MedicalXpress

Brineura approved for rare genetic illness affecting kids

April 28 2017

(HealthDay)—Brineura (cerliponase alfa) has been approved by the U.S. Food and Drug Administration to treat a specific form of Batten Disease, a rare set of genetic disorders that typically begin in childhood between ages 2 and 4, the agency said in a news release.

Initial symptoms of the CLN2 form of Batten, for which the drug is approved, usually include language delay, seizures and coordination problems. Affected children also develop vision loss and motor-skill issues, including trouble walking. Many require a wheelchair by late childhood and often don't survive past their teens, the agency said.

Batten is rare, occurring in as many as four of every 100,000 births in the United States. Brineura is an enzyme replacement drug; its primary ingredient is a lab-developed form of the human enzyme TPP1, administered directly into the cerebrospinal fluid by means of a surgically implanted reservoir, the FDA said.

Brineura was evaluated in clinical studies involving 24 children ages 3 to 8 with CLN2 disease. The most common side effects included fever, heart problems, vomiting, seizures, headache and irritability.

The drug hasn't been evaluated in children under age 3, the FDA said. As a condition of approval, its manufacturer will be required to evaluate the drug in younger children, and to study for a minimum of 10 years its long-term health effects.

Approval of Brineura was given to BioMarin Pharmaceutical, based in San Rafael, Calif.

More information: The FDA has more about this approval.

Copyright © 2017 HealthDay. All rights reserved.

Citation: Brineura approved for rare genetic illness affecting kids (2017, April 28) retrieved 24 April 2024 from https://medicalxpress.com/news/2017-04-brineura-rare-genetic-illness-affecting.html

This document is subject to copyright. Apart from any fair dealing for the purpose of private study or research, no part may be reproduced without the written permission. The content is provided for information purposes only.