Lineage Announces FDA Clearance of IND Amendment for OPC1 Cell Transplant for the Treatment of Spinal Cord Injury

February 13, 2024

The DOSED Clinical Study Will Evaluate a Novel Delivery Device in Subacute and Chronic Spinal Cord Injury Patients

CARLSBAD, Calif.--(BUSINESS WIRE)--Feb. 13, 2024-- Lineage Cell Therapeutics, Inc. (NYSE American and TASE: LCTX), a clinical-stage biotechnology company developing allogeneic cell therapies for unmet medical needs, announced today the clearance, by the U.S. Food and Drug Administration (FDA), of its Investigational New Drug amendment (INDa) for OPC1, an investigational allogeneic oligodendrocyte progenitor cell transplant for the treatment of spinal cord injury (SCI). Pursuant to the INDa, Lineage has initiated activities to open its first clinical site in the DOSED (Delivery of Oligodendrocyte Progenitor Cells for Spinal Cord Injury: Evaluation of a Novel Device) study to evaluate the safety and utility of a novel spinal cord delivery device in subacute and chronic SCI patients. Initial site opening is expected to occur in the second quarter of 2024, following customary trial preparations and submission in the first quarter of a grant application to the California Institute for Regenerative Medicine (CIRM) for potential partial financial support of the DOSED clinical study.

“Lineage’s oligodendrocyte cell transplants are designed to replace or support cells that are absent or dysfunctional due to traumatic injury, with the goal of helping to improve the quality of life and restore or augment functional activity to persons suffering from traumatic cervical or thoracic spinal cord injuries. The clearance of our INDa and the initiation of OPC1 clinical testing under our sponsorship represents a significant milestone for this program, and reflects our commitment to developing modern cell therapy product candidates,” stated Brian M. Culley, Lineage CEO. “We are excited by the opportunity to build upon the promising results achieved with OPC1 in previous trials, and to continue to seek improvements in how our therapy is prepared and administered. The DOSED clinical study in particular is intended to evaluate the safety and performance of a new delivery device, which is compatible with our forthcoming immediate-use formulation, and which does not require cessation of patient ventilation during administration. We believe these improvements can lead to a safer surgical procedure for surgeons and patients. This study also will mark the first time that OPC1 is administered to chronic SCI patients, and we will be collecting efficacy assessments in addition to the primary outcome measures of safety. Completing this regulatory step also enables us to proceed with our planned CLIN-2 grant application to CIRM to request external financial support for the OPC1 program.”

OPC1 has been tested in two clinical trials to date: a five patient Phase 1 clinical safety trial in acute thoracic SCI, where all subjects have been followed for at least 10 years; as well as a 25 patient Phase 1/2a multicenter dose-escalation clinical trial in subacute cervical SCI, where all subjects were evaluated for at least two years, one of the first cell therapy clinical trials supported by the California Institute for Regenerative Medicine under Proposition 71. Results from both studies have been published in the *Journal of Neurosurgery: Spine.* The data from the Phase 1/2a clinical study of OPC1 in subacute cervical SCI is available here and the data from the Phase 1 clinical study of OPC1 in acute thoracic SCI is available here.

