



NEWS RELEASE

# Savara to Discontinue Exploratory ENCORE Study Evaluating Molgradex for the Treatment of NTM Lung Infection in People Living With Cystic Fibrosis (CF)

2020-09-03

Study Stopped Enrolling in March 2020 Due to Limitations Caused by COVID-19

AUSTIN, Texas--(BUSINESS WIRE)--Sep. 3, 2020-- Savara Inc. (Nasdaq: SVRA), an orphan lung disease company, today announced the decision to discontinue ENCORE, a 48-week, open-label, non-controlled Phase 2a exploratory clinical study of Molgradex for the treatment of nontuberculous mycobacterial (NTM) lung infection in people living with cystic fibrosis (CF). Molgradex, an inhaled formulation of recombinant human granulocyte-macrophage colony-stimulating factor (GM-CSF), was also evaluated for the treatment of NTM in non-CF patients in the Phase 2a OPTIMA study that completed in March 2020.

The decision to discontinue ENCORE was based on confounding factors that compromised the ability of the study to achieve its primary purpose of investigating the efficacy of Molgradex on NTM sputum culture conversion to negative. Such factors included the impact of COVID-19 on patient recruitment and continued participation in the study as well as the availability of the new triple-combination CFTR modulator, approved during the treatment period of ENCORE, that has become a preferred treatment option for many CF patients. Study recruitment was terminated at the end of March with 14 patients enrolled out of a total target of 30. Additionally, nine out of 14 patients were on the triple-combination CFTR modulator. Eight patients started it during the study and one patient was on the triple-combination modulator from baseline. The decision to discontinue the study was not based on safety concerns.

Due to the early discontinuation of the study, not all patients have completed the planned 48-week treatment period. Based on preliminary data as of September 2020<sup>1</sup> from 12 patients who progressed at least beyond 20



weeks of treatment, five patients on the triple-combination CFTR modulator achieved a sputum culture conversion, defined as at least three consecutive sputum samples without growth of NTM. All of those patients had started the triple-combination modulator during the study prior to culture conversion. Sputum culture conversions were not observed in patients who were on Molgradex without the triple-combination modulator.

“Discontinuing the exploratory ENCORE study is very disappointing and I extend our sincere gratitude to the patients who participated, especially during the trying times of this pandemic,” said Badrul Chowdhury, Chief Medical Officer, Savara. “Many factors contributed to this decision, most notably the effects of COVID-19, which resulted in small patient numbers and operational disruptions at clinical sites. The approval of the triple-combination CFTR modulator, which transformed the standard-of-care for this patient population, further confounded our ability to evaluate the potential effect of Molgradex in this study as we are unable to differentiate which drug was primarily responsible for the observed culture conversions. While the results of ENCORE are interesting, additional controlled studies would be required to accurately understand the therapeutic potential of Molgradex in combination with the triple-combination modulator treatment.”

Based on the results of the exploratory ENCORE and OPTIMA studies, the Company will continue to focus development efforts of Molgradex on its lead indication, autoimmune pulmonary alveolar proteinosis, and does not plan to conduct further development activities related to Molgradex in NTM.

## About Savara

Savara is an orphan lung disease company with a pipeline comprised of three investigational compounds, all of which use an inhaled delivery route. Our lead program, Molgradex (molgramostim nebulizer solution), is an inhaled granulocyte-macrophage colony-stimulating factor (GM-CSF) in Phase 3 development for autoimmune pulmonary alveolar proteinosis (aPAP). Apulmiq is an inhaled liposomal ciprofloxacin in Phase 3 development for non-cystic fibrosis bronchiectasis (NCFB). AeroVanc is an inhaled vancomycin in Phase 3 development for persistent methicillin-resistant *Staphylococcus aureus* (MRSA) lung infection in people living with cystic fibrosis. Savara’s strategy involves broadening its pipeline through indication expansion, strategic development partnerships and product acquisitions, with the goal of becoming a leading company in its field. Our 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 approval 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 that additional controlled studies would be required to accurately understand the therapeutic potential of Molgradex in combination with the triple-combination modulator treatment, that Savara will continue to focus development efforts of Molgradex on its lead indication, and that Savara does not plan to conduct further development activities related to Molgradex in NTM. 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. 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 impact of the COVID-19 pandemic on our business and operations, the outcome of our ongoing and planned clinical trials for our 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 planned clinical development programs, 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 that are safe and effective for use as human therapeutics, and the timing and ability of Savara to raise additional 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.

<sup>i</sup> Data in this press release have not been validated and are subject to the final study report.

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## Savara Inc. IR & PR

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

(512) 851-1366

Source: Savara Inc.