



NEWS RELEASE

Savara Announces Start of Pivotal Phase III AVAIL Study of AeroVanc

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AeroVanc is the First Inhaled Antibiotic Developed for MRSA Lung Infection in Cystic Fibrosis
AUSTIN, TX -- (Marketwired) -- 09/26/17 -- **Savara, Inc.** (NASDAQ: SVRA), a clinical-stage specialty pharmaceutical company focused on the development and commercialization of novel therapies for the treatment of serious or life-threatening rare respiratory diseases, today announced the initiation of the **AVAIL** study, a Phase III, randomized, double-blind, placebo-controlled study of AeroVanc (vancomycin hydrochloride inhalation powder) for the treatment of persistent methicillin-resistant Staphylococcus aureus (MRSA) lung infection in individuals living with cystic fibrosis (CF).

Inhaled antibiotics are standard of care for gram negative *Pseudomonas aeruginosa* lung infection in individuals living with CF. However, there are no approved inhaled treatment options for gram positive MRSA lung infection despite the increase in MRSA prevalence, now affecting approximately 26% of people with CF in the United States.

"Persistent MRSA infection is associated with increased use of intravenous antibiotics, increased hospitalizations, a faster decline of lung function, as well as shortened life expectancy. Based on the completed Phase II study, AeroVanc represents a promising opportunity to make significant advancement in the treatment of this debilitating infection," said Elliott Dasenbrook, MD, MHS, Assistant Professor of Medicine and Director of the Adult Cystic Fibrosis Program at Cleveland Clinic, Cleveland, OH. Dr. Dasenbrook served as the Coordinating Investigator in the AeroVanc Phase II study, conducted in the United States in 87 subjects with CF.

"Consistent with our prior milestone timing guidance, we are very pleased to announce that patient enrollment is underway in our pivotal AVAIL study of AeroVanc," stated Rob Neville, CEO of Savara. "The initiation of the AVAIL study represents a substantial milestone for Savara and serves to underscore our team's commitment to deliver on time. We believe positive results from the AVAIL study would set us up for submission of an NDA, and we are

excited to begin turning our attention towards commercialization."

About the AVAIL Study

The AVAIL study will enroll approximately 200 subjects (150 subjects \leq 21 years old, 50 subjects $>$ 21 years old), at more than 80 clinical study sites across the United States and Canada. During Period 1 of the study, subjects will be randomly assigned in a blinded 1:1 fashion to receive either AeroVanc (30 mg) twice daily, or placebo, by inhalation for 24 weeks or 3 dosing cycles. A dosing cycle is defined as 28 days of treatment followed by 28 days of observation. During Period 2 of the study, subjects will receive open-label AeroVanc (30 mg) twice daily for an additional 24 weeks or 3 dosing cycles, to evaluate the long-term safety of AeroVanc. The Coordinating Investigator of the study is Dr. Patrick Flume, Professor of Medicine and Director of the Cystic Fibrosis Program, Medical University of South Carolina, Charleston, SC.

The primary efficacy endpoint in the AVAIL study is the mean absolute change in FEV1 percent predicted from baseline, which will be analyzed sequentially at week 4 (the end of cycle 1) and at week 20 (the end of cycle 3). The primary efficacy analysis will be based on patients between 6 - 21 years of age, using all observed data at weeks 4 and 20, as appropriate. Secondary efficacy endpoints include: (i) time to use of another antibiotic medication (oral, IV, and/or inhaled) for pulmonary infection, (ii) the number of successful FEV1-response cycles a subject achieves over Period 1 (weeks 4, 12, and 20), (iii) relative change from baseline in FEV1 percent predicted at weeks 4 and 20, (iv) change from baseline Cystic Fibrosis Questionnaire-Revised scores at weeks 4 and 20 and (v) change from Baseline in Cystic Fibrosis Respiratory Symptom Diary-Chronic Respiratory Symptom Score scores at weeks 4 and 20.

