

# What do FDA Delays Mean for Rare Disease Patients?

Congress previously created faster approval pathways for serious and life-threatening conditions, but they aren't using them.



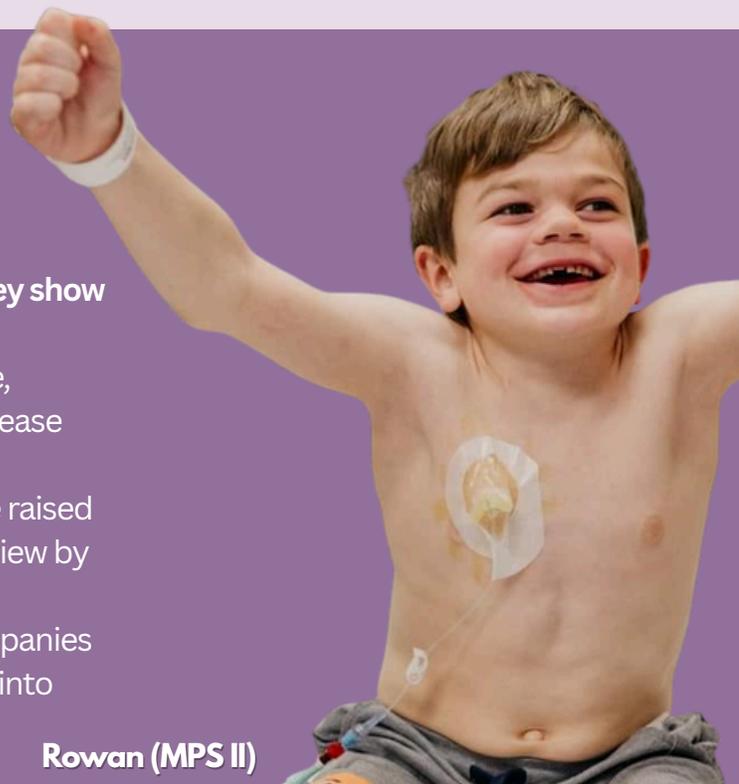
Kelley (MPS I)

78%

of drug applications for rare genetic neurodegenerative diseases were denied by the FDA in 2025

For the rare disease community, FDA rejection has looked like...

- Treatments or trials have been delayed **even when they show improvement in patients.**
- Pharmaceutical companies are asked to conduct large, traditional trials that are **impossible** in smaller rare disease patient populations.
- Concerns with manufacturing or paperwork errors are raised late in the FDA review process, often delaying final review by **months.**
- FDA regulatory expectations appear to shift after companies have already invested **hundreds of millions of dollars** into trials, manufacturing, etc.



Rowan (MPS II)

# How can Congress help?

Congress created Accelerated Approval, the Orphan Drug Act, and the 21st Century Cures Act. These programs were designed specifically for diseases like MPS, Batten Disease, and others, where waiting for perfect treatments would mean irreparable damage and lost lives. **These tools are not functioning as intended, and we need Congressional oversight to get them back on track.**

## Our asks are simple:

#1

### Use Accelerated Approval as Congress Intended

When there's clear evidence that a treatment improves the biology of a fatal disease, patients should be allowed **urgent** access to prevent further, irreversible disease progression.

#2

### Approve Innovative Trial Designs for Rare Diseases

Rare disease populations are small, and regulators **must** accept alternative study designs that adapt to smaller patient populations.

#3

### Address Manufacturing Issues Early

Requests for additional paperwork should not stop access or slow the process. Requests should be made **early**, not at the finish line, when there is evidence that a drug is safe and effective.

#4

### Provide Clear Expectations from the Start

Companies and families **need** predictability—not a shifting finish line.