Chicago Alliance to Fund Retinal Research

SIGHT QUEST NEWSLETTER

Autumn, 2016 A publication of SEARCH FOR VISION
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GOLF CLASSIC 2016 SUMMARY

A record-breaking year has had at the Parent Petroleum 19th Annual Golf Classic
Fueling Research to Cure Blindness

The 19th Annual Parent Petroleum Golf Classic produced a record-breaking turnout leading to Parent's largest fundraising event to date! Held at the Historic St. Andrews Golf & Country Club in West Chicago, IL, the thirty-six hole course with contoured greens provided an inspiring setting for a great day of golf. Guests were encouraged to visit the Lagunita's Beer Tent to sample a variety of locally crafted beer. The afternoon was capped off with a delightful dinner reception, which included raffle drawings, a silent

auction and the presentation of awards. This year's Celebrity guest's former Bears defensive end Ed O'Bradovich, and the 1985 Bears Super bowl defensive tackle Steve McMichael hosted a fun-filled hour of Bears talk. The all-day event raised funds to help support Dr. Michael Grassi and his team of scientists at the Eye and Ear Infirmary of U.I.C.College of Medicine. A highly motivated organization that is dedicated to finding cures for blindness through scientific research. A special thanks goes to Ann Rasch, the Greater Chicago Community of Search for Vision, BP, Castrol, the Motor Werks of Barrington and all of our Parent Petroleum, suppliers, customers and friends who helped raise funds for this worthy cause. A complete list of Raffle Winners is attached - Congratulations! To learn more please visit www.searchforvision.org or http://chicago.medicine.uic.edu/departments/eye.

SEARCH FOR VISION & PARENT PETROLEUM PRESENTS A

$5,000.00 RAFFLE

$20.00 per ticket or 6 for $100

DRAWING TO BE HELD THURSDAY, AUGUST 25, 2016 AT THE PARENT PETROLEUM GOLF OUTING

THE WINNING RESULTS WILL BE LISTED BELOW:

		NAME	TICKET NO.
FIRST PRIZE -	$2,500.00	MOBIL TEL	0039
SECOND PRIZE -	$1,000.00	MOBIL TEL	0100
THIRD PRIZE -	$550.00	STEPHEN CHACKO	1696
FOURTH PRIZE -	$250.00	DOLORES UGALDE	1929
FIFTH PRIZE -	$250.00	JOSHY MATHEW	1355
SIXTH PRIZE -	$150.00	JAY NANTHIKATTU	1253
SEVENTH PRIZE -	$150.00	DREW RASCH	0400
EIGHTH PRIZE -	$150.00	ANDY NOVAK	1145

(Winners need not be present to claim prize)

Search For Vision and the Chicago Community are dedicated to raise awareness, funding of treatments and cures for blinding maladies with particular focus on retinal degenerative diseases.

OPHTHALMOLOGY SERVICES AT UNIVERSITY of Illinois COLLEGE OF MEDICINE CHICAGO

The Retina Service specializes in treating patients with both medical and retinal vascular and vitreoretinal disorders, such as: age-related macular degeneration, vein occlusion, sickle cell eye disease, ocular complications of diabetes, retinal detachment, vitreomacular adhesion, retinopathy of prematurity, retinitis pigmentosa, intraocular tumors and severe eye trauma. The doctors in this service are skilled specialists in laser and other medical treatment of the retina, vitreoretinal surgery and complex retinal detachment repair.

Report from the 19th Retina International World Congress Taipei July 2016

Claudette Medefindt, Deputy President of Retina International, gave a paper on the progress of the RDD project to find the causative gene mutation in South African retinal degenerative (RD(families. We have 287 families where a full result has been delivered and from these 197 families stand to benefit from current clinical trials. This progress was praised by Retina organizations and researchers and has opened the door to some exciting new collaborative pathways. The 2 day scientific program was very inspiring with the some of the best researchers in the world renewing our hope and resolve to support research efforts in every way.

Patricia Zilliox CEO of the Clinical Research Institute of the Foundation Fighting Blindness [USA] gave an excellent and comprehensive summary of therapies in pre-clinical or clinical trials. These included:

- 2 drug and 3 Gene therapy trials for the RPE65 form of Leber Congenital Amaurosis [LCA] and 1 drug trial for the LRAT form of LCA

- 3 drug, 2 gene therapy and 1 stem cell therapy for the ABCA4 form of Stargardt or Cone Rod Dystrophy

- 3 gene therapies for X linked Retinoschises

- 4 gene therapies for Achromotopsia

- 1 gene therapy for the PDE6b form of Retinitis Pigmentosa [RP] or punctuate albescens

- 3 gene therapies for the Rhodopsin form of Dominant RP

- 6 gene therapies for the RPGR form of X linked RP

 4 gene therapies for the RLBP1 form of Recessive RP

 1 growth factor, 1 Optogenetic, 3 stem cell and 1 drug therapy for RP which are not gene specific

 1 gene therapy for the Myosin 7a gene form of Usher Type 1

 2 gene therapies for Choroideremia

Of these 37 therapies under investigation 36 require a genetic diagnosis - either because the therapy is for a specific gene or that patients are being selected by a specific gene type.

