

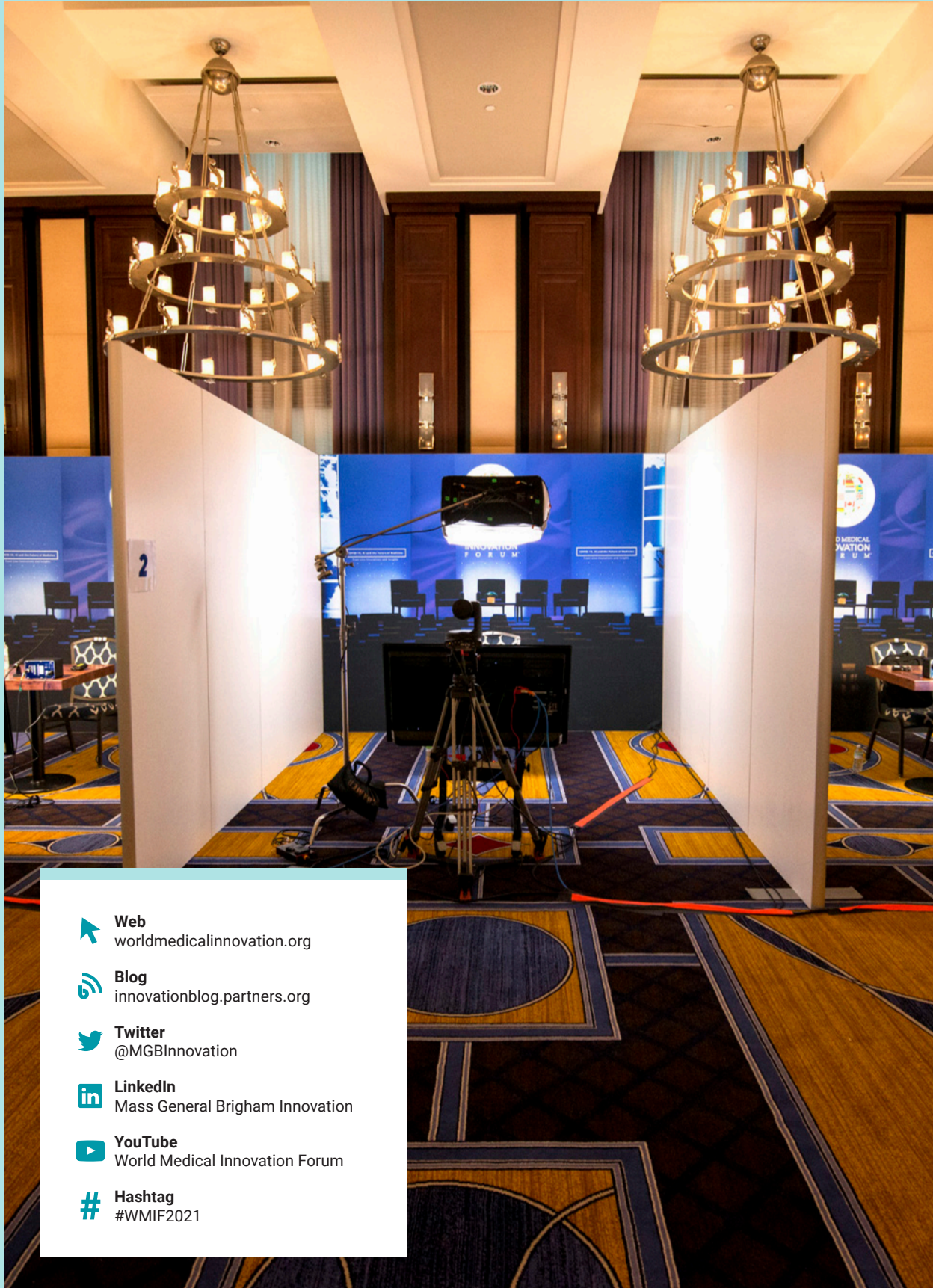


WORLD MEDICAL INNOVATION FORUM

gene and cell therapy

May 19–21, 2021

worldmedicalinnovation.org



Contents

Affiliations

BWH | Brigham and Women's Hospital
MGH | Massachusetts General Hospital
MEE | Massachusetts Eye and Ear
HMS | Harvard Medical School

6	Welcome
11	Steering Committee
14	Sponsors
16	Gene and cell therapy at a crossroads
24	Mass General Brigham Innovation
26	Innovation Growth Board
28	Commercialization Council
45	Planning Committee
47	2022 World Medical Innovation Forum



Reimagining treatment of genetic diseases

Novartis Gene Therapies is reimagining medicine to transform the lives of people living with rare genetic diseases. Utilizing cutting-edge technology, we are working to turn promising gene therapies into proven treatments.

