



As many of you know, Orphazyme has been engaged with the global NPC community for a number of years, in line with our objective to develop new therapies for patients where there are no or limited treatment options available. Since mid-2019 our team has been in discussions with regulatory agencies and local authorities in the United States (US) and Europe, regarding our intent to seek approval of our investigational drug, arimoclomol, as a treatment for NPC.

As this process progresses, we have also been working diligently towards making arimoclomol available to NPC patients through an early access program (EAP). In many countries, EAP (also known as expanded access or managed access programs) provides a pathway for patients with serious, life-threatening diseases or conditions who lack therapeutic alternatives to gain access to investigational drugs, such as arimoclomol, before they are approved.

Despite our best efforts, I regret to share with you that we will not meet our goal of introducing an EAP this fall. However, I can assure that development of the program is very much under way and we look forward to announcing its availability in early 2020.

Orphazyme's primary aim is to obtain approval for arimoclomol as a treatment for NPC in the US and EU, with the long-term goal of achieving access for all patients in need. To ensure we maintain the focus and resources to achieve these goals, early access to arimoclomol as an investigational therapy must necessarily be made available on a country-by-country rolling basis. For these reasons, Orphazyme's EAP will plans to initially provide access to arimoclomol pre-commercially to individuals with NPC in the United States. 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.

At this time, we are not able to comment on timelines or progress with specific countries outside of the US. However, we remain committed to doing what we can for NPC patients in need and we will continuously review our ability to make arimoclomol available through EAP to additional countries.

As always, we thank the patients and families who have been involved with our clinical program, many of whom continue to receive arimoclomol through an open-label extension study. We also thank the many others who have supported us through participation in research surveys, telephone interviews and who have shared insights with us about the realities of living with NPC – the truth of which grounds us in the urgency of our work.

Please know that we fully appreciate the urgent need to bring arimoclomol to patients with NPC and embrace our responsibility to achieve this goal.

Sincerely,

Thomas Blaettler, MD
Chief Medical Officer
Orphazyme A/S