# Lineage Cell Therapeutics Provides Update on OPC1 Cell Therapy Program for the Treatment of Spinal Cord Injury

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**Major Improvements Made to OPC1 Production, Including to Process, Purity, Potency, and Scale**

CARLSBAD, Calif.--(BUSINESS WIRE)--Dec. 8, 2020-- Lineage Cell Therapeutics, Inc. (NYSE American and TASE: LCTX), a clinical-stage biotechnology company developing allogeneic cell therapies for unmet medical needs, today provided an update on OPC1, its oligodendrocyte progenitor cell (OPC) allogeneic transplant for the treatment of acute spinal cord injuries (SCI). The Company reported it has developed an enhanced differentiation process, leading to major improvements in production and quality of its OPC1 cell therapy drug product, including:

- A new ready-to-inject formulation of OPC1, which enables clinical use at a much larger number of spinal cord treatment centers, accelerating enrollment for a larger and potentially registrational clinical trial.
- Elimination of dose preparation, reducing overall preparation time from 24 hours to 30 minutes and cutting logistics costs by approximately 90%.
- A 10 to 20-fold increase in OPC1 production scale, sufficient to support late-stage clinical development and which can be further scaled to meet initial commercial use.
- A 50-75% reduction in product impurities.
- Improvements in OPC1 functional activity, as assessed by cellular migration and secretion of key growth factors.
- Development of 12 new analytical and functional methods for in-process quality control and release of improved product.
- Elimination of all animal-based production reagents resulting in a xeno-free final product formulation, consistent with guidelines preferred by U.S. and European regulatory agencies.
- Filing of patent applications on the process and product which, if allowed, are anticipated to have expiration dates in 2039 and 2040.

“Manufacturing is the foundation of cell therapy and the significant enhancements we have achieved with OPC1 marks the second time we have successfully transformed a research-grade production process into one capable of supporting a successful commercial product. The first instance was with OpRegen, our dry AMD program, from which we now can generate 5 billion 99% pure RPE cells in a single 3-liter bioreactor. We’ve achieved similar value-creating improvements with the production of oligodendrocytes for our spinal cord program and I expect we also will be successful with our next endeavor, modernizing the production of allogeneic dendritic cells to support our immuno-oncology platform,” stated Brian M. Culley, Lineage CEO. “Our objective is to be the premier allogeneic cell therapy company and our dedication to manufacturing excellence allows us not only to reduce or eliminate certain regulatory and commercial hurdles, but also establish strong competitive barriers in our field. Looking ahead, we are reviewing our options to return OPC1 to clinical testing in a late-stage, comparative clinical trial and evaluating bespoke delivery solutions for our OPC1 cells.”

A further discussion of the manufacturing improvements made to the OPC1 program will be available today at 12:00 pm Eastern Time / 9:00 am Pacific Time on an analyst-led webinar hosted by FORCE Wealth. Mr. Culley and Brandi Roberts, Lineage’s CFO, will participate in a fireside chat hosted by Joseph Pantginis, Ph.D., Managing Director, Equity Research at H.C. Wainwright & Co., LLC. Interested investors can access the live presentation on the Events and Presentations section of Lineage’s website and an archived presentation will be available for 30 days. Additional videos are available on the Media page of the Lineage website, located at www.lineagecell.com/media/.

**About Spinal Cord Injuries**

A spinal cord injury (SCI) occurs when the spinal cord is subjected to a severe crush or contusion and frequently 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. There are no FDA-approved drugs specifically for the treatment of SCI. The cost of a lifetime of care for a severe spinal cord injury can be as high as $5 million.

**About OPC1**

OPC1 is an oligodendrocyte progenitor cell (OPC) transplant therapy designed to provide clinically meaningful improvements in 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. 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 administered 21 to 42 days post-injury in patients with subacute motor complete (AIS-A or AIS-B) cervical (C-4 to C-7) acute spinal cord injuries (SCI). Patient enrollment in this study is complete; 96% of patients reported one level of improved motor function and 33% of patients reported two levels of improved motor function. Patients continue to be evaluated on a long-term basis. Patients enrolled in the SCiStar study 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.

**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 Lineage's manufacturing improvements and anticipated patent expiration dates. 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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