FDA Approves Expanded Use of Vonvendi for von Willebrand Disease, Including for Certain Uses for Children

The U.S. Food and Drug Administration today approved expanded use of Vonvendi [von Willebrand factor (Recombinant)] for routine preventative (prophylactic) use in adults (age 18 years and older) with all types of von Willebrand disease (VWD) and on-demand and treatment of bleeding episodes and perioperative use in children with VWD.

Previously, Vonvendi was approved only for on-demand treatment of bleeding episodes and perioperative use in adults and preventative use only in adults with Type 3 VWD, the most serious type.

"This approval demonstrates FDA flexibility in evaluating applications of therapeutics to treat rare diseases," **said FDA Vinay Prasad, M.D., M.P.H., Director of the FDA's Center for Biologics Evaluation and Research**. "When we see the trifecta of plausible mechanism of action, robust pharmacology/biologic science, and supportive clinical study data, we promptly act even if that data is derived from a small sample size study."

Von Willebrand disease is a bleeding disorder in which a person's blood doesn't clot properly. People with the disease have low levels of von Willebrand factor (VWF), a protein that helps blood clot, or the protein doesn't perform as it should.

Vonvendi is the only recombinant (non-plasma derived) VWF product approved for VWD in the U.S., and this is the first recombinant VWF product approved for pediatric patients in the U.S. Prior to this approval, only plasma derived VWF products were available to the pediatric population.

"Today's approval is a testament to the collaborative interactions between CBER review teams and product developers, aimed at making innovative therapies available for use in pediatric populations, at the same time as adults," **said Vijay Kumar, M.D., acting director of the CBER Office of Therapeutic Products**.

Efficacy of Vonvendi was demonstrated in multiple clinical studies that showed success in treatment control of bleeding episodes and use in perioperative management in patients with VWD of all ages. It also proved successful for prevention of bleeding episodes in adults with VWD.

The most common adverse reactions observed in greater than, or equal to, 2% of patients in clinical trials with Vonvendi were headache, vomiting, nausea, dizziness and itchy skin (generalized pruritus).

This approval was completed under Priority Review and the product received Orphan Drug Designation.

The FDA granted approval of this product to Takeda.

FDA News released Sep 5, 2025. www.fda.gov.

FDA Grants Accelerated Approval to First Treatment for Barth Syndrome

New Treatment for Barth Syndrome Showcases FDA's Commitment to Bringing Effective and Safe Medications to Patients in Need

Today, the U.S. Food and Drug Administration granted accelerated approval to Forzinity (elamipretide) injection as the first treatment for Barth syndrome, in patients weighing at least 30 kg. Barth syndrome is a rare, serious and life-threatening disease of the mitochondria (the energy-producing parts of cells).

"The FDA remains committed to facilitating the development of effective and safe therapies for rare diseases and will continue to work diligently to help ensure patients with rare diseases have access to innovative treatments," **said George Tidmarsh, M.D., Ph.D., Director of the FDA's Center for Drug Evaluation and Research**.

Barth syndrome primarily affects males, typically starts with severe heart failure in infancy, and causes premature death. Patients who survive into adolescence and adulthood often have fatigue, poor stamina, and exercise intolerance. The quality of life and daily functioning of patients with Barth syndrome are significantly affected throughout their lives

Forzinity works by binding to the inner part of the mitochondria, improving mitochondrial structure and function. FDA granted Forzinity accelerated approval. This pathway can allow earlier approval of medications that treat serious conditions and fill an unmet medical need on the basis of a measure that is considered reasonably likely to predict patient benefit but does not directly assess the benefit to the patient.

Forzinity's accelerated approval is based on improved strength of the muscle used to straighten the

leg at the knee. FDA considers this improvement reasonably likely to predict patient benefit, such as an ability to stand more easily or walk farther. As a condition of accelerated approval, FDA is requiring the manufacturer of Forzinity to conduct a post-approval randomized, double-blind, placebo-controlled trial to confirm that the changes seen on knee muscle strength translate into patient benefit.

Forzinity is administered subcutaneously (under the skin) once daily. The most common side effects identified in clinical trials were mild-to-moderate injection site reactions. Serious reactions to Forzinity have also been reported.

