Medical Xpress

Mepsevii approved for rare enzyme disorder

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(HealthDay)—Mepsevii (vestronidase alfa-vjbk) has been approved by the U.S. Food and Drug Administration to treat a rare, genetic disorder called mucopolysaccharidosis type VII, sometimes called Sly syndrome.

Affecting fewer than 150 people worldwide, the extremely rare disorder typically causes various skeletal abnormalities that worsen with age, including short stature. Life expectancy and symptoms and vary widely, but also may include heart valve problems, enlarged liver and narrowed respiratory airways, the agency said Wednesday in a news release.

Mepsevii is designed to reverse an enzyme deficiency that can lead to the buildup of toxic substances in the body's cells.

"Prior to today's approval, patients with this rare, inherited condition had no approved treatment options," said Dr. Julian Beitz, a director from one of the agency's drug evaluation departments.

Clinical studies of the treatment involved 23 people ranging from 5 months to 25 years old. The most common side effects included allergic-like reactions at the infusion site, diarrhea, rash and severe anaphylaxis reaction.

The treatment is produced by Novato, Calif.-based Ultragenyx Pharmaceutical, which as a condition of approval will conduct additional research to evaluate its long-term safety.

More information: The FDA has more about this approval.