Novartis Gene Therapies is proud to sponsor the 2021 World Medical Innovation Forum.





Welcome

Welcome to the seventh annual World Medical Innovation Forum.

This year we gather to discuss the extraordinary opportunities in gene and cell therapy and the profound impact they will have on medicine. In more than thirty sessions over the course of three days, Mass General Brigham's Harvard faculty, industry experts and leading investors and entrepreneurs will explore the technologies and challenges of gene and cell therapy as we continue to bring life-changing breakthroughs to our patients and millions around the world.

The World Medical Innovation Forum was established to reaffirm the importance of collaborative innovation—academia, industry and government working together to create solutions for medicine's great challenges. Our goal is to provide actionable insights for Forum participants. We are grateful to the nearly 200 senior executives, investors, Harvard clinicians and investigators who will share their perspectives as speakers.

We welcome thousands of audience participants from nearly every state and dozens of countries around the globe, and we are grateful to our many sponsors representing some of the most innovative companies in health care. Our Steering Committee and Planning Team has devoted countless hours and made outstanding contributions to this year's program. We recognize our Co-chairs Nino Chiocca, MD, PhD, Neurosurgeon-in-Chief and Chairman, Neurosurgery, Brigham and Women's Hospital; Harvey W. Cushing Professor of Neurosurgery, Sue Slaughaupt, PhD, Scientific Director, Mass General Research Institute; Professor, Neurology, Ravi Thadhani, MD, CAO, Mass General Brigham; Professor, Medicine and Faculty Dean, and Luk Vandenberghe, PhD, Grousbeck Family Chair, Gene Therapy, Massachusetts Eye and Ear; Associate Professor, Ophthalmology for all their contributions.

We hope that you will join us next year in-person on May 2-4, 2022 when we reconvene to continue exploring the transformations in gene and cell therapy.

Thank you for joining us for this year's event.



Anne Klibanski, MD
President and CEO, Mass
General Brigham;
Laurie Carrol Guthart
Professor of Medicine,
HMS



Christopher Coburn
Chief Innovation Officer,
Mass General Brigham





Thank you for joining us. This year's Forum features the executives, investors, clinicians, entrepreneurs and decision makers who will transform the application of gene and cell therapies to the clinic. Our enduring goal remains to provide an environment where principals can directly share their insights and priorities. This is an exciting time.

The Forum is brought to you by Innovation, the global business development arm of Mass General Brigham. Its mission is the commercial application of the breakthroughs and unique capabilities of the system's 6200 Harvard faculty —bringing benefits to patients worldwide and generating new resources to further the nation's largest academic research enterprise in bringing breakthroughs to patients. Our work continues as we help to collaboratively shape the care of the future.

We express our deep appreciation to the many individuals who made this Forum possible and are particularly grateful to our speakers for sharing their passion, expertise and unique perspectives. Generous support by our Presenting sponsor Novartis, Stakeholder sponsors Bayer and GE Healthcare, Strategic sponsors Astellas, Biogen Fujifilm, and Collaborators Amplifybio, Boston Scientific, Canon, Catalent, InterSystems, NLVC, One Medical, Recon Strategy, Siemens Healthineers, ThermoFisher and Vertex— underpins - this event.

Many thanks to the Steering Committee members whose insights made the Forum possible and the Planning Team's dedicated work.

Enjoy the Forum and we look forward to seeing you in person May 4-6, 2022 in Boston!



Nino Chiocca, MD, PhD

Neurosurgeon-in-Chief and Chairman, Neurosurgery, BH; Harvey W. Cushing Professor of Neurosurgery, HMS (Co-Chair)



Susan Slaughaupt, PhD

Scientific Director, Mass General Research Institute, MGH; Professor, Neurology, HMS (Co-Chair)



Ravi Thadhani, MD

CAO, Mass General Brigham; Professor of Medicine and Faculty Dean for Academic Programs, HMS (Co-Chair)



Luk Vandenberghe, PhD

Grousbeck Family Chair in Gene Therapy, Massachusetts Eye and Ear; Associate Professor, Ophthalmology, HMS (Co-Chair)



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The background of the advertisement features a dark, atmospheric photograph of three mountaineers standing on a rocky mountain peak, silhouetted against a twilight sky. They are holding hands in a gesture of triumph. In the bottom left corner, there is a large, out-of-focus, glowing molecular model with blue and green spheres. The text is overlaid on this image.

