Lineage Cell Therapeutics Announces Vitelliform Maculopathy Patient Treated With OpRegen® Under Named Patient Compassionate Use

March 29, 2021

CARLSBAD, Calif.--(BUSINESS WIRE)--Mar. 29, 2021-- Lineage Cell Therapeutics, Inc. (NYSE American and TASE: LCTX), a clinical-stage biotechnology company developing novel cell transplants for serious medical conditions, today announced that a patient suffering from adult-onset vitelliform macular dystrophy (AVMD) had recently been treated with its lead product candidate, OpRegen, at Hadassah-Hebrew University Medical Center in Jerusalem, using a named patient compassionate use approval granted by the Israeli Ministry of Health. OpRegen is an investigational cell therapy consisting of allogeneic retinal pigment epithelium (RPE) cells administered to the subretinal space and is currently being investigated in a 24-patient phase 1/2a clinical trial for the treatment of dry age-related macular degeneration (AMD) with geographic atrophy (GA).

"Lineage is pioneering a new branch of medicine, consisting of the directed differentiation and transplant of specific cell types to replace damaged or dying cells with the goal of restoring or improving function lost to injury or disease," stated Brian M. Culley, Lineage CEO. "With OpRegen, we are transplanting new retina cells to replace old cells that were lost or damaged to disease, with a goal of providing stability or functional improvements to vision. As outlined more fully on our recent earnings call, we believe there are many potential applications of Lineage's core technology and intend this year to demonstrate that although we currently have three clinical-stage product candidates, those assets and our underlying platform may have utility in additional settings. For example, our RPE cells may be useful for treating additional retinal diseases, such as AVMD or Stargardt's Disease. Similarly, our spinal cord program may be applicable to other conditions characterized by demyelination, and our oncology platform may have application across many different tumor types, depending on which antigen we elect to present to the patient's immune system."

Mr. Culley continued, "In this first instance, we treated a patient with AVMD, because it closely resembles dry AMD and similarly involves impaired RPE function and progressive vision loss. When the team at Hadassah approached us about treating their existing AVMD patient with OpRegen on a compassionate use basis, we were supportive of the request and saw it as an opportunity to investigate a new application for our OpRegen product candidate."

This patient presented to the Department of Ophthalmology at Hadassah-Hebrew University Medical Center in late December 2020 with sudden and severe visual acuity decreases in one eye. BCVA in the worse vision eye was measured at 20/200, compared to 20/40 in the patient's contralateral eye. After an onset of blurred vision in 2018, evaluation and imaging diagnosed the patient as suffering from AVMD. Because AVMD is a disease of impaired RPE function leading to atrophy and shares similar characteristics to dry AMD, the team at Hadassah approached Lineage about the potential to treat this patient on a compassionate use basis. Lineage submitted a request on behalf of Hadassah-Hebrew University Medical Center which was approved by the Israeli Ministry of Health. Following approval from the University's Ethics Committee, the patient was treated in February 2021. The delivery of OpRegen RPE cells via pars plana vitrectomy was successful, with no complications arising during the procedure and the patient remains in follow-up.

About Adult-onset Vitelliform Maculopathy (AVMD)

AVMD is an eye disorder that can cause progressive vision loss and usually begins after age 40. AVMD affects an area of the retina called the macula, which is responsible for sharp central vision. The condition causes a fatty yellow pigment to accumulate in cells underlying the macula, eventually damaging the cells. Some people remain without symptoms throughout their life while others may slowly develop blurred and/or distorted vision, that can progress to central vision loss over time. There is currently no effective treatment for vitelliform macular dystrophy.

About OpRegen

OpRegen is currently being evaluated in a Phase 1/2a open-label, dose escalation safety and efficacy study of a single injection of human retinal pigment epithelium cells derived from an established pluripotent cell line and transplanted subretinally in patients with advanced dry AMD with GA. The study enrolled 24 patients into 4 cohorts. The first 3 cohorts enrolled only legally blind patients with best corrected visual acuity (BCVA) of 20/200 or worse. The fourth cohort enrolled 12 better vision patients (vision from 20/65 to 20/250 with smaller areas of GA). Cohort 4 also included patients treated with a new "thaw-and-inject" formulation of OpRegen, which can be shipped directly to sites and used immediately upon thawing, removing the complications and logistics of having to use a dose preparation facility. The primary objective of the study is to evaluate the safety and tolerability of OpRegen as assessed by the incidence and frequency of treatment emergent adverse events. Secondary objectives are to evaluate the preliminary efficacy of OpRegen treatment by assessing the changes in ophthalmological parameters measured by various methods of primary clinical relevance. OpRegen is a registered trademark of Cell Cure Neurosciences Ltd., a majority-owned subsidiary of Lineage Cell Therapeutics, Inc.

About Lineage Cell Therapeutics, Inc.

Lineage Cell Therapeutics is a clinical-stage biotechnology company developing novel cell therapies for unmet medical needs. Lineage's programs are based on its robust proprietary cell-based therapy platform and associated in-house development and manufacturing capabilities. With this platform Lineage develops and manufactures specialized, terminally differentiated human cells from its pluripotent and progenitor cell starting materials. These differentiated cells are developed to either replace or support cells that are dysfunctional or absent due to degenerative disease or traumatic injury or administered as a means of helping the body mount an effective immune response to cancer. Lineage's clinical programs are in markets with billion dollar opportunities and include three allogeneic ("off-the-shelf") product candidates: (i) OpRegen ®, a retinal pigment epithelium transplant therapy in Phase 1/2a development for the treatment of dry age-related macular degeneration, a leading cause of blindness in the developed world; (ii) OPC1, an oligodendrocyte progenitor cell therapy in Phase 1/2a development for the treatment of acute spinal cord injuries; and (iii) VAC, an allogeneic dendritic cell therapy platform for immuno-oncology and infectious disease, currently in clinical development for the treatment of non-small cell lung cancer. For more information, please visit www.lineagecell.com or follow the Company on Twitter @LineageCell.

Forward-Looking Statements

Lineage cautions you that all statements, other than statements of historical facts, contained in this press release, are forward-looking statements. Forward-looking statements, in some cases, can be identified by terms such as “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “design,” “intend,” “expect,” “could,” “plan,” “potential,” “predict,” “seek,” “should,” “would,” “contemplate,” project,” “target,” “tend to,” or the negative version of these words and similar expressions. Such statements include, but are not limited to, statements relating to the potential conditions and diseases applicable to Lineage’s clinical-stage product candidates. Forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause Lineage’s actual results, performance or achievements to be materially different from future results, performance or achievements expressed or implied by the forward-looking statements in this press release, including risks and uncertainties inherent in Lineage’s business and other risks in Lineage’s filings with the Securities and Exchange Commission (SEC). Lineage’s forward-looking statements are based upon its current expectations and involve assumptions that may never materialize or may prove to be incorrect. All forward-looking statements are expressly qualified in their entirety by these cautionary statements. Further information regarding these and other risks is included under the heading “Risk Factors” in Lineage’s periodic reports with the SEC, including Lineage’s most recent Annual Report on Form 10-K filed with the SEC and its other reports, which are available from the SEC’s website. You are cautioned not to place undue reliance on forward-looking statements, which speak only as of the date on which they were made. Lineage 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 required by law.

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Source: Lineage Cell Therapeutics, Inc.