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Dear Sanfilippo Community,

Ultragenyx recognizes that you are closely following the UX111 program, an investigational AAV gene therapy for patients with Sanfilippo syndrome type A (MPS IIIA), and have requested timely updates.

The U.S. Food and Drug Administration (FDA) has issued a Complete Response Letter (CRL) for our Biologics License Application (BLA) for UX111. We were very recently informed of this news as the BLA has been under review by the FDA. A CRL is a communication from the FDA, indicating that an application cannot be approved in its current form, and is the method that the FDA uses to provide sponsors with the specific observations that need to be addressed for potential approval in the future.

In the CRL for UX111, the FDA requested that Ultragenyx provide additional information and improvements related to specific aspects of chemistry, manufacturing and controls (CMC) and observations from the recently completed manufacturing facility inspections. It's important to know that we believe that the FDA observations are readily addressable and we have already addressed many of them. The FDA observations are related to facilities and processes and are not directly related to the quality of the product. The CRL did not note any review issues related to the clinical data package nor clinical inspections.

We understand the significant need to deliver effective therapies to children with Sanfilippo syndrome type A. While the CRL will delay the potential approval of UX111 to 2026, we are moving with urgency. The company will be working with the FDA over the next few months to resolve the observations. Once a resolution is achieved, we expect to resubmit the BLA and would then anticipate up to a 6-month review period after the resubmission.

We recognize the impact of this news on the Sanfilippo community and families who are waiting for a first-ever treatment. Ultragenyx remains unwavering in our commitment to the Sanfilippo community and UX111 program.

We are in close contact with patient advocacy organizations to ensure that the Sanfilippo community can remain updated on this news and what it means for the UX111 program. We also respect that you may have questions for Ultragenyx, and you can contact us at [mpsiiiia@ultragenyx.com](mailto:mpsiiiia@ultragenyx.com).

On behalf of the Ultragenyx team,

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