Professor Elise Heon [Toronto, Canada] opened the Scientific program by giving an excellent overview of Retinal Degeneration. Her presentation made the immensely complex subject totally understandable. The growing importance of the cone viability factor that the rod photoreceptors produce, was highlighted in her presentation and in many others. The maintenance of central, cone vision, will mean prolonged useful vision for patients with RP, irrespective of the many gene mutations responsible for photoreceptor cell death. Professor Heon reminded us that the natural history of vision loss in RD is critical and too many patients neglect to see their ophthalmologists on a regular basis. Throughout the congress the speakers reminded us that identifying the underlying causative genes and in particular the missing genes remain an urgent priority. It was gratifying to know that the path we have chosen- that of putting all of our resources into finding the gene mutations for RD unique to our diverse population, was the right one.

Professor Eberhart Zrenner gave a comprehensive overview of the various artificial retinal implants. These silicon- based implants restore some visual function to RD patients with severe vision loss. There are 3 main categories - epi-retinal, sub retinal and supra –choroidal denoting exactly where the implants are placed in the retina. The most successful systems are the Argus 2 from Second Sight [USA], the Alpha IMS from Retina AG [Germany] and the Pixium System [Paris]. All of the systems use the intact neural pathway to transport the visual signal to the visual cortex in the brain. The difficulty of assessing the effectiveness of an intervention also received attention at the congress. In RD, the degeneration is often slow and clinical trial endpoints such as Visual Acuity or loss of field do not always provide useful measures. Other methods, including patient relevant outcomes are receiving attention. One of the more promising endpoints may be a mobility based assessment. A maze with variable paths, obstacles and light levels can quickly assess how much functional vision patients actually have. The importance of the Lutein group of supplements was emphasized in an excellent paper by Professor Bernstein from the University of Utah. We in South Africa are fortunate to have a moderately priced supplement Retina Plus that has all the right carotenoids and anti- oxidants that showed a

definite slower rate of degeneration in animal models. It is also important to have a diet rich in these and other carotenoids and vitamins. Leafy green vegetables are rich in the Lutein group of carotenoids. [The recommended diet of 200 gm of veggies and 2 fruits daily plus 2 portions of oily fish weekly is what we should all aspire to- ED] Dr Bernstein reiterated that some risk factors for AMD were unmodifiable - Age, race and genetics, but some were modifiable- Smoking, Cardio vascular disease, lipid status and hypertension, alcohol consumption, light exposure and nutrition. He also emphasized the important role of supplements in AMD. He recommended a maximum of 10mg Lutein and 2mg Zeaxanthin daily.

Dr Francesca Testa from Naples, Italy gave a good overview of gene therapy - From experimental to Clinical Trials. He reminded us that over 200 genes had already been linked to RD and most of these expressed in the photoreceptors and to a lesser degree in the pigment epithelium. He also emphasized that the identification of the disease genes has paved the way to gene based therapies. He gave a concise and simple explanation on how gene therapy is done and how transgenic animals are produced. He explained the role of vectors in gene therapy and how gene transfer worked.

Dr Juliana Salum from Brazil, plus various other speakers gave us interesting updates on AMD treatments. We were reminded that prior to 1980 there were no treatments for AMD and that the anti VEGF treatments now available do, in many cases save the vision of Wet AMD patients.
The work of many outstanding researchers was discussed including the pioneering work of:

 Professor Masayo Takahashi who in 2014 was the first researcher to use iPSC [induced pluripotent stem cells] to grow Pigment Epithelial cells for AMD therapy.

 Dr Robert Lanza, who made the cover of Time magazine as 2014's most influential person, using hESC derived Pigment Epithelial cells for AMD.
This pioneering work served as the springboard for many other researchers and 5 clinical trials for AMD using both types of stem cells are now in progress.

Other interesting papers given were:

 Visual Restoration by Optogenetic Therapy by Dr Serge Picaud (France), the genetic transfer of light sensitive molecules to the retinal neural layer of the RCS rat.

