

No evidence to support stem cell therapy for pediatric optic nerve hypoplasia

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A study performed at Children's Hospital Los Angeles found no evidence that stem cell therapy improves vision for children with optic nerve hypoplasia (ONH). Their results are reported in the *Journal of the American Association for Pediatric Ophthalmology and Strabismus (AAPOS)*.

ONH, an underdevelopment of optic nerves that occurs during fetal development, may appear either as an isolated abnormality or as part of a group of disorders characterized by brain anomalies, developmental delay, and endocrine abnormalities. ONH is a leading cause of blindness in children in North America and Europe and is the only cause of childhood blindness that shows increasing prevalence. No treatments have been shown to improve [vision](#) in these children.

With no viable treatment options available to improve vision, ophthalmologists are becoming aware that families with children affected by ONH are travelling to China seeking stem cell therapy, despite lack of approval in the United States and Europe or evidence from controlled trials. The American Association for Pediatric Ophthalmology and Strabismus has also expressed its concern about these procedures. In response to this situation, pediatric neuro-ophthalmologist Mark Borchert, MD, Director of both the Eye Birth Defects and Eye Technology Institutes in The Vision Center at Children's Hospital Los Angeles, realized that a controlled trial of sufficient size was needed to evaluate whether stem cell therapy is effective at improving [optic nerve](#) function in children with ONH. He

agreed to conduct an independent study when asked by Beike Biotech, a company based in Shenzhen, China, that offers treatment for ONH using donor umbilical cord [stem cells](#) injected into the cerebral spinal fluid.

Beike Biotech agreed to identify 10 children with bilateral ONH (ages 7-17 years) who had volunteered to travel to China for stem cell therapy and who agreed to participate in the study; Children's Hospital was to find case matched controls from their clinic. However, only two case-controlled pairs were evaluated because Beike Biotech was only able to recruit two patients. Treatments consisted of six infusions over a 16-day period of umbilical cord-derived mesenchymal stem cells and daily infusions of growth factors. Visual acuity, optic nerve size, and sensitivity to light were to be evaluated one month before [stem cell therapy](#) and three and nine months after treatment.

No therapeutic effect was found in the two case-control pairs that were enrolled. "The results of this study show that children greater than 7 years of age with ONH may have spontaneous improvement in vision from one examination to the next. This improvement occurs equally in children regardless of whether or not they received treatment. Other aspects of the eye examination included pupil responses to light and optic nerve size; these did not change following treatment. The results of this research do not support the use of stem cells in the treatment of ONH at this time," says lead author Cassandra Fink, MPH, program administrator at The Vision Center, Children's Hospital Los Angeles.

Confounding the trial was that subjects received additional alternative therapies (acupuncture, functional electrical stimulation, and exercise) while receiving stem cell treatments, which was contrary to the trial protocol. The investigators could not determine the effect of these additional therapies.

"This study underscores the importance of scientifically testing these

procedures to validate them and also to ensure their safety. Parents of afflicted [children](#) should be aware that the science behind the use of stem cell technology is unclear. This study takes a step toward testing this technology and finds no beneficial effect," says William V. Good, MD, Senior Associate Editor, *Journal of AAPOS* and Clinical Professor of Ophthalmology and Senior Scientist at the Smith-Kettlewell Eye Research Institute.

More information: "Failure of stem cell therapy to improve vision in children with optic nerve hypoplasia," by Cassandra Fink, MPH, Pamela Garcia-Filion, PhD, Mark Borchert, MD, [DOI: 10.1016/j.jaapos.2013.06.012](#). It appears in the *Journal of AAPOS*, Volume 17, Issue 5 (October 2013)

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