A team of researchers, led by Bo Chen, PhD, Associate Professor of Ophthalmology, Icahn School of Medicine at Mount Sinai, has reversed congenital blindness in mice by changing the supportive Müller glia cells in the retina into functional rod photoreceptors—light-sensitive cells in the retina that signal the brain when activated.

The breakthrough research, published online in the August 15, 2018, issue of *Nature*, is expected to advance efforts toward retinal regeneration for diseases of the eye, such as age-related macular degeneration, degenerative glaucoma, and retinitis pigmentosa.

Funded in part by the National Eye Institute (NEI), an arm of the National Institutes of Health, the study drew praise from NEI Program Director Thomas N. Greenwell, PhD. “This is the first report of scientists reprogramming Müller glia to become functional rod photoreceptors in the mammalian retina,” says Dr. Greenwell. “Rods allow us to see in low light, but they may also help preserve cone photoreceptors, which are important for color vision and high visual acuity. Cones tend to die in later-stage eye diseases. If rods can be regenerated from inside the eye, this might be a strategy for treating diseases of the eye that affect photoreceptors.”

Scientists have long studied the regenerative potential of Müller glia cells because in species such as zebrafish, they divide in response to injury and can turn into photoreceptors and other retinal neurons. In the lab, scientists have coaxed mammalian Müller glia to behave as they do in the fish, but not without injuring the tissue. Since injured

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Lab Coat Ceremony Kicks Off PhD Training

The Graduate School of Biomedical Sciences at the Icahn School of Medicine at Mount Sinai recently held its inaugural PhD Lab Coat Ceremony—becoming the only institution in New York City, and one of the few in the country, to honor its matriculating class of PhD students in this fashion. The event, symbolizing the start of their graduate training, recognized 55 new students enrolled in Mount Sinai's PhD programs in Biomedical Sciences, Neuroscience, and the MD/PhD Medical Scientist Training Program.

The ceremony, held Monday, September 17, in Goldwurm Auditorium, was a jubilant event for the students who received their lab coats, as well as for their families and friends, and Icahn School of Medicine at Mount Sinai leadership and faculty.

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The Graduate School separately presented honorary plaques to 40 PhD and MD/PhD students who have officially joined a lab and confirmed their PhD candidature by passing their thesis proposal exams.

“We value the innovation and creativity that drive transformative scientific discoveries at Mount Sinai, but we also love the symbolism of the white lab coat, which embodies scientific rigor, objectivity, and professionalism,” said Marta Filizola, PhD, Dean of the Graduate School of Biomedical Sciences, the Sharon and Frederick Klingenstein/Nathan Kase, MD Professor, and Professor of Pharmacological Sciences, and Neuroscience.

During the ceremony, the students recited an oath for doctoral candidates—a set of guiding principles—written by Matthew O’Connell, PhD, Senior Associate Dean for Curriculum, Recruitment, and Admissions. “I willingly pledge to uphold the highest levels of integrity, professionalism, scholarship, and honor,” they each read, as they affirmed to conduct their research and professional endeavors with honesty and objectivity.

Dennis S. Charney, MD, Anne and Joel Ehrenkranz Dean, Icahn School of Medicine at Mount Sinai, and President for Academic Affairs, Mount Sinai Health System, welcomed the incoming students and spotlighted their strong academic credentials and their significant research experience.

Eric J. Nestler, MD, PhD, Dean for Academic and Scientific Affairs, reminded guests: “Although science today is under attack from extremists of many political stripes, science still represents promise for the future: economic advancement, improved health, and better lives. So, as we put on that lab coat—figuratively or literally—we accept several obligations to carry out research that is rigorous, reproducible, robust, and responsible.” Dr. Nestler is also Director of The Friedman Brain Institute and Nash Family Professor of Neuroscience.

World-renowned microbiologist Peter Palese, PhD, Chair of the Department of Microbiology and the Horace W. Goldsmith Professor of Medicine, introduced keynote speakers and discussed the importance of scientific discovery and collaboration.

Mount Sinai Receives Award from VNSNY CHOICE

VNSNY CHOICE SelectHealth, a New York State Department of Health Special Needs Plan for Medicaid-eligible New Yorkers living with HIV, recently awarded the Mount Sinai Health System $360,000 for its efforts at successfully improving the overall health status of its members. Mount Sinai’s Institute for Advanced Medicine, which directs all of the Mount Sinai Health System’s HIV prevention and treatment programs, serves more than 1,100 HIV-positive VNSNY CHOICE SelectHealth members annually. The award will be used to support Mount Sinai’s quality initiatives that serve this population.

