Project Alive Urges FDA to Prioritize Approvals for Life-Saving Rare Disease Treatments

The organization is reaching out to the FDA for support

Project Alive is fighting for a solution to Hunter Syndrome, also known as MPS II.

CRESTLINE, CA, UNITED STATES, August 6, 2025 /EINPresswire.com/ -- Kristin McKay, President and Executive Director of Project Alive, is calling on the U.S. Food and Drug Administration (FDA) to prioritize timely reviews and approvals for therapies targeting rare, life-threatening diseases, including Hunter syndrome (MPS II), a disorder that has deeply affected her own family.

Kristin is both the mother and sister of individuals diagnosed with Hunter syndrome, an ultra-rare genetic disorder with no cure and limited treatment options. Like many families in the rare disease community, she has followed the progress of promising new therapies with hope—particularly those showing encouraging results in clinical trials.

"We are grateful to the FDA for recognizing the importance of rare disease treatment and for acknowledging the strength of the data behind these therapies," said McKay. "However, we are increasingly concerned about recent delays in the approval process that, while not related to safety or efficacy, could significantly affect outcomes for children battling rapidly progressing diseases."

Recent delays in the approval of therapies for conditions like Sanfilippo syndrome—a disorder with similarities to Hunter syndrome—highlight the broader impact such slowdowns can have on rare disease communities. For children with MPS disorders, even short-term delays can result in permanent loss of mobility, language, and cognitive abilities.

"We understand the FDA has a responsibility to ensure treatments are both safe and effective," McKay added. "At the same time, we urge them to consider the unique urgency of rare pediatric

diseases, where time lost cannot be regained."

FDA Commissioner Dr. Makary has publicly stated his intention to expedite approvals for rare disease therapies, aiming to act in weeks rather than months. Project Alive and other advocacy organizations respectfully ask that this commitment be put into action by prioritizing therapies for Hunter syndrome and similar conditions.

"We believe in partnership and transparency," McKay said. "We are eager to collaborate with the FDA and stand ready to meet with Commissioner Makary at his earliest convenience. We've made multiple requests for a meeting and hope to confirm a date soon. Our children's futures depend on it."

Project Alive is also voicing solidarity with families affected by other rare diseases—including Barth syndrome, Hereditary Angioedema, and Duchenne Muscular Dystrophy—who are experiencing similar regulatory delays. The organization calls for a coordinated effort to ensure that promising treatments reach the patients who need them most, without unnecessary barriers.

About Project Alive
Project Alive is a leading patient advocacy and research organization dedicated to finding a cure for Hunter syndrome (MPS II). Through funding research, raising awareness, and supporting families, Project Alive works to accelerate meaningful progress in treatment and care for those affected by this devastating disease.

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