 RD Cure: CNGA3-Achromatopsia trial by Prof Eberhart Zrenner, University of Tübingen (Germany) - Achromotopsia is also known as Day- blindness, the symptoms are total photophobia, low visual acuity, no colour vision and nystagmus. In the patients treated there is a good safety profile and some early signs of efficacy, but more data is needed to assess consistency.

 Various papers on stem cells covered this exciting and evolving research. Some therapies are already in clinical trials and others will soon follow. One of the most important aspects of stem cells is being able to grow the disease in a dish. To be able to study various forms of RD in cellular models will mean greater understanding of the mechanism of the specific disease which will enable researchers to look at intervention strategies.

Professor Gerald Chader gave an elegant summary of the congress and as always reduced all the complex data into a digestible take home message. His presentation -Moving RD treatments from the laboratory bench to the patient bedside, is always a highlight of RI congresses. Professor Chader covered Cell transplantation, Artificial vision, Optogenetics, neuroprotection, antioxidants and gene therapy. He elucidated the state of the research and also what the immediate future might hold. In closing he had this important message for us. “Progress has been made. At the recent meeting of the RI Scientific & Medical Advisory Board in Seattle WA, a significant milestone was reached. At past meetings, we usually had about 15 short reports on basic RD research and progress towards clinical trials. This year, all talks reported on clinical trials with no time for news on basic studies. This is tremendous progress, pointing to upcoming treatments for all retinal degenerative diseases – Professor Jerry Chader – Secretary RI SMAB

OTHER SCIENCE NEWS

Stargardt/ AMD drug intervention Alkeus [USA] has launched a Multi-center phase II clinical trial for the drug ALK-001, for Stargardt Disease where the basis of the disease is a buildup of a toxic form of Vitamin A. The drug targets this waste-management problem. The ALK-001 replaces hydrogen atoms in Vitamin A with deuterium and this “burns cleaner” than the natural form. Deuterium is a safe, naturally occurring, non-radioactive form of hydrogen which is present in the human body. “The Phase II study will hopefully tell us more about the drug’s potential for slowing vision loss in people with Stargardt disease,” says Dr. Scholl. “If it works, we may have an opportunity to try it for other macular conditions, including dry age-related macular degeneration.” See more at: http://www.blindness.org/blog/index.php/arvo-2016-emerging-drug-targets-toxic-build-up-in-stargardt-disease/#more-4840

GENE EDITING

Researchers at Cedars-Sinai Medical Center in Los Angeles have used the new and revolutionary CRISPR/Cas9 gene editing approach to prevent vision loss in a rodent model of autosomal dominant retinitis pigmentosa (adRP). They have “shut down” the S334ter-3 mutation in the gene rhodopsin (RHO), a common cause of adRP. The success of the study is a significant step forward in developing a gene-editing therapy for saving and restoring vision for people affected by adRP as well as other inherited retinal diseases. CRISPR/Cas9

corrects only the defect in the recipient's mutated gene. This technique repurposes the naturally occurring defense system that bacteria use to find and disable invading viruses. One major advantage of this gene editing is that it gets around the problem of delivering large genes linked to retinal diseases—CEP290 or USH2A, for example—that won't fit in the viral delivery systems designed to carry them into retinal cells. Also, CRISPR may be a simpler approach to treating diseases in which delivering a whole new gene is not necessary; simply shutting down or repairing the bad gene may be enough to save vision. Shaomei Wang, M.D., Ph.D., the principal investigator for the Cedars-Sinai study, is cautiously optimistic about the potential of this emerging approach to gene correction. "Additional research is needed to show the feasibility of delivering the treatment at different stages of disease and demonstrating long-term safety and efficacy," she says. "But with rapid development of this powerful technology, CRISPR/Cas9-based therapies will likely be used in clinical trials in the foreseeable future." The gene-editing company Editas is currently developing a therapy to correct mutations in the gene CEP290, which can cause retinitis pigmentosa and Leber Congenital Amaurosis.

OPTOGENETIC UPDATE

RetroSense reported this week that 2 more patients have received a low dose of the RST-01 - the gene therapy to confer light sensitivity to the ganglion cells which remain intact even as photoreceptor cells degenerate and die. They plan to inject more patients with a higher dose within the next month. The benefit of Photoswitch therapy is that it is not gene specific. The trial is being conducted at the Retina Foundation of the Southwest in the USA.

The above article was excerpted from:
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EDITOR: Claudette Medefindt

NOTICE

Anyone wishing to receive Sight Quest Newsletter can contact Jay or Lorraine Popek at popek@att.net or call 708-652-4614.