

**Investor news**

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**Orphazyme assumes sponsorship of Phase II/III sIBM trial**

On December 14, 2017, Orphazyme A/S, University of Kansas Medical Center, and UCL announced that Orphazyme formally assumes the sponsorship of the Phase II/III arimoclomol trial for the neuromuscular disease sporadic Inclusion Body Myositis (sIBM).

The trial was conceived by expert investigators at KU Medical Center and UCL to assess the efficacy and safety of arimoclomol for the treatment of sIBM. The KU Medical Center and UCL teams bring long-standing knowledge on the basic and clinical science of sIBM.

The multi-centre trial initiated patient enrolment in August 2017 in the USA and Europe and so far, 9 patients have been enrolled. The trial is intended to support a registration of arimoclomol for the treatment of sIBM. Results from the trial are expected in H1 2020.

Anders Hinsby, CEO of Orphazyme said: *"With the first dosing of patients in the sIBM study, we mark an important milestone in the development of our pipeline. We hope this trial, conducted with the foremost experts in the field, will lead to the first effective treatment for this devastating disease."*

Richard Barohn, Vice Chancellor for Research and Professor of Neurology at the KU Medical Center said: *"This trial is the culmination of a sustained, investigator-led effort to move from cells, through animal studies, to patients. We are happy to work with Orphazyme to explore arimoclomol's potential."*

Professor Michael Hanna, Director of the MRC Centre for Neuromuscular Diseases and the UCL Institute of Neurology, said: *"Arimoclomol has strong potential benefit in patients with this disabling disease. We made the case quite strongly that arimoclomol should be properly tested in a large trial, and it is most exciting that we have now started the next step in the clinical development."*

Professor Mazen M. Dimachkie, Principal Investigator of the study and Director of the Neuromuscular Division at KU Medical Center said: *"The situation is dire for the patients as they progressively lose dexterity, mobility and independence. We do not have any effective treatment for this major unmet medical need, and I am excited to be leading this important trial."*

**For additional information, please contact****Orphazyme**

Anders Hinsby, CEO

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**About sIBM**

The disease is one of several neuromuscular diseases and is one of the most common wasting muscle disorders, affecting patients in the later stages of life, typically over the age of 50 years. The size of the patient population in Europe and the United States is not fully elucidated but has been conservatively estimated to be between 7,000 and 15,000 individuals. No effective treatment is currently available.

**About arimoclomol**

Arimoclomol is a new chemical entity with a very favorable safety and tolerability record in humans: Seven Phase I clinical studies have been conducted in healthy volunteers. Arimoclomol is administered orally, three times daily, and can be easily dissolved in liquids or food for best possible patient comfort and compliance. Orphazyme has obtained orphan drug designation for arimoclomol in NPC, ALS, and sIBM from both the FDA and EMA.

**About Orphazyme A/S**

Orphazyme is a Danish biotech company listed on Nasdaq Copenhagen with a late-stage drug pipeline, developing new treatment options for orphan protein-misfolding diseases. The company was founded in 2009 based on early scientific discovery in heat-shock proteins. The company is headquartered in Copenhagen and currently has 30 employees. The lead candidate arimoclomol is in development as a potential treatment for four orphan diseases; two neuromuscular diseases, sporadic Inclusion Body Myositis ("sIBM") and Amyotrophic Lateral Sclerosis ("ALS"), and two lysosomal storage diseases, Niemann Pick Type C ("NPC") and Gaucher disease. For more information, please visit [www.orphazyme.com](http://www.orphazyme.com).

**About University of Kansas Medical Center**

The University of Kansas Medical Center's mission is to educate exceptional health care professionals through a full range of undergraduate, graduate, professional, postdoctoral, and continuing education programs in the schools of Medicine, Nursing, and Health Professions. KU Medical Center also advances the health sciences through world-class research programs; provides compassionate and state-of-the-art patient care in an academic medical center environment; and works with communities in every Kansas county to improve the health of Kansans.

**About UCL**

UCL was founded in 1826. We were the first English university established after Oxford and Cambridge, the first to open up university education to those previously excluded from it, and the first to provide systematic teaching of law, architecture, and medicine. We are among the world's top universities, as reflected by performance in a range of international rankings and tables. UCL currently has over 39,000 students from 150 countries and over 12,500 staff. Our annual income is more than £1 billion. [www.ucl.ac.uk](http://www.ucl.ac.uk) | Follow us on Twitter @uclnews | Watch our YouTube channel [YouTube.com/UCLTV](https://www.youtube.com/UCLTV).