INTERNATIONAL ALLIANCE OF ALS/MND ASSOCIATIONS

BrainStorm Cell Therapeutics – NurOwn
Spring 2020

Background

The BrainStorm treatment regimen consists of a person's own stem cells (called autologous) being removed from bone marrow and then grown outside of the body in the presence of a chemical owned by the company called NurOwn, which aims to increase the stem cells' ability to make and secrete protective substances called growth factors. The stem cells are then injected into the fluid that bathes the brain and spinal cord (called cerebrospinal fluid or CSF) with a needle (called intrathecal or IT injection) at multiple intervals. The hope is that these treated stem cells will be able to slow the progression of motor neuron degeneration and hence, the progression of ALS symptoms.

In 2016, the first peer reviewed publication appeared, demonstrating preliminary, positive safety data in combination with BrainStorm completing a phase 2 clinical trial at three renowned US clinical sites.

The phase 2 trial data involved 48 ALS patients (36 treated and 12 placebo) and was published in December 2019 in the journal Neurology, titled "A single-dose transplantation of MSC-NTF cells is safe and demonstrated early promising signs of efficacy." The current phase 3 clinical trial is testing a multidose of 261 participants at six sites in the United States. On October 11, 2019, BrainStorm announced that this trial was fully enrolled and recently they reported the "phase 3 trial remains on track for topline data in Q4-2020 ." Public announcement of results will occur sometime after this date, and is currently expected at the end of 2020. The trial is double-blinded, meaning neither researchers nor participants know if they are on active treatment or placebo.

While BrainStorm has continually been in ALS-related news since 2012, the past year has again elevated the visibility of the company and treatment to the public. Some of the people participating in the phase 3 clinical trial have stated positive effects on social and mainstream media, while others have suggested their lack of noticeable effect is indication of being given placebo. It is important to await the results of the trial since such anecdotal claims have been connected to ALS treatments before, that have ultimately failed in clinical trial.

Recommendation

Based on preliminary published data, the SAC recommends to the Alliance that there is reason to be cautiously optimistic that NurOwn is safe and to hope it may provide some ability to affect disease course of ALS, but until the results of the current phase 3 clinical trial are available, it is very important to understand that no one yet knows if it works. The SAC remains hopeful that it does work, as with any therapy currently in testing. Based on preliminary, published trial data, and the support of multiple prominent ALS investigators, everyone should remain scientifically objective, but also hopeful.