

ZHENG-RONG LU, PhD

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IMPACT WISH:

“To preserve the vision of kids with Stargardt disease, easing the burden that blindness is to the individual, their family, and society.”

A RARE APPROACH TO A RARE DISEASE

FOCUS: *Developing a non-viral gene therapy for Stargardt disease, the most common form of inherited juvenile macular degeneration.*

As with most of the scientists profiled in these pages, the work for which Dr. Lu received his Gund-Harrington award didn't stem from a particular career goal. It is often a mix of driven scientific curiosity, specific areas of expertise, and happenstance. "I've always aimed to figure out better ways to help people suffering from all kinds of diseases, pure and simple," Dr. Lu says. "My work investigating novel drug delivery therapies, nanomedicine, biomaterials, etc. has led me to Stargardt disease."

Usually appearing in childhood or adolescence, almost everyone with Stargardt has extremely poor visual

acuity, in the range of 20/200 to 20/400, and it is not correctable with prescription eyeglasses or refractive surgery.


Gene therapy, where genetic material is introduced into cells, is a promising approach to diseases which, like Stargardt, are caused by a mutation in a single gene. However, the Stargardt gene is too large for current gene therapy techniques to correct the mutation.

"We have designed a non-viral system using novel lipid DNA nanoparticles," Dr. Lu says. "In a mouse model of Stargardt, the nanoparticles have demonstrated the safety and efficacy

necessary for delivering the large therapeutic gene to the retina, in turn slowing down the progression of the disease."

With help from the Harrington Discovery Institute, the goal is to optimize the nanoparticles for clinical translation, perform safety studies required by the FDA, eventually running clinical trials in patients.

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