



January 6, 2020

Today, Orphazyme is pleased to announce the availability of an Early Access Program (EAP) in the United States (US) for our investigational drug arimoclomol for the treatment of patients with NPC. Further details regarding this announcement can be seen in a [company press release](#). Orphazyme's EAP will provide access to eligible patients in the US as determined by their treating physician.

We recognize that availability of this program may raise a number of questions for those living with NPC and their families. To this end, we hope to provide some context for how information and communication pertaining to investigational medicines are governed in the US. To ensure patient safety, the FDA dictates how investigational medicines are developed and approved for use, as well as how information about these medicines is communicated. Orphazyme abides strictly by these guidelines, which are put in place to guard patient safety before an investigational medicine has been approved.

In practice, this means that sometimes Orphazyme is not able to communicate in ways that patients and their families would ideally like us to. For example, we recognize that some of you may be interested to learn additional details about the arimoclomol EAP, such as inclusion/exclusion criteria, concomitant medication use, the process to request early access, or other program specifics. All of these issues and questions should be directed to your physician. Together, you can discuss your care options and make decisions that are best for your unique needs.

Detailed information about the program can be made available to interested healthcare professionals upon their request. Healthcare professionals interested in more information may contact Clinigen, a company engaged by Orphazyme to administer our EAP: +1-877-768-4303, usmapoperations@clinigengroup.com.

We are pleased to initiate early access to arimoclomol for those living with NPC in the US through this program. It is our intent to offer early access to a number of additional countries over time, contingent upon discussions with local authorities and our progress towards filing for regulatory approval or obtaining reimbursement. As additional details pertaining to our regulatory filing with FDA to seek approval become available, we will continue to share updates with the community through the NNPfD and other patient organizations.

Sincerely,

Thomas Blaettler, MD
Chief Medical Officer
Orphazyme A/S