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

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Removal of Enrollment Stagger and Opening of Leading Ophthalmology Clinical Sites at Cincinnati Eye Institute and Wills Eye Hospital Expected to Significantly Accelerate Patient Enrollment

CARLSBAD, Calif.--(BUSINESS WIRE)--Feb. 6, 2020-- Lineage Cell Therapeutics, Inc. (NYSE American and TASE: LCTX), a clinical-stage biotechnology company developing novel cellular therapies for unmet medical needs, today provided an update 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). Dry AMD is a leading cause of adult blindness in the developed world with no FDA-approved treatment options.

Based on promising preliminary results from the ongoing clinical study, including using Lineage's new thaw-and-inject formulation of OpRegen with Gyroscope Therapeutics' Orbit Subretinal Delivery System (SDS), the Company has been informed by the study's independent data safety monitoring board (DSMB) that the protocol-mandated treatment stagger can be removed. In addition, Lineage is opening two new clinical sites to accelerate patient enrollment and broaden surgical experience among dry AMD experts.

All five patients in Cohort 4, those with better baseline vision and which represent the Company's intended patient population, have shown increases in best corrected visual acuity (BCVA). Notably, the first Cohort 4 patient dosed with both the Company's new thaw-and-inject formulation and Orbit subretinal delivery system, gained 25 readable letters (or 5 lines) at 6 months following administration of OpRegen RPE cells as assessed by the Early Treatment Diabetic Retinopathy Scale (ETDRS). This positive outcome represents an improvement in visual acuity from 20/250 to 20/100 in the patient's treated eye. There was no change in BCVA in the patient's untreated eye. To date, improvements have become most evident approximately three to six months after treatment with improvements in BCVA lasting at least 15 months with longer timepoints still to be collected. Safety data and evidence of successful engraftment of transplanted RPE cells is available for as long as three years in some patients. Because no unexpected complications have been observed with the new formulation and delivery system, the study's DSMB has removed the protocol-mandated enrollment stagger, which will permit Lineage to accelerate the rate of patient enrollment by opening additional clinical sites, as well as to treat patients with less advanced disease and smaller areas of geographic atrophy than patients in Cohort 1 through Cohort 3, which were legally blind due to more progressive disease.

The Company is opening two new clinical sites, Cincinnati Eye Institute and Wills Eye Hospital, both global leaders in ophthalmology research. Principal investigators at the new clinical sites are Christopher D. Riemann, M.D., Vitreoretinal Surgeon and Fellowship Director, Cincinnati Eye Institute and University of Cincinnati School of Medicine; Clinical Governance Board, Cincinnati Eye Institute and Allen C. Ho, M.D. FACS, Wills Eye Hospital Attending Surgeon and Director of Retina Research, Professor of Ophthalmology, Thomas Jefferson University. Both Drs. Riemann and Ho are renowned therapeutic area experts and have direct experience with the Orbit SDS as they helped to develop and refine the device.

"Having been part of the team that originally designed the subretinal delivery system, I am looking forward to delivering Lineage's RPE cells to the subretinal space of patients suffering from dry AMD," stated Christopher D. Riemann, M.D. "I believe that this combination will provide superior dose control and safety and may lead to superior outcomes when compared to alternate approaches. I am encouraged by the results observed in patients treated to date with OpRegen and I look forward to participating in this study."

"We continue to be excited about the data we are collecting in dry AMD, which includes safety and engraftment data persisting as long as three years from time of transplantation of our RPE cells. Additionally, we are honored by the interest our approach is garnering from retinal surgeons at leading ophthalmology research centers such as Cincinnati Eye and Wills Eye," stated Brian M. Culley, CEO of Lineage. "With the removal of the enrollment stagger and clearance to dose patients with vision as good as 20/65, we believe we can significantly accelerate our rate of enrollment on the OpRegen study. Our objective is to complete patient enrollment in the Orbit SDS portion of our clinical study as soon as this quarter. The sooner we treat the next four patients, the more mature and informative our clinical update will be at the Association for Research in Vision and Ophthalmology (ARVO) medical conference, which we plan to attend in Baltimore this May. We believe that by combining the best cell line, the best production process, and the best delivery system, we are positioning OpRegen as the front-runner in the race to address the unmet opportunity in the potential billion-dollar dry AMD market."

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 his or her 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 Dry AMD

Dry age-related macular degeneration (AMD) is a leading cause of adult blindness in the developed world. There are two forms of AMD: wet AMD and dry AMD. Dry AMD is the more common of the two types, accounting for approximately 85-90% of cases. Wet AMD is the less common of the two types, accounting for approximately 10-15% of cases. Global sales of the two leading wet AMD therapies were in excess of $10 billion in 2018. There currently are no FDA-approved treatment options for dry AMD. Nearly all cases of wet AMD begin as dry AMD. Dry AMD typically affects both eyes.

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. Until February 2020, staggered intervals within and between cohorts were 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 enrollment 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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