



Lineage Cell Therapeutics Completes Patient Enrollment in Phase 1/2a Clinical Study of OpRegen® Cell Therapy for the Treatment of Dry Age-related Macular Degeneration

November 11, 2020

- **OpRegen Data Update to be Featured in Presentation by Principal Investigator Christopher D. Riemann, M.D., at 2020 AAO Annual Meeting on November 15, 2020**
- **Therapeutic Expert Call to Discuss Results Scheduled for November 17, 2020**

CARLSBAD, Calif.--(BUSINESS WIRE)--Nov. 11, 2020-- [Lineage Cell Therapeutics, Inc.](#) (NYSE American and TASE: LCTX), a clinical-stage biotechnology company developing three novel cell therapies for serious conditions, today announced the successful completion of enrollment in its 24-patient Phase 1/2a study of its lead product candidate, [OpRegen®](#). OpRegen is an investigational cell therapy consisting of retinal pigment epithelium (RPE) cells administered to the subretinal space for the treatment of dry age-related macular degeneration (AMD) with geographic atrophy (GA). Updated interim results from the ongoing Phase 1/2a study will be presented at the [2020 American Academy of Ophthalmology Annual Meeting \(AAO 2020\)](#) during the OP02V Retina, Vitreous Original Papers Session on November 15, 2020 at 10:40am Eastern Time / 7:40 am Pacific Time by [Christopher D. Riemann](#), M.D., Vitreoretinal Surgeon and Fellowship Director, [Cincinnati Eye Institute](#) and University of Cincinnati School of Medicine. Dr. Riemann also will participate in a call to discuss the interim results on November 17, 2020 at 4:00 pm Eastern Time / 1:00 pm Pacific Time. Interested parties can access the event on the [Events and Presentations](#) section of Lineage's website.

"Completion of patient enrollment in our OpRegen study is a significant achievement for our team and reflects the focused commitment we have made to advancing our three cell therapy product candidates. We believe the potential for transplanted cells to safely and durably treat serious diseases and conditions, particularly where traditional molecular approaches have failed, will usher in a new treatment paradigm for modern medicine. Demonstrating this potential in clinical trials is a vital step in that process and we are thankful to have reached this important milestone," stated Brian Culley, CEO. "As a result of increased awareness of our study and promising data we reported recently, including the first known demonstration of retinal tissue restoration in a clinical setting, we were able to enroll and treat the final five patients in cohort 4 in just five weeks. We also surpassed our original goal by utilizing the Gyroscope Orbit SDS in 7 of the last 9 patients. We extend our gratitude to the participating patients, their families, and the study investigators and coordinators in both the US and Israel. With this milestone reached, our focus turns toward collecting safety and efficacy data on the most recently treated patients, evaluating options for later-stage clinical development, including with potential partners, and approaching the FDA to discuss our next steps. Our objective is to position the OpRegen program as a front-runner in the race to address an unmet need in what is widely expected to be a multi-billion-dollar dry AMD therapeutic market."

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 patients with vision as high as 20/64. 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. Additionally, for the patients in Cohort 4 that receive subretinal delivery of OpRegen utilizing the Gyroscope Orbit Subretinal Delivery System (Gyroscope Orbit SDS), objectives will include the evaluation of the safety of delivery of OpRegen using the Gyroscope Orbit SDS.

Recently, Lineage [reported](#) the first known finding of retinal tissue regeneration in a patient receiving OpRegen for the treatment of atrophic dry AMD. This unprecedented finding supports the view that dry AMD is not an irreversible, degenerative condition and that some portion of diseased retinal tissue may be recoverable in atrophic end-stage disease patients. These findings were initially observed by an independent external advisor using multiple imaging technologies and were subsequently confirmed by the reading center and additional experts in the field of retinal imaging. The Company also has [observed](#) evidence of benefit in other patients, including increases in Best Corrected Visual Acuity (BCVA), reduction in the growth of GA, and increases in reading speed.

OpRegen is a registered trademark of Cell Cure Neurosciences Ltd., a majority-owned subsidiary of Lineage Cell Therapeutics, Inc.

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 2019. Nearly all cases of wet AMD begin as dry AMD. Dry AMD typically affects both eyes. There are currently no U.S. Food and Drug Administration (FDA) or European Medicines Agency (EMA) approved treatment options available for patients with dry AMD.

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](https://twitter.com/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 for cell therapy generally and the expected addressable market for OpRegen. 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 12, 2020 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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