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Many thanks to the members of the Steering Committee for their leadership in shaping the Forum agenda, identifying speakers and securing sponsors.



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Susan Slaughaupt, PhD

Scientific Director and Elizabeth G. Riley and Daniel E. Smith Jr., Endowed Chair, Mass General Research Institute; Professor, Neurology, HMS (Co-Chair)



Ravi Thadhani, MD

CAO, Mass General Brigham; Professor, Medicine and Faculty Dean, HMS (Co-Chair)



Luk Vandenberghe, PhD

Grousbeck Family Chair, Gene Therapy, Massachusetts Eye and Ear; Associate Professor, Ophthalmology, HMS (Co-Chair)



Xandra Breakefield, PhD

Geneticist, MGH; Professor, Neurology, HMS



Reid Huber, PhD

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Chief, Diabetes Section and Director, Diabetes Program, BWH; Lecturer on Medicine, HMS



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Chairman, Department of Neurosurgery, MGH; William and Elizabeth Sweet Professor of Neurosurgery, HMS



Ole Isacson, MD, PhD

Director, Neuroregeneration Research Institute, McLean; Professor, Neurology and Neuroscience, HMS



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Co-Director Pediatric Stroke and Cerebrovascular Program, MGH; Assistant Professor of Neurology, HMS



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Marcela Maus, MD, PhD

Director, Cellular Immunotherapy, Cancer Center, MGH; Associate Professor of Medicine, HMS



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www.novartis.com/our-company/novartis-pharmaceuticals/novartis-gene-therapies

A photograph of a large, ornate event space, likely a ballroom or auditorium, set up for a formal event. The room features high ceilings with several large, multi-tiered chandeliers. The stage area is decorated with a blue backdrop and white partitions. Professional lighting equipment, including spotlights and tripods, is visible on the stage. The floor is covered with a patterned carpet in shades of blue, yellow, and red.

The 2021 World Medical Innovation Forum was made possible through the sponsorship of some of health care's most important companies.

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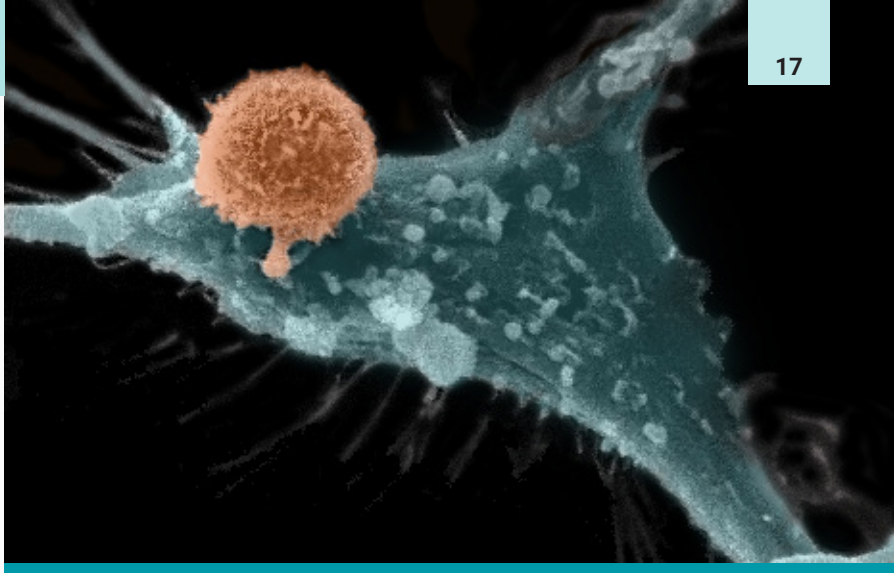
Gene and cell therapy at a crossroads

After glimpsing the promise of gene and cell therapy, Mass General Brigham researchers are now enhancing the technology to drive new treatments for rare and common diseases

Replacing defective genes or cells with healthy ones has been a goal of modern medicine for decades. And while that aspiration may have seemed simple in principle, the practical path toward realization has been longer and more complex than ever imagined. But now, the initial wave of gene and cell therapies has reached the clinic, with remarkable health benefits for a subset of patients that affirms the potential of these precision medicines for more widespread applications. With scores of gene and cell therapies now under development, the field stands at a major crossroads.

