Therapeutic Update

2020 is a year none of us will soon forget. As we turn a corner into 2021, we are pleased to respond to the INPDA's request for an update on our regulatory filings and early access programs. As always, Regan Sherman, Head of Global Patient Advocacy Relations, is available to INPDA members for additional dialogue: res@orphazyme.com

We begin by saying, "thank you." Orphazyme's manuscript "Clinical disease progression and biomarkers in Niemann–Pick disease type C: a prospective cohort study" has been accepted for publication in Orphanet Journal of Rare Diseases (OJRD-D-20-00391R1). We are thrilled to have this knowledge about NPC published and are grateful to the patients and families who participated in this study. Thank you for your commitment to advancing research; we hope this work will support future efforts to find treatments for NPC.

Update on FDA & EMA Application Status

In November 2020 we announced the submission of a marketing authorization application (MAA) to the European Medicines Agency (EMA). The timing of this submission is consistent with our previously communicated timelines, an achievement we are proud of in a year when all were challenged by the COVID-19 pandemic. Arimoclomol has been granted Orphan Drug Designation in Europe and we look forward to working with EMA as they complete their review of our application. We anticipate a regulatory decision from EMA in 2021.

In December 2020 we announced that the U.S. Food and Drug Administration (FDA) had requested an extension to the review period of our New Drug Application (NDA). The standard extension period of 90-days was requested; we understand this additional time is necessary for the FDA to complete its review. Our application retains its priority review status and we anticipate a decision by the updated Prescription Drug User Fee Act (PDUFA) target action date of June 17, 2021.

Early Access Programs

Orphazyme's Early Access Program (EAP) in the United States is now active at multiple institutions. To get the latest information on the US NPC EAP, please visit https://clinicaltrials.gov/ct2/show/NCT04316637.

We have also established EAP programs for patients living with NPC in France and Germany. We are investigating Early Access opportunities in some additional European countries; however, are not able to comment on timelines or progress at this time. As always, 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 in additional countries.

To learn more about Orphazyme's policy on Early Access, please visit www.orphazyme.com. Any questions regarding access to arimoclomol should be discussed with a qualified medical professional.

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