



NEWS RELEASE

# Savara Completes Enrollment in Molgradex OPTIMA Clinical Study for the Treatment of NTM

2018-10-31

Interim Results Expected in Q4 2018

AUSTIN, Texas, Oct. 31, 2018 (GLOBE NEWSWIRE) -- **Savara Inc.** (Nasdaq: SVRA), an orphan lung disease company, today announced completion of the target enrollment of 30 patients in **OPTIMA**, a Phase 2a clinical study evaluating its lead product candidate Molgradex, an inhaled formulation of recombinant human granulocyte-macrophage colony-stimulating factor (GM-CSF) for the treatment of nontuberculous mycobacterial (NTM) lung infection. Savara remains on track to report interim results from the OPTIMA study in the fourth quarter of 2018, with top line results expected in the second quarter of 2019.

Molgradex is also being investigated in a global pivotal Phase 3 clinical study, called **IMPALA**, for the treatment of autoimmune pulmonary alveolar proteinosis (aPAP). Enrollment in the study is complete and top line results are expected in the second quarter of 2019.

"People with NTM lung infections urgently need more effective treatment options, and we believe the rapid enrollment completion in OPTIMA reflects a high interest in our innovative, anti-infective immunotherapy candidate," said Rob Neville, Chief Executive Officer, Savara. "Antibiotics currently used with this patient population are poorly tolerated, frequently fail to eradicate the infection and are associated with a high recurrence rate. Molgradex has the potential to fundamentally change the treatment paradigm of NTM infections by stimulating the innate immune system in the lungs, instead of targeting bacteria directly, thereby avoiding problems of antibiotic resistance."

The interim analysis of OPTIMA will focus on safety and tolerability, as well as efficacy as initially assessed by early microbiological results in a subset of subjects who have completed the 24-week treatment period and have culture

results available up to at least the 16-week timepoint. Due to the generally slow growth of NTM on culture media, a sputum sample can be determined negative for NTM only after eight weeks of observation, causing a corresponding lag time in the assessment of the culture data.

#### About the OPTIMA 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 enrolled subjects with chronic *Mycobacterium abscessus* or *Mycobacterium avium* complex (MAC) infection, with all subjects having either antibiotic refractory infection or intolerance to standard NTM antibiotics. Subjects with cystic fibrosis were not enrolled. The study comprises a 24-week treatment period and a 12-week follow up period. Two subgroups of subjects were recruited into the OPTIMA study. Sub-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. Sub-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 NTM Lung Infection

NTM lung infection is a rare and serious lung disorder associated with increased rates of morbidity and mortality. Nontuberculous mycobacteria are naturally-occurring organisms and NTM lung infection can occur when an individual inhales the organism from the environment and develops a slowly progressive and destructive lung disease. NTM lung infection is typically characterized by cough, fatigue and weight loss. NTM infection often becomes chronic, requires long courses of multiple antibiotics, and despite aggressive treatment regimens, treatment failure rates are high, and recurrence of infection common. Chronic NTM lung infection can have a significant impact on quality of life. There have been few advancements in new systemic treatments for NTM lung infection. However, in a recent large, multicenter clinical study, local delivery of an inhaled form of amikacin directly into the lungs was shown to be effective in approximately one third of treatment refractory patients with

pulmonary MAC infection. This suggests that administration of high, local concentrations of drug delivered directly to the site of infection provides an attractive new avenue to improve clinical outcomes in this and other difficult-to-treat chronic lung infections.

#### 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 mycobacteria, 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](https://twitter.com/SavaraPharma), LinkedIn: [www.linkedin.com/company/savara-pharmaceuticals/](http://www.linkedin.com/company/savara-pharmaceuticals/))

#### 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 the timing of top line data or interim results from our OPTIMA and IMPALA studies, the belief that the rapid enrollment completion in OPTIMA reflects a high interest in our innovative, anti-infective immunotherapy candidate, that Molgradex has the potential to fundamentally change the treatment paradigm of NTM infections by stimulating the innate immune system in the lungs, instead of targeting bacteria directly, thereby avoiding problems of antibiotic resistance, 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.

Contacts:

Savara Inc. IR:

Ioana C. Hone ([ir@savarapharma.com](mailto:ir@savarapharma.com))

(512) 961-1891

Savara Inc. PR and Media:

Anne Erickson ([anne.erickson@savarapharma.com](mailto:anne.erickson@savarapharma.com))

(512) 851-1366

For IR: Solebury Trout

Gitanjali Jain Ogawa ([Gogawa@troutgroup.com](mailto:Gogawa@troutgroup.com))

(646) 378-2949

Savara Inc.