Orphazyme announces Interim Report First Half 2018

The first part of 2018 has taken Orphazyme further along the path of bringing arimoclomol to rare disease patients in great need of new treatment options. The clinical development programs remain on track, with two major clinical milestones met by the initiation of the Gaucher Phase II trial and the ALS Phase III trial. Another major milestone is coming up in the very near term, with the reporting of top-line results from the Phase II/III trial of arimoclomol for the treatment of Niemann-Pick disease Type C (NPC).

Business Highlights First Half 2018

- Arimoclomol was granted Rare Pediatric Disease Designation for the treatment of NPC, entailing eligibility for a Priority Review Voucher
- A US subsidiary was established in Massachusetts, USA, to establish closer relationships with the medical, patient, and financial communities, headed by Chief Commercial Officer, Paul Merrigan
- First patient dosed in arimoclomol Phase II trial in Gaucher disease. Results expected in H1 2019

Subsequent Events

- First patient dosed in Phase III trial of arimoclomol for the treatment of Amyotrophic Lateral Sclerosis (ALS)

“The first half of 2018 has been very productive, and I am pleased that we have met our milestones with the progress of our clinical programs. We will keep working hard to maintain the forward momentum and remain fully dedicated to developing therapies for orphan diseases with high unmet needs. We are now looking forward to presenting the top-line results from our NPC trial, which are expected within weeks”, says Anders Hinsby, Chief Executive Officer of Orphazyme.

Financial Results First Half 2018

- For the first six months of 2018, Orphazyme reported a net loss of MDKK 108, or DKK 5.41 per share (basic and diluted) compared to a net loss of MDKK 52, or DKK 5.09 per share (basic and diluted) for the same period in 2017
- Research and development costs for the period totaled MDKK 94 compared to MDKK 47 for the same period in 2017
- General and administrative expenses for the period totaled MDKK 15 compared to MDKK 8 for the same period in 2017
- As of June 30, 2018, Orphazyme had cash and cash equivalents totaling MDKK 513 compared to MDKK 34 as of June 30, 2017 and MDKK 632 as of December 31, 2018

Outlook

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 2018. The presentation will be followed by a Q&A session.

The call will be held on: **Tuesday, August 28, 2018 at 11.00 AM CET.**

Dial-in details:

- Denmark: +45 35 15 81 21
- United Kingdom: +44 (0) 330 336 9411
- United States: +1 929-477-0324

Event Title: Orphazyme Interim Report First Half 2018

Confirmation code: **2088051**

The presentation will also be available via webcast: [https://edge.media-server.com/m6/p/5hejv92c](https://edge.media-server.com/m6/p/5hejv92c)

After the call, the presentation will be available by using the following dial-in details:

- Denmark: +45 70 14 50 87
- United Kingdom: +44 (0) 207 660 0134
- United States: +1 719-457-0820

Confirmation code: 2088051

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](http://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.