Ultragenyx Announces Approval of FAYUVI™ Gene Therapy, the First-Ever FDA-Approved Treatment for Sanfilippo Syndrome Type A (MPS IIIA)

September 17, 2026

FAYUVI is a highly anticipated, first-ever treatment option with the potential to stop or slow the devastating, irreversible neurologic progression and loss of function associated with Sanfilippo syndrome Type A

Ultragenyx's UltraCare® program will support access, and commercial product is expected to be available to ship to Qualified Treatment Centers within 30-60 days

FAYUVI marks the second gene therapy approval, and sixth FDA approval overall, for Ultragenyx

The Company received a Priority Review Voucher upon FAYUVI approval

Ultragenyx to Host Conference Call on September 17, 2026 at 5:30 p.m. Eastern Time

NOVATO, Calif., Sept. 17, 2026 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) today announced that the U.S. Food and Drug Administration (FDA) granted standard full approval of FAYUVI™ (rebisufligene etisparvovec-hopf), also known as UX111, for the treatment of pediatric patients with mucopolysaccharidosis type IIIA (MPS IIIA, Sanfilippo syndrome Type A). FAYUVI is the first-ever FDA-approved treatment for Sanfilippo syndrome Type A, a progressive and fatal neurodegenerative disease, and the second gene therapy approval for Ultragenyx. The Company received a Priority Review Voucher upon this approval.

"The approval of FAYUVI reflects years of research from scientists and developers, as well as unwavering support from so many families and patient organizations in the face of a devastating, universally fatal disease with no treatment options. This is a historic milestone for a community that has waited far too long, but has never given up hope," said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. "We recognize the profound urgency of making this therapy available to families, and our focus now is on supporting timely access in the U.S. as we work closely with treatment centers and payers to support families on the gene therapy treatment journey. FDA approval is an important first step toward our long-term goal to bring this treatment option to families of children with Sanfilippo syndrome Type A around the world."

"The U.S. FDA approval of FAYUVI is a milestone that the Sanfilippo syndrome Type A community spent decades fighting to achieve: the first-ever treatment for a disease that relentlessly steals a child's abilities, independence, and future," said Glenn O'Neill, president and co-founder of the Cure Sanfilippo Foundation, and Terri Klein, CNPM, MPA, president and chief executive officer of the National MPS Society. "This remarkable scientific achievement is the culmination of decades of advocacy, fundraising, collaboration, and perseverance across the Sanfilippo community along with researchers, clinicians, and industry partners who never lost faith that progress was possible. We celebrate by honoring every family who contributed and remembering the children we lost while waiting for this day. Together, we look ahead with renewed hope knowing that this treatment is now approved for children and families affected by this heartbreaking disease."

About Sanfilippo Syndrome Type A and FAYUVI
Sanfilippo syndrome Type A is an ultra-rare, fatal lysosomal storage disease that primarily affects the brain and is marked by rapid, progressive neurodegeneration beginning in early childhood. Children with Sanfilippo syndrome Type A typically experience progressive global developmental delay, followed by the loss of cognitive, language, and motor function, ultimately leading to early death. Sanfilippo syndrome Type A is estimated to affect approximately 3,000 to 5,000 patients in commercially accessible geographies, with a median life expectancy of 15 years. The disease is caused by a deficiency of the sulfamidase (SGSH) enzyme, which results in the accumulation of heparan sulfate substrate in cells and progressive damage to the central nervous system. FAYUVI is a single-dose intravenous AAV9 gene therapy designed to deliver a functional copy of the deficient enzyme gene that can express and replace the SGSH enzyme.

"This gene therapy addresses a pressing unmet clinical need and offers families a promising therapeutic option," said Kevin M. Flanigan, M.D., director of the Center for Gene Therapy at Nationwide Children's Hospital and principal investigator on the study that led to its approval. "It is additionally gratifying in that this vector was first developed at Nationwide Children's more than a decade ago, and its approval highlights our commitment to developing therapies that meaningfully impact children's health."

The final delivery of this therapy did not come without tremendous difficulties during its development, and the Company hopes this approval will revitalize the investment in other ultra-rare gene therapies. The therapy was developed by Haiyan Fu, PhD, and Doug McCarty, PhD, during their tenures at Ohio State University/Nationwide Children's Hospital and was licensed to Abeona. When funding constraints arose despite positive clinical data, Abeona made the pivotal decision to out-license the asset to Ultragenyx, ensuring this vital treatment reached the finish line for patients. Ultragenyx thanks the researchers, the development and leadership team at Abeona, and so many families, patient advocacy groups, and investigators who worked tirelessly through so many obstacles over the many years to lead the Company to this moment of shared success.

