Therapeutic Update

ORPHAZYME

Orphazyme Global Update: Progressing Arimoclomol in NPC

Despite the continued global impact of the coronavirus pandemic, our teams around the world have remained focused on our commitment to impact the lives of people living with neurodegenerative orphan diseases and their families. We are pleased that clinical development and regulatory filing activities for our investigational treatment, arimoclomol, have continued as scheduled this year, with minimal interruptions to our timelines.

Below is a summary of our latest activities.

Orphazyme Progress in the US

In September, the U.S. Food and Drug Administration (FDA) accepted our New Drug Application (NDA) for priority review. As we await feedback from the FDA, we are simultaneously expanding our US footprint, infrastructure, and team support, in preparation for potential commercialization. Meanwhile we have assembled three steering committees (payers, HCPs, and NPC caregivers) to share their valuable experience and feedback to ensure our plans are designed to effectively support the US NPC community.

Our Early Access Program (EAP) is now active at multiple US institutions. COVID-related challenges impacted the pace of activation for some locations; however, we continue to work diligently to bring additional locations online while prioritizing patient safety and providing a pathway to access arimoclomol prior to FDA action.

To get the latest information on the US NPC EAP, please visit clinicaltrials.gov/ct2/show/NCT04316637.

To learn more about Orphazyme's policies around Early Access Programs, please visit orphazyme.com.

Orphazyme Plans in Europe

As our filing in the US progresses, we are also preparing for a marketing authorization submission to the European Medicines Agency (EMA) in the EU. We remain on schedule to complete this submission before the end of the year.

We believe that commercial approvals will provide the best pathway for people living with NPC to gain access to arimoclomol. At the same time, as indicated earlier this year, we have been exploring the possibility of early access in a limited number of EU countries where an established system for early access exists, such as France, Italy, and Germany. We are not able to comment on timelines or progress at this time. However, we are committed to doing what we can for people living with NPC and will continue to review our ability to make arimoclomol available through EAPs to additional countries.

Growing the Orphazyme Team

We are building a robust footprint to support these efforts in the US and EU. Our team has grown to include experienced professionals in the areas of product strategy, manufacturing and supply, operations, marketing, market access, and a number of other critical functions. In addition to our headquarters in Copenhagen, Denmark, we have also established key offices in Zug, Switzerland and Chicago, IL, USA.

While we remain a small company with a lean team, we are committed to making arimoclomol available to people and families living with NPC.

Continuing Community Support

As we enter October, we look forward to introducing a new NPC awareness campaign in conjunction with Global Niemann-Pick Awareness Day on October 19th. We are excited to share this family-friendly resource with you and hope you will help us to spread the word about this initiative with your constituents. More to come!

In the meantime, we are pleased to release a new video aimed to improve awareness and understanding of NPC. Thank you to the families who supported development of this resource by sharing their experiences with NPC. Please feel free to share this video with your constituents as you **see** appropriate.

We are dearly missing the opportunity to connect with community leaders in person at annual events. We are grateful to our patient organization partners, including NNPDF and NPUK, for creating new platforms to stay connected and engage virtually in these challenging times. If your organization has planned virtual activities that you would like Orphazyme to be a part of, please do reach out to let us know.

We look forward to the opportunity to gather virtually to exchange ideas, share updates and learn from each other at events in the future. In the meantime, we look forward to sharing updates through the INPDA and other NPC partners as new information becomes available.

As always, if you have questions or would like to connect with us, please contact Regan Sherman, Head, Global Patient Advocacy Relations – res@orphazyme.com.

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