“VNSNY CHOICE SelectHealth shares with our Mount Sinai provider partners the conviction that HIV infection is now a readily treatable condition from which no New Yorker needs to suffer or die,” says Jay Dobkin, MD, Medical Director for VNSNY CHOICE SelectHealth. “We are gratified that our efforts in support of the Mount Sinai program have been so successful, and hope to build even more effective collaborations in the future.”
We value the innovation and creativity that drive transformative scientific discoveries at Mount Sinai, but we also love the symbolism of the white lab coat, which embodies scientific rigor, objectivity, and professionalism.

— Marta Filizola, PhD

speaker Vincent Racaniello, PhD, Higgins Professor of Microbiology and Immunology at Columbia University’s Vagelos College of Physicians and Surgeons. Dr. Racaniello, a noted virologist who focuses on the molecular biology of picornavirus replication and pathogenesis, earned his doctorate from Mount Sinai in 1980 and was the first PhD graduate mentored by Dr. Palese.

"Forty-three years ago this month, I was in the same place as you are now," Dr. Racaniello said, addressing the students. "Looking back, I now realize I was completely clueless, but four decades in science have taught me many important lessons. The one worth telling you is that science is not about you: It is not about building a big lab, scoring many research grants, publishing papers in prominent journals, or even winning a Nobel Prize. It is about discovery."

Lab Discovery Leads to a Remedy

A drug that recently received approval from the U.S. Food and Drug Administration (FDA) for the treatment of pain associated with the gynecological disorder endometriosis had its genesis two decades ago in the laboratory of Stuart Sealfon, MD, at the Icahn School of Medicine at Mount Sinai.

The drug, Orilissa™, approved by the FDA in July, is the first oral regimen that specifically helps to ease the moderate to severe pain that accompanies endometriosis, a condition where the tissue that forms in the lining of the uterus continues to grow outside the uterus. The disorder, which affects roughly one in ten women of reproductive age, negatively impacts quality of life, since the excess tissue growth is often accompanied by pain during menstruation, intercourse, or urination.

“Orilissa is a drug that resulted from the basic research we conducted at Mount Sinai, and it will help millions of women,” says Dr. Sealfon, Sarah B. and Seth M. Glickenhaus Professor and Chair Emeritus of the Department of Neurology. “At Mount Sinai, we discovered how to clone the drug target that was needed to develop this new drug.”

Indeed, as a young researcher more than two decades ago, Dr. Sealfon led the Mount Sinai team that cloned the gonadotropin-releasing hormone receptor (GnRHR) and genetically engineered host cells that express GnRHR. Gonadotropin-releasing hormone (GnRH), which is secreted by the hypothalamus, plays a key role in controlling reproduction, and acts via its receptor GnRHR.

The cloning procedure and primary structure of the receptor were described in two studies authored by Dr. Sealfon in 1992 and 1995, which were published in Molecular Endocrinology and Molecular and Cellular Endocrinology, respectively. The research provided a better understanding of the complex interplay of hypothalamic, pituitary, and gonadal hormones, which underlie pharmacotherapy and the reproductive system. At the time, Dr. Sealfon says, a career-development grant provided him with the funding he needed to conduct his research. Two U.S. patents, in 1998 and 1999, assigned these inventions to Mount Sinai.

The oral application of Orilissa—also known by its generic name, elagolix—enables women to dial down the reproductive system. The dose-dependent drug suppresses the luteinizing hormone and the follicle-stimulating hormone, which leads to decreased blood concentrations of estradiol and progesterone. This reduces the growth of excess tissue, or lesions that form on the ovaries, fallopian tubes, or areas near the uterus, including the bowel and bladder that characterize endometriosis and cause pain.

The 20 years it took for elagolix to move from Dr. Sealfon’s laboratory to the marketplace demonstrates the length of time it can take for basic scientific discoveries to bear fruit, experts say. The drug was released by AbbVie, a global pharmaceutical company, in cooperation with Neurocrine Biosciences, Inc.

Endometriosis is considered one of the most common gynecologic disorders in the United States, but women can sometimes go years before having the laparoscopic procedure needed to render a proper diagnosis. In addition to the use of oral contraceptives, treatments have included nonsurgical anti-inflammatory drugs, and opioids. In more extensive cases, women may undergo surgical procedures, including a hysterectomy.

In two Phase 3 clinical trials, Orilissa has been shown to be helpful in the treatment of uterine fibroids, as well. Fibroids are a common benign tumor that causes bleeding or pain in millions of women, and for which there are, currently, limited nonsurgical treatment options.

In the years since his initial discovery, Dr. Sealfon’s lab has continued to study GnRH receptor-mediated gonadotropin regulation and help guide future work in the field.

