BrainStorm Issues 2020 Letter to Shareholders

NEW YORK, Dec. 28, 2020 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of cellular therapies for neurodegenerative diseases, today announced the following letter from its Chief Executive Officer, Chaim Lebovits, to its shareholders and the investment community.

Dear Valued Shareholders,

I am incredibly proud of the tremendous progress BrainStorm made as a company in 2020. The many scientific and corporate milestones we have achieved this year are a testament to the talent and unwavering commitment of our employees, partners and investigators. I deeply appreciate the work they've done, and their ability to execute in this challenging COVID environment. I want to thank especially the patients and caregivers, who showed a steadfast dedication to our clinical trials despite the pandemic. We remain committed to bringing NurOwn® to patients with neurodegenerative diseases as a much-needed treatment option. In addition, we are expanding our pipeline with the application of our exosome platform technology to include new indications that have significant unmet medical need.

BrainStorm's most significant clinical milestone of 2020 was the timely completion of the NurOwn® Phase 3 ALS clinical trial, despite the challenges of COVID-19. We believe the totality of evidence that we have generated are supportive of NurOwn's clinical benefit. We are diligently pursuing next steps, including active discussions with the United States Food and Drug Administration (FDA) to identify regulatory pathways that may support NurOwn's approval in ALS.

While we did not demonstrate statistical significance in the primary efficacy endpoint in the full study population, pre-specified analyses suggest that NurOwn® has a clinically meaningful treatment effect. For example, in a pre-specified participant subgroup with early disease and preserved function based on the ALSFRS-R baseline score, NurOwn® showed the expected numerically superior treatment response compared to placebo. Furthermore, phase 3 cerebrospinal fluid neurodegenerative and inflammatory biomarkers were consistently modified by NurOwn treatment, supporting NurOwn's proposed mechanism of action in ALS. We are thankful to the FDA for prioritizing our IND, as we and the Agency clearly understand the urgency needed for ALS patients.

Following the completion of our Phase 3 ALS clinical trial, we recently initiated an Expanded Access Program (EAP) to provide NurOwn® treatment for patients who completed the Phase 3 trial and meet specific eligibility requirements. This EAP was developed in partnership with the FDA and will take place at the six clinical centers of excellence that participated in the trial. Initiating the EAP immediately after the Phase 3 trial's completion speaks to the promise of NurOwn's phase 3 clinical data, the urgent unmet medical need for ALS patients and our commitment to patients. The ALS physician, advocacy and patient community feedback on the EAP has been extremely positive.

In parallel with the clinical programs to advance NurOwn, Brainstorm is also working to establish the manufacturing capability necessary for a commercial ramp up. To this effect, we engaged in strategic partnerships to expand our Current Good Manufacturing Practice (cGMP) capabilities. Our tech transfer to Catalent has already been initiated and will allow for continuous supply of NurOwn® for future clinical trials and initial commercialization. Our work with Rapid Reshore & Development, to help BrainStorm establish in-house manufacturing capabilities, will accelerate once a regulatory pathway is clear. These agreements will help to ensure an ongoing cGMP clinical supply of NurOwn® and will be integral to ensuring rapid access for patients following a potential regulatory approval. We also signed a lease agreement with the Tel Aviv Sourasky Medical Center (Ichilov Hospital) in Tel Aviv, Israel, to produce NurOwn® in three state-of-the-art cleanrooms. The new facility will significantly increase our manufacturing capacity, positioning us to strategically enter the European and Israeli markets. In addition to our clinical data review, we are also in active dialog with the FDA around this component of NurOwn's regulatory review. I am happy to share with you that we have recently had a successful meeting with the FDA regarding Chemistry, Manufacturing and Controls (CMC). We will provide an update when the minutes are finalized.

We have made excellent progress in our NurOwn clinical program in progressive MS, despite some delays in clinical trial enrollment due to COVID-19 healthcare restrictions. The next milestone will be the generation of top line results from our fully dosed Phase 2 trial which we expect by the end of first quarter 2021. Detailed analyses of the full study clinical and biomarker dataset can help demonstrate the potential of NurOwn technology in progressive MS. The broader objective for NurOwn is to continue to grow a strong body of evidence for NurOwn in ALS, MS in addition to other neurodegenerative diseases.

As part of our strategy to advance the NurOwn technology and expand our pipeline, we announced earlier this

year a new clinical program in Alzheimer's disease. NurOwn has the capability to simultaneously target multiple relevant Alzheimer's disease pathways and bring a comprehensive approach that addresses both neurodegeneration and neuroinflammation. This is a fundamentally different approach to many previous Alzheimer's therapies which have focused on a single target such as tau or beta-amyloid. We intend 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. The trial will be conducted at the Brain Research Centers affiliated with the Alzheimer Center Amsterdam, Pitié-Salpêtrière Hospital (Paris), and several other clinical trial sites in the Netherlands and France.

We were also pleased to announce a new program utilizing our exosome-based platform-technology for the treatment of severe COVID-19 infection and released proof-of-concept results from a pre-clinical study. In the pre-clinical study in mice, we demonstrated that intratracheal administration of exosomes extracted from Mesenchymal stem cells resulted in a statistically significant improvement in multiple lung parameters. The results from this study have been accepted and will be published in a peer-reviewed medical journal in 2021. We are actively evaluating next steps to determine the best path forward with our exosome platform.

We made important additions to the senior management team during 2020 including Dr. David Setboun as Chief Operating Officer and Dr. Stacy Lindborg as Head of Global Clinical Research. Dr. Setboun has extensive experience in the biopharmaceutical industry having held key leadership roles in commercial development and product launches at Biogen. Dr. Lindborg is an experienced healthcare executive and globally recognized medical statistician with over two decades of multinational experience in R&D, regulatory drug approvals, strategy development, analytics and big data at Lilly and Biogen. Prof. Jacob Frenkel joined us as Chairman of our Board earlier in the year. These appointments, as well as the other recent hires, are part of a strategic initiative to build a senior team with proven commercial capabilities, as we transition to potentially becoming a commercial organization.

Looking ahead to 2021, our main focus areas are to reach agreement with FDA on a path for regulatory approval NurOwn® in ALS, to generate top-line results for our Phase 2 MS study in the first quarter, to advance our Phase 2 Alzheimer's disease trial, to expand our exosome-based technology, and to further develop our cellular technology and manufacturing capabilities and infrastructure.

In 2021, we will leverage our strong financial position with over $40 million in cash and our experienced team will continue to be inspired by the ALS patients to bring our innovative treatment option forward while delivering value to our shareholders.

I would like to thank you for your continued support.

Sincerely,

Chaim Lebovits
Chief Executive Officer
BrainStorm Cell Therapeutics Inc.

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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