June 2, 2020

A COVID-19 Update from Orphazyme on its Early Access Program for Niemann-Pick Disease Type C:

We are now several months into the COVID-19 pandemic, and the Orphazyme team in the United States (U.S.) has been adjusting, just like the millions of American families seeking to maintain normal activities. We recognize the unique burden the public health crisis places on families managing treatment of rare conditions.

In January, we announced an Early Access Program (EAP) in the U.S. for Niemann-Pick Disease Type C (NPC). (To learn more about our Orphazyme's policies around Early Access Programs, please see the link to the policy directly from our main page at www.orphazyme.com.)

We now want to provide healthcare providers and the NPC community with an update on the program and the modifications we are making to help protect the safety of participants.

At the discretion and within the guidelines set by each site, for the duration of the public health crisis, healthcare providers at participating sites can conduct baseline evaluations and subsequent visits of patients through telemedicine. Treatment can also be shipped directly from the site to the patient's home.

Also, at the discretion of the site, if an in-person visit isn't necessary during this time, the healthcare provider can waive the requirement for a physical examination for patients seen relatively recently. In addition, the healthcare provider can have the required lab work collected at a facility close to the patient's home.

The up to date list of participating sites for the NPC EAP is available at https://clinicaltrials.gov/ct2/show/NCT04316637, along with additional information on the program.

The NPC EAP in the U.S. is expected to remain open until the investigational drug arimoclomol, an orally administered small molecule, becomes commercially available in the U.S. On May 28th, 2020 Orphazyme initiated the submission of its New Drug Application (NDA) for a rolling review by the U.S. Food and Drug Administration (FDA) for arimoclomol. In addition, arimoclomol has received fast track and breakthrough* therapy designations, as well as orphan drug and rare pediatric disease designations from the U.S. FDA.

Orphazyme has partnered with Clinigen Group to administer the NPC EAP and support healthcare providers interested in participation. Healthcare providers can obtain details about the NPC EAP by calling the Clinigen customer service team at + 1-773-770-6888, or by sending an email to the organization at usmapoperations@clinigengroup.com.

Inquiries not related to the EAP should be directed to usmedicalaffairs@orphazyme.com, and patients seeking medical information should contact their physician.

Please stay safe, and please know that Orphazyme remains relentlessly focused on pioneering the heat-shock protein response to deliver on our commitment to the patients and communities we serve.

We look forward to sharing additional updates in the months ahead.

Sincerely,
Molly Painter
U.S. President, Orphazyme

*https://www.fda.gov/patients/learn-about-drug-and-device-approvals/fast-track-breakthrough-therapy-accelerated-approval-priority-review

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. For more information, please visit www.orphazyme.com .