



NEWS RELEASE

# Savara Provides Enrollment Update on Molgradex Impala and OPTIMA Clinical Studies and Reaffirms Guidance for Topline Data

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AUSTIN, Texas, Sept. 27, 2018 (GLOBE NEWSWIRE) -- **Savara Inc.** (Nasdaq: SVRA), an orphan lung disease company, today provided patient enrollment updates for its Molgradex pivotal Phase 3 clinical study, **IMPALA**, for the treatment of autoimmune pulmonary alveolar proteinosis (aPAP), as well as its Molgradex Phase 2a clinical study, **OPTIMA**, for the treatment of nontuberculous mycobacterial (NTM) lung infection, both of which were guided to complete enrollment by the end of Q3 2018. Patient enrollment in the IMPALA study is currently at 129 patients out of a total target of 135 patients, with sufficient patients in screening to complete enrollment within the coming weeks and topline data still anticipated in Q2 2019. Patient enrollment in the OPTIMA study is currently at 25 patients out of a total target of 30 patients, with sufficient patients in screening to complete enrollment within the coming weeks. Interim results from the OPTIMA study are still on track to be reported in Q4 2018 and topline data is anticipated in Q2 2019.

## About the IMPALA Phase 3 Clinical Study

The IMPALA study is a randomized, double-blind, placebo-controlled study in 135 subjects, being conducted in 18 countries worldwide, designed to compare the efficacy and safety of Molgradex with placebo in patients with aPAP. Patients are randomized to receive treatment for 24 weeks in one of three treatment arms: 1) Molgradex 300 µg administered once daily, 2) Molgradex 300 µg and matching placebo administered daily in 7-day intermittent cycles of each, or 3) inhaled placebo administered once daily. The primary endpoint in the IMPALA study is the absolute change from baseline in arterial-alveolar oxygen gradient, a measure of patient's oxygenation status, following 24 weeks of treatment. In addition, the FDA will focus its review on three key secondary endpoints assessed to show improvement in clinical symptoms and function, including six-minute walk distance, St. George's respiratory questionnaire, and the time to need of whole lung lavage.

### About the OPTIMA Phase 2a Clinical Study

OPTIMA is an open-label, non-controlled, multi-center, Phase 2a clinical study of Molgradex in 30 subjects ( $\geq 18$  years of age) with persistent pulmonary NTM lung infection. OPTIMA is enrolling subjects with chronic M. abscessus or Mycobacterium avium complex (MAC) infection, with all subjects having either antibiotic refractory infection or intolerance to standard NTM antibiotics. Subjects with CF will not be enrolled. The study comprises a 24-week treatment period and a 12-week follow up period. Two subgroups of subjects are being recruited into the OPTIMA study. Group 1 consists of subjects who remained sputum culture positive while currently on a multidrug NTM guideline based anti-mycobacterial regimen, which had been ongoing for at least six months prior to the baseline visit. Group 2 consists of subjects who remained sputum culture positive, but either stopped a multidrug NTM guideline based anti-mycobacterial regimen at least 28 days prior to screening due to lack of response or intolerance, or never started such treatment. The primary endpoint in the study is sputum culture conversion, defined as at least three consecutive sputum samples without growth of NTM. Secondary endpoints include: (i) the number of subjects with sputum smear conversion to negative, defined as at least three consecutive negative acid-fast bacilli (AFB) stained sputum smears on microscopy among subjects who were smear positive at baseline, (ii) the number of subjects with durable sputum culture conversion, defined as sputum culture conversion at or before week 24 and culture still negative for growth of NTM at 12-week follow up, (iii) the number of subjects with durable sputum smear conversion, defined as sputum smear conversion at or before week 24 and AFB stained smear still negative for NTM at 12-week follow up among subjects who were smear positive at baseline, and (iv) other microbiological indicators, exercise capacities and patient reported outcomes.

### About Savara

Savara is an orphan lung disease company. Savara's pipeline comprises Molgradex, an inhaled granulocyte-macrophage colony-stimulating factor, or GM-CSF, in Phase 3 development for autoimmune pulmonary alveolar proteinosis, or aPAP, in Phase 2a development for nontuberculous mycobacterial, or NTM, lung infection, and in preparation for Phase 2a development in cystic fibrosis, or CF, affected individuals with chronic NTM lung infection; and AeroVanc, a Phase 3 stage inhaled vancomycin for treatment of persistent methicillin resistant staphylococcus aureus, or MRSA, lung infection in CF. 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. The most recent acquisition is aerosolized amikacin/fosfomycin, a Phase 2 ready, proprietary combination antibiotic, which has demonstrated potent and broad-spectrum antibacterial activity against highly drug resistant pathogens. Savara's management team has significant experience in orphan drug development and pulmonary medicine, 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)

## 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 regarding having sufficient patients in screening to complete enrollment in our Molgradex Phase 3 IMPALA and Phase 2a OPTIMA studies within the coming weeks, the timing of topline data or interim results from our IMPALA and OPTIMA studies, and Savara's strategy. 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 and planned clinical trials for our product candidates, the ability to successfully identify exploratory product pipeline candidates, the ability to successfully execute our strategy for amikacin/fosfomycin, 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 ability to successfully develop our product candidates, the risks associated with the process of developing, obtaining regulatory approval for and commercializing drug candidates such as Molgradex, AeroVanc and amikacin/fosfomycin that are safe and effective for use as human therapeutics and the timing and ability of Savara to raise additional equity capital as needed to fund continued operations. 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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