

**KRISHANU SAHA, PhD**

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IMPACT WISH:

“We hope to improve vision for patients afflicted by inherited genetic disorders, with low potential for adverse events; and that this work will be a springboard to further discoveries in genomic medicine.”

THE EYES ARE THE

WINDOW TO BREAKTHROUGHS

FOCUS: *Developing gene-editing therapies that can help or cure a far greater number of people whose sight is afflicted by inherited genetic disorders.*

“The most exciting part of my work is to meet my former trainees and hear about their successes,” Dr. Saha says. “I’m always impressed by how much my trainees accomplish after they leave my lab.”

Considering the breakthrough work going on in that lab, no doubt his trainees depart with a sense that they can achieve most anything.

Dr. Saha’s work is aimed at genetic disorders that afflict the retinal pigment epithelium (RPE), the cell layer just outside the neurosensory retina. Its many functions include light absorption, supplying nutrients to photoreceptors, converting light to electrical signals, and removing pathogens and cell debris.

These disorders, such as some forms of retinitis pigmentosa, Best disease and congenital amaurosis, all greatly hinder eyesight, some leading to blindness.

The usual current therapy, the viral treatment of delivering lab-formed DNA molecules into the cells, has limitations, and some patients’ gene makeup isn’t suited for this approach.

Dr. Saha and his team are working on generating nanocarriers of gene-editing machinery that sidestep the safety issues of viral delivery. “Advancements have enabled precision gene-editing directly in patient tissues,” Dr. Saha says.

“But beyond helping disorders that affect the eyes, our approach could set the foundation for a new paradigm in genomic medicine, expanding the types of tissues that could be edited, and hence the spectrum of disease where genomic medicine could have an impact.”

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