At Mass General Brigham, our faculty are at the forefront of this extraordinary revolution. As part of a world-leading healthcare system that spans the full spectrum of the biomedical research enterprise and works collaboratively with industry to fuel innovation, Mass General Brigham scientists and physicians are working to bring the next generation of gene and cell therapies to the clinic. We highlight a few of their stories here, ranging from rare, genetic diseases to more common conditions that lack effective treatments.

Bryan Choi, MD,
PhD, Mark Leick,
MD, and Marcela
Maus, MD, PhD



Driven to solve devastating rare diseases

Susan Slaughaupt, PhD, the Scientific Director of the Mass General Research Institute, first began working on a rare genetic disease called mucopolidosis IV (ML4) in the late 1990's as a junior faculty member at MGH. At the time, very little was known about the condition, which results in early motor and cognitive delays, followed by loss of sight, and eventually, death, often in the third or fourth decade of life.

In 2000, Slaughaupt and her colleagues made a transformative discovery: they identified the culprit gene. That gave them a critical handle on the molecular roots of the disease, one of about 40 so-called lysosomal disorders that together represent the most common cause of neurodegenerative disease in children. Still, there remained many questions about the biology of ML4 and if — and how — a gene therapy strategy should be crafted.

"This is a very slowly progressing neurological disease, which, from a research perspective makes it a very difficult nut to crack," said Slaughaupt, who is also a professor of neurology at HMS. "We're committed to cracking it because of our deep relationship with the ML4 patients and their families."

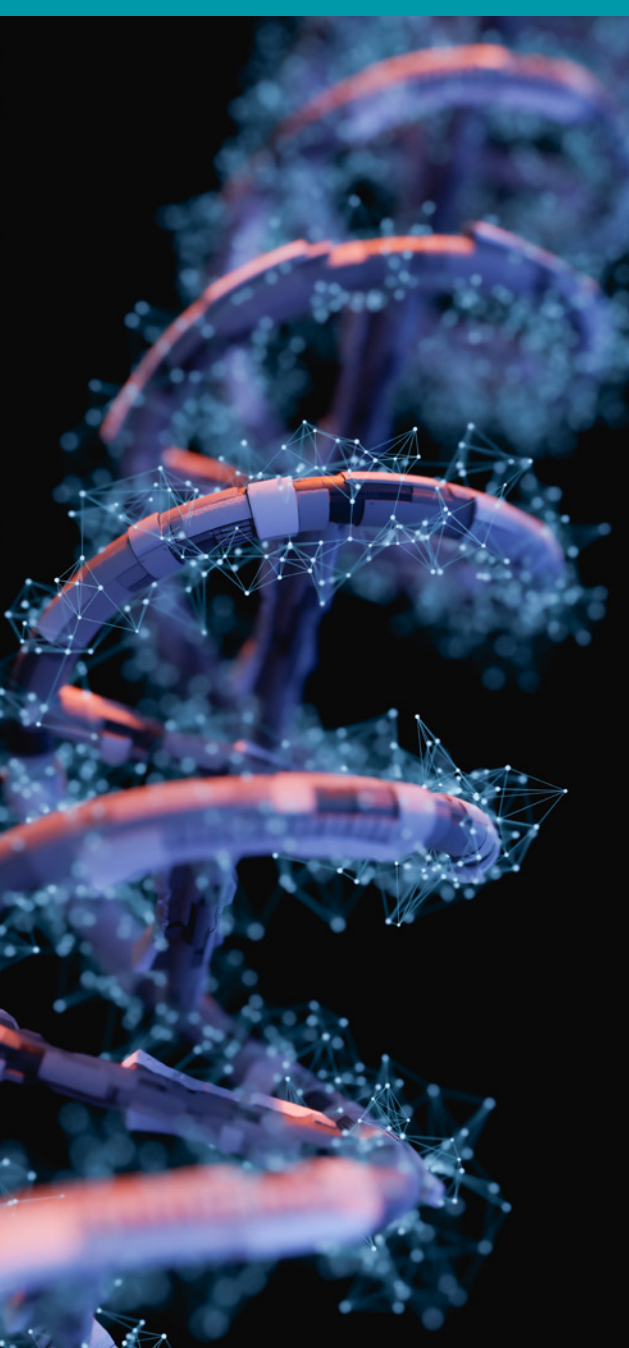
Yulia Grishchuk, PhD, an assistant professor of neurology at MGH, who trained as a postdoctoral fellow in Slaughaupt's lab, is now carrying the project forward in her own lab. Her focus: to not only unlock the biology of the disease but also fix it.

