

The following information was shared with NNPDF by Mandos Health and is also available at mandoshealth.com/communications

Mandos Health Communication, September 1, 2021

To The NPC1 Community,

September 6th will mark 60 days since we closed on the acquisition of adrabetadex*. Wow! We said at the time that we expected some busy days and weeks ahead and that has certainly proven to be true. We've been touched by everyone who has reached out to us with so much encouragement and passion and our whole team is proud to be working on this program.

As shared on our website at www.mandoshealth.com, we are committed to equitable communications, which means disseminating as much information as we can across the entire NPC community simultaneously, so we hope you will find this monthly update to our website helpful, timely and effective. We wanted to use this communication to provide some details on what we have been doing to date and also what we have planned in the coming months.

Integration Work (Mallinckrodt to Mandos)

- Transferred the Investigational New Drug application (IND) for the adrabetadex clinical program to Mandos
- Resolved partial clinical hold placed on adrabetadex clinical trial program. The hold has been lifted, and patients in clinical trial receiving benefit are able to continue on treatment
- Transferred full clinical database for adrabetadex from Mallinckrodt to Mandos
- Amended contracts with multiple Expanded Access Program (EAP) sites to provide for additional patients to be treated with adrabetadex via expanded access program
- Initiated work with contract research organizations and suppliers to support existing expanded access program and potential clinical development needs
- Coordinating with Mallinckrodt as they close out clinical studies 301, 302, and 001
- Preparing to enroll clinical patients who are currently receiving benefit and wish to continue treatment into the EAP

We would like to extend our thanks to the NPC team at Mallinckrodt who have worked tirelessly throughout this transition period thus far, and we look forward to completing the transfer of responsibilities in the near future with their continued dedication and enthusiasm. We are pleased to announce that not only have we avoided any interruption to patient treatments but have also been able to provide access to several new patients who were in urgent need of treatment.

Review of existing data from Mallinckrodt and Expanded Access Program

- We have taken an innovative approach to our review of the data and are performing an in-depth review of the totality of adrabetadex data that is available to us
- We are also evaluating the full breadth and manifestations of the shortcomings in the clinical trial design
- We are very much looking forward to presenting our analysis to the community, regulators, and other stakeholders in the future
- We are happy to engage with as many key opinion leaders and treating investigators as possible to better understand the full range of responses observed to adrabetadex. For any physicians who would like to be in touch but haven't connected with us yet, please [contact us here](#).

We will continue to expeditiously and thoughtfully analyze and report on both the data signals and the trial design implications.

External Engagement Activities included

- Press release announcing closing of acquisition
- Website (www.mandoshealth.com) launched
- Introduction of Mandos to community at National Niemann-Pick Disease Foundation (NNPDF) and International Niemann-Pick Disease Alliance (INPDA) annual conferences

Future Development AND EAP Planning

- We are continuing to evaluate potential regulatory pathways for development
- We initiated outreach with the US Food and Drug Administration (FDA) regarding discussion towards a potential development path as well as to discuss plans for Expanded Access Program supply that support Mandos' desire to enable continued patient access through the EAP beyond October 20, 2021

We would like to take this opportunity to emphasize that we are working diligently to make information about our progress available to the NPC community on a regular basis.

Summary DASHBOARD of Expanded Access Program Efforts (as of September 1, 2021)

Current Sites

- 16 sites are currently active in EAP

New/Pending Sites

- 4 pending new sites for EAP

New patients enrolled in Expanded Access Program since July 1, 2021

- 3 new patients

Current patients enrolled in Expanded Access Program

- 43 patients

We are committed to supporting continued access to adrabetadex where possible. We have posted our broader expanded access policy on our website at www.mandoshealth.com. As noted above, we are proud to have been instrumental in providing adrabetadex treatment to several patients who met the eligibility criteria, with urgent need, through the Expanded Access Program. We appreciate the efforts of all the investigators, institutions, regulators, and clinical partners who worked to make those access opportunities a timely reality for patients in need.

In summary, over the coming months, we anticipate completing our evaluation of the totality of the data available to us relevant to adrabetadex, which puts us in a position to then request a formal meeting with FDA to discuss potential paths forward for adrabetadex. We will also explore the possibility of supporting additional EAP sites to reduce patient and family travel burdens where possible.

We are very excited about the progress we have made thus far. We also hope this update demonstrates our aim to be transparent and committed to providing information about adrabetadex and our access and development plans to the NPC community.

Sincerely,

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