Project Alive Urges FDA to Prioritize Approvals for Life-Saving Rare Disease Treatments Fof RGX-121 Gene Therapy


Project Alive's Executive Director Kristin McKay

CRESTLINE, CA, UNITED STATES, August 22, 2025 /EINPresswire.com/ -- Project Alive, a leading nonprofit dedicated to advocating for children and families impacted by Hunter syndrome (MPS II), is calling on the U.S. Food and Drug Administration (FDA) to prioritize timely approvals of life-saving rare disease treatments. This urgent call comes in light of the FDA's recent decision to extend the Prescription Drug User Fee Act (PDUFA) date for REGENXBIO's RGX-121, an investigational gene therapy for Hunter syndrome, by three months.

RGX-121 has shown strong safety and efficacy signals in clinical trials, including sustained reductions in toxic glycosaminoglycans in the brain and decreased reliance on burdensome enzyme replacement therapy. Families and clinicians have been eagerly awaiting its approval as the first potential one-time treatment targeting the central nervous system for Hunter syndrome.

However, the FDA's decision to delay review until February 8, 2026, has left the Hunter syndrome community devastated. Each day of delay means further progression of this neurodegenerative disease in children—progression that cannot be reversed. For families already facing the relentless decline of their children's abilities, time is not a luxury.

"Our children cannot wait. Every day without access to therapies like RGX-121 means more irreversible damage to their brains and bodies," said Kristin McKay, Executive Director of Project Alive and mother to a child living with Hunter syndrome. "We urge the FDA to use every tool available to expedite approvals for treatments that have demonstrated safety and the potential

to alter the trajectory of these devastating diseases."

Troubling Pattern of FDA Delays in Rare Disease

The delay of RGX-121 follows other recent setbacks, including the FDA's issuance of a Complete Response Letter (CRL) to Ultragenyx for its gene therapy candidate UX111 for Sanfilippo syndrome (MPS IIIA) earlier this summer. These actions have raised deep concerns within the rare disease community that children with rapidly progressive neurodegenerative conditions are being left behind, even when therapies have demonstrated compelling safety and efficacy signals.

For REGENXBIO's official statement on the FDA's RGX-121 review extension, see their press release here.
For Ultragenyx's statement regarding the FDA's CRL for UX111, see their press release here.

Fundraising Campaign to Amplify National Awareness

In response to the delay, Project Alive has launched a national fundraising campaign to support the production of media packages that will amplify the voices of families and highlight the urgent need for timely FDA action. These awareness efforts will be used to engage policymakers, regulators, and the broader public in advocating for children with rare diseases.

Supporters can donate and join the campaign here: https://projectalive.org

About Hunter Syndrome (MPS II)

Hunter syndrome is a rare, life-limiting genetic disorder that affects nearly every system in the body. Caused by a deficiency in the enzyme iduronate-2-sulfatase, it leads to the buildup of toxic substances in cells, resulting in progressive damage to the brain and body. Current treatment options are limited and do not address the neurological decline that steals children's futures.

About Project Alive

Project Alive is a 501(c)(3) nonprofit organization dedicated to advancing research, advocacy, and community support for families impacted by Hunter syndrome. Founded by parents, the organization is driven by a deep commitment to accelerate the development of life-saving

therapies and to ensure every child has a chance at a brighter future.

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