In 2016, she began developing a gene therapy approach for treating ML4. Now, after several years of rigorous pre-clinical studies, Grishchuk and her colleagues are looking for an industry partner to help carve a path toward clinical translation.

"Overall, working in the rare disease space has posed hurdles at every level: getting grants, publishing papers, and finding commercial partners to collaborate with us and help sustain the translational work," said Grishchuk. Despite these hurdles, she remains deeply focused on studying ML4 and getting an effective treatment to the patients who need it.

"I started working on ML4 because of its underlying biology and my own background in lysosomes and neurodegeneration," said Grishchuk. "But after getting to know the ML4 community and the patients who suffer from the disease, they are what drive me."

Jeannie Lee, MD, PhD, a molecular biologist at MGH and professor of genetics at Harvard Medical School, is studying another devastating rare disease, known as Rett syndrome. She and her colleagues are pioneering a novel form of epigenetic therapy for the condition, which is a rare, X-linked disorder that affects roughly 1 in 10,000 girls. Rett syndrome is particularly devastating because the girls appear normal for the first year or two of life, and then begin to deteriorate rapidly, losing language and motor skills, and developing behavioral and cognitive difficulties. Eventually, they require constant, lifelong care. Unfortunately, there are no treatments for these patients.



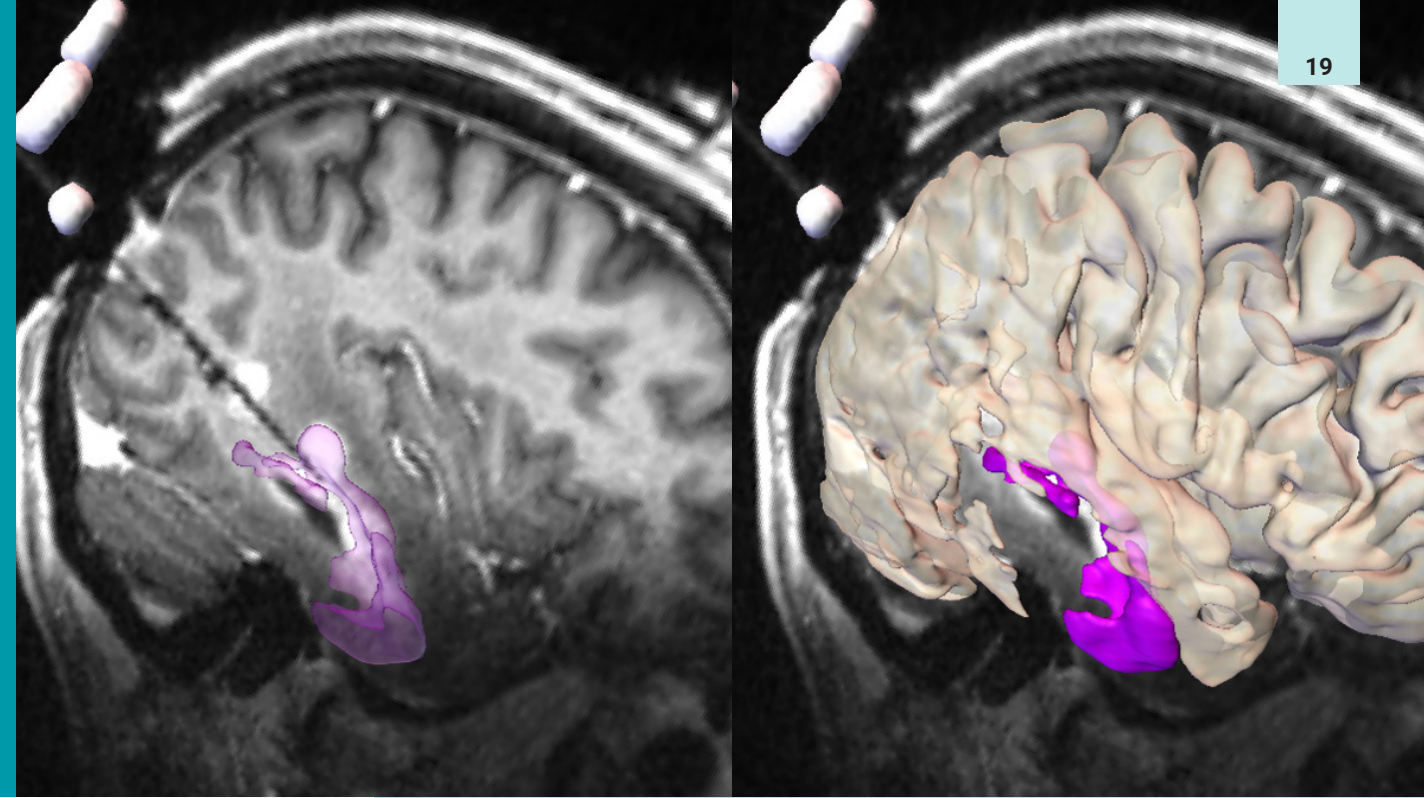
The gene responsible for Rett syndrome is called *Mecp2*, and affected girls carry one defective copy and one healthy copy. Ordinarily, that healthy copy might suffice, but since it sits on the X-chromosome, it is rendered inactive about half the time. That's because females randomly inactivate one of their X chromosomes in cells throughout the body as a way to cope with the double dose of genes from the two X chromosomes — males, by comparison, have just one X chromosome. So, in females with Rett syndrome, roughly 50 percent of their cells allow the healthy copy of *Mecp2* to remain active, while the other 50 percent choose the damaged copy — an unlucky role of the dice that sets the disease in motion.

