

# Mandos Health

**November 2022**  
**To The NPC1 Community,**

We're excited to share some important updates on our progress:

## **One Year Anniversary & EAP Update**

It has been more than one year since Mandos officially joined the NPC Community with adrabetadex\*. We understand that the community continues to be concerned about the possibility of losing adrabetadex, this message was heard loud and clear at the NNPfD conference.

*We want to reinforce that we will continue to provide adrabetadex for the North American Expanded Access Program (EAP) per the criteria regarding risk/benefit from FDA.*

## **Community Update**

Cathy Traz has joined Mandos as our Patient and Community Engagement lead. Please feel free to contact her at [patientadvocacy@mandoshealth.com](mailto:patientadvocacy@mandoshealth.com)

Mandos is now planning to attend a number of NPC conferences to further our engagement. This is all possible due to the fast and dedicated efforts on the NPC development program and our continued commitment to moving the development program forwards.

Mandos attended the 30th Annual NNPfD/INPDA Conference recently with great success. Presentations and more information on the conference can be found here: <https://www.nnpdfconf.org/>

Mandos also attended the NPUK Annual Family Conference & Interactive Workshop on Niemann-Pick Diseases 2022 in Bedfordshire, England. More information can be found here: <https://www.npuk.org/about-us/>

We recognized October as NPC Awareness Month, and shared with our team information at <https://nnpdf.org/npc-disease-awareness> and <https://www.inpda.org/>

## **Development Program Update**

We are excited to be working with our partners on gaining access to sources of data that stem from natural history databases and registries (i.e., YODA, INPDR Gateway Ltd, NIH).

Firsts... Natural history hasn't been examined to this extent. And for many collaborators this is the first time working with a sponsor like this. These are terrific examples of the community coming

together and we want to say thank you. This also exemplifies just how expansive our strategy is and we're very excited to continue moving ahead.

We are in the final stages of compiling all the data needed, then we can complete all our analyses which support our development plans.

We intend to engage with the FDA for input on development plans to inform a potential path forward.

As we move ever deeper into data gathering, analyses, and regulatory preparations, you can continue to expect to see regular updates from us. As always, we are working as diligently and expeditiously as we can in our analysis without sacrificing data quality and look forward to continuing to keep everyone updated on our progress.

Every single day matters to this community. We will ensure no stone is left unturned.

## **Stay up to date with our progress**

We are committed to building a positive patient experience as a science-driven, transparent organization engaging all stakeholders with updated communications, conference attendance, and patient advocacy organization meetings.

Stay up to date with us at  
[www.mandoshealth.com](http://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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