BrainStorm Cell Therapeutics and FDA Agree to Potential NurOwn® Regulatory Pathway for Approval in ALS

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NEW YORK, Feb. 11, 2020 (GLOBE NEWSWIRE) -- BrainStorm Cell Therapeutics, Inc., (NASDAQ:BCLI), a leading developer of adult stem cell therapies for neurodegenerative diseases, today announced that the Company recently held a high level meeting with the U.S. Food and Drug Administration (FDA) to discuss potential NurOwn® regulatory pathways for approval in ALS. Repeated intrathecal administration of NurOwn (autologous MSC-NTF cells) is currently being evaluated in a fully enrolled Phase 3 pivotal trial in ALS (NCT03280056).

In the planned meeting with senior Center for Biologics Evaluation and Research (CBER) leadership and several leading U.S. ALS experts, the FDA confirmed that the fully enrolled Phase 3 ALS trial is collecting relevant data critical to the assessment of NurOwn efficacy. The FDA indicated that they will look at the "totality of the evidence" in the expected Phase 3 clinical trial data. Furthermore, based on their detailed data assessment, they are committed to work collaboratively with BrainStorm to identify a regulatory pathway forward, including opportunities to expedite statistical review of data from the Phase 3 trial.

Both the FDA and BrainStorm acknowledged the urgent unmet need and the shared goal of moving much needed therapies for ALS forward as quickly as possible.

“This is a key turning point in our work toward providing ALS patients with a potential new therapy,” said Chaim Lebovits, President and CEO of BrainStorm. “We commend the FDA for its commitment to the ALS community and to facilitating the development, and we ultimately hope, the approval of NurOwn. The entire BrainStorm team is grateful for the ongoing and conscientious collaboration in the quest to beat ALS.”

Ralph Kern, MD, MHSc, Chief Operating Officer and Chief Medical Officer, stated, “The entire team at BrainStorm has collectively worked to ensure that we conduct the finest, science-based clinical trials. We had the opportunity to communicate with Senior Leadership at the FDA and discuss how we can work together to navigate the approval process forward along a novel pathway. We appreciate their willingness and receptiveness to consider innovative approaches as we all seek to better serve the urgent unmet medical needs of the ALS community.”

Brian Wallach, Co-Founder of I AM ALS stated: “There is nothing more important to those living with ALS than having access to therapies that effectively combat this fatal disease. We have been working with BrainStorm for months now because we believe that NurOwn is a potentially transformative therapy in this fight. We were privileged to represent the patient voice at this meeting and are truly grateful to the company and the FDA for this critical agreement. This is a truly important moment of hope and we look forward to seeing both the Phase III data and the hopeful approval of NurOwn as soon as is possible.”

About NurOwn®

NurOwn® (autologous MSC-NTF cells) represent a promising investigational 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. 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. NurOwn® is currently being evaluated in a Phase 3 ALS randomized placebo-controlled trial and in a Phase 2 open-label multicenter trial in Progressive MS.

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® Cellular Therapeutic Technology Platform used to produce autologous MSC-NTF cells through an exclusive, worldwide licensing agreement as well as through its own patents, patent applications and proprietary know-how. Autologous MSC-NTF cells have received Orphan Drug status designation from the U.S. Food and Drug Administration (U.S. FDA) and the European Medicines Agency (EMA) in ALS. BrainStorm has fully enrolled the Phase 3 pivotal trial in ALS (NCT03280056), investigating repeat-administration of autologous MSC-NTF cells at six sites in the U.S., supported by a grant from the California Institute for Regenerative Medicine (CIRM CLIN2-0989). The pivotal study is intended to support a BLA filing for U.S. FDA approval of autologous MSC-NTF cells in ALS. BrainStorm received U.S. FDA clearance to initiate a Phase 2 open-label multi-center trial of repeat intrathecal dosing of MSC-NTF cells in Progressive Multiple Sclerosis (NCT03799718) in December 2018 and has been enrolling clinical trial participants since March 2019. For more information, visit the company's website.

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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