



Kadimastem - AstroRx September 2020

Background

Kadimastem is a company located in Israel that is currently testing an “off-the-shelf”, clinical grade astrocyte cell product called AstroRx®. AstroRx® consists of astrocytes in suspension, derived from human embryonic stem cells. Astrocytes are neighbouring (glial) cells that provide support to motor neurons when healthy.

Data from an initial group of treated individuals (Cohort A) is presented in a [press release](#) from September 24th, 2019. Cohort A consisted of five participants with ALS treated intrathecally (in cerebrospinal fluid) with one dose of AstroRx® cells and evaluated for rate of disease progression over six months compared to the progression observed over the three months prior to treatment. The results stated that there was a “statistically significant decline in disease progression during the first 3-4 months after treatment was started” and that it serves as evidence for AstroRx® to provide “meaningful clinical benefit”. Kadimastem states that these results are “suggesting that a higher dose and/or repeated treatments may achieve prolonged efficacy”.

On December 23rd, 2019, Kadimastem announced that treatment with a higher dose of AstroRx® cells was provided to all five individuals in Cohort B with evaluation set for the next six months. Cohort C has been enrolled and involves repeated dose administration separated by 2-3 months. On August 3rd, 2020, results from Cohort B were provided in a [press release](#) that indicated the primary objective of safety was met, but also included claims about the efficacy. However, it should be noted that this was a study of only 5 people in each cohort and was primarily designed to test safety of the treatment. It is therefore very important that the data communicated in this release is not extrapolated by the public to indicate evidence about AstroRx® cells’ ability to slow ALS/MND progression.

A number of messages provided by Kadimastem could stimulate questions from people affected by ALS/MND. Kadimastem uses the slogan “cells to cure diseases” and the website explains that the goals of AstroRx® treatment include “replacing malfunctioning cells” and “restoring functionality”. The Chief Scientist states online that “This regenerative cell therapy promises to bring treatment and cure to millions of patients worldwide.” AstroRx® is also called a “breakthrough cell therapy”.

Recommendation

The SAC acknowledges the potential ability of healthy astrocytes to provide support to motor neuron health. Given the small size of Cohorts A and B, and the open label treatment of participants there is insufficient evidence to determine efficacy of AstroRx® in ALS/MND. There is presently no adequate evidence of efficacy with AstroRx treatment, neither has evidence been presented showing that AstroRx® could replace malfunctioning cells or restore functionality in humans. Caution should be taken when interpreting the results so far and should continue until a larger, properly controlled clinical trial has been completed.