This application was granted priority review, and Forzinity was granted a rare pediatric disease designation.

The FDA granted the accelerated approval of Forzinity, as well as a rare pediatric disease priority review voucher, to Stealth Biotherapeutics Inc.

FDA News released Sep 19, 2025. www.fda.gov.

FDA Approves Gene Therapy for Treatment of Spinal Muscular Atrophy

The U.S. Food and Drug Administration today approved Itvisma (onasemnogene abeparvovec-brve) for the treatment of spinal muscular atrophy (SMA) in adult and pediatric patients 2 years of age and older with confirmed mutation in the survival motor neuron 1 (*SMN1*) gene. Itvisma is an adeno-associated virus (AAV) vector-based gene therapy.

"Today's approval shows the power of gene therapies and offers treatment to patients across the SMA disease spectrum, including patients at various ages, SMA symptoms, and motor functional levels," **said Vinay Prasad, M.D., M.P.H., the FDA's Chief Medical and Scientific Officer and Director of the Center for Biologics Evaluation and Research**. "This exciting area of science continues to change the lives of patients and the FDA is committed to expediting the development of products for unmet medical needs."

SMA is an autosomal-recessive neurodegenerative disorder caused by mutations in the *SMN*1 gene, characterized by irreversible and progressive motor neuron loss, leading to progressive muscle atrophy and weakness, and subsequent paralysis and death in the most severe cases. SMA has an incidence of approximately 4-10 per 10,000 live births. Prior to the availability of effective treatment, SMA was considered one of the leading causes of infant mortality due to genetic disease in the U.S.

Itvisma demonstrated substantial evidence of effectiveness for the treatment of SMA in pediatric patients 2 years of age and older with a confirmed mutation in the *SMN1* gene based on primary evidence of effectiveness from the adequate and well controlled Phase 3 study, and the confirmatory evidence of effectiveness from data characterizing the mechanism of the product's action, as well as efficacy findings from Zolgensma (onasemnogene abeparvovec-xioi) which contains the same active ingredient in an intravenous formulation. The applicant provided adequate justification to support expanding the indication beyond the pivotal study population to include adult patients with SMA, however, warnings and precautions are warranted due to the potentially increased risks of adverse events of special interest (e.g., hepatotoxicity and cardiotoxicity) in adult patients with preexisting chronic medical conditions.

The active ingredient (drug substance) in Itvisma is identical to Zolgensma but formulated at a different concentration. Zolgensma is administered intravenously based on patient weight to pediatric patients less than 2 years of age with SMA due to biallelic mutations in the *SMN1* gene. Itvisma is a concentrated formulation in a smaller delivery volume, administered directly to the central nervous system via a single intrathecal injection independent of patient weight, which expands treatment options available to patients with SMA older than 2 years of age.

The direct administration of Itvisma into the cerebrospinal fluid surrounding the spinal cord (site of action) allows for delivery to motor neurons with a lower dose of vector, without the need to adjust for the patient's body weight. This provides a treatment with rapid onset and direct targeting of the genetic root cause of SMA. By addressing the root cause of SMA, Itvisma restores SMN protein production and halts further disease progression.

The FDA review team worked collaboratively to leverage Zolgensma safety data and most of the side effects of Itvisma are consistent with identified risks associated with Zolgensma. Information from the hepatotoxicity boxed warning in the Zolgensma label is retained in the Itvisma label with appropriate modifications. This approach is supported by clinical data showing hepatotoxicity in Itvisma clinical studies.

"Significant unmet need remains in SMA, particularly for patients across various ages and motor function levels, predominantly those 2 years of age and older." **said Vijay Kumar M.D., Acting Director, Office of Therapeutic Products in the FDA's**

Center for Biologics Evaluation and Research. "This approval shows our continued commitment to supporting and facilitating treatments for patients with rare diseases."

The FDA granted this application Fast Track, Breakthrough Therapy, and Priority Review designations. Itvisma also received Orphan Drug designation, which provides incentives to encourage the development of drugs for rare diseases.

Itvisma is manufactured by Novartis Gene Therapies, Inc.

FDA News released Nov 24, 2025. www.fda.gov.

Source: FDA

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