# BioTime to Present SCiStar Clinical Study Top-Line Data at the 26th Annual American Society for Neural Therapy and Repair Conference

April 26, 2019

ALAMEDA, Calif.--(BUSINESS WIRE)--Apr. 26, 2019-- BioTime, Inc. (NYSE American and TASE: BTX), a clinical-stage biotechnology company developing new cellular therapies, announced today that Edward D. Wirth, III, M.D., Ph.D., Chief Medical Officer of BioTime, will present at the 26th Annual American Society for Neural Therapy and Repair (ASNTR) Annual Conference on April 26th, 2019 at 10:30am EDT as part of Session 6: "*Spinal Cord Injury*". Dr. Wirth's presentation is entitled " *Top-line 12-month Results from the SCiStar Study - A Phase 1/2a Trial of Human Embryonic Stem Cell-Derived Oligodendrocyte Progenitor Cells (OPC1) in Patients with Subacute Cervical Spinal Cord Injury*". ASNTR will be held April 25 – 27, 2019 at the Sheraton Sand Key Resort in Clearwater Beach, Florida.

"We believe the primary goals of the SCiStar Study, which were to observe the safety of OPC1 in cervical spinal cord injury patients as well as other important metrics including related to the optimal timing of OPC1 injection, tolerability of the immunosuppression regimen, engraftment of OPC1 cells, and rates of motor recovery observed among different study subpopulations, have all been successfully achieved," stated Dr. Wirth. "We now are in the process of analyzing the full data set from the SCiStar Study to inform how best to proceed with this promising program. We expect to propose a clinical plan to the U.S. Food and Drug Administration later this year and expect to share the outcome of those discussions when they are available."

"We appreciate the support of the California Institute for Regenerative Medicine, the world's largest institution dedicated to bringing the future of cellular medicine closer to reality, whose generous grant funding to date of $14.3 million has helped advance the clinical development of our OPC1 program and generate these encouraging clinical results in patients with traumatic spinal cord injuries," stated Brian M. Culley, Chief Executive Officer of BioTime. "We look forward to continuing our partnership with CIRM and will support their mission to accelerate stem cell treatments to patients with unmet medical needs and fast-track the development of the most promising stem cell technologies."

The SCiStar Study is an open-label, single-arm trial testing three sequential escalating doses of OPC1 administered 21 to 42 days post-injury, at up to 20 million OPC1 cells in 25 subjects with subacute motor complete (AIS-A or AIS-B) cervical (C-4 to C-7) acute spinal cord injuries (SCI). These individuals have essentially lost all movement below their injury site and experience severe paralysis of the upper and lower limbs. AIS-A subjects have lost all motor and sensory function below their injury site, while AIS-B subjects have lost all motor function but may have retained some minimal sensory function below their injury site. 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 as measured by upper extremity motor scores and motor level on International Standards for Neurological Classification of Spinal Cord Injury (ISNCSCI) examinations at 30, 60, 90, 180, 270, and 365 days after injection of OPC1.

Below are a summary of key findings from the SCiStar Study. A copy of Dr. Wirth's presentation will be available on the Events section of BioTime's website concurrent with his presentation at ASNTR.