Clinical Program Supporting FAYUVI
The approval of FAYUVI is supported by data from the pivotal *Transpher A* trial and long-term follow-up studies, which demonstrated clinical benefit relative to the decline observed in natural history, along with durable treatment effect across clinical assessments and multiple biomarkers while maintaining an acceptable safety profile. Clinical data now extend to up to nearly 8 years of follow-up.

Biochemical efficacy in replacing the missing enzyme was demonstrated by a reduction in accumulated cerebral spinal fluid (CSF) heparan sulfate (HS) levels throughout the study and across all age groups. Clinical efficacy was assessed based on patients' mean change in Bayley-III Cognitive raw score from 24 to 60 months of age. FAYUVI-treated patients from the modified intention-to-treat (mITT) population (N=17) were compared to untreated patients with Sanfilippo syndrome Type A from an external, comparable natural history cohort (N=27). FAYUVI-treated patients (mITT) demonstrated a 23.5 point higher ($p<0.0001$) cognitive score over natural history during the period of study, providing the efficacy basis for standard full approval.

Enabling Access for Eligible Patients
Ultragenyx will provide support to help enrolled patients and caregivers navigate access to treatment through its UltraCare® program, which now

includes specially trained UltraCare® Gene Therapy Guides to help understand insurance coverage, assist in obtaining treatment support, and answer questions about the treatment process. Dedicated in-house UltraCare Gene Therapy Guides are available Monday through Friday from 9 a.m. to 8 p.m. Eastern Time at 888-756-8657. More information is available at www.ultracaresupport.com.

FAYUVI will be available through a network of Qualified Treatment Centers (QTCs), which are U.S.-based healthcare institutions with specialized expertise and training to administer gene therapy. Ultragenyx expects commercial product will be available for shipment to QTCs within 30-60 days.

FAYUVI is manufactured entirely within the U.S., at Ultragenyx's Gene Therapy Manufacturing Facility in Bedford, Massachusetts, and Andelyn Biosciences in Columbus, Ohio.

Additional details, including information on the QTC network, will be available on fayuvi.com, which is expected to be live within the coming days.

Investor Conference Call
Ultragenyx will host a conference call today at 5:30 p.m. Eastern Time/2:30 p.m. Pacific Time to discuss the FAYUVI approval. The live and replayed webcast of the call will be available through the company's website at https://ir.ultragenyx.com/events-presentations.

INDICATION
FAYUVI™ (rebisufligene etisparvovec-hopf) is an adeno-associated virus (AAV) vector-based gene therapy indicated for the treatment of neurologic manifestations of mucopolysaccharidosis type IIIA (MPS IIIA, Sanfilippo syndrome type A) in pediatric patients with preserved neurodevelopmental function.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

Hepatotoxicity
Elevated liver enzymes (ALT, AST, and GGT) were observed in clinical studies of FAYUVI. Prior to FAYUVI infusion, assess liver function by clinical examination and laboratory testing (ALT, AST, GGT, and total bilirubin), and evaluate liver-related medical history. Administer corticosteroids to all patients before and after the FAYUVI infusion. If abnormalities are observed, adjust the corticosteroid treatment regimen, including increasing the dose and/or prolonging the corticosteroid taper period.

Closely monitor ALT, AST, GGT, and total bilirubin levels after FAYUVI administration until 2 weeks after the corticosteroid taper is complete and as clinically indicated. Continue to monitor liver function in all patients who develop elevated liver enzymes until levels return to baseline.

Thrombocytopenia
Decreased platelet counts were observed in clinical studies of FAYUVI.
Prior to FAYUVI infusion, assess platelet counts. Monitor platelet counts weekly for the first 4 weeks, then monthly for 6 months following infusion. Continue monitoring as clinically indicated.

Thrombotic Microangiopathy
Thrombotic microangiopathy (TMA) has been reported in association with AAV gene therapies. While there have been no cases of TMA associated with FAYUVI in clinical studies, laboratory and clinical monitoring for TMA following FAYUVI infusion is recommended.

Monitor platelet counts closely within the first 4 weeks following FAYUVI infusion. Signs and symptoms of TMA may include, but are not limited to, thrombocytopenia, hemolytic anemia, easy bruising, hypertension, seizures, decreased urine output, and renal dysfunction. If TMA is suspected, immediately consult a pediatric hematologist and/or nephrologist for further evaluation and management as clinically indicated.