"*Mecp2* is used within the cells that make it, so while some cells have a fully functioning gene, their activity can't help other cells in which the healthy copy of *Mecp2* is inactive," explained Lee.

She has studied X-inactivation for 25 years and her laboratory discovered many of the core molecular components. Lee and her colleagues realized that the answer to treating Rett lies in the bodies of the affected girls. "In the cells that are sick, there is a normal copy of *Mecp2*, it's just locked up on the inactive X chromosome," said Lee. "But what if we could reactivate it?"

That reversal requires a novel kind of gene-based therapy, known as an epigenetic therapy, which relies on two key elements: an antisense oligonucleotide that targets one of the essential factors within the X-inactivation machinery and silences it, and an inhibitor of an epigenetic process known as DNA methylation. Together, these factors can help target *Mecp2* and turn it back on. "What we've learned is that it doesn't take 100 percent of *Mecp2* function to have an impact, based on various mouse studies, so even just a little bit of reactivation might make a big difference for Rett patients."

Lee and her colleagues have spent several years developing and vetting their approach in animal models and are now looking for an industry partner to embark on clinical translation. They are also working to adapt their approach for other X-linked disorders. "We're pressing ahead," said Lee. "We know we can get this therapeutic cocktail to work in human cells. Now the question is can we get it to work in Rett patients."



Nino Chiocca, MD, PhD

Toward gene therapies for hearing loss

Luk Vandenberghe, PhD, the Grousbeck Family Chair of Gene Therapy at Massachusetts Eye and Ear and an Associate Professor of Ophthalmology at HMS, has built his career studying one of the molecular workhorses of gene therapy, the adeno-associated virus (AAV). Although this viral vector is the predominant delivery agent for the handful of gene therapies now used in the clinic, its biology is not deeply understood.

"It really is a black box therapeutic," said Vandenberghe. "We don't yet know why different AAVs do what they do."

To close these knowledge gaps, his lab has been systematically comparing various AAVs found in nature and designing new ones from first principles using computational tools. Several years ago, the researchers discovered that one of these *in silico* AAVs infected an unexpected group of cells: the sensory cells in the inner ear, called hair cells. These cells carry out highly specialized functions in the ear that are essential for normal hearing. When hair cells malfunction or degenerate, the result is hearing loss and eventually, total deafness.

The unusual affinity of the Vandenberghe's computer-generated virus, known as Anc80, meant that the researchers could uniquely target hair cells with gene therapies. "Hearing is one of our major forms of communication with the outside world," said Vandenberghe. "Unfortunately, its loss can be very debilitating and isolating."

While there have been some recent breakthroughs in treating hearing loss, such as cochlear implants, "these seem fairly rudimentary for 21st century biomedicine," he said.

