US FDA declines to approve Ultragenyx's gene therapy for rare genetic disorder

Signage is seen outside of the Food and Drug Administration (FDA) headquarters in White Oak, Maryland, U.S., August 29, 2020.

July 11 (Reuters) - The U.S. Food and Drug Administration has declined to approve Ultragenyx Pharmaceutical's (RARE.O), opens new tab experimental gene therapy to treat a rare inherited disorder, citing manufacturing concerns, the company said on Friday.

Shares of the California-based biopharma company fell 5.4% in extended trading.

The FDA in its "complete response letter" sought additional information related to the company's production processes and facilities. The regulator's decision comes more than a month ahead of the previously announced action date of August 18.

The agency's observations, related to facilities and processes, are addressable and not directly related to the therapy's quality, Ultragenyx said, adding that many of the issues have already been addressed.

"It looks like this is a speed bump to approval, rather than a roadblock," Leerink Partners analyst Joseph Schwartz said in a client note.

The therapy, UX111, was developed to treat a common type of Sanfilippo syndrome — a group of genetic conditions that begin in early childhood causing severe brain damage and early death.

The treatment involves managing symptoms as there are currently no approved disease-modifying medicines.

Ultragenyx said the FDA did not cite any review issues related to the clinical data submitted as part of the marketing application.

The company's application for UX111 was based on trial data that showed the gene therapy significantly reduced toxic buildup in the brain and improved cognitive and communication skills in children with Sanfilippo syndrome type A.

It plans to resubmit updated clinical data from current patients after resolving the FDA's concerns. A new review could take up to six months once the revised application is filed.

Ultragenyx acquired the global rights of the therapy from Abeona Therapeutics (ABEO.O), opens new tab through an exclusive license agreement on 2022.

News Source:

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