



## THE VISION of CHILDREN

### WE ARE WINNING THE BATTLE

**Great news – the first successful human gene therapy trials in the history of the world have just occurred!!!** For the first time, scientists have used gene therapy to dramatically improve sight for people who have a form of Leber's Congenital Amaurosis (LCA).

Leber's Congenital Amaurosis is a group of hereditary degenerative blinding diseases that affects light receptors in the retina. Finding a cure for LCA should ultimately lead to a cure for all genetically caused vision disorders. LCA begins to affect individuals in early childhood and eventually causes complete blindness by age twenty to thirty. In this form of blindness, LCA photo receptor cells are unable to respond to light due to mutations in the RPE65 gene. The defective RPE65 proteins are unable to produce sufficient vitamin A molecules necessary for good, healthy vision.

In the clinical studies, each of the patients, nine young adults ages 19-26, experienced a form of LCA involving a defective RPE65 gene. The scientists used a genetically engineered adeno-associated virus to transmit a normal version of the RPE65 gene via surgical procedure. Scientists injected millions of copies of the normal RPE65 beneath the retina in the back of an eye.

*Leading eye surgeon, Dr. James Bainbridge, a presenter at The Vision of Children Foundation World Symposium in 2006, was intimately involved with the first gene therapy trials this past spring at Moorfields Eye Hospital in London.*

Additional trials took place at The Children's Hospital of Philadelphia and at the University of Florida, Gainesville with techniques used in the study taken from an earlier gene therapy trial led by Professor Robin Ali at Moorfields Eye Hospital in London.

*All patients reported a vast improvement in their vision, with some reporting the ability to read up to three lines on an eye chart!* After treatment, the patients' eyesight and light sensitivity were periodically measured. All the patients stated that their vision in dim light improved greatly. The researchers reported that each injected eye became approximately three times more sensitive to light and each one was comparatively better than the untreated eye. In addition, patients experienced less nystagmus.

These studies signify a stunning achievement for gene therapy and for modern medicine. The Vision of Children Foundation and the medical community have strived for 17 years to achieve these results.

This breakthrough gives great hope to all patients with genetically caused vision disorders. 🙄

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#### CALENDAR OF EVENTS

#### FEB 27-MAR 1, 2009

World Symposium  
On Ocular Albinism

#### MAY 2, 2009

Rock Concert  
to Benefit VOC

San Diego Kids Trick or Treat for VOC



# RESEARCH

## NEWS FROM THE LAB

Last newsletter we reported on a new researcher, Dr. Alex Yuan. Supported by The Vision of Children, Dr. Yuan and his team have gained significant insight on rescuing defects present in ocular albinism.

Many human disorders, including ocular albinism, are due to the expression of a defective gene resulting in either a poorly functioning or a non-functional protein. These disorders can theoretically be cured by replacing the defective gene with copies of the normal gene. Methods to deliver genes have been perfected in the laboratory. However, gene delivery is in its infancy as a tool to cure human disease. Current methods of gene transfer involve the use of viruses or artificial particles and lipids. These delivery methods have the disadvantage of introducing either infectious material or expensive artificial molecules into the human body with unknown adverse effects.

Dr. Yuan and the Jules Stein Eye Institute at UCLA aim to conduct studies on a potentially new method of gene delivery. This new method

involves the use of biological material called microvesicles. Microvesicles are normally produced by human cells and may serve as “delivery trucks” to ferry genetic material and proteins from cell to cell. Dr. Yuan and his group have been studying the composition of microvesicles released by embryonic stem cells because he wishes to gain a better understanding of their role in normal human physiology. Dr. Yuan has found that microvesicles released from one cell attach themselves to other neighbor cells and pass to them their content. He hopes to take advantage of the normal physiological functions of microvesicles by developing methods to deliver normal genetic material to malfunctioning cells. In this case, since Dr. Yuan is studying a mouse model for ocular albinism, the microvesicles will be delivering their cargo to the retinal pigment epithelial cells. Once delivered, the genetic material should produce normal copies of the defective gene causing the disorder, thereby altering the behavior of the affected cells. The team will try to correct the defects present in the ocular albinism mouse model using microvesicles delivering normal genetic material, leading to crucial new steps to a cure for vision disorders! 🐾

## WELCOMING OUR NEW TEAM MEMBER

We are happy to introduce a new addition to the VOC family. Michelle Pius joins us after volunteering for several years with many charities in the San Diego area including Rady Children’s Hospital’s Rancho Santa Fe Auxiliary Unit. During her five years as a board member, she dedicated her time and talents to raise much needed funds. Michelle has a “can do” attitude that is present in every project she undertakes. She embarks on all her tasks with creativity, integrity, and an almost limitless amount of energy.

