

SOLUTIONS TO DIFFICULT DISEASES WILL BE HERE SOON



The patients who linger in our minds the longest are those we cannot help. Despite the advances in our field, we still fail some of our most vulnerable patients. We can offer only limited relief to patients presenting with Leber congenital amaurosis, choroideremia, or other inherited retinal diseases (IRDs).

Our field has had its share of victories on this front. The work of Mark S. Humayun, MD, PhD, and his team to develop the Argus II Retinal Prosthesis (Second Sight) is a breakthrough in medical engineering that deserves recognition for its genius. (The White House agreed: It awarded Dr. Humayun the National Medal of Technology and Innovation in 2016.)

The Argus II was one of the first cracks in the dam—and, as a number of therapies for IRDs glide through the clinical trial phase, it looks like the dam is about to break.

The authors in this issue of *Retina Today* discuss pathologies that are rare and difficult to treat. Four of the six pieces in our cover series address IRDs; the other two take us to the OR and the clinic for updates on optic disc pit maculopathy and uveitis.

Daniel Chao, MD, PhD, kicks off the cover series with a crash course in IRDs with his article, “Inherited Retinal Diseases for the Retinal Subspecialist: Where to Start.” Dr. Chao’s introduction to the topic is a primer for physicians approaching gene therapy for the first time. In “Treating IRDs: Gene-Specific and Gene-Independent Approaches,” Alessandro Iannaccone, MD, MS, FARVO, explains from a strategic standpoint the framework that IRD researchers use. After reading these first two articles, you’ll have a capital of knowledge on which to draw as the conversation gets granular.

Ophthalmology has a unique distinction: It is the first medical field to use a gene therapy approved by the US FDA. Aaron Nagiel, MD, PhD, reflects on his use of voretigene neparvovec-rzyl (Luxturna, Spark Therapeutics) in “One Year in: Perspectives on Voretigene.” Dr. Nagiel is one of

approximately two dozen surgeons in the world cleared to administer this treatment. By sharing his center’s experience with voretigene, he offers readers a preview of the forthcoming 1-year postapproval data.

Thomas A. Ciulla, MD, MBA, in discussing the pipeline of treatments for Stargardt macular dystrophy, shows how researchers dare to be creative when treating IRDs. The number of avenues pursued—visual cycle modulation, complement inhibition, gene therapy, and subretinal transplantation of stem cell–derived retinal pigment epithelial cells—underscores how hard researchers are working to find a solution to treating a disease that affects 1 in 10,000 people.

Changing gears, Barbara Parolini, MD, discusses optic disc pit maculopathy. This challenging presentation sometimes flummoxes surgeons. Dr. Parolini offers a detailed history of work performed on optic disc pits and reminds surgeons of a simple and easily forgotten rule: Rely on your surgical knowledge and research to choose the course of action best suited to the patient when he or she enters the OR.

Steven Yeh, MD, closes out the cover series by sitting down with *RT* to discuss the state of uveitis in 2019. There are several treatment options for uveitis (unlike the IRDs discussed elsewhere in this issue), yet it continues to confound our field. Dr. Yeh distills data from the latest clinical trials into easy-to-understand nuggets that our readers will appreciate.

A final note before we depart: Half of the cover stories in this issue were born of lectures delivered at live meetings attended by *RT*’s editorial team. When you see our editors at an upcoming meeting, say hello. They’re friendly, and they want to know what you think of the publication. ■

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