Vast’s Lead Drug Candidate Receives Orphan Drug Designation for Treatment of Respiratory Infection for Cystic Fibrosis

Phase 1 program set to begin Q4 2025 in the US

MORRISVILLE, NC, UNITED STATES, July 16, 2025 /EINPresswire.com/ -- Vast Therapeutics, a clinical-stage life science company, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to the active pharmaceutical ingredient used in our investigational ALX1 drug product.

> Orphan drug designation marks an important milestone in our mission to address critical challenges in lung health”
>
> *Paul Bruinenberg MD MBA, Chief Medical Officer of Vast Therapeutics*

This designation reflects Vast’s commitment to delivering first-in-class therapies to transform the lives of patients facing serious lung diseases. The FDA grants orphan drug designation to investigational therapies that address rare medical diseases or conditions that affect fewer than 200,000 people in the U.S.

"Orphan drug designation marks an important milestone in our mission to address critical challenges in lung health,” said Paul Bruinenberg MD MBA, Chief Medical Officer of Vast Therapeutics. “People with bronchiectasis, and more specifically those with cystic fibrosis, often live with chronic bacterial colonization in their lungs that significantly impacts both quality of life and life expectancy. These patients deserve novel treatment approaches with the goal of eradicating the pathogen and improving lung function.”

Orphan drug designations from the FDA offer several key benefits to life science companies aiming to treat patients with rare diseases. The potential incentives include tax credits, waiver of PDUFA fees, and an additional 7-years of market exclusivity.

About ALX1
Clinical evidence shows that people with certain chronic respiratory diseases, like cystic fibrosis, have significantly reduced nitric oxide production that impairs their ability to mount an effective defense against microbial challenges and regulate chronic inflammation. Our lead candidate, ALX1, contains a first-in-class small molecule prodrug designed to efficiently restore nitric oxide levels in the lung. Delivered via a hand-held, portable inhalation device, ALX1 is nebulized daily

as a fine mist into the lungs. Nitric oxide’s dual mechanism of action targets the vicious cycle of infection and inflammation in these patients.

About Vast Therapeutics
Vast is a clinical-stage life science company committed to creating transformative medicines for patients with serious lung diseases. The debilitating cycle of chronic infection and inflammation affects lungs across the entire spectrum of human life, ranging from children with rare orphan diseases like cystic fibrosis (CF) to adults with the highly prevalent chronic obstructive pulmonary disease (COPD). Our drug candidates target the nitric oxide insufficiency in these diseases to provide both meaningful patient benefits as well as address underlying causality.

Nathan Stasko, Chief Executive Officer
Vast Therapeutics, Inc.
nstasko@vasttherapeutics.com
Visit us on social media:
LinkedIn
