FDA pressed by Congressmen to approve controversial Duchenne drug

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As a crucial deadline nears for a closely watched regulatory decision, two Republican senators are urging the Food and Drug Administration to approve a controversial drug to treat Duchenne muscular dystrophy.

In a letter, the senators expressed “disappointment” that an FDA advisory panel voted not to recommend eteplirsen to combat the disease, which is a rare and fatal genetic disorder that causes muscles to waste away. About 13,000 children, mostly boys, are afflicted and they typically die before turning 25 years old. The panel determined the drug was not effective.

The FDA’s decision is being closely watched as a litmus test for the agency, which is grappling with increasingly assertive patient groups that want the agency to take a more expansive view toward approving medicines for unmet medical needs.

In this instance, patient advocates and their many backers want the FDA to use what is called the accelerated approval process to endorse eteplirsen. The senators maintain that accelerated approval was designed for circumstances such as those posed by eteplirsen.

Read full, original post: Senators urge FDA to approve Sarepta drug for Duchenne