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Boys Town Scientists Provide Key Pre-Clinical Evidence for Treating Usher Syndrome with Gene Therapy

Omaha, Neb—A team of scientists from Boys Town National Research Hospital, in collaboration with Oxford BioMedica, have received Food and Drug Administration (FDA) approval for the first treatment ever developed for Usher Syndrome.

Boys Town scientists Dominic Cosgrove, Ph.D., Director of the Center for the Study and Treatment of Usher Syndrome; Marisa Zallocchi, Ph.D., and You-Wei Peng, M.D., Ph.D, conducted the pre-clinical research of the investigational drug UshStat[®], giving evidence for FDA approval.

Usher Syndrome results in deafness at birth associated with delayed onset and progressive blindness. The newly approved treatment involves the introduction of a functioning copy of the defective gene into retinal cells. The treatment is aimed at the most common of the most severe clinical sub-type of Usher syndrome (USH1B), which has early onset and rapid progression of retinal disease. The disorder affects about 1 in 30,000 people in the United States and Europe.

The clinical trial will involve up to 18 patients with Usher syndrome type 1B and will be performed at Oregon Health and Science University's Casey Eye Institute in Portland, Oregon. The study will be led by Professor Richard Weleber and will evaluate safety, tolerability, and aspects of the biological activity of the treatment. Based on the pre-clinical data, it is anticipated that a single application of UshStat[®] to the retina could provide long term or potentially permanent stabilization of vision. Clinical trials may be initiated by the end of 2011.

Boys Town National Research Hospital has been a leader in research on Usher syndrome for 25 years and played an important role in isolation of the gene mutation for Usher type 1B that may now be corrected by gene therapy.

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Boys Town Scientists Treating Usher Syndrome

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This research has been supported by the Foundation Fighting Blindness (a public charity), the National Institutes of Health and the Nebraska Tobacco Settlement Biomedical Research Development Fund. This is the third program for retinal gene therapy developed by Oxford BioMedica to enter clinical development under the Phase I/II collaborative agreement signed with Sanofi in April of 2009.

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Boys Town National Research Hospital, located in Omaha, Nebraska, is internationally recognized as a leader in clinical and research programs focusing on childhood deafness, visual impairment and related communication disorders. The hospital has developed national medical programs that are now instituted in hospitals and clinics across the country.

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