

**AEROVATE THERAPEUTICS EMERGES FROM RA CAPITAL INCUBATOR WITH \$72.6 MILLION FUNDING FOR POTENTIAL DISEASE-MODIFYING PULMONARY ARTERIAL HYPERTENSION THERAPY**

***AV-101 is a Proprietary Inhaled Version of Imatinib, Which Has Proven Efficacy Treating PAH; Aerovate Was Founded and Seeded by RA Capital Management; Series A is Led by Sofinnova Investments; Includes New Investors Atlas Venture, Cormorant Asset Management, Surveyor Capital and Osage University Partners***

**BOSTON (August 6, 2020)** – Aerovate Therapeutics, Inc., has closed on \$72.6 million in Series A funding to advance trials of AV-101, an inhaled, dry powder aerosol version of imatinib to treat pulmonary arterial hypertension (PAH). An oral version of imatinib showed significant efficacy in a phase 3 trial as a disease-modifying therapy for PAH, but it was poorly tolerated by PAH patients because of systemic side effects.

The Series A for Aerovate, which was founded and incubated by RA Capital Management, is led by Sofinnova Investments, a new investor in the company. Atlas Venture, Cormorant Asset Management, Surveyor Capital (a Citadel company) and Osage University Partners are the other new investors in the round.

“Aerovate is a biotechnology company focused on developing drugs that meaningfully improve the lives of patients with rare cardiopulmonary disease. It is exciting to secure financing from such a strong syndicate to progress AV-101, our lead program,” said Aerovate founding President Benjamin Dake, PhD. He noted that the financing will take AV-101 through a clinical efficacy read-out, with a registrational trial scheduled to begin during the first half of 2021 with PAH patients. “Sufferers of PAH desperately need a disease-modifying therapy,” Dake added.

AV-101 is an easy-to-use, proprietary dry powder formulation of imatinib that delivers the medicine directly to the lungs. This approach should decrease the systemic adverse effects that hampered the oral version in PAH trials -- by reducing systemic exposure to the drug while simultaneously increasing the dose of medicine delivered directly to diseased lung tissues. AV-101 is administered with a simple inhaler, providing a convenient solution for patients compared to more cumbersome nebulized drugs currently approved in PAH. Those often require prolonged dosing protocols lasting many minutes, multiple times a day, including complicated device assembly and filling – likely reducing patient adherence and impacting real-world efficacy.

Aerovate emerged out of RA Capital’s seed and incubation program. The concept and proprietary drug product underlying AV-101 were co-invented by the RA Capital venture group and supported by its incubator, Carnot Pharma.

“With the support of the high caliber syndicate around the table, I believe Aerovate is poised to move this important treatment into the clinic immediately. This financing keeps the company on a rapid path towards offering this potentially transformational therapeutic to patients with

real need,” said RA Capital Managing Director and Aerovate Board Chair Joshua Resnick, MD. “AV-101 strives to provide PAH patients with the benefits of a disease modifying drug – in an easy-to-use format, free from the inconvenience of nebulized products – without the cancer-drug-like side effects of the oral version. This is extremely encouraging news for sufferers of this disease,” said Resnick.

“We are excited about AV-101’s potential because the core molecule, imatinib, has shown real efficacy for this use, including cases of hemodynamic remission in severely ill PAH patients. Aerovate’s team and their deep experience with PAH and aerosol development suggest that AV-101 may progress quickly and successfully through trials,” said Sofinnova General Partner and Aerovate Board Director Maha Katabi, PhD.

“Pulmonary arterial hypertension is a chronic disorder with a poor quality of life and five-year survival rate of only 57%. In multiple trials, imatinib showed strong clinical benefit for PAH patients that lasted for many months; they experienced improved exercise capacity, lower pulmonary vascular resistance, decreased pulmonary artery pressure and increased cardiac output. Imatinib addresses the underlying cause of the disease, unlike currently available vasodilator therapies,” said Aerovate Chief Medical Officer Hunter Gillies, MD. “Building on imatinib’s established efficacy, our research gives us strong confidence that AV-101, through targeted delivery to the lungs, will minimize systemic adverse effects, provide good tolerability and potentially be even better at treating the underlying disease than the oral version.”

“Given the significant unmet need in PAH, I am excited about leveraging my experience in developing novel products for underserved pulmonary diseases. Having successfully led the Esbriet team through approval for the treatment of IPF in the US, I look forward to working with Aerovate’s talented team to develop AV-101 to augment the current standard of care in PAH,” said Aerovate independent Board Director Jonathan Leff, MD.

The Aerovate board will include Atlas Venture Partner David Grayzel, MD, along with Dake, Katabi, Leff and Resnick.

## **About PAH**

PAH is a disorder that progresses rapidly and afflicts approximately 80,000 people worldwide and for which there are, to date, no approved disease-modifying therapies. PAH is a proliferative disorder of the pulmonary vasculature characterized by remodeling, constriction and occlusion of the small pulmonary arteries resulting in elevated blood pressure in the pulmonary circulation. PAH causes significant strain on the heart, leading to limited physical activity, heart failure and reduced life expectancy. Even with the current standard of care vasodilator therapies, which dilate pulmonary vessels but do not directly modify the disease, PAH often progresses at a fast pace. Aerovate estimates that, based on \$5 billion annual sales from the currently approved vasodilator drugs, the market for disease-modifying PAH therapies is significant.

## **About Aerovate Therapeutics**

Aerovate Therapeutics is a biotechnology company focused on developing drugs that meaningfully improve the lives of patients with rare cardiopulmonary disease. Aerovate's lead program is a potentially disease modifying drug, AV-101, for people who suffer from pulmonary arterial hypertension (PAH). AV-101 is a proprietary inhaled dry powder formulation of the drug imatinib that delivers this medicine directly to diseased lung tissues while reducing systemic adverse side effects. Aerovate was founded and incubated by RA Capital Management, a multi-stage investment manager focused on healthcare and life sciences. For more information, please visit [www.aerovatetx.com](http://www.aerovatetx.com)

## **About RA Capital Management**

RA Capital Management is a multi-stage investment manager dedicated to evidence-based investing in public and private healthcare and life science companies developing drugs, medical devices, and diagnostics. For more information, please visit [www.racap.com](http://www.racap.com)

## **About Sofinnova Investments**

Sofinnova Investments is a biopharmaceutical investment firm with approximately \$2.2B in assets under management. Sofinnova invests in both private and public therapeutics-focused biotech companies, in both the U.S. and Europe, across all stages of company formation and growth seeking to build world class companies that aspire to dramatically improve the current state of medical care today and ultimately, the lives of patients. For more information, please visit [www.sofinnova.com](http://www.sofinnova.com)

Media Contact:

Michael-Jon Romano

Sommerfield Communications

[Michael-jon@sommerfield.com](mailto:Michael-jon@sommerfield.com)

+1 (212) 255-8386

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