FDA delay raises slim hope for muscular dystrophy drug

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Federal health regulators will take more time to review a highly-contested drug for muscular dystrophy that has become a flashpoint in the debate over patient access to experimental medicine.

Drugmaker Sarepta Therapeutics Inc. said the Food and Drug Administration will miss its goal of issuing a decision on the drug by Thursday. While the FDA does not comment on such delays, the extended timeline raises the possibility that regulators may ultimately approve the company's medication.

The Cambridge, Massachusetts-based company said FDA regulators said they will "strive to complete their work in as timely a manner as possible."

Company shares rocketed nearly 17 percent to $21.56 in midday trading.

Sarepta's drug eteplirsen has become rallying point for patients and families stricken by the deadly inherited disease, which causes muscle weakness and eventually the loss of all basic movement. The disease affects one of every 3,600 boys worldwide and usually causes death by age 25, according to the National Institutes of Health. There are no drugs that treat the underlying disease, though steroid drugs can slow muscle degeneration.

Wall Street viewed the delay as a positive indicator for the drug's prospects. But some analysts cautioned against that interpretation.

"We wonder whether the FDA delay is designed to ensure that it appears that the agency is adequately factoring in every possible stakeholder's input before rejecting the application," said Leerink Swann analyst Joseph Schwartz, in a research note. He holds a negative rating on Sarepta's stock.

Last month, dozens of parents, patients and physicians packed an FDA meeting to urge the agency to approve the injectable drug. The public push came despite an overwhelmingly negative review by FDA scientists which found virtually no evidence that eteplirsen was effective in treating the disease.

Ultimately, a panel of FDA advisers voted 7-3 against the drug's effectiveness, though three panelists abstained from casting a vote. The FDA is not required to follow such recommendations.

Sarepta's drug is thought to produce a protein called dystrophin, which plays a key role in muscle fibers.

However, the FDA found numerous problems with the company's research that made it difficult to determine how much dystrophin the drug actually produces, and what, if any, benefit that produces for patients. The company's primary study included just 12 patients and appeared to show an increase in dystrophin of less than 1 percent.

FDA staff emphasized that the agency "strongly encouraged" Sarepta to conduct a larger, more comprehensive study of its drug with a randomly selected control group of patients receiving a placebo, considered the gold-standard of study design.

Sarepta pointed to the challenges of enrolling and tracking large numbers of children with a rare disease in such studies.