In a randomized, double-blind, placebo-controlled Phase II study in CF patients with persistent MRSA infection, AeroVanc reduced MRSA density in sputum, and showed encouraging trends of improvement in lung function, and respiratory symptoms, as well as prolongation of the time to use of other antibiotics, with best responses in subjects under 21 years of age.

AeroVanc has been granted Orphan Drug Designation and Qualified Infectious Disease Product, or QIDP, status for the treatment of persistent MRSA lung infection in CF patients in the United States. The Orphan Drug Designation makes AeroVanc eligible for seven years of exclusivity from approval in the United States, and ten years of exclusivity in the European Union and the QIDP designation makes AeroVanc eligible for an additional five years of exclusivity in the United States. In 2017, a composition of matter patent covering AeroVanc was issued by the United States Patent and Trademark Office which affords Savara important protection for the program in the U.S., the largest market for the product, augments the Company's market protection strategy, and will expire no earlier than 2032.

About Savara

Savara Inc. is a clinical-stage specialty pharmaceutical company focused on the development and commercialization of novel therapies for the treatment of serious or life-threatening rare respiratory diseases. Savara's pipeline comprises: Molgradex, a Phase III stage inhaled granulocyte-macrophage colony-stimulating factor, or GM-CSF, for PAP; AeroVanc, a Phase III stage inhaled vancomycin for treatment of MRSA infection in Cystic Fibrosis; and, Aironite, an inhaled sodium nitrite for HFpEF in Phase II development. Savara's strategy involves expanding its pipeline of potentially best-in-class products through indication expansion, strategic development partnerships and product acquisitions, with the goal of becoming a leading company in its field. Savara's management team has significant experience in orphan drug development and pulmonary medicine, in identifying unmet needs, developing and acquiring new product candidates, and effectively advancing them to approvals and commercialization. More information can be found at www.savarapharma.com. (Twitter: [@SavaraPharma](https://twitter.com/SavaraPharma))

Forward Looking Statements

Savara cautions you that statements in this press release that are not a description of historical fact are forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements may be identified by the use of words referencing future events or circumstances such as "expect," "intend," "plan," "anticipate," "believe," and "will," among others. Such statements include, but are not limited to, statements that AeroVanc is a promising opportunity to make significant advancement in the treatment of a debilitating infection, our belief that positive results from the AVAIL study would set us up for submission of an NDA, turning our attention towards commercialization, that the Phase II study in CF patients with persistent MRSA infection showed encouraging trends of improvement, that the composition of matter patent affords Savara important protection for the program and the timing, planned enrollment and endpoints of its AVAIL study for AeroVanc. Savara may not actually achieve any of the matters referred to in such forward looking statements, and you should not place undue reliance on these forward-looking statements. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. These forward-looking statements are based upon Savara's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, risks and uncertainties associated with the outcome of our ongoing clinical trials for our product candidates (including AeroVanc), the ability to project future cash utilization and reserves needed for contingent future liabilities and business operations, the availability of sufficient resources for Savara's operations and to conduct or continue planned clinical development programs (including our AVAIL study for AeroVanc), the ability to obtain the necessary patient enrollment for our AVAIL study and for our product candidates in a timely manner, the timing and ability of Savara to raise additional equity capital to fund continued

operations; the ability to successfully develop our product candidates, and the risks associated with the process of developing, obtaining regulatory approval for and commercializing drug candidates such as Molgradex, AeroVanc and Aironite that are safe and effective for use as human therapeutics. All forward-looking statements are expressly qualified in their entirety by these cautionary statements. For a detailed description of our risks and uncertainties, you are encouraged to review our documents filed with the SEC including our recent filings on Form 8-K, Form 10-K and Form 10-Q. You are cautioned not to place undue reliance on forward-looking statements, which speak only as of the date on which they were made. Savara undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made, except as may be required by law.

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