US FDA approves Regeneron's rare bone disorder drug

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Bengaluru: The U.S. FDA on Wednesday approved Regeneron's drug for a rare genetic disorder after it significantly reduced abnormal bone formation in certain soft tissues.

New York-based Regeneron's shares rose 4%.

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In a 56-week trial involving 63 participants, Pasatru reduced the development of new bone abnormalities by 94% in patients treated with a 3 mg per kg dose and by 90% in the case of a 10 mg per kg dose, when compared with placebo.

It works by blocking Activin A, a protein involved in triggering abnormal bone growth in patients with FOP.

Susan Rhee, a member of Regeneron's clinical team, told last week that the company is planning to start a trial for children later this year.

The drug will compete with French drugmaker Ipsen's oral treatment Sohonos, which in 2023 became the only other treatment to be approved by the U.S. Food and Drug Administration.

Incyte and partner Mirum Pharma as well as privately held Ashibio are also developing treatments for the condition.

In 2020, Regeneron paused dosing in a mid-stage trial of the drug after five patient deaths, ultimately discontinuing the study and working with global regulators to design the late-stage trial, according to the company.

The condition affects roughly 1 in 2 million people worldwide, with around 800 to 900 active diagnosed cases globally, data from the National Institutes of Health showed.

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