

FDA Approves New Treatment

Denali Therapeutics is pleased to share that AVLAYAH™ (tvidenofusp alfa-eknm) is now approved under the U.S. Food and Drug Administration’s (FDA) Accelerated Approval pathway, becoming a new treatment option.

Who is AVLAYAH for?

AVLAYAH is a prescription medicine approved for the treatment of neurologic symptoms in pediatric patients weighing at least 5 kg with Hunter syndrome prior to advanced neurologic disease. This approval is based on a reduction of heparan sulfate (HS) in the cerebrospinal fluid (CSF) surrounding the brain and spinal cord. Studies are ongoing to confirm how well it works in improving clinical symptoms.

This milestone represents meaningful progress for the MPS II community and reflects years of collaboration among patients, families, advocates, researchers, and clinicians. Thank you for your partnership.

Learn More About What This Approval May Mean for You or Your Family



Speak with a doctor about whether this treatment might be right for you or your loved one and to understand specific care considerations that may apply



Visit Denali’s website for updates and important information about the new treatment at DenaliTherapeutics.com



Contact Denali Patient Services for personalized support as you consider treatment options and navigate insurance coverage and care center coordination

- **Phone:** 844-DNLI365 (844-365-4365)
- **Fax:** 650-456-1875
- **Email:** patientservices@care.dnli.com

If you or a family member are currently participating in a clinical study, please speak with your study doctor or principal investigator (PI) about next steps.

When May This New Treatment Be Available?

Following approval, the treatment is expected to become available for physicians to order within a few weeks. Timing will vary for each individual as insurance coverage is confirmed and care center coordination takes place. Denali Patient Services is available to help guide you through these steps.

As more information becomes available, it will be shared with advocacy organizations and the broader community.

EXAMPLE ACCESS PATHWAY*:



**Experiences may vary. Contact Denali Patient Services for support.*

Availability Outside the U.S.

At this time, the treatment is approved for use only in the United States. We understand the urgent need for treatment options worldwide and remain committed to working with regulatory authorities to explore pathways that can help enable sustainable access globally.

Para información y recursos en español, llame al 844-365-4365

WHAT IS AVLAYAH?

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AVLAYAH is not recommended for use in combination with other enzyme replacement therapies for the treatment of Hunter syndrome.

IMPORTANT SAFETY INFORMATION

AVLAYAH may cause serious side effects, including hypersensitivity (allergic) reactions such as anaphylaxis which can be life-threatening; infusion-associated reactions (IARs); anemia (low red blood cell count); and Membranous Nephropathy (kidney disorder that affects the filters that help remove wastes and fluids from the kidney). Anaphylaxis can occur early in treatment and after many doses. Seek immediate medical care if symptoms occur.

The most common side effects (in 20% or more of patients) were infusion-associated reactions, upper respiratory infection, ear infection, fever, anemia (low red blood cell count), cough, vomiting, diarrhea, rash, COVID-19, runny or congested nose, falls, headache, skin injuries, and hives.

Contact your healthcare provider right away if you experience any side effects. These are not all the possible side effects of AVLAYAH. You may report side effects to FDA at www.fda.gov/medwatch or call [1-800-FDA-1088](https://www.fda.gov/1800). You may also report side effects to Denali Therapeutics at [1-833-ONE-DNLI \(1-833-663-3654\)](https://www.dnli.com).

Please see the full [Prescribing Information](#) for additional Important Safety Information, including serious side effects.