About the DOSED Clinical Study

The Delivery of Oligodendrocyte Progenitor Cells for Spinal Cord Injury: Evaluation of a Novel Device (DOSED) clinical study is an open label, multi-center, device safety study, in approximately 3-5 subacute and 3-5 stable chronic subjects with complete (ASIA Impairment Scale A) or incomplete (ASIA Impairment Scale B), traumatic, focal SCI affecting either cervical (C4-C7) or thoracic (T1-T10) vertebrae. The primary objective of this study is to evaluate the safety of a novel spinal cord delivery device to administer OPC1 to the spinal parenchyma. The primary endpoint is safety, as measured by the frequency and severity of adverse events (AEs) through 30 days following OPC1 injection that are related to the injection procedure. Secondary endpoints are safety and tolerability, as measured by the frequency and severity of AEs, including AEs of special interest, through 90 days following OPC1 injection, that are related to OPC1 and/or the concomitant short-term immunosuppression. Safety parameters will be evaluated by magnetic resonance imaging (MRI) data evaluating evidence of deterioration or changes in the following: intramedullary hemorrhage, cerebral spinal fluid (CSF) leak, epidural abscess, infection; evidence of an expanding cyst or mass at the injection site or elsewhere in the central nervous system (CNS); evidence of inflammatory lesion(s) at injection site or elsewhere in CNS and evidence of CSF flow obstruction. Exploratory endpoints include: (1) potential improvements in neurological impairment, function, and pain, evaluated by changes from baseline on the following endpoints: changes in neurological function as measured by sensory and motor scores and motor level on International Standards for Neurological Classification of Spinal Cord Injury (ISNCSCI) examinations; changes in post-injection pain, defined as a worsening of pain or neuropathic pain of greater than 7 days duration from baseline levels, as assessed by the International Spinal Cord Injury Pain Basic Data Set or occurrence of allodynia; (2) changes from baseline at 30, 90 and 365 days post-OPC1 injection in: ISNCSCI, SCIM, International Spinal Cord Injury Pain Questionnaire; and (3) patient and clinician impressions of changes in quality of life as reported by changes from baseline at 30, 90, and 365 days post-OPC1 injection.

About OPC1

OPC1 is an oligodendrocyte progenitor cell (OPC) transplant therapy designed to provide clinically meaningful improvements to motor recovery in individuals with 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 most often 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 or interventions 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 California Institute for Regenerative Medicine (CIRM)

CIRM, California's Stem Cell Agency, was created by the voters of California in 2004 with the passing of Proposition 71, which authorized $3 billion in funding for stem cell research in California. The agency funds stem cell research at institutions and companies throughout California (as well as

institutions and companies outside of the state that conduct a portion of their research in California) with the goal of accelerating treatments to patients with unmet medical needs. In 2020, California voters approved to continue funding California's Stem Cell Agency through the passage of Proposition 14. CIRM's mission is to accelerate world class science to deliver transformative regenerative medicine treatments in an equitable manner to a diverse California and world as well as to accelerate stem cell treatments to patients with unmet medical needs. By promoting and encouraging the growth of the stem cell biotechnology sector, the agency is also helping attract the best scientists to the state and establishing California as a global leader in stem cell research. For more information, please visit https://www.cirm.ca.gov/ and follow the agency on Twitter: @CIRMnews.

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 and preclinical programs are in markets with billion dollar opportunities and include five allogeneic ("off-the-shelf") product candidates: (i) OpRegen ®, a retinal pigment epithelial cell therapy in Phase 2a development for the treatment of geographic atrophy secondary to age-related macular degeneration, is being developed under a worldwide collaboration with Roche and Genentech, a member of the Roche Group; (ii) OPC1, an oligodendrocyte progenitor cell therapy in Phase 1/2a development for the treatment of acute spinal cord injuries; (iii) VAC2, a dendritic cell therapy produced from Lineage's VAC technology platform for immuno-oncology and infectious disease, currently in Phase 1 clinical development for the treatment of non-small cell lung cancer; (iv) ANP1, an auditory neuronal progenitor cell therapy for the potential treatment of auditory neuropathy; and (v) PNC1, a photoreceptor neural cell therapy for the potential treatment of vision loss due to photoreceptor dysfunction or damage. 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," "aim," "may," "will," "estimate," "continue," "anticipate," "design," "intend," "expect," "could," "can," "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 ability of cell transplant therapies, including OPC1, to improve recovery, allow a patient to regain more function than what could otherwise be expected, or improve a patient's quality of life; the potential future achievements of our clinical, preclinical and development programs, the planned initiation of clinical trials, including the expected initial site opening for the DOSED clinical study in the second quarter of 2024; that the improvements in the delivery device for OPC1 can lead to a safer surgical procedure; and the timing and availability of clinical data updates related to our programs. 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, but not limited to, the following risks: that positive findings in early clinical and/or nonclinical studies of a product candidate may not be predictive of success in subsequent clinical and/or nonclinical studies of that candidate; and those risks and uncertainties inherent in Lineage's business and other risks discussed in Lineage's filings with the Securities and Exchange Commission (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 most recent Annual Report on Form 10-K filed with the SEC 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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Source: Lineage Cell Therapeutics, Inc.