Matthew Baney, Senior Director of the Institute for Advanced Medicine, says, “We have been successful in starting and maintaining our patients on medications to keep them healthier. All of our sites have initiated outreach efforts and care coordination activities directed at finding patients who fall out of care and getting them back into treatment. We also have a fair amount of patients in the Mount Sinai Health Home Program, which is a free service that integrates and coordinates health care for people on Medicaid—and that has a significant impact on suppression rates.”
Immunotherapy has been a game changer in treating some cancers, but it does not work for every patient. Building mathematical models that might predict a patient’s response is central to the work of Benjamin D. Greenbaum, PhD, Assistant Professor of Medicine (Hematology and Medical Oncology), Oncological Sciences, and Pathology, at The Tisch Cancer Institute at the Icahn School of Medicine at Mount Sinai. For his efforts, he recently won the Pershing Square Sohn Prize for Young Investigators in Cancer Research.

Dr. Greenbaum, a computational biologist, was among six researchers to receive the award from the nonprofit Pershing Square Sohn Alliance for Cancer Research. His laboratory will receive $200,000 yearly for the next three years. He was also recognized by The Mark Foundation for Cancer Research, which will provide an additional $50,000 a year.

Dr. Greenbaum’s work “will be instrumental in understanding what types of T cells are required for generating effective anti-tumor immunity and how to design immune therapies that selectively induce their development,” says a longtime colleague and mentor, Nina Bhardwaj, MD, PhD, the Ward-Coleman Chair in Cancer Research, and Director of Cancer Immunotherapy, at The Tisch Cancer Institute at the Icahn School of Medicine at Mount Sinai.

Dr. Greenbaum began his career studying the evolution of viruses and later became interested in checkpoint blockade inhibitor immunotherapies, which help the body recognize and kill cancer cells, and for which the Nobel Prize was recently awarded. He led a group that created the first predictive mathematical model demonstrating how a set of melanoma and lung cancer patients would respond to certain immunotherapies, a finding described in November 2017 in the journal *Nature*. To further improve such models, “we work with clinicians, oncologists, immunologists, geneticists, and others to try to round out the full picture of how the immune system interacts with cancer,” Dr. Greenbaum says. “This is a very vibrant time in cancer immunotherapy.”

Mount Sinai Researcher Wins Young Investigator Award

Reprogramming Retina Cells to Restore Vision (continued from page 1)

When the researchers took another step forward by testing the treatment in congenitally blind mice that were born without functional rod photoreceptors, the results were positive once again. The light responses recorded from retinal ganglion cells—neurons that carry signals from photoreceptors to the brain—and measurements of brain activity confirmed that the newly formed rods were integrating into the visual pathway circuitry, from the retina to the primary visual cortex in the brain.

“Our findings underscore that we are closer than ever to developing new therapies for people with severe degenerative eye disease,” says Dr. Chen. “Mice that were blind from birth were now able to see light for the first time following treatment.” Dr. Chen says his next step will be determining whether the technique works on cultured human retinal tissue.

In addition to receiving NIH funding, Dr. Chen’s research was supported by a $2 million grant from the McGraw Family Foundation.
A Birthday Party for Amos and Professor Bunsen Honeydew

Two beloved members of Mount Sinai Kravis Children’s Hospital—sweet-natured golden doodles Amos and Professor Bunsen Honeydew—were treated to a Hawaiian-themed birthday party in August. Patients, families, and staff gathered at the hospital to show their love and support for the dogs, while enjoying crafts activities, eating cupcakes, and signing a celebratory banner in honor of Amos’ and Professor’s third birthdays. The dogs are related, but were not part of the same litter. The excitement of the party appeared to tire out the four-legged guests of honor—they both fell asleep one hour into the festivities, with Hawaiian music playing in the background.

Under the direction of skilled clinical handlers in the Child Life and Creative Arts Therapy Department, Professor works with patients in the Blau Center for Children’s Cancer and Blood Disease and the Alice Gottesman Bayer Pediatric Intensive Care Unit. Amos comforts pediatric inpatients and is a popular attendee at Kravis’ coffee hour, a weekly offering for the parents and families of patients.

The dogs are full-time employees of Mount Sinai Kravis Children’s Hospital through the Paws & Play facility dog program. The program—supported by PetSmart Charities® and the Mount Sinai Auxiliary Board—is the first of its kind in New York State.