Michelle has a passion for non-profits. We are excited to have her on our team and look forward to the new paths she will create. “I am delighted to join VOC and I look forward to meeting and working with all of our supporters” 🐾

## VOC NEWS

### EYESITE ON WEBSITE

The Vision of Children Foundation has been working overtime to perfect our look, our services, and our research. In November, The Vision of Children is unveiling its fresh website at [www.visionofchildren.org](http://www.visionofchildren.org).

Visit VOC’s website for our latest scientific research, upcoming events, and most importantly, services intended specifically for you and your family.

Visit us at: [www.visionofchildren.org](http://www.visionofchildren.org) 🐾

## FAMILY NETWORK

### KID OF THE MONTH

One of the most enjoyable experiences for us at VOC is reading personal stories and comments from our Family Network members and learning more about those we serve. Please continue to send us your stories, victories, challenges, and life experiences. We are here to provide support and encouragement. We love hearing how the children of the future are growing and enjoying life; we especially love hearing of their triumphs over the challenges sometimes presented by their visual impairments.

This month, George F. Triebenbacher and his mother remind us how little tikes keep us all busy.

George is a warm and affectionate four-year-old boy who lives in Pleasantville, New York. When George was born with congenital nystagmus, his parents, George and Lorenza Triebenbacher, found support and advice from Sam and Vivian Hardage and other VOC families. *(continues on next page...)*



Now, four years later, George is a very busy little boy, who enjoys attending preschool, playing soccer on his team, and practicing karate. He also loves mini golf, riding his bike, and playing with his eight-year-old sister, Marina. George, who has a very pleasant, easy-going personality, and always impresses his parents with his affection and sense of humor, enjoys listening to all kinds of music (especially Andrea Bocelli)\* and “reading” books. He is very bright and great at completing mazes and puzzles. But what George loves best is hanging out with his family! 😊

*\*You may remember that George’s parents were instrumental in planning and ensuring the great success of VOC’s Andrea Bocelli fundraising concerts in New York in 2006 and 2007. What a blessing to have the entire Triebenbacher family in the VOC Family Network!*

## VISION HEROES HOST ANNUAL “FUN” RAISER



“Vision Heroes” award recipients, Laura and Richard Saker continue to help further the mission of The Vision of Children Foundation. They have demonstrated their tireless efforts and visionary leadership these past two summers by designating the VOC as the beneficiary of their ShopRite promotion, a program in which ShopRite employees ask shoppers to make direct donations to the VOC.

Richard and Laura Saker have spent a lifetime sharing their blessings. As President and CEO of Saker Holding, the parent company of Saker ShopRites, Inc. and Saker Realty Enterprises, Richard has harnessed the power of this huge enterprise to benefit countless lives and the quality of life for the residents of Central New Jersey. Together with Laura, they raise millions of dollars to benefit the hungry and those suffering from ALS, provide support for individuals and their families dealing with vision disorders, and raise money to promote the arts, conservation, healthcare, and animal welfare.

Richard’s business leadership and professional expertise and Laura’s governance responsibilities on the boards of several area non-profits, have followed a decades-long Saker Family tradition of giving back to the communities in which they work and live.

Proud of its longstanding reputation for excellence, Saker ShopRites World Class Supermarkets have been voted “Best Supermarket” 10 years in a row, by major newspapers in Central New Jersey.

