NATIONAL NIEMANN-PICK DISEASE FOUNDATION, INC.

NOVEMBER 24

Latest Xenpozyme Supply Updates

Dear ASMD Community,

Yesterday, NNPDF representatives participated in our ongoing discussions with Sanofi regarding the immediate U.S. supply shortage and the longer-term global manufacturing and supply challenges. We know how important it is to keep our community informed, and we want to continue sharing updates as new information becomes available. Please see the latest updates below:

Patient Outreach and Infusion Coordination

Sanofi has provided an encouraging update regarding the immediate U.S. supply disruption affecting **20 mg Xenpozyme vials**. These vials are anticipated to ship this weekend and to be available for infusions around the U.S. beginning either Monday, November 30 or Tuesday, December 1.

This anticipated shipment does not resolve the ongoing shortage of 4 mg vials or the longer-term global supply challenges. NNPDF continues to monitor both situations closely and advocate for timely information and continuity of treatment.

Immediate U.S. Supply Update

Sanofi has confirmed that its Case Management and Patient Education Liaison (PEL) teams are proactively contacting patients whose upcoming infusions may be affected. Sanofi's medical teams are also communicating with healthcare providers to support treatment planning and coordination.

Sanofi is continuing to assess upcoming infusion schedules to identify potential disruptions and support patients and their care teams as the situation evolves.

If you have an infusion scheduled in the coming days, we encourage you to remain in close communication with your treating clinician and Sanofi Case Manager, if applicable, to confirm your individual treatment arrangements.

Any decisions regarding changes to treatment or infusion schedules should be made in consultation with your healthcare provider.

Longer-Term Manufacturing and Regulatory Updates

While the immediate U.S. supply situation is being actively addressed, important questions remain regarding the longer-term global manufacturing and supply challenges.
At the time of today's meeting, there were no new updates from the FDA or EMA regarding Sanofi's proposed mitigation plan, and no confirmed timeline for a regulatory response.

NNPDF continues to seek additional information and transparency regarding the Waterford manufacturing facility, the steps being taken to address production challenges, and the anticipated timeline for longer-term supply stability.

We will continue raising these questions directly with Sanofi and sharing updates as information becomes available.

ASMD Updates and Resources: A Timeline

We recognize that significant information has been shared over the past several weeks. For those who may have missed earlier discussions, we have gathered the following resources in one place:

- **November 10 | Initial Xenpozyme Supply Announcement:** NNPDF informed the ASMD community of Sanofi's announcement of a global Xenpozyme supply disruption related to manufacturing capacity and began coordinating with clinicians, Sanofi, and global patient advocacy partners. **Read the November 10 announcement**

- **November 11 | ASMD Community Webinar with Sanofi:** Sanofi representatives provided an overview of the Xenpozyme supply situation, including manufacturing challenges, and responded to community questions. **Watch the recording**

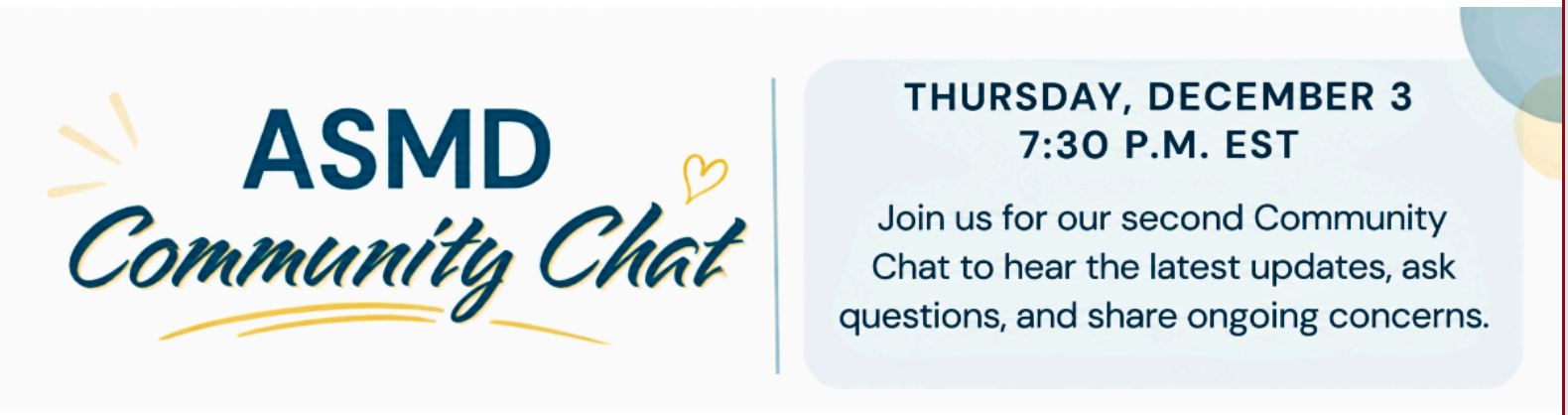
- **November 13 | NNPDF Community Email:** Xenpozyme Supply and Temporary Bridge Guidance. NNPDF shared suggested interim dosing and monitoring considerations for discussion with treating clinicians while awaiting regulatory guidance. **Read the communication**

- **November 17 | CheckRare Discussion:** Additional information about the Waterford manufacturing facility, production challenges, and Sanofi's efforts to address the supply situation. **View the discussion**

- **November 18 | ASMD Community Chat:** Dr. Josh Baker, Dr. Melissa Wasserstein, and Dr. Justin Hopkin joined the community for an open discussion about the supply situation, patient concerns, and advocacy efforts. **Watch the recording**

- **November 19 | ASMD Community Chat Follow-Up:** NNPDF shared the November 18 Community Chat recording, clinical expert contacts, and Sanofi patient support resources. **Read the communication.**

Staying Connected | Save the Date



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Please save the date for our next **ASMD Community Chat on Thursday, December 3, at 7:30 p.m. Eastern Standard Time**, when we plan to be joined by **Dr. Joshua Baker and Dr. Justin Hopkin**.

We hope to have further updates and greater clarity on several outstanding questions we continue to pursue with Sanofi. This conversation will be an opportunity to share what we have learned and discuss ongoing concerns.

In the meantime, please continue communicating with your healthcare team regarding upcoming infusions. **Do not make changes to your dose or infusion schedule without first speaking with your treating clinician. Our clinical experts discourage patients from stopping infusions as a drug conservation strategy.**

If you are experiencing challenges, have questions, or need assistance navigating available resources, please contact our Family Services Manager, Allie Stavrolakis, at astavrolakis@nnpdf.org or 920-542-4029.

We know this uncertainty continues to weigh heavily on individuals and families. While important questions remain, please know that NNPDF is actively engaged, seeking answers, and advocating for our community every step of the way.

We are here for you, and you are not alone. We will continue to share updates as new information becomes available.

With regards,



Heather Teal, M.Ed.
NNPDF Executive Director

Supporting One Another. Supporting Our Community.

NATIONAL NIEMANN-PICK DISEASE FOUNDATION, INC.

PO Box 49, Fort Atkinson, WI 53538-0049
877-287-3672 | nnpdf@nnpdf.org | nnpdf.org

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National Niemann-Pick Disease Foundation, Inc. | P.O. Box 49, 401 Madison Avenue, Suite B | Fort Atkinson, WI 53538-0049 US

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