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BRAINSTORM CONSIDERING RIGHT TO TRY FOR ALS THERAPY

BY STEVE USDIN

BrainStorm Cell Therapeutics Inc. (NASDAQ:BCLI) is considering offering NurOwn to treat amyotrophic lateral sclerosis under the recently enacted federal right-to-try law, BrainStorm President and CEO Chaim Lebovits told BioCentury.

He also discussed the company's plans on a conference call Thursday with over 200 patients, caregivers and investors. While BrainStorm hasn't made a final decision about providing NurOwn under right to try, Lebovits made it clear that he hopes to do so if legal, ethical and business challenges can be resolved. "I think you can hear between the lines where our heart is," he said.

Responding to the wife of an ALS patient on the conference call, Lebovits said: "I understand you have no time. Patients currently have no time to wait for the end of Phase III. We understand that."

He told BioCentury that BrainStorm "will make a decision very soon, the pressure is too high to wait long."

NurOwn consists of autologous mesenchymal stromal cells induced to differentiate into neurotrophic factor-secreting cells.

In a double-blind, placebo-controlled, U.S. Phase II trial in 48 ALS patients, a single treatment of NurOwn was well tolerated with no treatment-related serious adverse events reported. At week 12 posttreatment, 40% of patients receiving NurOwn experienced an improvement in ALS Functional Rating Scale (ALS-FRS) slope of ≥50% from baseline vs. 17% of patients receiving placebo. In a subgroup of 23 rapidly progressing ALS patients, 94% of patients receiving NurOwn achieved a 100% improvement in ALS-FRS slope at week two vs. 20% of patients receiving placebo (p=0.0027). At week 24, 22% of patients in the subgroup receiving NurOwn achieved a 100% improvement in ALS-FRS slope vs. 0% of patients receiving placebo.

BrainStorm is conducting a U.S. Phase III trial of NurOwn in 200 ALS patients ages 18 to 60. The company received a $15.9 million grant from the California Institute for Regenerative Medicine (CIRM) to conduct the trial. BrainStorm's trial is the only Phase III trial that is actively enrolling ALS patients in the U.S., according to ClinicalTrials.gov.

BrainStorm has received a "flood" of requests for access to NurOwn under right to try, Lebovits told BioCentury. Several patients and caregivers who said on the conference call that they were anxious to obtain NurOwn reported that they were ineligible for the Phase III trial because they were older than 60, the cut-off date for inclusion.

Lebovits told BioCentury and patients on the conference call that BrainStorm will not provide access under right to try if doing so compromises its ability to complete the Phase III trial or submit a BLA.

He also said that if it provides NurOwn under right to try, BrainStorm will charge for the therapy. The law does not restrict companies' abilities to charge for unapproved therapies.

Lebovits declined to estimate the cost for NurOwn beyond saying that cost of manufacturing and delivering the therapy is similar to CAR T cancer therapies. Yescarta, a CAR T therapy from the Kite Pharma Inc. unit of Gilead Sciences Inc., has a wholesale acquisition cost (WAC) of $373,000.

Insurers are unlikely to cover the cost of an unapproved therapy, and BrainStorm is grappling with the ethics of pricing and access, Lebovits told BioCentury. "Will it be right to try for the rich only? Or if the poor can't afford it, should the rich die? This is a very, very hard decision," he said.

On the conference call, he said BrainStorm "won't go forward [with providing access under right to try] unless we have at least a partial answer to those who cannot afford it."

BrainStorm has received over 1,500 requests to provide NurOwn to ALS patients under right to try or under a pending application to offer it in Israel under the country's hospital exemption regulation, Lebovits said on the call. He told BioCentury the company could potentially offer NurOwn under right to try to 40 or 50 patients per year.

BrainStorm is consulting ethicists, patients and clinicians to determine which patients would be given access if the company decides to make it available under right to try, Lebovits told BioCentury.

NurOwn used in clinical trials is being manufactured by the City of Hope, Lebovits said. City of Hope would not

manufacture NurOwn for distribution under right to try, and BrainStorm is exploring the potential for manufacturing the product at other sites, he said on the call.

BrainStorm "has not heard from FDA" about providing NurOwn under right to try, Lebovits told BioCentury. He said the company hopes to organize a round table discussion with FDA, manufacturers of drugs for other conditions who are considering right to try, and legislators.