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Congress of the United States
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August 20, 2025

Dr. Marty Makary
Commissioner
U.S. Food and Drug Administration
10903 New Hampshire Ave.
Silver Spring, MD 20993

Dear Commissioner Makary,

I am writing to encourage the Food and Drug Administration (FDA) to expeditiously review the resubmitted Biologics License Application (BLA) for Ultragenyx's UX111 gene therapy for mucopolysaccharidosis type III, also known as Sanfilippo syndrome.

As you know, Sanfilippo syndrome is a terminal neurodegenerative illness that primarily affects children at an early age. Gene therapies, like UX111, have shown promising results in clinical trials and present a hopeful future for families affected by diseases like Sanfilippo syndrome. I encourage the FDA to consider the clinical value of these treatments and prevent unnecessary delays in reviewing future applications.

In July, the FDA issued a Complete Response Letter (CRL) for UX111, requesting that Ultragenyx provide additional data and improvements on chemistry, manufacturing, and controls, as well as address observations from recent facility inspections. These facility- and process-related observations are readily addressable and not directly related to the quality or efficacy of the treatment. As such, Ultragenyx resubmitted its BLA in August and is now awaiting an updated decision from the FDA.

Historically, considerations of BLAs have taken up to six months, and, unfortunately, that is time that many families do not have. Acting swiftly to approve UX111 could save the lives of thousands of children who are running out of time. As such, I encourage the FDA to review the resubmitted BLA for Ultragenyx's UX111 treatment as soon as possible and pledge to work with the rare disease community to ensure no child is denied access to lifesaving treatment.

The work the FDA staff do every day is essential to protecting our public health, improving our quality of life, and developing treatments for rare diseases that impact our loved ones. I

appreciate your time and consideration, and please do not hesitate to reach out to me or my staff with any questions.

Sincerely,

A handwritten signature in blue ink, reading "Nikki Budzinski". The signature is written in a cursive, flowing style.

Nikki Budzinski
Member of Congress