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#### Children's Low Vision Resource Center

By Appointment Only

Paula Korelitz  
Outreach Director

Rosemary Blaszkievicz  
Parent Advisor

4064C W. 13 Mile Road  
Royal Oak, Michigan 48073  
1-800-788-2020  
ropard@yahoo.com

**ROPARD is an association dedicated to eliminating the problems of low vision and blindness in children caused by premature birth and retinal diseases.**

**ROPARD has available for parents, family and professionals many items that are useful for the development of children with retinopathy of prematurity and low vision.**

**DVD I:** "Management of Retinopathy of Prematurity and Other Pediatric Related Diseases" \$30

**Booklet:** "Looking Ahead: A Parent's Guide to the Development of their Child with Retinopathy of Prematurity" \$10

**Brochures:** "A Parent's Guide to Their Premature Infant's Eyes" Pkg of 100--\$25. (available in English or Spanish)

#### Holiday Cards

Don't forget to order your Holiday Cards!

Package of 12 for \$15.

They are also available in Braille for \$22.

Custom imprinting upon request.

Order at [www.ROPARD.org](http://www.ROPARD.org); or call 1-800-788-2020.

#### Your Donation Can Make a Difference!!!

Show your concern for this cause by donating to:  
ROPARD, P.O. Box 250425, Franklin, Michigan 48025

Name \_\_\_\_\_

Address \_\_\_\_\_

City \_\_\_\_\_ State \_\_\_\_\_ Zip \_\_\_\_\_

**For further information, please call  
1-800-788-2020**

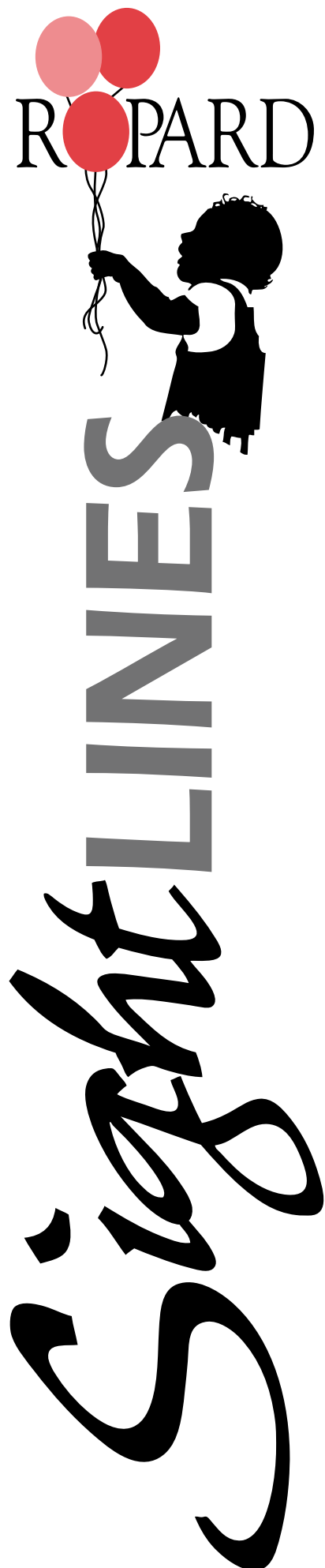
Your tax-deductible contribution will be used to support the highest levels of clinically relevant research.



**Susan Campbell,**  
Editor

**Paula Korelitz,**  
Website Administrator

Should you have any questions regarding this issue, please contact ROPARD at 1-800-788-2020 or visit our website at <http://www.ropard.org> or e-mail us at [ropard@yahoo.com](mailto:ropard@yahoo.com)



### Gene Therapy

Gene therapy first received a lot of press in the late 1980s and early 90s when viral gene transfer was being investigated. Some early successes were followed by very disappointing results. The premature media-hype added to the disappointment and set gene therapy back a few years. Thankfully, a few dedicated scientists and clinicians quietly persisted in their endeavor for successful gene transfer of DNA as a therapy. We now have a successful clinical trial treating patients with an inherited disease of blindness (Leber's Congenital Amaurosis - LCA) with gene replacement.

The basic description of gene therapy is the stable transfer of genetic material into a host cell. Although it sounds simple it is a daunting task. While some diseases are perfect targets for gene therapy most are not. There are three questions that must be addressed before designing a gene therapy.

1. Is the disease causing gene known? Surprisingly, most diseases are not caused by a single gene defect which limits the diseases that can be targeted. For instance, Retinitis Pigmentosa has over 9 genes that may be involved, and a single gene may have multiple mutations. The most successful gene therapy will address a disease that has the most patients affected by a single genetic mutation, such as Congenital X-Linked Retinoschisis (the RS1 gene).
2. Is it possible to package the DNA encoding the gene therapy? There are a number of ways to introduce the genetic material of interest to the host, both viral and non-viral. The viral vectors have been utilized the most because of their

natural affinity for host cell invasion and their ability to integrate the DNA for long-term therapy. It is possible to package the genetic therapy into a viral shell and then use that virus' own mechanisms for entering a cell. The non-viral options use either synthetic capsules for transfer or cell-based systems. Neither of these options, however, allow for long-term stable transfer of a gene.

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### SAVE THE DATE:

#### The Children's Vision Award and Visions of the Future

On May 8, 2010 ROPARD will honor Dr. Jean Bennett and Dr. Albert Maguire with the Children's Vision Award. Drs. Bennett and Maguire have used gene therapy to reverse near blindness. The award ceremony and dinner will take place at the Rivera Court in The Detroit Institute of Arts, Detroit, Michigan.



