

Update from Orphazyme

NNPDF has received the following communication from Orphazyme regarding this week's [press release](#) on arimoclomol programs for NPC:

Arimoclomol clinical trial and expanded access programs will continue for the current time

Dear Joslyn and Justin:

We are writing to inform you that the CT-ORZY-NPC-002 trial activities as well as ongoing early-access and named-patient programs for arimoclomol in NPC will continue for the current time.

As you may have seen, Orphazyme participated in an ad-hoc expert group meeting and an oral explanation last month as part of the evaluation of the European marketing authorization application for arimoclomol in NPC. The Committee for Medicinal Products for Human Use (CHMP) subsequently informed us of a negative trend vote following the oral explanation. As a consequence, we have carefully assessed all options and decided to withdraw the Marketing Authorization Application in EU for arimoclomol prior to the formal CHMP opinion. This decision enables us to consider the best possible path forward for arimoclomol in EU.

Considering the Company's financial position, Orphazyme has now entered in-court restructuring. This is a process designed to assess the basis to secure the activities of the company going forward including a basis for injecting further capital and/or a potential sale of all or some assets in the company.

We firmly believe that arimoclomol has the potential to help NPC patients and we are motivated to explore options for a regulatory path forward in both Europe and the US. We remain focused on preparing for the Type C meeting with the FDA. Our core team of employees is committed to facilitating continued access to arimoclomol to NPC patients during this time, and we aim to keep you informed should there be any significant changes to these programs.

Our motivation has always been the aim to provide NPC patients with a potential new treatment option. We therefore deeply appreciate all the hard work you are doing to help NPC patients, the NPC community, and the arimoclomol program.

Please do reach out to Christine or myself if you have any questions.

Sincerely,

Tara Greene

*Sr. Director, US Medical Operations and US Patient Advocacy
Orphazyme US, Inc.*