US FDA reverses course on Regenxbio's rare-disease gene therapy, backs accelerated approval bid

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Bengaluru: Regenxbio said on Monday the U.S. FDA has indicated that existing data for its rare-disease gene therapy could support an accelerated approval application, reversing course months after declining to approve the treatment.

Regenxbio shares were up 16%. The decision is the latest in a series of regulatory reversals that have raised hopes the U.S. Food and Drug Administration may be taking a more flexible approach to therapies for rare diseases.

Last week, uniQure said the FDA reversed course on its gene therapy for Huntington's, clearing the path for an accelerated approval filing after previously calling for a new trial.

Separately, Replimune said in May it planned to seek approval for its experimental skin cancer drug for a third time after reaching an agreement with the U.S. regulator.

Barclays analyst Eliana Merle said the shift and other recent FDA stance changes signal increased flexibility, adding that despite senior leadership being in flux, the agency is likely to be "more friendly to industry moving forward."

The FDA's latest feedback marks a welcome turnaround for the company, after the agency declined to approve Navsunli, its experimental treatment for Hunter syndrome, citing uncertainty over the trial design.

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Raymond James analyst Sean McCutcheon said that while "some wrinkles" remain based on the justifications in the Navsunli complete response letter, the development signals "a new approach which is significantly more flexible and portends a favorable outcome for Regenxbio and patients with Hunter Syndrome.

The company said the FDA had now indicated it would not need to enroll additional patients or conduct new studies, including a previously requested placebo-controlled trial. Regenxbio plans to meet with the agency in July and expects to resubmit its application in the third quarter.

The company said the FDA would review its resubmitted application on an expedited basis. The Wall Street Journal reported the development earlier on Monday.

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