- **Overall safety profile of OPC1 to date is excellent**
  - Magnetic resonance imaging (MRI) scans at 12 months post-injection of OPC1 has shown no evidence of adverse changes in any of the 25 SCiStar study subjects treated with OPC1.
  - To date, there have been no unexpected serious adverse events (SAEs) related to the OPC1 cells.
  - No concerning safety issues and no intraoperative complications have been noted.
  - No SCiStar study subjects had worsening of neurological function post-injection.
  - No adverse findings observed on follow-up MRI scans.
  - Immunosuppression with tacrolimus (an immunosuppressive drug utilized mainly after allogeneic organ transplant to lower the risk of organ rejection) was well-tolerated.
- **Majority of SCiStar subjects who received 10M or 20M OPC1 cells exhibited robust motor recovery in upper extremities**
  - Three subjects (Cohort 1) received a sub-therapeutic dose of 2M cells to evaluate the initial safety of injecting OPC1 into lesions in the cervical spinal cord. All other subjects (Cohorts 2-5) received 10M or 20M cells.
  - At 12 months, 95% (21/22) of SCiStar study subjects in Cohorts 2-5 recovered at least one motor level on at least one side and 32% (7/22) of these subjects recovered two or more motor levels on at least one side. The average improvement in upper extremity motor score as measured by the ISNCSCI scale for these subjects was 8.9 points.
  - Notably, no SCiStar study subjects saw decreased motor function following administration of OPC1 and subjects either retained for 12 months the motor function recovery seen through 6 months or experienced further motor function recovery from 6 to 12 months.
- **MRI scans consistent with durable engraftment through 1 year post-injection**
  - All three SCiStar study subjects in Cohort 1 and 95% (21/22) of SCiStar study subjects in Cohorts 2 to5 have MRI scans at 12 months consistent with the formation of a tissue matrix at the injury site, which is encouraging evidence that OPC1 cells have engrafted at the injury site and helped to prevent cavitation, a destructive process that occurs within the spinal cord following spinal cord injuries, and typically results in permanent loss of motor and sensory

function.

**About OPC1**

OPC1 is an oligodendrocyte progenitor cell (OPC) therapy currently being tested in a Phase I/IIa clinical trial known as SCiStar for the treatment of acute spinal cord injuries. 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. The clinical development of 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 for the treatment of acute SCI and has been granted Orphan Drug designation from the U.S. Food and Drug Administration (FDA).

**About BioTime, Inc.**

BioTime is a clinical-stage biotechnology company developing new cellular therapies for degenerative retinal diseases, neurological conditions associated with demyelination, and aiding the body in detecting and combating cancer. BioTime's programs are based on its proprietary cell-based therapy platform and associated development and manufacturing capabilities. With this platform BioTime 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. BioTime'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, the 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. For more information, please visit www.biotimeinc.com.

**Forward-Looking Statements**

BioTime 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 timing of when we propose a clinical plan to the U.S. Food and Drug Administration and the sharing of the outcome of those discussions when they are available, and that MRI results are supportive evidence showing that OPC1 cells have durably engrafted to help prevent cavitation at the injury site. Forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause BioTime'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, without limitation, risk and uncertainties related to: BioTime's ability to raise additional capital when and as needed, to advance its product candidates; BioTime's ability to develop and commercialize product candidates; the failure or delay in starting, conducting and completing clinical trials or obtaining FDA or foreign regulatory approval for BioTime's product candidates in a timely manner; the therapeutic potential of BioTime's product candidates, and the disease indications for which BioTime intends to develop its product candidates; BioTime's ability to conduct and design successful clinical trials, to enroll a sufficient number of patients, to meet established clinical endpoints, to avoid undesirable side effects and other safety concerns, and to demonstrate sufficient efficacy of its product candidates; developments by BioTime competitors that make BioTime's product candidates less competitive or obsolete; BioTime's ability to manufacture its product candidates for clinical development and, if approved, for commercialization, and the timing and costs of such manufacture; the performance of third parties in connection with the development and manufacture of BioTime's product candidates, including third parties conducting clinical trials as well as third-party suppliers and manufacturers; the potential of BioTime's cell therapy platform, and BioTime's plans to apply its platform to research, develop and commercialize our product candidates; BioTime's ability, and the ability of its licensors, to obtain, maintain, defend and enforce intellectual property rights protecting BioTime's product candidates, and BioTime's ability to develop and commercialize its product candidates without infringing the proprietary rights of third parties; BioTime's ability to recruit and retain key personnel; and BioTime's ability to successfully integrate the operations of Asterias into BioTime. BioTime'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. For a detailed description of BioTime's risks and uncertainties, you are encouraged to review its documents filed with the SEC including its recent filings on Form 8-K, Form 10-K and Form 10-Q. You are cautioned not to place undue reliance on forward-looking statements, which speak only as of the date on which they were made. BioTime 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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