BrainStorm Announces High-Level FDA Feedback on NurOwn® ALS Clinical Development Program

FDA recommended generating additional clinical data to meet FDA's BLA requirements

NEW YORK, Feb. 22, 2021 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of adult stem cell therapies for neurodegenerative diseases, announced today that it recently met with senior leadership from the U.S. Food and Drug Administration (FDA) and received FDA feedback on a high-level data summary from the NurOwn (autologous MSC-NTF cells) ALS Phase 3 clinical trial. The FDA concluded from their initial review that the current level of clinical data does not provide the threshold of substantial evidence that FDA is seeking to support a Biologics License Application (BLA). In addition, the FDA advised that this recommendation does not preclude Brainstorm from proceeding with a BLA submission.

"Brainstorm will first consult with principal investigators, ALS experts, expert statisticians, regulatory advisors, and ALS advocacy groups to assess the benefit/risk of a BLA submission before making a final decision," said Chaim Lebovits, CEO Brainstorm Cell Therapeutics. "We want to thank our independent advisors for their candor and unwavering commitment to our ALS program. We also would like to give a heartfelt thank you to all ALS patients and their families that were involved in this clinical research program. The company will continue to build on the strength of its wonderful, professional and dedicated team while being well funded at a current position of over $40M in cash. We will soon announce our next flagship product and program for other diseases with unmet needs."

"Many of us with longstanding experience in ALS therapy development agree that there was evidence of benefit from NurOwn cell therapy and hope that there will be an opportunity for further assessment of this modality in ALS," said Robert Brown, DPhil, MD, Director of the Program in Neurotherapeutics at the University of Massachusetts Medical School.

"ALS is a devastating disease with worse outcomes than most forms of cancer," said Anthony Windebank MD, Jean and Judith Pape Adams Professor of Neuroscience, Mayo Clinic College of Medicine and Science. "The clear signal in this trial that some patients with ALS respond to treatment with NurOwn is a light at the end of the tunnel. The careful study of biomarkers associated with response will help lead us forward towards a broadly effective therapy. The patients and their families who have brought us to this point are true heroes."

"I share my profound gratitude and thanks to the patients and families that participated in this phase 3 trial and all the teams at the six sites," said Merit Cudkowicz MD, Chief of Neurology and Director of the Healey & AMG Center for ALS at Mass General Hospital, and the Julieanne Dorn Professor of Neurology, Harvard Medical School. "We learned a lot about the efficacy and safety of NurOwn in people with ALS in this well conducted trial. We also learned some of the challenges with the use of ALSFRS-R at the lower end of the scale. Additional discussions with the community and sharing all the data in a peer reviewed publication are critical next steps."

"We will complete all ALS phase 3 study analyses to support scientific communication of the phase 3 data, including a peer-reviewed manuscript," said Ralph Kern MD MHSc, President and Chief Medical Officer Brainstorm Cell Therapeutics. "The results of the phase 3 clinical trial are very important, and we are confident that the analysis of key ALS disease biomarkers will contribute to our understanding of ALS. Our focus is to advance our proprietary cellular technology platform and to prioritize product development efforts based on data and business priorities."

About NurOwn®

The NurOwn® technology platform (autologous MSC-NTF cells) represents 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.

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 designation status 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 completed a Phase 3 pivotal trial in ALS (NCT03280056); this trial investigated the safety and efficacy of repeat-administration of autologous MSC-NTF cells and was supported by a grant from the California Institute for Regenerative Medicine (CIRM CLIN2-0989). BrainStorm is also conducting an FDA-approved 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) completed dosing in December 2020, and topline results are expected by the end of the first quarter 2021.

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 NurOwn® manufacturing and clinical development plans, 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, the prospects for regulatory approval of BrainStorm's NurOwn® treatment candidate, the initiation, completion, and 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, or to use third parties 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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