Pediatrician Honored as a “Literacy Champion”

Leora Mogilner, MD, Associate Professor of Pediatrics, Icahn School of Medicine at Mount Sinai, recently received a Literacy Champion award from Reach Out and Read of Greater New York, part of a national nonprofit group that promotes early literacy. Dr. Mogilner is medical director of the regional chapter and was instrumental in its founding 20 years ago. She accepted the award at a benefit gala from Chelsea Clinton, who was representing the Clinton Global Initiative. “The idea is that you teach parents about the importance of reading to children from the time they are babies,” Dr. Mogilner says. “In my practice at Mount Sinai Pediatric Associates, we give out brand new books, and we also have volunteers in the waiting room who read to kids and model reading aloud for parents.” Her dream for the future: “I would love to see this at every clinic and every practice in New York City.”

Celebrating the Completion of Treatment

Patients who undergo radiation therapy for cancer may have treatment that can last multiple weeks and include up to five sessions per week—a physically and emotionally trying experience. So, when patients finally do complete treatment, it is certainly worthy of a celebration, determined the staff in the Department of Radiation Oncology at Mount Sinai West—which delivered more than 11,000 radiation therapy treatments in 2017. Now, as patients exit the treatment area after their final session, they can jubilantly ring a ceremonial bell, a celebration that also includes the radiation oncologist, nurses, radiation therapists, and supportive team members who cared for them. Patients also receive an Achievement of Excellence certificate signed by their care team. “It is often an emotional moment and a feeling of immense relief,” says Natosha Houston, Technical Supervisor, Radiation Oncology. “It is also rewarding for our staff to celebrate this significant milestone with patients as they move onto their next step in treatment or return to their daily routine.”

Patient Viveca White rang a ceremonial bell to signify she completed her treatment.
7th Annual SINA Innovations
Theme: Innovation in Science and Medicine

Sessions and keynote speakers include:

**Cell and Gene Engineering**
Charles Murry, MD, PhD, Chair, Institute for Stem Cell and Regenerative Medicine, University of Washington
Katherine A. High, MD, President, Spark Therapeutics

**The Arc of Innovation, From Concept to Cure**
Raymond Schinazi, PhD, Director, Center for AIDS Research, Emory University School of Medicine
Tom Maniatis, PhD, Scientific Director and Chief Executive Officer, New York Genome Center

**Data-Driven Innovation That Is Transforming Medicine**
Eric Dishman, Director, All of Us Research Program, National Institutes of Health
Sri Madabushi, PhD, Business Development Director, Google AI Healthcare

**Caring for the Community Through the Lifespan**
David Blumenthal, MD, President, The Commonwealth Fund
Kelly J. Kelleher, MD, Distinguished Professor of Pediatrics, Psychiatry, and Public Health, Nationwide Children’s Hospital

Tuesday, October 23 – Wednesday, October 24
Annenberg, Stern Auditorium

Email: Sinainnovations@mssm.edu
http://icahn.mssm.edu/about/sinainnovations
Registration is free, but required.

3rd Annual Mount Sinai Health Hackathon
Creating Novel Technology Solutions for Health Care
Theme: Rare Diseases

A 48-hour fun and high-energy, team-based, multidisciplinary competition focused on creating novel technology solutions for problems in health care. Three finalist teams will be awarded $2,500.

Friday, October 19 – Sunday, October 21
Hess Center for Science and Medicine
Davis Conference Center

Registration is $10.

For more information, email health-hackathon@mssm.edu.

Disability Awareness Fairs

In recognition of Disability Awareness Month in October, the Office for Diversity and Inclusion will host two informational fairs designed to connect employees and the community to organizations and resources that aid in the care of people with disabilities. All staff, faculty, and students are welcome to attend.

**Wednesday, October 17**
11 am - 2 pm
Mount Sinai West
Main Lobby

**Thursday, October 25**
11 am - 3 pm
The Mount Sinai Hospital
Guggenheim Pavilion Atrium

For more information, e-mail the Office for Diversity and Inclusion at diversity@mountsinai.org.

12th Annual Cardiology Nurse Practitioner Symposium

Mount Sinai Heart presents a one-day program designed to enhance knowledge about the prevention, intervention, and management of cardiovascular diseases. Presentations will include “Advanced Heart Failure,” “Establishing Genetic Links in Coronary Artery Disease,” and “Left Ventricular Assist Device as Therapy for Complex Valve Disease.”

To learn more about the program, fees, accreditation, and registration, go to mountsinaiheartnp.org.

Friday, November 9
7 am - 5:45 pm
Stern Auditorium

Mount Sinai
Transformation Update

For the most recent updates on Mount Sinai’s downtown transformation, please go to:
http://www.mountsinai.org/locations/downtown

The Mount Sinai Health System complies with applicable Federal civil rights laws and does not discriminate, exclude, or treat people differently on the basis of race, color, national origin, age, religion, disability, sex, sexual orientation, gender identity, or gender expression.