World Ophthalmology Congress®
Berlin, Germany

AAD Congress 2010
3 - 6 June 2010

XXXII International Congress
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5 - 9 June 2010

World Ophthalmology Congress 2010

Gene Therapy for Blindness: First Successes in Hereditary Retinal Diseases

Berlin, April 2010 – Patients with rare hereditary eye diseases, such as Leber congenital amaurosis, suffer from severe visual impairment from birth. Indeed, they often go completely blind before reaching adulthood. Gene therapy can help to improve visual performance and prevent blindness. Ophthalmologists from 120 countries will be discussing the first study findings at the World Ophthalmology Congress (WOC® 2010), which takes place in Berlin in June.

Doctors have achieved some first success with two hereditary retinal diseases using gene therapy: on the one hand with a rare form of Leber congenital amaurosis (LCA), from which some 200 patients in Germany suffer. In these patients, a defect in the so-called RPE65 gene affects the formation of visual pigment. “Sufferers have severely impaired vision in the first two years after birth. They gradually lose their sight and are often completely blind before reaching the age of 20,” reports Professor Dr. med. Birgit Lorenz, Head and Chairman of the Department of Ophthalmology, Justus-Liebig-University Giessen, Germany.

As part of the gene therapy approach, viral vectors carrying a correct version of RPE65 are injected under the retina. Viral vectors, also known as “gene shuttles”, are transport vehicles used to transfer foreign genetic material to a living recipient cell. The vectors deposit the gene in the pigment cells of the retina, which then form the correct proteins. Subsequently, formation of functional visual pigment resumes. “The first application of this therapy on 18 patients in England and the USA has shown that visual performance improves in the long term, particularly among young patients,” Lorenz reports in the run-up to WOC® 2010. A total of six studies are currently either in progress or in preparation.

Gene therapy can also help people suffering from retinitis pigmentosa (RP). Likewise a hereditary disease, it leads to degeneration of the retina and also blindness in its later stages. In contrast to LCA, doctors currently do not try to eliminate the gene defect head-on. Rather, they implant a small capsule

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in the eye. This contains genetically modified cells which form the protein CNTF, the “ciliary neurotrophic factor”. CNTF prevents cell death in the retina. “Here, too, the first findings are promising. Stabilisation or improvement of vision has been observed in the ten people taking part in the study,” Lorenz explains. Lorenz will be discussing the possibilities of gene therapy in ophthalmology with her colleagues at WOC® 2010.

A further study with CNTF-producing capsules has in the meantime also been carried out on patients with the dry form of age-related macular degeneration (AMD). Publication of the findings is still pending. Should gene therapy be successful for this disease, it could be an option for a large number of patients. AMD is the most common cause of severe visual impairment and blindness in industrialised countries.

In case of publication, we kindly request you to send us a specimen copy.

Date worth noting:

WOC Symposium: Leber Congenital Amaurosis: Classification and Treatment of Childhood Onset Severe Retinal Dystrophies
Tuesday, 8 June 2010, 16.00 to 17.30, Room 014, ICC Berlin, Neue Kantstraße/Ecke Messedamm, 14057 Berlin

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Germany plays host in 2010 to the largest international ophthalmologic congress with the World Ophthalmology Congress (WOC® 2010). In addition to the International Congress of Ophthalmology (ICO), the Annual Congress of the German Society of Ophthalmology (DOG) and the German Academy of Ophthalmology (AAD) will take place under the umbrella of WOC® 2010. From 3 to 6 June, 2010, AAD courses will be held in German. The international program in English will follow from 5 to 9 June. The organizers expect over 8,000 attendees from some 120 countries.