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Newsletter**

Clinical Trials: Where Patients make a Difference

Two years ago, BioTime, a biotech company based in Israel, began a clinical trial involving patients with dry AMD. Nine blind patients suffering from advanced dry AMD (best corrected vision worse than 20/200) were transplanted with retinal pigment epithelial (RPE) cells derived from human embryonic stem cells. If there were no significant adverse events to those patients one year after surgery, an additional 6 patients with a best corrected vision of 20/100 would be treated.

The retina cells transplantation is performed using a needle injection into the retina. Subjects are given immunosuppression drugs from one week prior to surgery for one year after the transplant. The primary goal of the study is to determine the

safety of the procedure. Secondary outcomes will be to see if the graft survives, and if there are changes in areas of geographic atrophy.

Recent reports are promising. In subjects transplanted with retinal cells more than one year ago (either 50K retina cells or 200K retinal cells), the treatments were well tolerated. These patients were blind at the beginning of the study, so there is no expectation they would regain eyesight. However, in the majority of subjects changes in subretinal pigmentation were noted on high tech imaging. The investigators are hopeful that this suggests the transplanted RPE cells are surviving and may be integrating in areas where previous cell death had resulted in geographic atrophy. Healthy new cells that are successfully engrafted may halt the progression of AMD. The next cohort of six study subjects will undergo the transplant surgery in the U.S. and Israel.

The NIH recently awarded BioTime a \$1.5 million as part of its Small Business Innovation Research (SBIR) program. SBIR grants are given to companies that collaborate with academic centers to advance technology. BioTime has partnered with researchers at Michigan State University and Univ. of California – Irvine for this new grant. Dr. Magdalene Seiler, PhD from UCI has been successfully transplanting retinal cells in small animals. She will be sharing some of her techniques to assess visual function and structure in transplanted retinal tissue with the BioTime scientists.

While years away from clinical availability, clinical trials like this are essential steps in developing techniques to treat vision loss from dry AMD.