## Gene Therapy Continued

3. Are the host cells dividing or non-dividing? This is important in selecting the method of gene transfer. Most viruses infect actively dividing cells and only a few are able to enter a non-dividing cell. Central neurons, such as those found in the retina, are non-dividing cells which limits the choice of viral vectors available. The viral vector must also be tolerated by the host cell and not be seen as an infection by the body's immune system.

All of these factors make gene therapy a difficult task. The gene therapy for LCA uses a recombinant adeno-associated virus that encapsulates the *RPE65* gene. The viral vector creates a minimal immune response, is able to infect non-dividing cells, and transfers the gene to the cell nucleus for processing. The successful treatment of LCA with gene transfer technology was carried out by Dr. Jean Bennett and Dr. Albert Maguire who are the recipients of the 2010 Children's Vision Award.



In June, Lois Smith, MD, PhD spoke on *ROP* in 2009 at the ROPARD Lecture Series.

## Parent Information Seminar

The annual meeting of Michigan Association for the Education and Rehabilitation of the Blind and Visually Impaired (MAER) will be held in Livonia, MI on April 29 & 30, 2010. A special session will be arranged for parents. Drs. J. Baker, A. Capone, P. Droste, E. O'Malley and M. Trese, will be there to lead the discussion. For location and time please go to [www.ropard.com](http://www.ropard.com).

## [www.ropard.com](http://www.ropard.com)

A new version of the ROPARD website was recently launched. This version, designed to be more user-friendly, provides an easy format for providing comments to ROPARD including suggestions about the website.

Some of the new features include a more thorough internal search engine and a *News Flash* section where up-to-date news and information is placed on the home page. In an effort to be more responsive to visitors, we will be launching a section of Frequently Asked Questions (FAQs). Parents are urged to provide us with questions they have had difficulty in finding answers for when their child was first diagnosed with retinopathy of prematurity and during and after medical or surgical intervention.

The old email list has been disbanded and visitors to our new site are encouraged to sign up for our current email list so that they can stay informed. We encourage you to let us know if you think there should be other additions to the website because we are trying to make ROPARD's website truly beneficial to parents all over the world.

## Events Around The World

ROPARD is supported by volunteers who raise funds to prevent blindness in children. This year, golf tournaments were held in Ohio, by the Tiffany Rose Foundation, and Texas, hosted by the Skipper family. "Volley for Vision", a fund-raising volleyball tournament set up by the Bruno family in Pittsburgh, expanded to include two games.

In the Philippines, the Parent Advocates for Visually Impaired Children (PAVIC) continues its work in support of the needs of visually handicapped children and has expanded its mandate to include a transitional program for multiply-handicapped children.

In India, the ROP tele-medicine program continues to expand, to identify and treat more infants. The use of the Apple iPhone to allow easy access to the uploaded images from remote areas is being studied. At the world ROP meeting, November 21-23, in New Delhi, several projects funded by ROPARD will be discussed.

## Layla Bodnar

By Jon Andrews, *Bayside Leader*, March 5, 2009, used with permission.

"Everyone is special in their own way," is the philosophy of a remarkable girl. Layla Bodnar is the five year old daughter of Konji and Max Bodnar of Melbourne, Australia. Layla was born with FEVR (Familial Exudative Vitreoretinopathy). She can only see objects that are close-up, making her legally blind and being classified as disabled. However, her disability has not slowed her zest for life nor her fearlessness in enjoying new experiences.

At first, it was difficult for her parents to be reconciled with her condition. "The early years were quite tough for us as we didn't know what was ahead," Konji said. "As parents, we were learning as well, just like Layla was." The family was obviously ready for the challenges presented to them. Like any other family, Max, Konji, Layla and her younger brother Vaughn do what every other family does—enjoy their time together. Ms. Bodnar said ROPARD and Vision Australia had been superb in offering help, advice, guidance and tireless devotion to Layla and her family.



Although it may take Layla longer to learn, she has a strong desire to succeed. She swims, does gymnastics, goes camping and loves plane rides. Currently Layla attends kindergarten and she loves it. She is very popular with all the girls (it is a girls school). She enjoys reading and drawing, but her real love is music and dancing. Next month she will be dancing as a lion in a performance at the local theater. Next year, she is looking forward to attending a coed primary school.

"She takes on challenges just like every other child," Konji said. "We include her in everything; everyone does and she lives a rich and full life. She doesn't see her life as any different—why should we?"

## Dr. Jean Bennett and Dr. Albert Maguire

Dr. Jean Bennett, MD, PhD, is Professor of Molecular Ophthalmology at the University of Pennsylvania. Dr. Bennett's research effort focuses on developing somatic gene delivery for treatment of ocular diseases.

Dr. Albert Maguire, MD, is associate professor at Scheie Eye Institute, Department of Ophthalmology, University of Pennsylvania where he is a member of the Retina Service. He is an attending physician at multiple institutions including Children's Hospital of Philadelphia where Dr. Maguire is a pediatric eye surgeon.

Since early in their careers, Dr. Maguire and Dr. Bennett, who are married, have worked together to develop the foundation for many ocular gene therapy studies at the University of Pennsylvania. They have recently collaborated in a study to replace a retinal gene that is defective in Leber's Congenital Amaurosis which can cause blindness.



3 Dr. Jean Bennett and Dr. Albert Maguire