

March 2020

A message from Orphazyme to the global community of patients, family members and healthcare providers impacted by the corona virus pandemic (COVID-19):

This is an uncertain and difficult time for us all. As we face these challenges, Orphazyme's top priority is the health and safety of patients and our employees. We have taken responsible measures, in line with local guidance, to protect the health of our colleagues and local communities. At the same time, we are being proactive in our approach to collaboration with clinical and business partners to mitigate the spread of COVID-19 while continuing to advance our clinical development programs in Niemann-Pick Type C (NPC), Amyotrophic Lateral Sclerosis (ALS), sporadic Inclusion Body Myositis (sIBM) and Gaucher disease.

At this time, our clinical trials are expected to remain ongoing with expert guidance and support from investigators and partners around the world. Our trials were designed to limit the burden of participation for patients and their families where possible. As such, we are already utilizing novel measures such as telehealth, home nursing services, 'direct to patient' shipment of investigational medicinal product (IMP) and limiting the need for patient travel to try and minimize the potential for exposure to the virus.

Orphazyme has also established a network of clinical sites and research organizations to ensure that we are aware of the most recent COVID-19 requirements and restrictions. Finally, for individuals with NPC living in the United States (US), we remain committed to making arimoclomol available through an [early access program \(EAP\)](#). Please speak to your healthcare provider regarding questions about the EAP in the US.

We recognize that for individuals and families managing complex medical conditions, this is an especially worrisome time and encourage patients with specific medical questions related to COVID-19 to contact their healthcare providers for more information.

In the days and weeks to come, Orphazyme will continue to communicate openly with patient organizations and clinical trial investigators around the globe to share the latest information regarding our efforts as the situation develops. Meanwhile please utilize attached FAQs to answer questions if any about our trials.

Please be safe and stay well,

Thomas Blaettler, MD
Chief Medical Officer, Orphazyme

About Orphazyme: Orphazyme is a Danish biopharmaceutical company with a late-stage drug pipeline, developing new treatment options for orphan protein-misfolding diseases. Orphazyme develops new therapies that target the body's heat shock response by aiming to mobilize a group of stress response proteins, called heat shock proteins. Heat-shock proteins are believed to restore balance to cells affected by stress arising from misfolded proteins, protein aggregates and lysosomal dysfunction.

Please go to www.orphazyme.com to learn more.