Orphazyme Update Re: Arimoclomol Clinical Development Program

As the global coronavirus pandemic has reached nearly every corner of the globe, its effect has challenged each of us in different ways. For many members of INPDA's global community of Niemann-Pick disease advocates, we know that this time has created unique challenges.

For Orphazyme, we're doing what we can to support our communities and to stay focused on our mission. Know that we are maintaining momentum for all our programs for our investigational therapy arimoclomol for the treatment of Niemann-Pick Disease type C (NPC).

With over 150 employees located in Denmark, Sweden, Switzerland and Unites States, like many of you, we are relying on video conferences to stay connected and learning how to contribute meaningfully to the community in virtual forums. We are also partnering closely with our clinical research partners, including investigators and institutions around the world, to ensure our clinical development programs continue while prioritizing patient safety.

Arimoclomol development program
Clinical Trial Updates

- Future NPC planned study: <2 years old (Infant Study) planned to start second half of 2020

We plan to participate in community congresses and events throughout 2020, regardless of their format. A schedule of planned events is listed below. Please contact us if you are interested in speaking with our colleagues in conjunction with any of the meetings.

- 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

Making progress toward registration
On May 29, Orphazyme initiated the submission of its New Drug Application (NDA) for a rolling review by the US Food and Drug Administration (FDA) for arimoclomol, in NPC. Orphazyme expects to complete submission of the remaining portions of the NDA to the FDA in the next couple of months.

Arimoclomol has received fast track, orphan drug, rare pediatric disease and -breakthrough therapy designations from the U.S. FDA for NPC. Breakthrough Therapy Designation* is a U.S. program intended to expedite the development and review of drugs to treat serious or life-threatening diseases in cases where preliminary clinical evidence shows that the drug may provide substantial improvements over available therapy.

In Europe, we are still on schedule to submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) later this year.

Early Access Program
In January, we announced an Early Access Program (EAP) in the United States (U.S.) for NPC.
For the duration of the public health crisis, we have put in place modifications for the U.S. EAP to protect the safety of participants.

Healthcare providers at participating sites – and at the discretion and within the guidelines set by each

institution – are able to use telemedicine systems to conduct visits and are able to have treatment shipped directly from the site to the patient's home.

The get the latest information on the U.S. NPC EAP, please visit https://clinicaltrials.gov/ct2/show/NCT04316637.

It is our intent to offer early access to a limited number of additional countries over time, contingent upon discussions with local regulatory authorities and our progress towards filing for regulatory approval or reimbursement

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

Supporting the community

We continue to add to our team by expanding our commercial and medical teams in the Europe and U.S.! Recently Dr. Stefan Kolb joined us as Senior Global Medical Affairs Director for Lysosomal Storage Diseases.

At Orphazyme, our people, science, discipline and drive are all focused on one thing—the patient. We're committed to helping make a meaningful difference by collaborating with the community through scientific and therapeutic innovation and ultimately in bringing a treatment to those living with rare diseases.

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.

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www.orphazyme.com

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