

**Orphazyme Update Re: Arimoclomol Clinical Development Program**

To the INPDA's global community of Niemann-Pick disease advocates: thank you for the opportunity to provide you with an update on our team's work to progress the investigational drug candidate arimoclomol for the treatment of Niemann-Pick Disease type C (NPC).

In January, Orphazyme reported positive data from the 12-month open label extension portion of our phase 2/3 trial investigating arimoclomol as a potential treatment for NPC. As we have publicly reported, these new data *demonstrate a sustained effect on slowing disease progression over a two-year period. Data also show that study participants who were initially randomized to placebo in the placebo-controlled trial experienced a 90% reduction in disease progression compared to the previous year when switched to arimoclomol treatment in the open label extension. Importantly, there were no new safety issues detected.*

With this encouraging new data and following positive conversations with regulators over the course of 2019, Orphazyme is preparing to file a New Drug Application for arimoclomol in NPC with the US Food and Drug Administration (FDA) towards the end of the first half of 2020. We plan to submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) in the second half of this year.

Also in January, Orphazyme announced the availability of an early access program (EAP) for arimoclomol for the treatment of NPC in the US. 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 will continuously review our ability to make arimoclomol available through EAP to additional countries.

Since our last update, we have welcomed several leaders to our executive team. We look forward to opportunities to introduce you to these new team members over the course of the coming year:

- Kim Stratton, Chief Executive Officer
- Molly Painter, US President
- Angus Hogg, Head of Global Product Strategy
- Julia Barr, Head of Global Strategy & Operations

Our team is eager to engage with NPC advocates throughout 2020. A schedule of planned conference participation is provided below. Please contact Regan Sherman if you are interested to speak with our colleagues in conjunction with any of these meetings:

- *WORLDSymposium* Lysosomal Disease Conference, February
- Society for Inherited Metabolic Disorders Annual Meeting, April
- Ara Parseghian Medical Research Fund (APMRF) Annual Conference, May
- National Niemann-Pick Disease Foundation (NNPDF) Annual Family Conference, July
- Niemann-Pick United Kingdom (NPUK) Annual Conference, September
- Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Congress, August
- Child Neurology Society Congress, October

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](mailto:res@orphazyme.com).