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FDA approves first drug for pediatric patients with systemic lupus erythematosus

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Belimumab is the first drug approved by the Food and Drug Administration (FDA) to treat systemic lupus erythematosus (SLE) in children. Intravenous (IV) belimumab was approved for children ages 5 years to 17 years with active, seropositive SLE receiving standard care.

Childhood-onset SLE is a rare disease characterized by significant morbidity and mortality with widespread organ involvement, including the kidneys and brain. Because pediatric SLE is a rare disease, drug development is particularly challenging. Very few SLE medications that have been studied in adults have been studied in children.

The approval of belimumab was supported by a randomized, controlled trial that evaluated the efficacy, safety and pharmacokinetics of 10 mg/kg IV belimumab vs. placebo in 93 pediatric patients. No novel safety issues were discovered.

Due to the limited number of children with SLE, the pediatric study was not powered to make formal statistical inferences on its own. Because the disease is sufficiently similar in adults and children, the FDA relied on extrapolation of efficacy from adult studies with belimumab and a novel post-hoc Bayesian analysis that borrowed information from the adult belimumab program to provide additional efficacy estimates for children.

The results of this unique analysis supported the conclusion that IV belimumab is efficacious in children 5 years of age and older with SLE. This innovative approach, especially when prespecified, may expedite development of treatments for pediatric rheumatic diseases.

Resources

- Belimumab prescribing information
- Additional FDA Update columns