Merck Receives Priority Review from FDA for New Biologics License Application for Sotatercept, an Activin Signaling Inhibitor to Treat Adults with Pulmonary Arterial Hypertension (PAH)

9/28/2023

Application based on clinically meaningful results from the Phase 3 STELLAR trial

If approved, sotatercept would be the first in its class, bringing a novel approach to address a rare and progressive disease of the pulmonary arteries

RAHWAY, N.J.--(BUSINESS WIRE)-- Merck (NYSE: MRK), known as MSD outside of the United States and Canada, announced today that the U.S. Food and Drug Administration (FDA) has accepted for priority review a new Biologics License Application (BLA) for sotatercept, Merck's novel investigational activin signaling inhibitor, for the treatment of adult patients with pulmonary arterial hypertension (PAH) (WHO Group 1). The FDA has set a Prescription Drug User Fee Act (PDUFA), or target action, date of March 26, 2024.

PAH is a rare, progressive and ultimately life-threatening disease characterized by the narrowing of blood vessels in the lungs, causing significant strain on the heart. The application for sotatercept is based on data from the Phase 3 STELLAR trial, in which sotatercept on top of background therapy demonstrated a statistically significant and clinically meaningful improvement in 6-minute walk distance (6MWD) and eight of nine secondary outcome measures. These results were presented at ACC.23/WCC and published in The New England Journal of Medicine.

“Despite advances in the treatment of PAH over the last two decades, there is still a significant need to improve outcomes for patients,” said Dr. Joerg Koglin, senior vice president, global clinical development, Merck Research...
Laboratories. “The FDA’s acceptance of this application is an exciting milestone in our journey to bring this novel activin signaling inhibitor to patients. Based on the profound improvements across primary and secondary outcome measures in the Phase 3 STELLAR trial, we believe sotatercept has the potential to transform the treatment of patients with PAH. We look forward to working closely with the FDA to bring sotatercept to patients in need.”

About STELLAR

STELLAR (NCT04576988) was a pivotal Phase 3, randomized, double-blind, placebo-controlled, multicenter, parallel-group study to evaluate the safety and efficacy of sotatercept in adult patients with PAH (WHO Group 1) being treated with background therapy with WHO Functional Class (FC) II or III. The primary endpoint of the study was exercise capacity, as measured by change from baseline in week 24 6MWD. Nine secondary endpoints, tested hierarchically in the following order, were multicomponent improvement, change in pulmonary vascular resistance (PVR), N-terminal pro-B-type natriuretic peptide (NT-proBNP) level, improvement in WHO FC, time to clinical worsening or death, French risk score, and the PAH-SYMPACT® Physical Impacts, Cardiopulmonary Symptoms and Cognitive/Emotional Impacts domain scores; all assessed at week 24 except clinical worsening, which was assessed when the last patient completed the week 24 visit.

About pulmonary arterial hypertension (PAH)

Pulmonary arterial hypertension (PAH) is a rare, progressive and life-threatening blood vessel disorder characterized by the constriction of small pulmonary arteries and elevated blood pressure in the pulmonary circulation. Approximately 40,000 people in the U.S. are living with PAH. The disease progresses rapidly for many patients. PAH results in significant strain on the heart, leading to limited physical activity, heart failure and reduced life expectancy. The five-year mortality rate for patients with PAH is approximately 43%.

About sotatercept

Sotatercept is a potential first-in-class activin signaling inhibitor biologic being studied for the treatment of PAH (WHO Group 1). PAH is a rare disease caused by hyperproliferation of cells in the arterial walls in the lung, leading to narrowing and abnormal constriction. In pre-clinical models, sotatercept has been shown to modulate vascular cell proliferation, reversing vascular and right ventricle remodeling.

In addition to STELLAR, the sotatercept clinical development program includes multiple Phase 2 and 3 trials across a broad range of patients. Studies are underway in adult patients with PAH (WHO Group 1) at intermediate or high risk of disease progression or mortality, as well as with pulmonary hypertension due to left heart disease (WHO Group 2).
Sotatercept has been granted Breakthrough Therapy Designation and Orphan Drug designation by the U.S. Food and Drug Administration (FDA), as well as Priority Medicines designation and Orphan Drug designation by the European Medicines Agency for the treatment of PAH. Merck acquired exclusive rights to sotatercept in the pulmonary hypertension field through the acquisition of Acceleron Pharma Inc. Sotatercept is the subject of a licensing agreement with Bristol Myers Squibb.

About Merck

At Merck, known as MSD outside of the United States and Canada, we are unified around our purpose: We use the power of leading-edge science to save and improve lives around the world. For more than 130 years, we have brought hope to humanity through the development of important medicines and vaccines. We aspire to be the premier research-intensive biopharmaceutical company in the world – and today, we are at the forefront of research to deliver innovative health solutions that advance the prevention and treatment of diseases in people and animals. We foster a diverse and inclusive global workforce and operate responsibly every day to enable a safe, sustainable and healthy future for all people and communities. For more information, visit [www.merck.com](http://www.merck.com) and connect with us on [Twitter](https://twitter.com), [Facebook](https://www.facebook.com), [Instagram](https://www.instagram.com), [YouTube](https://www.youtube.com) and [LinkedIn](https://www.linkedin.com).

Forward-Looking Statement of Merck & Co., Inc., Rahway, N.J., USA

This news release of Merck & Co., Inc., Rahway, N.J., USA (the “company”) includes “forward-looking statements” within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. These statements are based upon the current beliefs and expectations of the company’s management and are subject to significant risks and uncertainties. There can be no guarantees with respect to pipeline candidates that the candidates will receive the necessary regulatory approvals or that they will prove to be commercially successful. If underlying assumptions prove inaccurate or risks or uncertainties materialize, actual results may differ materially from those set forth in the forward-looking statements.

Risks and uncertainties include but are not limited to, general industry conditions and competition; general economic factors, including interest rate and currency exchange rate fluctuations; the impact of the global outbreak of novel coronavirus disease (COVID-19); the impact of pharmaceutical industry regulation and health care legislation in the United States and internationally; global trends toward health care cost containment; technological advances, new products and patents attained by competitors; challenges inherent in new product development, including obtaining regulatory approval; the company’s ability to accurately predict future market conditions; manufacturing difficulties or delays; financial instability of international economies and sovereign risk; dependence on the effectiveness of the company’s patents and other protections for innovative products; and the exposure to litigation, including patent litigation, and/or regulatory actions.
The company undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events or otherwise. Additional factors that could cause results to differ materially from those described in the forward-looking statements can be found in the company’s Annual Report on Form 10-K for the year ended December 31, 2022 and the company’s other filings with the Securities and Exchange Commission (SEC) available at the SEC’s Internet site (www.sec.gov).

Media Contacts:

Julie Cunningham  
(617) 519-6264

Ayn Wisler  
(917) 691-6218

Investor Contacts:

Peter Dannenbaum  
(732) 594-1579

Damini Chokshi  
(732) 594-1577

Source: Merck & Co., Inc.