

Scholar Rock gets first FDA nod for rare muscle-wasting disease

The injectable treatment, sold as Isembyld, is the first muscle-targeted therapy designed to improve motor function for adults and children with spinal muscular atrophy who have been treated with existing therapies for the condition.



Bengaluru: The U.S. Food and Drug Administration approved Scholar Rock's therapy on Friday to treat a rare genetic condition that causes muscle weakness, marking the first regulatory approval for the company.

The injectable treatment, sold as Isembyld, is the first muscle-targeted therapy designed to improve motor function for adults and children with spinal muscular atrophy who have been treated with existing therapies for the condition.

Scholar Rock shares rose over 23% in after-market trading. The company said Isembyld will be available in the coming days but did not immediately respond to a Reuters request for comment on pricing details.

Spinal muscular atrophy is a genetic disorder that damages motor neurons, causing progressive muscle weakness and problems with movement, breathing and swallowing.

The condition is estimated to affect roughly 10,000 children and adults in the United States, according to the Muscular Dystrophy Association.

Cantor analysts said Isembyld's approval sets up Scholar Rock to be one of the best long-term growth stories in biotech based on geographic, indication and product line expansions.

Chemically known as apitegromab, Isembyld selectively blocks activation of myostatin, a protein that limits muscle growth, with the aim of increasing muscle mass and strength.

The approval was based on late-stage data that showed the therapy significantly improved motor function for patients with the condition compared with placebo.

It will compete with Biogen's Spinraza, Novartis' gene therapy Zolgensma and Roche's oral Evrysdi.

The company has previously said it would use a U.S.-based manufacturing facility for Isembyld.

BMO Capital Markets analyst Evan Seigerman forecasts 2035 adjusted worldwide peak sales of \$2.1 billion for the drug.

Scholar Rock is also testing the drug to preserve muscle in obesity, competing with more than a dozen companies developing treatments aimed at minimizing muscle loss during weight loss.

News Source:

<https://pharma.economictimes.indiatimes.com/news/drug-approvals-and-launches/scholar-rock-gets-first-fda-nod-for-rare-muscle-wasting-disease/134230305>