THE WALL STREET JOURNAL.
WSJ.com

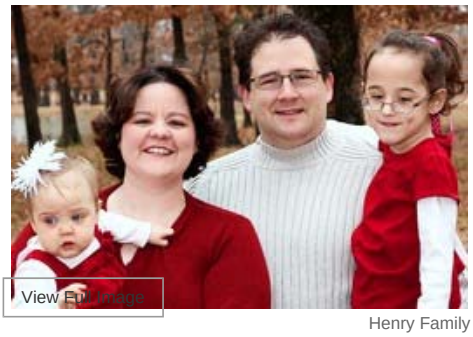
HEALTH INDUSTRY | FEBRUARY 17, 2011
HEALTH

Drug Shows Promise For Newborn Blindness

Treating Babies With Avastin Surpassed a Usual Therapy

By JENNIFER CORBETT DOOREN

An inexpensive drug therapy far surpassed a conventional laser procedure in fixing a leading cause of blindness in babies born prematurely, according to a new study.



Henry Family

The Henry family (from left), Evelyn, Kelly, Brad and Grace. Both girls were born premature. Grace is legally blind while Evelyn received drug therapy and her eyesight is developing normally.

Henry Family

Evelyn Henry

The results of the study, to be published this week in the New England Journal of Medicine, were so significant that the 15 hospitals participating in the research have stopped using lasers in favor of the drug, Avastin, which is injected into the eyes of the affected newborns. Avastin, made by Roche Holding AG's Genentech unit, is designed to be used for treating cancer, and the company doesn't promote its use for any eye conditions.

Babies born before 30 weeks of gestation have immature eyes and are at high risk of developing a condition called retinopathy of prematurity that is caused by uncontrolled growth of blood vessels in the eye. The blood-vessel growth can lead to scarring and detachment of the retina, which causes blindness.

The study's lead researcher, Helen Mintz-Hittner, an ophthalmology professor at the University of Texas Health Science Center at Houston Medical School, says retinopathy of prematurity is traditionally treated with a laser procedure that requires general anesthesia. Some babies need several treatments over time, and in most cases peripheral vision can't be saved.

Dr. Mintz-Hittner estimates there are about 3,000 to 4,000 cases of retinopathy of prematurity in the U.S. each year. The numbers are increasing as more premature babies survive.

An injection of Avastin stopped blood-vessel growth. In many cases, the drug was able to successfully treat retinopathy in premature babies with just one treatment. Babies get a small amount of anesthetic to numb the eye before being injected.

Avastin was designed to help treat cancer by choking off blood vessels to tumors, and doctors have been using it to treat some eye conditions. Genentech also markets a drug called Lucentis that's similar to Avastin and is

approved by the Food and Drug Administration to treat age-related macular degeneration, which is the leading cause of blindness in older adults.

Dr. Mintz-Hittner says such a small dose of Avastin is used for premature babies that it costs about $40 to treat both eyes. A comparable dose of Lucentis would be about $2,500, she says. Avastin also is preferable to Lucentis for retinopathy in infants because of the way it's designed. Avastin is considered a large-molecule drug, so it can't easily travel outside the eye area. Dr. Mintz-Hittner said a theoretical concern with Lucentis, which is a smaller molecule, is that it might travel to other parts of the body and impact necessary blood-vessel growth in babies.

A Genentech spokesman said there's limited safety information about uses of Avastin in treating eye diseases. "We don't support or promote the off-label use of Avastin," he said, noting that Avastin is not approved for use in the eye.

About 150 premature babies were enrolled in the Houston-led study from March 2008 through August 2010. Half were randomized to receive Avastin and the other half received laser treatment. Seven babies died of causes unrelated to the eye treatments. The remaining 143 babies were followed for several weeks after their initial treatments to see if the retinopathy returned.

Overall, retinopathy recurred in four infants treated with Avastin compared with 19 infants treated with the laser, which translates into a 20% reduction in the risk of recurrence. The results were even more significant for babies with a harder-to-treat form of the disease called zone 1, in which abnormal blood vessels form at the very back of the eye. Among infants with this form of the disease, the recurrence rate was 6% with Avastin compared with 42% for laser therapy.

James D. Reynolds, an ophthalmology professor at the University of Buffalo, wrote in an accompanying editorial that Avastin should be the treatment of choice for babies with zone 1 retinopathy of prematurity.

Bradley and Kelly Henry, who had two daughters born prematurely, saw first hand the outcomes of the different treatments. Their daughter Grace was born in 2004 at 24 weeks' gestation, weighing under two pounds. She was later diagnosed with retinopathy of prematurity and had several laser treatments under Dr. Mintz-Hittner's care. Grace's left eye eventually burst after she developed glaucoma, and it had to be replaced with a glass eye. She has enough vision in the right eye to attend regular school but is considered legally blind."It's hard to watch your daughter walk into a flagpole because she can't see," Ms. Henry says.

By the time the Houston-area family's other daughter, Evelyn, was born early last year at 26 weeks' gestation, the Avastin study was under way. Evelyn wasn't enrolled but was able to receive Avastin after she was diagnosed with retinopathy.

Ms. Henry says she remembers being "elated" that Evelyn was able to receive Avastin. "I knew what the effects of laser treatment were," she says. Evelyn, now 13 months old, had just one treatment with Avastin and so far appears to be developing normally, Ms. Henry and Dr. Mintz-Hittner say.

The oldest children in the Avastin study are now nearly 3-years old and appear to be developing normally, Dr. Mintz-Hittner says, adding that so far she hasn't seen any significant side-effects from the drug. She says doctors need to be careful not to administer Avastin too early before the abnormal blood vessels fully develop, nor too late after the blood-vessel growth causes the retina to detach.