Hypersensitivity and Infusion Reactions
Infusion reactions, including hypersensitivity reactions and anaphylaxis, may occur with infusion of FAYUVI. Symptoms may include, but are not limited to, hypotension, pyrexia, palpitation, nausea, vomiting, chills, or headache.

Closely monitor patients for clinical signs and symptoms of infusion reactions, including hypersensitivity reactions, and monitor vital signs during and after completion of FAYUVI infusion as clinically indicated. In the event of an infusion reaction during administration, pause the infusion and provide supportive care according to clinical practice. If the infusion is paused and continued administration is appropriate, restart at a slower rate after the infusion reaction has resolved.

Risk of Malignancy
Malignancy may occur following treatment with FAYUVI due to potential integration of AAV vector DNA into the genome.

In the event of a malignancy, contact Ultragenyx Pharmaceutical Inc. at
1-888-756-8657.

ADVERSE REACTIONS
The most common adverse reactions are (≥5%): liver enzyme increased (85%), vomiting (67%), abnormal behavior (56%), diarrhea (48%), pyrexia (41%), white cell count decreased (30%), Cushingoid features (30%), decreased appetite (22%), platelet count decreased (19%), anemia (19%), constipation (15%), nausea (11%), amylase increased (11%), alkaline phosphatase increase (11%), seizure (11%), hepatomegaly (11%), muscle spasticity (7%), hypokalemia (7%), gait disturbance (7%), and adrenal insufficiency (7%).

VACCINATIONS
Prior to FAYUVI administration, consider the patient's vaccination status. Vaccines should be avoided 30 days prior to treatment with FAYUVI (and use of corticosteroids) and while on corticosteroid therapy.

PREGNANCY
There are no data on the use of FAYUVI in pregnant women. Women who are pregnant or desire to become pregnant should not be treated with FAYUVI. A negative serum pregnancy test must be confirmed before administration of FAYUVI in females of childbearing potential.

VECTOR SHEDDING
Temporary vector shedding of FAYUVI occurs primarily through bodily fluids and waste. Advise patients and/or caregivers on proper handling of patient bodily fluids and waste, including hand hygiene after direct contact. These precautions should be followed for 3 months after FAYUVI infusion.

Report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to Ultragenyx Pharmaceutical Inc. at 1-888-756-8657.
Please see the full Prescribing Information for FAYUVI.

About Ultragenyx
Ultragenyx is a biopharmaceutical company committed to bringing novel therapies to patients for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved medicines and treatment candidates aimed at addressing diseases with high unmet medical need and clear biology, for which there are typically no approved therapies treating the underlying disease.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx's strategy is predicated upon time- and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit the company's website at: www.ultragenyx.com.

Forward-Looking Statements and Use of Digital Media
Except for the historical information contained herein, the matters set forth in this press release, including statements regarding the commercial launch, availability, timing of shipment and market acceptance of FAYUVI; Ultragenyx's ability to supply FAYUVI to Qualified Treatment Centers; patient access to FAYUVI, including insurance coverage and reimbursement; the safety, efficacy, durability and potential benefits of FAYUVI; the potential commercial opportunity for FAYUVI; and Ultragenyx's ability to satisfy FDA requirements and maintain approval for FAYUVI, are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995.

Such forward-looking statements involve substantial risks and uncertainties that could cause actual results to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, risks and uncertainties related to the commercial launch and market acceptance of FAYUVI; the ability to identify eligible patients and establish and support a network of Qualified Treatment Centers; uncertainty related to insurance coverage and reimbursement; risks related to serious or undesirable side effects, including risks associated with AAV gene therapy; manufacturing risks and the ability to manufacture and supply FAYUVI in sufficient quantities and in compliance with regulatory requirements; the risk that the FDA may modify the approved indication, impose additional requirements or withdraw approval if applicable requirements are not satisfied; smaller than anticipated market opportunities; competition from other therapies or products; product liability; regulatory scrutiny; and other matters that could affect the availability or commercial potential of Ultragenyx's products and product candidates. Ultragenyx undertakes no obligation to update or revise any forward-looking statements.

For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of Ultragenyx in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on August 5, 2026, and its subsequent periodic reports filed with the SEC.

In addition to its SEC filings, press releases and public conference calls, Ultragenyx uses its investor relations website and social media outlets to publish important information about the company, including information that may be deemed material to investors, and to comply with its disclosure obligations under Regulation FD. Financial and other information about Ultragenyx is routinely posted and is accessible on Ultragenyx's Investor Relations website (https://ir.ultragenyx.com/) and LinkedIn website (https://www.linkedin.com/company/ultragenyx-pharmaceutical-inc-/).

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