



NEWS RELEASE

# Savara Provides Update on Case Reports of Inhaled Granulocyte-Macrophage Colony Stimulating Factor for the Treatment of Nontuberculous Mycobacteria Infection

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AUSTIN, Texas, May 29, 2018 (GLOBE NEWSWIRE) -- **Savara, Inc.** (NASDAQ:SVRA), an orphan lung disease company, today provided an update on two case reports exploring the use of aerosolized granulocyte-macrophage colony stimulating factor (GM-CSF) for the treatment of *Mycobacterium abscessus* (*M. abscessus*), a species of multidrug-resistant nontuberculous mycobacteria (NTM), in individuals living with cystic fibrosis (CF). Notably, culture conversion has now been achieved in both patients, along with meaningful clinical improvement.

The two case reports were initially published in the **European Respiratory Journal** (ERJ) by Mark E. Wylam, M.D., pulmonologist and critical-care specialist at the **Mayo Clinic College of Medicine**, and his coworkers. In the published case reports, inhaled GM-CSF eradicated *M. abscessus* infection in the first subject and precipitated a strong microbiological response in the second subject. Subsequent to the publication, the second subject has now achieved culture conversion without NTM antibiotic treatment.

"This is a remarkable result given that this second subject, unlike the first one, was not treated with NTM antibiotics," stated Mark E. Wylam, M.D., the senior author of the ERJ publication. "Both subjects remain on aerosolized GM-CSF and have experienced clinically meaningful improvements in weight gain and lung function. I believe these case studies strongly reinforce the scientific rationale for the treatment of NTM lung infection using inhaled GM-CSF, and I look forward to the results of Savara's ongoing OPTIMA study."

In Q1 2018 Savara initiated the **OPTIMA** study, a 30-patient, multi-center, open-label Phase 2a clinical trial to investigate the efficacy and safety of Molgradex for the treatment of chronic NTM lung infection. Subjects with



either *M. abscessus* or *Mycobacterium avium* complex (MAC) infection are being enrolled, with all subjects having either antibiotic refractory infection or intolerance to standard NTM antibiotics. The trial consists of 24 weeks of Molgradex treatment, followed by a 12-week observational period. The primary endpoint is sputum culture conversion defined as at least three consecutive negative sputum cultures. Secondary endpoints include other microbiological indicators, exercise capacity and patient-reported outcomes.

Patient enrollment in the OPTIMA study is on track to complete in Q3 2018 and topline results are expected in H1 2019. However, as OPTIMA is an open-label study, depending on enrollment and other factors, interim results from the study may be provided in 2018.

#### About NTM Lung Infection

NTM lung infection is a rare and serious lung disorder associated with increased rates of morbidity and mortality. NTM are naturally-occurring organisms and NTM lung infection can occur when an individual inhales the organism from their 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 and 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 are approximately 50,000 to 80,000 individuals affected by NTM lung infection in the U.S., the most common types involving MAC and *M. abscessus*. There have been few advancements in new systemic treatments for NTM lung infection. However, in a recent Phase 3 clinical trial by Insmed, local delivery of an inhaled form of amikacin directly to the lung was shown to be effective in approximately one third of treatment refractory patients with pulmonary MAC infection, suggesting administration of high local concentrations of drug directly at the site of infection provides an attractive new avenue to improve clinical outcomes in this and other difficult-to-treat chronic lung infections.

#### About Molgradex

Molgradex is an inhaled formulation of recombinant human GM-CSF, in Phase 3 development for autoimmune pulmonary alveolar proteinosis and in Phase 2a development for NTM lung infection. Molgradex is delivered via an investigational eFlow® Nebulizer System (PARI Pharma GmbH). Molgradex has been granted Orphan Drug Designation for the treatment of PAP in the United States and the European Union.

#### 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 and in Phase 2a development for NTM lung infection; and AeroVanc, a Phase 3 stage inhaled vancomycin for treatment of 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. 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 relating to the belief that the Mayo Clinic case reports strongly reinforce the scientific rationale for treatment of NTM lung infection using inhaled GM-CSF and looking forward to the results of the ongoing OPTIMA study, that patient enrollment in the OPTIMA study is on track to complete in Q3 2018, that topline results are expected in H1 2019, that interim results from the OPTIMA study may be provided in 2018, that in a recent Phase 3 clinical trial by Insmed, local delivery of an inhaled form of amikacin directly to the lung was shown to be effective in approximately one third of treatment refractory patients with pulmonary MAC infection, suggesting administration of high local concentrations of drug directly at the site of infection provides an attractive new avenue to improve clinical outcomes in this and other difficult to treat chronic lung infections 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 clinical trials for our product candidates (including the Phase 2a study of Molgradex for NTM), 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 Phase 2a study of Molgradex for NTM), the ability to obtain the necessary patient enrollment for our product candidates in a timely manner (including our Phase 2a study of Molgradex for NTM), 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 and AeroVanc 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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