Lineage Cell Therapeutics Provides Update on OPC1 Cell Therapy Program for Spinal Cord Injury

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- **Improvements Implemented in OPC1 Production Process**
- **Patent Position Strengthened to Protect the Processes, Product, and Methods of Use**
- **Company is Evaluating Novel Delivery Devices to Enhance and Ease Surgical Procedure**
- **FDA RMAT Meeting Planned by Year End to Discuss Manufacturing and Clinical Development**

CARLSBAD, Calif.--(BUSINESS WIRE)--Jun. 25, 2020-- Lineage Cell Therapeutics, Inc. (NYSE American and TASE: LCTX), a clinical-stage biotechnology company developing novel cell therapies for unmet medical needs, today provided an update on OPC1, the Company's oligodendrocyte progenitor cell (OPC) therapy for the treatment of acute spinal cord injury (SCI). The OPC1 program was obtained through Lineage's acquisition of Asterias Biopharmaceuticals in March 2019, and manufacturing has been completely transferred to the Company's current Good Manufacturing Practice (cGMP) facility in Israel, where key process improvements have been developed and implemented. Lineage also has strengthened its patent position in order to protect the OPC1 processes, product and composition, and methods of use. Lineage intends to meet with the U.S. Food and Drug Administration (FDA) to discuss further development of the OPC1 program by the end of 2020.

"We have worked diligently over the past year to transition all manufacturing activities for the OPC1 program to our in-house cGMP facility, where our experienced cell therapy production team could develop and deploy much-needed improvements and modernization to the production and analytical processes," stated Brian M. Culley, Lineage CEO. "This work has achieved significantly better efficiency and improved quality control, which we expect will enable a consistent supply of material to support a late-stage clinical trial of OPC1. With these necessary steps now completed, our focus turns to developing a "thaw-and-inject" formulation and superior delivery tools, to enable an easier surgical procedure and facilitate faster enrollment in the next clinical trial. We also are evaluating ways to return OPC1 to the clinic sooner than originally planned, reflecting our view of compelling clinical data which continues to read out from the 25-patient phase 1/2a SCiStar study. Our approach with OPC1 replicates our development strategy for OpRegen, our cell therapy program for dry AMD, in which we seek not only to provide cell-based regenerative benefits, but also commercially relevant solutions with competitive advantages in areas of scale-up, production costs, and delivery techniques. By analyzing every piece of the overall therapeutic landscape, we seek to position ourselves as the clear leader in the emerging field of cell therapy transplant medicine."

Key OPC1 Program Milestones Achieved Since the Asterias Acquisition Include:

- Technology transfer has been completed and cell production processes have been established at Lineage's cGMP cell therapy facility;
- The production process was improved to achieve greater efficiency and higher quality control, becoming compatible with larger-scale manufacturing, and Biologics Review Application (BLA) readiness;
- Multiple OPC1 batches have been successfully manufactured; GMP production is planned to begin in early 2021;
- New master and working pluripotent cell banks have been produced to supply consistent clinical and commercial batches of OPC1;
- New in-process controls and release testing have been introduced to the production and release process and have been updated to be more compatible with current and expected future FDA BLA review processes;
- Issuance of two new patents in 2019 related to methods for utilizing pluripotent stem cell-derived OPCs for the treatment of SCI and reducing cavitation in patients with acute SCI. Additional patent applications are pending which may further protect the processes, product and composition, and methods of use of OPC1.

Key 2020 Milestone and Development Plans for the OPC1 Program Include:

- Meeting with the FDA to discuss manufacturing improvements and the design of a late-stage comparative clinical trial;
- Introducing a new "thaw-and-inject" formulation to enable easier preparation and administration of cells to the spinal cord, avoiding the need for washing and other dose preparation steps one day prior to treatment and improving commercial positioning;
- Identifying a novel delivery system, to make the surgical procedure easier, faster, and more compatible with a thaw and inject formulation of OPC1, improving overall end-user experience for the surgeon.

About OPC1

OPC1 is an oligodendrocyte progenitor cell (OPC) transplant therapy designed to provide clinically meaningful improvements to motor recovery in individuals with acute spinal cord injuries (SCI). OPCs are naturally occurring precursors to the cells which provide electrical insulation for nerve axons in the form of a myelin sheath. SCI occurs when the spinal cord is subjected to a severe crush or contusion injury and typically results in severe functional impairment, including limb paralysis, aberrant pain signaling, and loss of bladder control and other body functions. There are approximately 18,000 new spinal cord injuries annually in the U.S. and there currently are no FDA-approved drugs specifically for the treatment of SCI. The OPC1 program has been partially funded by a $14.3 million grant from the California Institute for Regenerative Medicine. OPC1 has received Regenerative Medicine Advanced Therapy (RMAT) designation and Orphan Drug designation from the U.S. Food and Drug Administration (FDA).

About the OPC1 Clinical Study

The SCiStar Study of OPC1 is an open-label, 25-patient, single-arm trial testing three sequential escalating doses of OPC1 which was administered 21 to 42 days post-injury, at up to 20 million OPC1 cells in patients with subacute motor complete (AIS-A or AIS-B) cervical (C-4 to C-7) acute spinal cord injuries (SCI). These individuals had experienced severe paralysis of the upper and lower limbs. The primary endpoint in the SCiStar study was safety as assessed by the frequency and severity of adverse events related to OPC1, the injection procedure, and immunosuppression with short-term, low-dose tacrolimus. Secondary outcome measures included neurological functions measured by upper extremity motor scores (UEMS) and motor level on International Standards for Neurological Classification of Spinal Cord Injury (ISNCSCI) examinations through 365 days post-treatment. Enrollment is complete in this study; patients will continue to be evaluated on a long-term basis.

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 and in preclinical development for additional cancers and as a vaccine against infectious diseases, including SARS-CoV-2, the virus which causes COVID-19. 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 Lineage's plans for OPC1 development and clinical studies. 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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