – Orphazyme A/S (ticker: ORPHA.CO), a biopharmaceutical company dedicated to developing treatments for patients living with rare diseases, today announced its Annual Report for 2018. Below is a summary from the report of Orphazyme's business progress, financial performance for the year, and guidance for 2019. The full report is attached as a PDF file and can furthermore be found on the Company's website www.orphazyme.com.

Anders Hinsby, Chief Executive Officer, said: "*2018 was a very important year for Orphazyme, with the initiation of two clinical trials and the successful completion of our Phase II/III clinical trial in Niemann-Pick disease Type C (NPC). For almost 10 years, we have now been steadfast in our mission to develop a treatment option for patients suffering from this severely debilitating disease, and we are now closer than ever to achieving this goal. We are looking forward to 2019, during which we will start the preparations to bring arimoclomol to approval for NPC*".

Business progress in 2018

Priority	**√**	**Targeted milestone**
ALS	√	• Initiate Phase III trial in Q3
sIBM	√ –	• Enroll patients in both USA and Europe • Complete Phase II/III trial enrollment by year-end
NPC	√	• Phase II/III top-line results in Q3
Gaucher	√ –	• Initiate Phase II trial in Q2 • Complete trial enrollment by year-end

Financial performance
The operating loss of DKK 231.6 million was below the expected operating loss range of DKK 245-275 million. The difference to the outlook for 2018 was mostly due to slower patient enrollment in our clinical trials.

Research and development expenses totaled DKK 196.5 million in 2018 compared to DKK 99.0 million in 2017. The increase was mainly due to the on-going NPC trial, the ramp-up of the sIBM Phase II/III trial, the initiation of the Phase II trial for Gaucher disease in June, and the initiation of the Phase III trial for ALS in August.

General and administrative expenses totaled DKK 35.1 million in 2018 compared to DKK 32.0 million in 2017. The increase is mainly due to increased employee costs, the initiation of pre-commercial activities as well as increased investor relations activities.

As of December 31, 2018, Orphazyme had cash DKK 394.7 million compared to DKK 631.7 million as of December 31, 2017. The decrease in cash results from the increase in research and development spend, as described above.

Guidance 2019

MDKK	2019 guidance	2018 actual result	2018 guidance
Operating loss	(315) – (345)	(232)	(245) – (275)
Cash position at year-end	>50	395	>350

Conference call and webcast

The call will be held today at 11.00 AM CET**.**

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Event Title: Orphazyme Annual Report 2018

Confirmation code: 1127327

The presentation will also be available via webcast: https://edge.media-server.com/m6/p/et2opupq

[PDF file attached].

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 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.

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.