



NEWS RELEASE

Savara Announces Expedited U.S. Molgradex Development Strategy

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Ongoing IMPALA Study to be Expanded to Serve as Global Pivotal Study for Registration

AUSTIN, Texas, May 10, 2017 /PRNewswire/ -- **Savara Inc.** (NASDAQ: SVRA) today announced that it has received guidance from the U.S. Food and Drug Administration on the clinical program requirements for a New Drug Application submission in the U.S. for Molgradex, an inhaled formulation of recombinant human GM-CSF, for the treatment of autoimmune pulmonary alveolar proteinosis, or PAP. Based on the FDA's guidance, Savara will modify the endpoint hierarchy and statistical analyses of its ongoing IMPALA study, currently enrolling patients in Europe and Japan, to qualify the study as a pivotal Phase 3 study in the U.S. The total number of patients to be enrolled will be increased from 51 to 90 to support the modified design. If successful, the amended study would serve as the sole pivotal study for regulatory submission for marketing authorization of Molgradex for the treatment of PAP in the U.S. in addition to Europe and Japan.

"In the short time since we acquired Molgradex in July 2016 we have made tremendous progress, and having the opportunity to utilize our ongoing IMPALA study as a global Phase 3 study is a major win for the company, with the potential to considerably expedite the approval of the product in the U.S. by eliminating the need to conduct a separate Phase 3 study," stated Rob Neville, Chief Executive Officer of Savara. "This is very good news for the PAP community in the U.S., as we believe Molgradex can offer a game changing treatment alternative in a disease where the current standard treatment option is to periodically conduct an invasive whole lung lavage procedure requiring general anesthesia."

The IMPALA study is a randomized, double-blind, placebo-controlled study designed to compare the efficacy and safety of Molgradex with placebo in patients with PAP. The study, which began enrolling patients in Europe and Japan last year, will be expanded to enroll a total of 90 patients, half of which have already been enrolled. Patient



enrollment is expected to be completed by the first quarter of 2018, and top line data is expected to be available by the fourth quarter of 2018.

"Molgradex is a unique product that directly addresses the disease mechanism causing PAP – an autoimmune blockade of a naturally occurring signaling protein, GM-CSF, which is required to clear excess surfactant from the lungs," said Dr. Bruce Trapnell, M.D., Professor of Medicine and Pediatrics, Cincinnati Children's Hospital Medical Center, and Director of the NCATS-NHLBI Rare Lung Diseases Clinical Research Consortium. "PAP is a debilitating lung disease with a high clinical need for an effective medicinal treatment to reduce or eliminate the need for whole lung lavage. Inhaled GM-CSF restores the ability of lung cells to clear surfactant. Based on an increasing body of literature and clinical experience of explorative use of inhaled GM-CSF, I and my colleagues treating PAP patients worldwide are very optimistic about the potential of Molgradex as a truly transformative treatment option in PAP."

The primary endpoint in the IMPALA study will remain 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 that will be 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. Patients are randomized to receive treatment for up to 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.

About Pulmonary Alveolar Proteinosis (PAP)

PAP is a rare lung disease which is characterized by the build-up of lung surfactant in the alveoli, or air sacs, of the lungs. The surfactant consists of proteins and lipids, and is an important physiological substance that lines the inside of the alveoli to prevent the lungs from collapsing. In a healthy lung, the old and inactivated surfactant is cleared and digested by immune cells called alveolar macrophages. Alveolar macrophages need to be stimulated by GM-CSF to function properly in clearing surfactant, but in autoimmune PAP, GM-CSF is neutralized by antibodies against GM-CSF, rendering the macrophages unable to perform their tasks. As a result, an excess of surfactant accumulates in the alveoli, causing obstruction of gas exchange, and patients start to experience shortness of breath, and decreased exercise tolerance. Patients may also experience chronic cough, as well as episodes of fever, chest pain, or coughing blood, especially if secondary lung infection develops. In the long term, the disease can lead to serious complications, including lung fibrosis and the need for lung transplant.

About Molgradex

Molgradex, an inhaled formulation of recombinant human GM-CSF, is being developed for the treatment of autoimmune pulmonary alveolar proteinosis, or PAP. Savara is also pursuing indication expansion, with priority on the development of Molgradex in rare infectious lung diseases, where stimulation of the innate immune system has the potential to improve clinical outcomes. The Company expects to announce details related to its indication expansion strategy by the third quarter of 2017. Molgradex has been granted Orphan Drug Designation for the treatment of PAP in the United States and the European Union.

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, an inhaled granulocyte-macrophage colony-stimulating factor, or GM-CSF, in Phase 3 development, AeroVanc, an inhaled vancomycin in preparation for a Phase 3 study, and Aironite, an inhaled nebulized sodium nitrite in Phase 2 development. Savara's strategy involves expanding its pipeline of 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 regarding the Molgradex development strategy, potential to considerably expedite the approval of the product in the U.S., that Molgradex can offer a game changing treatment alternative, patient enrollment expected to be completed by the first quarter of 2018, and top line data expected to be available by the fourth quarter of 2018 and expecting to announce details related to its indication expansion strategy by the third quarter of 2017. 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 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 in a timely manner, the timing and ability of Savara to raise additional equity capital to fund continued operations; the ability to successfully develop Savara's product candidates, and the risks associated with the process of developing, obtaining regulatory approval for and commercializing drug candidates that are safe and effective for use as human therapeutics. Risks and uncertainties facing Savara are described more fully in Savara's filings with the Securities and Exchange Commission including the Form 8-K filed on April 27, 2017, other filings on Form 8-K, the Annual Report on Form 10-K for the fiscal year ended December 31, 2016, the Quarterly Report on Form 10-Q for the quarter ended March 31 2017 and the Registration Statement on Form S-4 related to the Mast/Savara merger. 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 any 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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