

Mandos Health

October 1, 2021

To The NPC1 Community,

As we approach the three-month mark since the closing of our acquisition of adrabetadex*, we want to provide a brief update on our continued progress. On September 16th, we were able to share with you all that we'd reached agreement with the FDA to officially continue the US Expanded Access Program (EAP) for intrathecal and/or intravenous administration of adrabetadex* beyond October 20th, 2021. Thank you for all the words of support and encouragement in response to that communication. It's humbling and gratifying to get these passionate reminders about how important this work is to so many and very motivating to our entire team.

We are also excited to share that we **have now received authorization from Health Canada's Special Access Program (SAP) to continue to provide adrabetadex to the EAP sites in Canada.** For NPC1 patients and families in Canada, here is the [link to our website](#) to contact us if you have any questions.

As part of our monthly communication to help keep the community up to date on our Expanded Access Program efforts, we are happy to share our most current dashboard summary below. As we have now been able to gain the regulatory authorizations in the US and Canada to move this forward and extend access beyond October 20th, we will be refreshing this dashboard next month.

Summary DASHBOARD of Expanded Access Program Efforts

(as of October 1, 2021)

Current Sites

15 sites are currently active in EAP

New/Pending Sites

5 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

42 patients

Transition

We are also pleased to share that while the formal transition period has ended, many of the

Mallinckrodt team have put in a great deal of time and effort, have been great partners to us, and we again wish to publicly appreciate their efforts.

Moving Forwards

We are engaging with many of the key opinion leaders in NPC and are encouraged by what we hear. We are continuing our evaluation of the totality of the data and we hope to be able to present findings from that over the coming months once we complete our analysis of external databases that may add to our understanding of patient and family experiences in NPC1.

We look forward to updating you all again soon.

Sincerely,

Scott Riccio
EVP, Patient & Community Engagement
Mandos, LLC
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**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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