NEWS RELEASE

# Positive Interim Results Of Aironite Phase 2 Study Presented At 4th Annual Drug Discovery And Development Symposium For Pulmonary Hypertension

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## Aironite Significantly Lowered Pulmonary Artery Pressures and Significantly Increased Pulmonary Arterial Compliance

AUSTIN, Texas, July 11, 2017 /PRNewswire/ -- (**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 positive interim results from an ongoing 50-patient open-label Phase 2 study of Aironite in patients with pulmonary hypertension (PH) from multiple different etiologies. The results were presented in an invited lecture at the 4$^{th}$ Annual Drug Discovery and Development Symposium for Pulmonary Hypertension on July 10$^{th}$, 2017 by lead investigator Marc A. Simon, M.D., M.S., F.A.C.C., Associate Professor of Medicine, Bioengineering, and Clinical Translational Science and Director, Heart Failure Research / Clinical Hemodynamics Core Facility, at the Pittsburgh Heart, Lung, and Blood Vascular Medicine Institute at the University of Pittsburgh.

In the 41 patients enrolled to date in the study, administration of Aironite significantly improved multiple hemodynamic measures, with most pronounced improvements in patients with pulmonary hypertension due to heart failure with preserved ejection fraction (PH-HFpEF). In the 10 PH-HFpEF patients analyzed, Aironite administration resulted in significant overall decreases in right atrial pressure, systolic and diastolic right ventricular pressure, systolic and diastolic pulmonary artery pressure, and pulmonary capillary wedge pressure. Of note, pulmonary capillary wedge pressure decreased by 7.5 mmHg (95% CI –9.0 to –6.0; baseline median value 18mmHg; $p < 0.05$) and mean pulmonary artery pressure decreased by 7.9 mmHg (95% CI –9.4 to –6.3; baseline median value 34 mmHg; $p < 0.05$). Pulmonary artery compliance (Cpa) was improved by 35% (+0.97 ml/mmHg, 95% CI +0.25 to

+1.68; p < 0.01). Aironite was generally well-tolerated and no significant safety concerns were identified, supporting the primary safety outcome of the study.

"These additional results build upon prior interim data published last year in the Journal of Clinical Investigation, and demonstrate that Aironite can significantly improve cardiopulmonary hemodynamics in HFpEF as well as Group 3 PH patients, both clinical conditions which are inadequately treated by currently approved medicinal treatments," stated Taneli Jouhikainen, M.D., Ph.D., Chief Operating Officer of Savara. "If the observed short-term improvements are translated into clinically meaningful functional improvements in our ongoing placebo-controlled studies in HFpEF patients, we believe the product will have exciting potential to be advanced towards Phase 3 studies and hopefully an eventual NDA filing."

## About the Phase 2 Study

This is an institution-sponsored, single-center, open-label Phase 2 study, the main objective of which is to investigate the hemodynamic effects and mechanisms of action of Aironite delivered in a single escalating dose to PH patients who undergo right heart catheterization. The study will enroll patients with PH from different etiologies, comprising 20 patients with advanced PH associated with heart failure with preserved ejection fraction (HFpEF-PH), 20 patients with pulmonary arterial hypertension who may be on standard PH therapy, and 10 patients with PH associated with advanced lung disease. Subjects receive a first dose of 45 mg of Aironite via nebulizer, with one subsequent escalation of dosage to 90 mg approximately 60 minutes after the first dose. During the study, right heart and pulmonary artery hemodynamics are measured continuously, and cardiac output is measured at 15 minute intervals, as well as noninvasive systemic blood pressure and pulse oximetry. Changes in hemodynamics and calculated pulmonary and systemic vascular resistances, as well as pulmonary artery compliance are performed utilizing standard formulas.

## 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 preserved ejection fraction (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.

Savara is also supporting two placebo-controlled institution-sponsored Phase 2 studies of Aironite in patients with HFpEF, referred to as the INDIE and the INABLE studies. The main objective of the INDIE study is to assess the efficacy of Aironite on maximum exercise capacity (peakVO2), activity levels, and quality of life after four weeks of

dosing, whereas the main objective of the INABLE study is assess the efficacy of Aironite on the same outcome measures when used in conjunction with supervised cardiac rehabilitation exercise training. Both studies are currently ongoing, and being conducted at leading U.S. based medical institutions.

## 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 3 stage inhaled granulocyte-macrophage colony-stimulating factor (or GM-CSF) for pulmonary arterial proteinosis (or PAP), AeroVanc, an inhaled vancomycin for MRSA infection in Cystic Fibrosis in preparation for Phase 3, and Aironite, an inhaled sodium nitrite in Phase 2 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, creating and acquiring new product candidates, and effectively advancing them to approvals and commercialization. More information can be found at **www.savarapharma.com**. (Twitter: **@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 relating to that these additional results demonstrate that Aironite can significantly improve cardiopulmonary hemodynamics in HFpEF as well as Group 3 PH patients, our belief that the product will have exciting potential to be advanced towards Phase 3 studies and hopefully an eventual NDA filing, and that Aironite has demonstrated encouraging results in Phase 2 clinical trials conducted to date. 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 ongoing clinical trials for Aironite and Savara's 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 Aironite and other 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 Savara's other product candidates, and the risks associated with the process of developing, obtaining regulatory approval for and commercializing drug candidates such as 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, 10-Q and 10-K. 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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SOURCE Savara Inc.

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