

BrainStorm Cell Therapeutics – NurOwn August 2019

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 stem cells with boosted abilities will be able to slow the progression of motor neuron degeneration and hence, the progression of ALS symptoms.

Starting in 2012, BrainStorm made global headlines with a news story focused on an individual who began walking and talking again after the stem cell treatment. Since then, related and unrelated media has increased the popular belief that stem cells are all-encompassing agents of healing, which has contributed to the clinical trials of NurOwn as one of the most publicly discussed experimental therapeutics in the field. In 2016, the first peer reviewed publication on the safety data appeared in combination with BrainStorm completing a Phase 2 clinical trial at three renowned US clinical sites. Following that trial, there was promising safety data and cautious optimism that there was some treatment effect, though the trial was not large enough to confirm this. The Phase 2 data is currently being prepared for publication, which will allow for more robust analysis of the study. In 2017, a 200 participant Phase 3 clinical trial was started at six sites in the United States, as chosen by the company. They are more than halfway done recruiting for this trial and results will hopefully become available in early 2020.

While BrainStorm has continually been in ALS-related news since 2012, recent months have again elevated the visibility of the company and treatment to the public. First, in February 2019, BrainStorm decided to provide the NurOwn treatment for free to Mr. Matt Bellina in the United States, through Right to Try. Since his treatments, he has been posting symptom improvement videos on social media. Others have also been claiming positive effects on social media, while some have been claiming their lack of noticeable effect is indication of being given placebo. The trial is double-blinded, meaning neither researchers nor participants know if they are on active treatment or placebo.

In March 2019, BrainStorm announced the launch of a Hospital Exemption program in Israel where 13 people with ALS will be treated with the NurOwn treatment, with 8 being free for Israeli citizens and 5 being international at an undisclosed cost.

Recommendation

As there is not yet a peer reviewed publication on the most recent Phase 2 clinical trial data and the studies leading up to the current Phase 3 trial have been too small to know if the treatment is effective with certainty, we do not know if it works. If the study goes well, the Phase 3 trial will inform the field as to whether the treatment is able to slow the progression of ALS. The SAC remains hopeful that it does work, as with any therapy currently in testing. Despite the difficulties in interpreting the many years and multiple sources of claims around NurOwn, based on anecdotal evidence from those involved in the trial and reported effects from participants, as well as the support of prominent investigators involved in the Phase 2 trial, there is reason for the field to be cautiously optimistic. The tone around the NurOwn Phase 3 should be scientifically objective, but also hopeful.