



NEWS RELEASE

# Savara Reports Completion of Enrollment in Phase 2 Clinical Trial of Aironite for the Treatment of HFpEF

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## Top-Line Results on Track for First Half of 2018

AUSTIN, TX -- (Marketwired) -- 10/18/17 -- **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 reported that patient enrollment is now complete in a multicenter, randomized, double-blind, placebo-controlled Phase 2 clinical trial of Aironite, a sodium nitrite solution for inhalation via nebulization, in patients with heart failure with preserved ejection fraction (HFpEF). The 100-patient study, known as the Inorganic Nitrite Delivery to Improve Exercise Capacity in HFpEF (INDIE-HFpEF) study, is sponsored by Duke Clinical Research Institute (DCRI) as the Coordinating Center for the Heart Failure Clinical Research Network (HFN) and is being conducted at various clinical centers that are part of the HFN. Savara expects that top-line results from the INDIE-HFpEF study will be available in the first half of 2018.

"We are pleased to report that patient enrollment has been completed ahead of schedule in the INDIE-HFpEF study of Aironite," stated Rob Neville, Chief Executive Officer of Savara, Inc. "We appreciate Duke's and the HFN's support throughout the study and their recognition of the potential of Aironite as an exciting new investigational treatment option for heart failure."

"Exercise intolerance is the major complaint contributing to HFpEF patients' symptoms and reduced quality of life," said Barry A. Borlaug, M.D., a leading cardiologist, who has performed and published other studies of Aironite in HFpEF patients. "A medication such as Aironite is particularly exciting for these patients due to its ability to affect central hemodynamic dysfunction as well as peripheral limitations."

Limitation in exercise capacity is the main factor affecting the function and quality of life in HFpEF patients.

Published results from two Aironite Phase 2a clinical trials, a randomized, double-blind placebo-controlled study,

and an open label study, have demonstrated promising improvements on both exercise hemodynamics in HFpEF patients, as well as on resting hemodynamics in patients with pulmonary hypertension associated with HFpEF.

### **About the INDIE-HFpEF Study**

The INDIE-HFpEF study is a multicenter, randomized, double-blind, placebo-controlled crossover study to evaluate the effect of Aironite on peak exercise capacity as assessed by cardiopulmonary exercise testing (CPET). The study is sponsored and conducted by the HFN, and Savara's wholly-owned subsidiary, Aires Pharmaceuticals, Inc., is providing the study drugs, nebulizers, as well as regulatory, technical and additional financial support for the study. One hundred patients with a diagnosis of HFpEF were enrolled across 17 clinical centers in the United States. The primary endpoint to be analyzed will be the peak oxygen consumption (VO<sub>2</sub>) after four weeks of treatment with Aironite or placebo as assessed by CPET performed at peak drug levels. Secondary objectives include (i) submaximal activity tolerance chronically, (ii) quality of life, (iii) chronic filling pressures as assessed by echocardiography and natriuretic peptide levels, (iv) ventilatory efficiency at maximal exercise, and (v) submaximal exercise capacity at peak drug levels; as well as evaluation of the safety and tolerability of Aironite.

### **About Aironite**

Aironite is a sodium nitrite solution for inhalation via nebulization that has demonstrated encouraging results in Phase 2 clinical trials conducted to date in patients with heart failure with HFpEF. Nitrite is a direct vasodilator and can be recycled in vivo to form nitric oxide (NO) independent of the classical NO synthase (NOS) pathway. Nitrite and Nitrite-mediated NO formation have multiple beneficial effects, including dilation of blood vessels, reduction in hemodynamic perturbations during exercise in the failing heart, and improvements in skeletal muscle bioenergetics and mitochondrial respiration, resulting in improved efficiency of oxygen utilization during submaximal exercise.

In addition to the INDIE HFpEF study, Savara is also supporting a complementary, double blind, placebo-controlled institution-sponsored Phase 2 study of Aironite in patients with HFpEF, referred to as the INABLE study. The main objective of the INABLE study is to assess the efficacy of Aironite on the same outcome measures as in the INDIE-HFpEF study, but when used in conjunction with supervised cardiac rehabilitation exercise training. Supervised exercise training for HFpEF patients is the only therapeutic intervention to have demonstrated improvements in exercise capacity and quality of life in patients managed with nonspecific heart failure medications, thus the INABLE study is designed to demonstrate Aironite's ability to amplify this benefit.

### **About the Heart Failure Clinical Research Network (HFN)**

The HFN was established by the National Heart, Lung, and Blood Institute (NHLBI) to expedite clinical research on treatments and strategies to improve the management of acute and chronic heart failure. The HFN's work is supported by a grant awarded by the NHLBI, part of the National Institutes of Health (NIH). The HFN provides a unique platform for collaborative research by bringing together many premier centers across North America. The

HFN is composed of nine Regional Coordinating Centers and their affiliated sites, whose investigators provide scientific leadership in the collaborative development of the HFN's scientific agenda. The HFN is recognized for robust enrollment in heart failure clinical trials and high scientific productivity. More information can be found on the HFN's website, <https://www.hfnetwork.org/>.

### **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](http://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 top-line results from the INDIE study will be available in the first half of 2018, that Aironite is an exciting new investigational treatment option for heart failure, that Aironite has demonstrated promising improvements on both exercise hemodynamics in HFpEF patients, as well as on resting hemodynamics in patients with pulmonary hypertension associated with HFpEF and that Aironite has demonstrated encouraging results in Phase 2 clinical trials. 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 Aironite and our other product candidates, 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, the

ability to obtain the necessary patient enrollment 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 Aironite and our other 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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