April 27, 2020

Citius Announces Pre-IND Submission to FDA Under the Coronavirus Treatment Acceleration Program for a Novel Stem Cell Therapy for Acute Respiratory Distress Syndrome (ARDS) in COVID-19

- Company's goal to initiate clinical trial in patients in 2020

- Innovative therapy candidate uses next generation mesenchymal stem cells (MSCs) derived from a master cell bank of induced pluripotent stem cells (iPSCs)

CRANFORD, N.J., April 27, 2020 /PRNewswire/ -- Citius Pharmaceuticals, Inc. ("Citius" or the "Company") (Nasdaq: CTXR), a specialty pharmaceutical company focused on developing and commercializing critical care drug products, today announced that it submitted a pre-IND meeting request and supporting briefing documents to the Center for Biologics Evaluation and Research ("CBER") of the FDA under the Coronavirus Treatment Acceleration Program (CTAP) on April 24. The Company has requested the Division's feedback to support the most expeditious pathway into the clinic to evaluate a novel cell therapy in patients suffering from COVID-19-related ARDS.

The cells, called NoveCite Cells or NC-MSCs, are made by Novellus, Inc. ("Novellus"), a Cambridge-based biotechnology company, using its patented mRNA-based cell-reprogramming process. NC-MSCs are mesenchymal stem cells derived from a single donor's fibroblasts that have been dedifferentiated into an induced pluripotent stem cell (iPSC) master cell bank, thereby avoiding the need to source additional donor cells. The iPSCs are then further differentiated into a mesenchymal stem cell (MSC) therapy. Citius and Novellus plan to develop NC-MSCs for the treatment of ARDS, and last month the companies signed an exclusive option agreement.

The Company plans a multi-center randomized placebo-controlled dose-finding study followed by an expansion phase to assess the safety, tolerability, and efficacy of NC-MSCs in patients with moderate to severe ARDS due to COVID-19. The proposed trial, a Phase 1b/2 clinical trial, is titled "A Randomized Placebo-Controlled Dose-Finding Study Followed by a Dose Level Expansion to Assess the Safety and Efficacy of NoveCite **M**SCs in Subjects with **A**cute **R**espiratory Distress Syndrome (ARDS) Due to SARS-CoV-2 Disease (**CO**VID-19)," or "**MARCO"**. The primary objectives of this study are to evaluate the safety and efficacy of NoveCite cells as a treatment for subjects with moderate-to-severe ARDS due to COVID-19 and to identify therapeutic doses.

"MSCs have an established track-record of clinical safety, and have shown promise in the

treatment of inflammatory lung disease," said Matt Angel, PhD, co-founder and Chief Science Officer at Novellus, Inc. "Our research has shown that the NoveCite cells, being derived from mRNA-reprogrammed iPSCs, secrete higher levels of immunomodulatory proteins than donor-derived MSCs, and have unique manufacturing advantages."

"We believe we have the key elements in place from a clinical design and manufacturing point of view to evaluate this novel cell therapy approach to deal with the current pandemic," said Myron Holubiak, Chief Executive Officer of Citius. "ARDS is a very serious complication for many patients suffering from COVID-19, and is believed to account for about 80% of the deaths in ventilated patients. There is no proven or FDA-approved treatment for it, other than oxygen therapy, including use of mechanical ventilation, and fluid management. Literature from previous investigational studies with MSCs in the treatment of lung injuries support the idea that MSCs could prove effective in treating COVID-19-related ARDS. We look forward to our FDA discussions and are excited to be at the cusp of what could be a novel and effective therapy for ARDS."

About Acute Respiratory Distress Syndrome (ARDS)
ARDS is a type of respiratory failure characterized by rapid onset of widespread inflammation in the lungs. ARDS is a rapidly progressive disease that occurs in critically ill patients – most notably now in those diagnosed with COVID-19. ARDS affects approximately 200,000 patients per year in the U.S., exclusive of the current COVID-19 pandemic, and has a 30% to 50% mortality rate. ARDS is sometimes initially diagnosed as pneumonia or pulmonary edema (fluid in the lungs from heart disease). Symptoms of ARDS include shortness of breath, rapid breathing and heart rate, chest pain (particularly while inhaling), and bluish skin coloration. Among those who survive ARDS, a decreased quality of life is relatively common.

About Coronavirus Treatment Acceleration Program (CTAP)
In response to the pandemic, the FDA has created an emergency program called the Coronavirus Treatment Acceleration Program (CTAP) to accelerate the development of treatments for COVID-19. By redeploying staff, the FDA is responding to COVID-19-related requests and reviewing protocols within 24 hours of receipt. The FDA said CTAP "uses every available method to move new treatments to patients as quickly as possible, while at the same time finding out whether they are helpful or harmful." In practice, that means developers of potential treatments for COVID-19 will benefit from an unusually faster track at the FDA to shorten wait times at multiple steps of the process.

About Citius Pharmaceuticals, Inc.
Citius is a late-stage specialty pharmaceutical company dedicated to the development and commercialization of critical care products, with a focus on anti-infectives and cancer care. For more information, please visit www.citiuspharma.com.

About Novellus, Inc.
Novellus is a pre-clinical stage biotechnology company developing engineered cellular medicines using its non-immunogenic mRNA, nucleic-acid delivery, gene editing, and cell reprogramming technologies. Novellus is privately held and is headquartered in Cambridge, MA. For more information, please visit www.novellus-inc.com.

Safe Harbor
This press release may contain "forward-looking statements" within the meaning of Section

27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934. Such statements are made based on our expectations and beliefs concerning future events impacting Citius. You can identify these statements by the fact that they use words such as "will," "anticipate," "estimate," "expect," "should," and "may" and other words and terms of similar meaning or use of future dates. Forward-looking statements are based on management's current expectations and are subject to risks and uncertainties that could negatively affect our business, operating results, financial condition, and stock price. Factors that could cause actual results to differ materially from those currently anticipated are: the risk of successfully negotiating a license agreement with Novellus within the option period; our need for substantial additional funds; the ability to access the FDA's CTAP program for the MARCO trial; the estimated markets for our product candidates, including those for ARDS, and the acceptance thereof by any market; risks associated with conducting trials for our product candidates, including those expected to be required for any treatment for ARDS and our Phase III trial for Mino-Lok; risks relating to the results of research and development activities; risks associated with developing our product candidates, including any licensed from Novellus, including that preclinical results may not be predictive of clinical results and our ability to file an IND for such candidates; uncertainties relating to preclinical and clinical testing; the early stage of products under development; risks related to our growth strategy; our ability to obtain, perform under, and maintain financing and strategic agreements and relationships; our ability to identify, acquire, close, and integrate product candidates and companies successfully and on a timely basis; our ability to attract, integrate, and retain key personnel; government regulation; patent and intellectual property matters; competition; as well as other risks described in our SEC filings. We expressly disclaim any obligation or undertaking to release publicly any updates or revisions to any forward-looking statements contained herein to reflect any change in our expectations or any changes in events, conditions, or circumstances on which any such statement is based, except as required by law.

Contact:
Andrew Scott
Vice President, Corporate Development
(O) 908-967-6677 x105
ascott@citiuspharma.com

View original content:http://www.prnewswire.com/news-releases/citius-announces-pre-ind-submission-to-fda-under-the-coronavirus-treatment-acceleration-program-for-a-novel-stem-cell-therapy-for-acute-respiratory-distress-syndrome-ards-in-covid-19-301047150.html

SOURCE Citius Pharmaceuticals, Inc.