

# Cell-based therapy shows promise in patients with Parkinson's disease

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A novel cell therapy using retinal pigment epithelial (RPE) cells attached to tiny gelatin bead microcarriers implanted in the brain can improve the symptoms of patients with moderate to advanced Parkinson's disease (PD).

Rush University Medical Center neurosurgeon Dr. Roy A. E. Bakay and colleagues from Emory University, Atlanta found the therapy Spheramine was well-tolerated and patients experienced improvement in Parkinsonian symptoms (tremor, rigidity, slowness of movements, and impaired balance and coordination.) These findings were presented at the Annual Meeting of the American Association of Neurological Surgeons in Chicago on April 28, 2008.

The pilot study was initiated at Emory University Hospital and followed six patients with moderate to advanced PD to investigate the safety, tolerability, and efficacy of the Spheramine implantation. The full patient group has been evaluated for four years, and several have been monitored for six years. Bakay and colleagues report long-term improvement or stabilization of symptoms, maintained for a minimum of two years after Spheramine implantation. They note no Spheramine-related serious adverse events were reported and that the most frequent adverse event was postsurgical headache, which spontaneously resolved within one to two weeks.

“The results of this study are very encouraging – Spheramine is well tolerated through several years of follow-up and improvement in

parkinsonian symptoms is sustained,” stated Bakay.

The cellular product Spheramine consists of RPE cells attached to microcarriers. RPE cells produce levodopa, the precursor of dopamine. Dopamine is a neurotransmitter produced by nerve cells in the brain that progressively declines as the disease progresses.

The RPE cells, which are normally found in the back of the eye, are cultured under standardized conditions and attached to the microscopic beads prior to implantation. The microcarriers are necessary for the cells to survive in the brain. The implanted cells serve as a new potential source of levodopa to enhance dopamine production where it is most needed.

The patients were selected based on disease stage, levodopa responsiveness, and severity of PD symptoms while off medication. An even distribution of Spheramine was surgically implanted into the more affected side of the brain, and patients left the hospital a few days later.

The primary efficacy measure in this trial is the motor score of the Unified Parkinson’s Disease Rating Scale (UPDRS) when the patient has been OFF antiparkinsonian medication for at least 12 hours. The researchers report clinical improvements were noted in both UPDRS motor scores off medication (44 percent improvement from baseline at 48 months) and patient-reported quality of life scores (23 percent improvement from baseline of total PDQ-39 score at 48 months). Several of these patients have been monitored for 6 years and the trial has been extended to 10 years of follow-up.”

Bakay said positive results in the pilot study prompted the initiation of a Phase IIb, multicenter, double-blind, randomized, sham surgery-controlled study (STEPS) to further evaluate the safety, tolerability and efficacy of Spheramine. Changes from the pilot study included

implantation in both sides of the brain and the addition of a sham surgery group. To date, 71 patients have been randomized for either Spheramine or sham surgery and results from the will become available later this year.

## **Parkinson's Disease**

Parkinson's disease is a progressive brain disorder that affects a person's motor skills which worsen as the disease advances. Early in the disease, there is a loss of brain cells that produce the chemical dopamine.

Normally, dopamine operates in a delicate balance with other neurotransmitters to help coordinate the millions of nerve and muscle cells involved in movement. Without enough dopamine, this balance is disrupted, resulting in tremor (trembling in the hands, arms, legs and jaw); rigidity (stiffness of the limbs); slowness of movement; and impaired balance and coordination – the hallmark symptoms of PD.

PD affects one in every 100 people over the age of 65. The latest epidemiology studies indicate that worldwide numbers will increase from an estimated 4.1 million in 2005 to 8.7 million people with PD by 2030. There were an estimated 19,500 PD-related deaths in the United States in 2005, an increase of 1,500 deaths from 2004.

It is estimated to cost \$23 billion a year in direct and indirect costs and lost productivity. Despite therapeutic advancements, oral medications provide insufficient symptom control after the disease has progressed and new approaches are needed.

Source: Rush University Medical Center

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