Lineage Provides Update on Patient Enrollment in Phase I/IIa Clinical Study of OpRegen® for the Treatment of Dry Age-Related Macular Degeneration

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CARLSBAD, Calif.--(BUSINESS WIRE)--Dec. 30, 2019-- Lineage Cell Therapeutics, Inc. (NYSE American and TASE: LCTX), a clinical-stage biotechnology company developing novel cellular therapies for unmet medical needs, today announced additional patient data from its ongoing Phase I/IIa clinical study of OpRegen®, the Company's retinal pigment epithelium (RPE) transplant therapy, for the treatment of dry age-related macular degeneration (dry AMD), a leading cause of adult blindness in the developed world with no FDA-approved treatment options.

The first Cohort 4 patient treated using both a new subretinal delivery system and the Company's new thaw-and-inject (TAI) formulation of OpRegen has continued to demonstrate notable improvements in vision, having gained 25 readable letters (or 5 lines) 6 months following administration of OpRegen RPE cells, as assessed by the Early Treatment Diabetic Retinopathy Scale (ETDRS). This represents an improvement in visual acuity from a baseline of 20/250 to 20/100 in the treated eye. A second Cohort 4 patient has been similarly dosed, and though early, the patient has shown a small improvement in visual acuity in the treated eye at just 14 days following treatment. To date, improvements have become most evident approximately three to six months after treatment. Both patients had rapid healing at the surgical site with no unexpected complications or any serious adverse events.

"We are increasingly optimistic about the data we are collecting in dry AMD," stated Brian M. Culley, CEO of Lineage. "We have treated five patients in Cohort 4, those with less advanced disease, which more closely match our intended patient population. At the longest-available assessment point for each patient, all five have shown an increase in the number of letters they can read on an ETDRS eye chart. Importantly, these gains have been maintained for as long as 15 months, which is the longest time point for which we have collected data in the better vision cohort. Notably, the first two patients dosed with the new sub-retinal delivery system by Gyroscope Therapeutics and our innovative TAI formulation of OpRegen had no unexpected complications, so we intend to request the removal of the enrollment treatment stagger from the protocol, which should permit us to significantly accelerate our rate of enrollment. Our objective is to combine the best cells, the best production process and the best delivery system, which we believe will position us as the front-runner in the race to address the unmet opportunity in the potential billion-dollar dry AMD market."

"We expect 2020 will be a year of major milestones for Lineage. Based on our existing cash and the current value of our marketable securities, we believe we will be able to achieve these milestones under our reduced 2020 spending plan," continued Mr. Culley.

"Having dosed a patient with the combination of Gyroscope's recently 510(k)-cleared Orbit Subretinal Delivery System alongside Lineage's new thaw-and-inject formulation of OpRegen RPE cells, I found the procedure to be relatively straightforward, leading to the successful delivery of RPE cells to the subretinal space," stated Judy Ju-Yi Chen, M.D., a retinal surgeon at West Coast Retina, San Francisco, CA. "I am hopeful that additional procedures will show that this combination provides superior dose control, safety, and efficacy compared to conventional procedures."

The ETDRS eye chart consists of a set of letters of diminishing size on each line. The more letters a patient can read, the better their vision. The Company also is collecting data on rate of geographic atrophy (GA) growth, best corrected visual acuity (BCVA), low-light visual acuity, reading speed, quality of life questionnaires, microperimetry, and assessing structural changes using optical coherence tomography (OCT), fundus autofluorescence (FAF), and color fundus photography.

About the Phase I/IIa Clinical Study

This is a Phase I/IIa 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 geographic atrophy. The study will enroll approximately 24 patients, divided into 4 cohorts. The first 3 cohorts consisted solely of legally blind patients, with best corrected visual acuity (BCVA) of 20/200 or worse. The fourth cohort will include approximately 12 patients with vision ranging from 20/250 to as high as 20/64. Cohort 4 also includes patients treated with one of two formulations of OpRegen; the first 3 patients were treated with a formulation which required plating and preparation of cells one day prior to use. The remaining patients on Cohort 4 will be treated with an "off-the-shelf" or "thaw-and-inject" formulation of OpRegen which can be shipped directly to sites and used immediately upon thawing, which removes the complications and logistics of having to use a dose preparation facility. Staggered intervals within and between cohorts are applied to ensure patient safety and welfare. The primary objective of the Phase I/IIa 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. Additionally, for the patients in Cohort 4 that receive subretinal delivery of OpRegen utilizing Gyroscope Therapeutics' Orbit Subretinal Delivery System (Orbit SDS), objectives will include the evaluation of the safety of delivery of OpRegen using the Orbit SDS.

About OpRegen®

OpRegen is a retinal pigment epithelium (RPE) transplant therapy in Phase I/IIa development for the treatment of dry AMD, a leading cause of adult blindness in the developed world. OpRegen consists of a suspension of RPE cells delivered subretinally as an intraocular injection. RPE cells are essential components of the back lining of the retina and function to help nourish the retina including photoreceptors. OpRegen has been granted Fast Track designation from the U.S. Food and Drug Administration. 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 proprietary cell-based therapy platform and associated 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 either to 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 assets include (i) OpRegen®, a retinal pigment epithelium transplant therapy in Phase I/IIa 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 I/IIa development for the treatment of acute spinal cord injuries; and (iii) VAC2, an allogeneic cancer immunotherapy of antigen-presenting dendritic cells currently in Phase I development for the treatment of non-small cell lung cancer. Lineage is also evaluating potential partnership opportunities for Renevia®, a facial aesthetics product that was recently granted a Conformité Européenne (CE) Mark. 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 development of Lineage's OpRegen program, as well as Lineage's spending plans. 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 (the 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 Annual Report on Form 10-K filed with the SEC on March 14, 2019 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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