BrainStorm Expands Clinical Pipeline to Evaluate NurOwn® for the Treatment of Alzheimer's Disease

Protocol for the proposed multi-national trial has been submitted for EU regulatory approval

Company will host a Key Opinion Leader call and webcast to discuss the newly announced Alzheimer's Disease program on July 8, 2020 at 8:15 AM ET

NEW YORK, June 24, 2020 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of adult stem cell therapies for neurodegenerative diseases, today announced a new clinical program focused on the development of NurOwn® as a treatment for Alzheimer's disease. As part of the newly announced program, the Company is planning a multi-national Phase 2 clinical trial to evaluate the safety and efficacy of NurOwn treatment in patients with prodromal to mild Alzheimer's disease.

"Our newly announced Alzheimer's program is an important strategic expansion of BrainStorm's clinical pipeline," said Chaim Lebovits, Chief Executive Officer of BrainStorm. "We see this as an ideal complement to our lead amyotrophic lateral sclerosis (ALS) program, as there are compelling data suggesting that the benefits observed with NurOwn in ALS patients may extend to other neurological conditions, including Alzheimer's disease. As we move towards completion of our pivotal Phase 3 ALS study, the newly announced Alzheimer's program, combined with our ongoing progressive multiple sclerosis (MS) program, will continue to drive the advancement of the NurOwn platform. Through the completion of our clinical trial programs, BrainStorm hopes to advance NurOwn to address unmet needs across a broad population of patients with neurodegenerative disease."

The lead investigators of the trial are two world renowned clinical experts in Alzheimer's disease, Philip Scheltens, M.D., Ph.D., and Bruno Dubois, M.D., Ph.D. Prof. Scheltens, the principal investigator (PI) of the study, is Professor of Cognitive Neurology and Director of the Alzheimer Centre at Amsterdam University Medical Centers. He has extensive experience as PI of many international clinical trials in this field. Prof. Dubois, the French national coordinator of the study, is Professor of Neurology at the Neurological Institute of the Salpétrière University Hospital. He is President of the Scientific Committee of France-Alzheimer and of IFRAD (International Fund Raising for Alzheimer's Disease) as well as a member of the European Alzheimer Disease Consortium (EADC). Profs. Scheltens and Dubois will discuss the proposed clinical trial during an upcoming Key Opinion Leader (KOL) webinar and call hosted by BrainStorm on July 8, 2020 at 8:15 AM ET.

Speaking on the trial Prof. Scheltens stated, "We are very eager to evaluate the efficacy of NurOwn in the treatment of Alzheimer's disease, because of its unique mechanism of action. While many previous Alzheimer's therapies have focused on a single target such as tau or beta-amyloid, NurOwn has the capability to simultaneously target multiple relevant biological pathways and bring a comprehensive approach to this multifactorial disease. Importantly, NurOwn's mechanism of action may also allow the therapy to enable synergistic combinations with anti-tau or anti-beta-amyloid treatments, further underscoring its potential to address critical unmet needs in Alzheimer's disease."

Prof. Dubois added, "In such a complex disease, addressing inflammation and neuro-protection is an innovative and very interesting approach. It is a first in the world for this technology and Brainstorm is leveraging encouraging results from the Phase 2 study in ALS."

Ralph Kern, M.D., MHSc., President and Chief Medical Officer of BrainStorm, noted, "In the proposed Phase 2 clinical trial, our goal is to confirm that the potential shown by NurOwn in our lead ALS program may be extended to patients with Alzheimer's disease. We now have a compelling set of biomarker data supporting NurOwn's mechanism of action, including important pharmacodynamic changes in cerebrospinal fluid (CSF) inflammatory mediators. Additionally, our innovative biomarker-driven precision medicine approach, which will focus on patients with early-stage disease and pre-defined levels of CSF tau and beta-amyloid proteins, may greatly facilitate Phase 2 clinical trial success."

Stacy Lindborg, Ph.D., Executive Vice President and Head of Global Clinical Research at BrainStorm, said, "We are excited to introduce a novel approach to Alzheimer's disease that targets both inflammation as well as neurotrophic factors. Given the acute need for vastly different treatment methodologies, we believe our broad targeting approach, combined with robust effects on CSF measures and a favorable safety profile, allows us to go forward with confidence. Additionally, compiling NurOwn safety data across diseases will allow us to enhance patient safety for each trial."

Phase 2 Trial to be Conducted at Leading Alzheimer's disease Centers in Europe

BrainStorm plans to conduct a 52-week, Phase 2 open-label, proof-of-concept clinical trial to evaluate NurOwn in 40 participants with prodromal to mild Alzheimer's disease. Study participants will receive three intrathecal NurOwn doses 8 weeks apart. In addition to meeting well-defined clinical criteria for prodromal to mild Alzheimer's disease, participants must also meet biomarker defined criteria for Alzheimer's disease. The clinical trial will evaluate safety and pharmacodynamics of NurOwn, including effects on inflammatory, Alzheimer's-specific, neurodegenerative, and synaptic biomarkers, as well as a range of key clinical measures of cognition and function. The clinical trial will be conducted at the Brain research Center affiliated with the Alzheimer Center Amsterdam, Pitié-Salpêtrière Hospital (Paris), and several other clinical trial sites in the Netherlands and France.

