Arnall Golden Gregory LLP

Client Alert

Contact Attorneys Regarding This Matter:

Clark G. Sullivan
404.873.8512 - direct
404.873.8513 - fax
clark.sullivan@agg.com

William H. Kitchens
404.873.8644 - direct
404.873.8645 - fax
william.kitchens@agg.com

Geoffrey K. Rogers
404.873.8104 - direct
404.873.8105 - fax
geoffrey.rogers@agg.com

Arnall Golden Gregory LLP
Attorneys at Law
171 17th Street NW
Suite 2100
Atlanta, GA 30363-1031
404.873.8500
www.agg.com

Stem Cell Therapy in 2009: From Lab to Market

Stem cell therapy has the potential to change dramatically the treatment of human disease. A number of adult stem cell therapies already exist, such as bone marrow transplants that are used to treat leukemia. In the future, medical researchers anticipate being able to use technologies derived from stem cell research to treat a wider variety of diseases including cancer, Parkinson's disease, spinal cord injuries, and multiple sclerosis.

On February 6th, President Obama gave lawmakers his "guarantee" that he will sign an executive order lifting federal regulations on the use of embryonic stem cells in medical research. Furthermore, Mr. Obama has said the executive order would be coordinated with legislation codifying his order, so as to prevent a future Executive Branch reversal of the policy. Despite these developments, there exists a great deal of uncertainty regarding how the investigation of stem cells, which many scientists see as a goal worthy in itself, will translate in a market for stem cell therapy. Success in the marketplace will depend not only on technological advances, but also the important steps of governmental approval, healthcare reimbursement, and patent protection. As discussed below, several leaders in the stem cell industry are taking these steps to create a commercial market for stem cell therapy.

FDA Approves First Clinical Trial with Embryonic Stem Cells

On January 23rd, Geron Corporation announced that the FDA had granted clearance of the company's Investigational New Drug (IND) application for the clinical trial of "GRNOPC1" in patients with acute spinal cord injury. GRNOPC1, Geron's lead therapeutic candidate, contains progenitor cells derived from embryonic stem cells. When injected into the spine, these cells have demonstrated nerve growth stimulating properties leading to restoration of function in animal models of acute spinal cord injury. The clearance enables Geron to move forward with the world's first study of a human embryonic stem cell therapy in man. Geron plans to initiate a trial that is designed to establish the safety of GRNOPC1 in patients with "complete" thoracic spinal cord injuries. The company will recruit 8-10 recently injured patients and inject them with small numbers of human embryonic stem cells manipulated to become the oligodendrocyte cells that insulate nerves and produce compounds to stimulate the growth of nerve cells.

Geron faced difficulties in passing the 4-year approval process. The company

had expected to begin the trial last May, but faced last minute objections from the FDA. Over the following months, Geron worked closely with the FDA to gain approval for the trial. The FDA's chief concerns were (1) whether the mixture of cells within the product were predictable and free from contamination, (2) the possibility for unwanted growth and differentiation both from the desired cell type and contaminating cells, (3) the potential unpredictability of cells, (4) the difficulty in retrieving and monitoring cells for harmful effects, and (5) the concern that the animal tests performed to evaluate the cells could be less predictive than animal tests for small molecule or protein drugs. To help meet these concerns, Geron collaborated with the FDA to prepare standards regarding what evidence is necessary to ensure the safety of stem cell products. The central purpose of the Phase I trial will be to show that the patients do not develop tumors or damage to their nervous system. Subsequent phases will be designed to show whether the stem cells might repair the damaged spinal cords. If successful, this clinical trial may hold great promise for other diseases such as multiple sclerosis or stroke. Additionally, the treatment has the potential to become very cost effective to mass produce because the cells can be grown in vats.

Many researchers view Geron's study as a test of whether embryonic stem cells are ready for clinical trials. Scientists caution that miracles should not be expected from this particular trial, as it was only designed to test safety. Regardless of outcome, the trial will provide valuable insight for moving stem cell research into the clinic.

Reimbursement – Proving the Value of Stem Cell Therapy

As with all advanced healthcare technologies, the commercial success of stem cell therapies will greatly depend on the extent of healthcare reimbursement. In his inaugural address, President Obama pledged to "restore science to its rightful place, and wield technology's wonders to raise health care's quality and lower its costs." This provides hope that federal health insurance programs will provide reimbursement for those stem cell therapies that can provide higher quality care at a lower cost.

In Europe, at least one stem cell therapy is already awaiting reimbursement approval. Bioheart, Inc., a company that develops cell therapies for the treatment of chronic and acute heart damage, announced on January 13th that it had filed applications for reimbursement in Germany, Italy, the Netherlands, and Switzerland for its MyoCell(r) myogenic cell therapy for heart failure. Previously, data from Bioheart's clinical trials indicated that about 84 percent of the myogenic stem cell treated patients improve in quality of life and exercise capacity, as compared to 31 percent of patients on heart failure drugs.

Bioheart's reimbursement application targets the sickest of heart failure patients, and the company estimates that approximately 250,000 of the 9 million European patients in heart failure will qualify. The applications seek a reimbursement level matching exactly that charged to provide bi-ventricular pacemakers for this same patient population. To obtain reimbursement, Bioheart seeks to prove that its stem cell technology provides a better quality of care at a comparable price. In recent studies, the MyoCell treatment resulted in 55 to 90 meters improvement in 6-minute walking distance, a significant improvement over traditional

pacemakers, with demonstrated a 16 to 20 meter improvement.

Stem Cell Patents – Protecting the Research Investment

Across the globe, billions of dollars per year have been poured into stem cell research. To create value from this substantial investment, companies around the globe have filed thousands of patent applications. By one count, these applications have resulted in over 1300 issued U.S. patents relating to stem cells products and their methods of use (www.stemcellpatents.com).

Geron's GRNOPC1 product is an excellent example of how patent rights are of primary importance in the commercialization of stem cell technology. Geron reports that its stem cell-related patent portfolio includes 36 issued or allowed U.S. patents, 68 patents granted or accepted in other countries, and over 130 applications pending worldwide. Of central importance to Geron are U.S. Patent Numbers 5,843,780 and 6,200,806, both of which are related to its lead therapeutic GRNOPC1. Due at least in part to the large potential value of these patents, the New York-based Public Patent Foundation and the California-based Foundation for Taxpayer and Consumer Rights requested that the patent office initiate reexamination proceedings in October of 2006. The patent office upheld the validity of these patents in two parallel reexamination proceedings spanning 16 months. Geron reports that this positive outcome will assist it in protecting the production and commercialization of GRNOPC1.

In addition to protecting their own research, stem cell companies also must consider the intellectual property rights of their competitors. This task, which is complicated due to the high number of stem cell related patents, requires clearing upcoming products through freedom-to-operate opinions, and obtaining licenses for necessary technology. Geron, for example, has obtained exclusive licenses to patents from the Wisconsin Alumni Research Foundation (WARF) and the University of California to protect its GRNOPC1 product. As Geron's product enters clinical trials, it may continue to face challenges from the patents of its competitors. For example, Neuralstem, Inc. announced on Monday, January 26th that its patent covering "Stable neural stem cell lines" had been allowed. As patent attorney Dr. Donald Zuhn has pointed out in his "Patent Docs" newsletter, the timing of this announcement is of interest because it immediately followed Geron's announcement on Friday, January 23rd, that its GRNOPC1 product has been approved to enter clinical trials.

For more information about clinical trials, reimbursement, or product protection issues, please contact Bill Kitchens, chair of Arnall Golden Gregory's Healthcare/Life Sciences practice (william.kitchens@agg.com).