Mandos Health

October 2024

To the NPC1 Community,

We are excited to share important updates on our progress and commitment to supporting novel therapies for Niemann-Pick Type C (NPC) Disease. Here is the overview of topics we cover in this newsletter:

1. Development Program Update
2. Community Update
3. Expanded Access Program

Development Program Update

We are pleased to announce we had a productive Type C meeting with FDA on October 10. The FDA were encouraged by our progress with the program, and we are grateful for the FDA's thoughtful collaboration on the development program.

As a PBC, we strive to include the voice of our stakeholders in our work. We would like to thank Dr. Liz Berry-Kravis (EAP), Dr. Denny Porter (NIH), & Dr. Justin Hopkin (NNPDF) for their partnership and attendance at this important meeting to represent our external stakeholders - the clinicians, the researchers, and especially the patients and their families.

Mandos Health is continuing our development program, and we look forward to sharing our data findings with the NPC community and thought leaders in the coming months.

Community Update

Mandos Health celebrated NPC Awareness Month this month with several updates and links to our team on the NNPDF and INPDA community social media posts focused on patients and caregivers (warriors), and awareness and education.

Mandos Health recently attended the Alliance for Patient Access (AfPA) Policy Summit, the Reagan-Udall Foundation (reaganudall.org) FDA Rare Disease Innovation Hub public meeting, and the NORD Breakthrough Summit.

We enjoy engaging all stakeholders with our communications, conference attendance, and patient advocacy organization meetings.

Expanded Access Program (EAP) Update

Mandos Health continues to work with the clinicians, researchers and sites who provide expanded access and care to the NPC patients and their families.

Stay up to date with our progress

We are committed to continuing to build a science-driven organization dedicated to NPC.
Stay up to date with us at **www.mandoshealth.com**

**Adrabetadex is an investigational drug that has not been approved by FDA, and FDA has not found it to be safe and effective for use to treat NPC1 or for any other use.*

Mandos Health

A world of support for all affected by NPC1

Research. Access. Collaboration.

#NoStoneLeftUnturned #TheRightThingToDo #BuiltForPatients

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