NurOwn is protected by patents granted in the United States, Europe, Israel, and elsewhere covering treatments of neurodegenerative diseases, including Alzheimer's disease. These include European patent no. EP2620493B1 (Mesenchymal stem cells for the treatment of CNS diseases).

Key Opinion Leader Call and Webcast

BrainStorm will host a call and webinar featuring Philip Scheltens, M.D., Ph.D., and Bruno Dubois, M.D., Ph.D., on July 8, 2020 at 8:15 AM ET. The webinar will be followed by a question-and-answer session with Professors Scheltens and Dubois.

Dial-in and Webcast Information

Register for Call/Webinar Here

About NurOwn®

NurOwn (autologous MSC-NTF cells) represent a promising investigational therapeutic approach to targeting disease pathways important in neurodegenerative disorders. MSC-NTF cells are produced from autologous, bone marrow-derived mesenchymal stem cells (MSCs) that have been expanded and differentiated ex vivo. MSCs are converted into MSC-NTF cells by growing them under patented conditions that induce the cells to secrete high levels of neurotrophic factors (NTFs). Autologous MSC-NTF cells can effectively deliver multiple NTFs and immunomodulatory cytokines directly to the site of damage to elicit a desired biological effect and ultimately slow or stabilize disease progression. BrainStorm has fully enrolled a Phase 3 pivotal trial of autologous MSC-NTF cells for the treatment of amyotrophic lateral sclerosis (ALS). BrainStorm also recently received acceptance from the U.S. Food and Drug Administration (FDA) to initiate a Phase 2 open-label multicenter trial in progressive multiple sclerosis (MS) and initiated enrollment in March 2019.

About BrainStorm Cell Therapeutics Inc.

BrainStorm Cell Therapeutics Inc. is a leading developer of innovative autologous adult stem cell therapeutics for debilitating neurodegenerative diseases. The Company holds the rights to clinical development and commercialization of the NurOwn technology platform used to produce autologous MSC-NTF cells through an exclusive, worldwide licensing agreement. Autologous MSC-NTF cells have received Orphan Drug status designation from the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for the treatment of amyotrophic lateral sclerosis (ALS). BrainStorm has fully enrolled a Phase 3 pivotal trial in ALS (NCT03280056), investigating repeat-administration of autologous MSC-NTF cells at six U.S. sites supported by a grant from the California Institute for Regenerative Medicine (CIRM CLIN2-0989). The pivotal study is intended to support a filing for U.S. FDA approval of autologous MSC-NTF cells in ALS. BrainStorm also recently received U.S. FDA clearance to initiate a Phase 2 open-label multicenter trial in progressive multiple sclerosis (MS). The Phase 2 study of autologous MSC-NTF cells in patients with progressive MS (NCT03799718) started enrollment in March 2019. For more information, visit the company's website at www.brainstorm-cell.com

Safe-Harbor Statement

Statements in this announcement other than historical data and information, including statements regarding future clinical trial enrollment and data, constitute "forward-looking statements" and involve risks and uncertainties that could cause BrainStorm Cell Therapeutics Inc.'s actual results to differ materially from those stated or implied by such forward-looking statements. Terms and phrases such as "may", "should", "would", "could", "will", "expect", "likely", "believe", "plan", "estimate", "predict", "potential", and similar terms and phrases are intended to identify these forward-looking statements. The potential risks and uncertainties include, without limitation, BrainStorm's need to raise additional capital, BrainStorm's ability to continue as a going concern, regulatory approval of BrainStorm's NurOwn treatment candidate, the success of BrainStorm's product development programs and research, regulatory and personnel issues, development of a global market for our services, the ability to secure and maintain research institutions to conduct our clinical trials, the ability to generate significant revenue, the ability of BrainStorm's NurOwn treatment candidate to achieve broad acceptance as a treatment option for ALS or other neurodegenerative diseases, BrainStorm's ability to manufacture and commercialize the NurOwn treatment candidate, obtaining patents that provide meaningful

protection, competition and market developments, BrainStorm's ability to protect our intellectual property from infringement by third parties, heath reform legislation, demand for our services, currency exchange rates and product liability claims and litigation,; and other factors detailed in BrainStorm's annual report on Form 10-K and quarterly reports on Form 10-Q available at http://www.sec.gov. These factors should be considered carefully, and readers should not place undue reliance on BrainStorm's forward-looking statements. The forward-looking statements contained in this press release are based on the beliefs, expectations and opinions of management as of the date of this press release. We do not assume any obligation to update forward-looking statements to reflect actual results or assumptions if circumstances or management's beliefs, expectations or opinions should change, unless otherwise required by law. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance or achievements.

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CONTACTS

Investor Relations:
Preetam Shah, MBA, PhD
Chief Financial Officer
BrainStorm Cell Therapeutics Inc.
Phone: + 1.862.397.1860
pshah@brainstorm-cell.com

Media:
Paul Tyahla
SmithSolve
Phone: + 1.973.713.3768
Paul.tyahla@smithsolve.com
