Therapeutic Update

Arimoclomol Clinical Development Program

Congratulations on the first publication of your new INPDA member newsletter! It was good to see many of you at the INPDA member meeting in June and again in Wyboston at the recent Niemann-Pick UK (NPUK) family conference. Thank you for engaging with our team at these events, for your thoughtful questions and counsel as we continue to work towards a new potential treatment option for those living with NPC.

The team at Orphazyme has been hard at work this summer preparing to file for approval of arimoclomol with regulators in the US (FDA) and Europe (EMA). At the same time, we are also preparing to launch an early access program (EAP) of arimoclomol in NPC and plan to begin the rollout of the program later this fall. More details will be made public via a company press release at the time of the program launch.

Some of you have contacted Orphazyme regarding our study of arimoclomol in young children, ages 6 months to <2 years. Enrolment of new patients is currently awaiting necessary updates to the study protocol. We will notify the community through INPDA once enrolment has resumed.

You may recall that Orphazyme is conducting a survey of individuals with NPC and their carers. We anticipate that approximately 60 patients or their carers will complete an online questionnaire, and that 30 will then participate in a telephone interview. The goal of the survey is to understand the impact of NPC is on a person's life; specifically, how each of the five domains of the 5-domain NPC Clinical Severity Score (NPCCSS) -- swallowing, ambulation, cognition, speech and fine motor skills -- and any other factors identified as important to the respondent, have an impact health related quality of life (HRQOL) and activities of daily living (ADLs).

Patient and carer participation in the survey will help us to understand how changes to a person's score on the NPCCSS make a difference in their daily lives. The importance of these changes will then be explored to understand what is clinically meaningful to people living with NPC. Additionally, study results will help us to better understand the experiences or "journey" of individuals and families with NPC, from initial symptoms to diagnosis and beyond. This will help us to identify major events impacting the person and/or family's life.

Our deep thanks go out to NPUK and NNPDF for their support of this important work. Responses will help us to further validate the 5-domain NPCCSS, the tool used in our clinical study to measure disease severity. Findings will also help regulatory authorities and payers to understand the impact of NPC and medical needs of individuals and families.

If you have questions or would like to discuss any of these updates, please contact Regan Sherman, Associate Director, Patient Advocacy Relations – res@orphazyme.com.