Several ShopRite stores went above and beyond the OPT IN promotion at the check out stands, and created a day of fun for kids. Melanee Gross, Steve Larsen and their team from the Marlboro store constructed a carnival for the day, with prizes, cupcakes, and games galore! Face painting, food, and fun led the way for families on this special day. Their hard work helped raise much needed funds for vision research and we thank them for their endeavors and enthusiasm. 😊



## PHILANTHROPY

### BOCCE BALL BONANZA

As one of the beneficiaries at the Del Mar-Solana Beach Sunrise Rotary Club’s 12th Annual Turf Bocce Ball Tournament, the VOC received a grant of \$6,600 to apply to its Computer Monitor program. The Computer Monitor program provides visually impaired children the tools to efficiently use a computer in the classroom. Our Computer Monitor program continues to grow and serve hundreds of thousands of visually

impaired children. The grant will provide monitors to seven elementary schools, one middle school, and two high schools. We thank Del Mar-Solana Beach Sunrise Rotary Club for their generous support which will help visually impaired children learn and compete more effectively in our technological, computer oriented society. 😊

### NATALIE’S WAY FOUNDATION RAISES \$15,000!!!!

**FORE! The Stasis are back!** In April, we introduced you to Robert and Annette Stasi, Natalie’s Way Foundation, and their annual golf tournament, and this time around we want to tell the tale of golfers, fans, and spectators alike.

The Vision of Children Foundation is grateful for the Stasi family and applauds their hard work as we continue our quest to help children have a clear view of the world around them. 😊

Robert and Annette of Staten Island, NY, hosted over 200 golf enthusiasts who spent the day on the links to raise money for OCA, OA and other eye disorders. As Sam Hardage presented Robert and Annette with our “Vision Heroes Award”, the couple was delighted to present VOC a check for \$15,000 from the day’s event. Robert commented on the moment, “Partnering with the Vision of Children brings us closer to finding a cure for visual impairment associated with albinism as well as all genetic vision disorders worldwide.”



# PAST EVENTS

## VOC CELEBRATES WITH SUPPORTERS

The breeze was warm and the sunset was casting a lovely glow as guests gathered in the courtyard at Cavaillon Restaurant for hors d'oeuvres, wine, and socializing before moving to the dining room for an intimate wine paired dinner hosted by Vivian and Sam Hardage.



Why all the fuss? The Vision of Children Foundation was honoring donors on the west coast for their long and steadfast support of the VOC's mission.

The evening featured a four-course French themed meal pleasingly paired with the latest Pinot Noir from the Emeritus Vineyards. Emeritus founder, Brice Cutrer Jones, made the

evening special with a formal wine tasting and an entertaining discussion of his life's work as one of the wine industry's most celebrated vintners.

Sam Hardage gave a very informative and moving update on the recent gene therapy trials and their immense success and the importance of increasing our efforts in the research arena to build upon this discovery.

The science has been proven and we now need to deliver this life changing therapy to the visually impaired community. 🙄



## ALL CHILDREN HAVE DIFFERENT EYES

The Vision of Children staff kept busy all summer long. In July they attended the NOAH (National Organization for Albinism and Hypopigmentation) conference in Las Vegas. As an exhibitor, VOC was able to interact with Family Network members and gather needed feedback from children who use our programs. We introduced our new social development book, "All Children Have Different Eyes." It was a huge hit! Children loved the colorful pages and parents appreciated the message. One mother reported how it was so hard to see her son being misunderstood at school and was eager to have him read "All Children Have Different Eyes," so he could

explore ways to explain that he learns in different ways than his "normally sighted" friends. The Vision of Children's goals, who we are, and what we do, have been conveyed to thousands of families, allowing us to gain valuable new relationships and to reach those who need our assistance. 🙄



## DAY AT THE RACES

Vision of Children volunteers, including high school students and employees from The Hardage Group, spent a day at the Del Mar Horse Races in San Diego to hand out ball caps and raise public awareness about our mission. Volunteers reported that people were eager to hear about recent medical and research breakthroughs in vision research. 🙄

## VOC IN D.C.

In April, The Vision of Children attended the American Association for Pediatric Ophthalmology and Strabismus meeting which focused on recent medical advances in the diagnosis, management, and treatment of conditions encountered in the practice of pediatric ophthalmology. Clinical research creates new, best possible treatment options for patients. The VOC acquired data on vision rehabilitation in children, information on pediatric neuro-ophthalmology, and statistics of in vivo studies of human extraocular muscle functions which will be posted on our informational website which can be accessed via [www.visionofchildren.org](http://www.visionofchildren.org)

The Vision of Children seeks relationships with doctors from all over the world and will provide them with information to share with their patients regarding our ready database of available resources to families and patients who need professional help.

The VOC provided informational packets to doctors' offices worldwide and continues to look for opportunities to expand our medical and research database! 🙄

