Vimizim approved for rare childhood disorder

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(HealthDay)—Vimizim (elosulfase alfa) has been approved by the U.S. Food and Drug Administration to treat a rare childhood disorder called Mucopolysaccharidosis Type IVA, also known as Morquio A syndrome.

The disorder is caused by a missing metabolic enzyme that leads to problems with bone development, growth and movement, the agency said in a news release. It affects about 800 people in the United States.

Vimizim replaces the missing enzyme, known as GALNS. The drug's safety and effectiveness were established in clinical trials involving 176 people, ranging in age from 5 to 57. The most common side effects included fever, vomiting, headache, nausea, abdominal pain, chills and fatigue, the FDA said.

The drug's safety and effectiveness weren't evaluated in children under age 5 years, the agency added. Vimizim's label will include a boxed warning to include the risk of anaphylaxis, an allergic-like reaction that could be life threatening.

Vimizim was the first drug granted a Rare Pediatric Disease Priority Review Voucher, an FDA effort to encourage development of new treatments for rare childhood diseases.

The drug is marketed by BioMarin Pharmaceuticals, based in Novoto, Calif.

More information: The FDA has more about this approval.