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Aerami Therapeutics Announces Orphan Drug Designation for Imatinib for the Treatment of Pulmonary Arterial Hypertension

- ***Pulmonary arterial hypertension (PAH) affects almost 68,000 patients worldwide, with treatment limited to vasodilators, none of which are disease-modifying***
- ***Dosing of patients in Phase 1 trial is currently ongoing***
- ***Plans to enter Phase 2/3 Trials in early 2022***

DURHAM, N.C., Aug. 25, 2021 (GLOBE NEWSWIRE) -- Aerami Therapeutics, Inc. a clinical stage biopharmaceutical company developing inhaled therapies to treat severe respiratory and chronic diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted the company orphan drug designation for imatinib for treatment of patients with pulmonary arterial hypertension (PAH). AER– 901, Aerami's drug-device combination product candidate for inhaled imatinib for the treatment of PAH is currently in a Phase 1 trial with completion targeted for the end of 2021.

The FDA's Office of Orphan Drug Products grants orphan status to support development of medicines for underserved patient populations, or rare disorders, that affect fewer than 200,000 people in the U.S. Orphan drug designation provides certain benefits, including the potential for a seven-year market exclusivity upon regulatory approval, exemption from FDA application fees and tax credits for qualified clinical trials.

Even though PAH patient numbers are low and currently approved products do not fundamentally modify or halt the progression of the disease, they have together created one of the largest specialty pharmaceutical markets with ~$5.5B in worldwide sales in 2020.

"Receiving orphan drug designation for imatinib in AER-901 is another important milestone to emerge from our PAH development program," said Steve Thornton, chief executive officer of Aerami "We are pleased with the progress we are making in our Phase 1 trial and believe that AER- 901, which is targeted to enter Phase 2/3 trials in the first half of 2022, might, for the first time, provide the opportunity to modify the course of this terrible disease, and offer an important therapeutic option for patients."

Oral imatinib is a tyrosine kinase inhibitor used to treat certain types of cancers which has been previously investigated in a Phase 3 clinical trial, IMPRES, as an oral formulation for the treatment of PAH, given its potential to reduce the tightening and stiffness of pulmonary arteries. Oral imatinib demonstrated clinically meaningful and statistically significant improvement in pulmonary hemodynamics and physical exercise capacity but the indication was not pursued because of substantial adverse events believed to be a result of systemic

toxicity associated with the high oral doses used in the study.

“By delivering Aerami’s proprietary inhaled imatinib, directly to the site of the disease, we believe AER-901 has the potential to significantly reduce the dose necessary to achieve therapeutic benefit thereby avoiding the adverse events seen with oral imatinib,” said Timm Crowder, president of Aerami, “Our nebulized formulation and delivery system has the potential to improve efficacy through deeper lung penetration and better drug uptake while reducing the potential for side effects like cough, which is commonly associated with other inhaled technologies such as dry powder formulations.”

About AER-901

AER-901 has been developed as a nebulized formulation to improve drug uptake and dosing consistency and to avoid or reduce commonly seen issues related to powdered forms for inhalation including cough, which would be problematic in this group of patients who suffer severe shortness of breath. AER-901 is designed to deliver consistent, therapeutically effective, and well tolerated levels of imatinib through once-a-day inhalation via the Fox® device, licensed from Vectura Group plc, which is both 510k cleared and CE marked. A version of this nebulizer is currently marketed to patients with a form of PAH in Europe. The Fox®, a breath actuated, smart nebulizer, is expected to enable inhaled imatinib to be deposited more deeply in the lungs and small airways than a powdered form. A high percentage of powdered inhaled products are deposited in the throat. The Fox® achieves deep lung deposition by administering drug only during inhalation and controlling the flow rate and volume during inspiration.

About Pulmonary Arterial Hypertension (PAH)

PAH is a devastating disease affecting almost 68,000 patients worldwide with limited treatment options. The disease causes blood vessels in the lungs to become narrowed, blocked or destroyed. This results in slowing of blood flow through the lungs and increases the blood pressure in the lung arteries. Over time, the heart must work harder to pump blood through the lungs, eventually causing the heart muscle to weaken and fail. PAH is often idiopathic and most commonly affects young women. Current therapeutic options focus mostly on vasodilation and have significant shortcomings including limited efficacy, inconsistent survival benefits, and significant side effects. Even with current medications, median survival is only seven to ten years.

About Aerami Therapeutics
A clinical stage biopharmaceutical company developing inhaled therapies to treat severe respiratory and chronic diseases. More information can be found at www.aerami.com

This press release contains “forward-looking statements” concerning the development and commercialization of Aerami’s product candidates, out-licensing arrangements, timing of clinical trials, the company’s business development efforts and its expectations regarding its prospects. Forward-looking statements are subject to risks, assumptions and uncertainties that could cause actual future events or results to differ materially from such statements, including risks associated with the clinical development process and the ability to obtain funding to support planned clinical activities. These statements are made as of the date of this press release. Actual results may vary. Aerami undertakes no obligation to update any forward-looking statements for any reason.

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