Now, Vandenberghe and his colleagues are applying their viral discoveries toward the development of gene therapies for hearing loss. They are beginning with rare genetic forms of hearing loss, such as those due to mutations in a gene called *otoferlin* as well as other genes. The team hopes to begin clinical trials early next year.

Vandenberghe believes the environment at MGB helps propel his work. "We often underestimate the complementarity that exists within this healthcare system," he said. "MGB is arguably the largest organization of its kind and it really goes from soup to nuts — from basic research to clinical care. For scientists, it's like a playground, and the challenge is ours to figure out how to best use it to serve patients."

‘Moving the needle’ for glioblastoma

Glioblastoma is the most common type of brain cancer in adult patients. Unfortunately, its outlook is too often grim. Most patients die within 12 to 18 months of diagnosis.

“We really haven’t moved the needle for glioblastoma in the last 30 years,” said Khalid Shah, PhD, who is the Vice Chair of Neurosurgery and directs the Center for Stem Cell Therapeutics and Imaging at Brigham Health. “We’re still treating patients with chemo and radiation, and eventually, these tumors just come back — something has to change.”

Now something is changing. Various teams across MGB are harnessing the tools of gene and cell therapy to develop novel treatments for glioblastoma. For example, Shah and his colleagues are wielding cancer cells as weapons against themselves. Their work draws inspiration from a surprising discovery nearly two decades ago, which found that cancer cells that have spread to distant sites in the body can find their way back to the original tumor. Shah’s team has taken this re-homing concept and, with the power of CRISPR genome editing technologies to molecularly rewire patients own tumor cells, designed the cells to be cancer seekers and slayers. Now, Shah’s group has enhanced these cells, giving them dual cancer-killing and immunomodulatory properties, and are gearing up to test their experimental approach in a phase one clinical trial.

“What the glioblastoma field needs is a cell therapy that not only kills tumor cells, but also gives the body long-term immunity against the cancer so it doesn’t return,” said Shah, who is also a professor at HMS. “That’s what we are building.”

Marcela Maus, MD, PhD and her colleagues are also working on a novel cell therapy. Their focus: developing next-generation CAR-T technologies that can target solid tumors like glioblastoma. CAR-T cells, which Maus’ team helped pioneer, first entered the clinic in 2017. The cells are created using patients’ own immune cells and are genetically engineered in the laboratory to give them therapeutic powers. They’ve proven remarkably potent for some forms of difficult-to-treat blood cancers, enabling some patients to survive for years cancer-free. Now, Maus has enhanced the cells, tweaking them to target not just one, but two molecules on glioblastoma cells. Such bispecificity should make the CAR-T cells better able to destroy solid tumors. The team is preparing to launch clinical trials later this year.

“At Mass General Brigham, we’re incredibly lucky to have such amazing scientific and clinical talent right here,” said Maus, who is Director of Cellular Immunotherapy at the MGH Cancer Center and an associate professor of medicine at HMS. “We have all the talent we need to complete the therapeutic lifecycle — to sketch an idea on the whiteboard, test it in preclinical models, and then collaborate with colleagues in the hospital to bring it to patients.”

Another Mass General Brigham team is also working on a new gene and cell therapy strategy to target glioblastoma. Nino Chiocca, MD, PhD, Neurosurgeon-in-Chief and Chairman of Neurosurgery at BWH, is designing specialized cancer-killing viruses to kill these tumors. Such oncolytic viruses have proven to be quite effective against melanoma, and now Chiocca and his colleagues are adapting them to target glioblastoma. Part of the enthusiasm for this approach lies in the observation that oncolytic viruses help recruit certain immune cells into glioblastoma tumors, transforming them from immunologically cold to hot. This transformation is critical for enabling tumors to respond to cancer immunotherapies, like checkpoint inhibitors. Now, in addition to testing oncolytic viruses in clinical trials, Chiocca’s team will also explore potential combinations with immunotherapy drugs.

“As scientists, we do a lot of research in the lab, but it rarely reaches patients,” said Chiocca, who is also the Harvey W. Cushing Professor of Neurosurgery at HMS. “It’s very exciting to see these potential therapies that could really make a difference for glioblastoma.”

Rebuilding the brain in Parkinson's disease

Parkinson's disease affects some 10 million people worldwide. The disease emerges because of a shortage of dopamine, an important signaling molecule for neurons in the brain that helps control movement. The dopamine-producing neurons slowly degenerate and die, for reasons that are not entirely clear but likely involve a mix of genes and environmental factors. Although drugs can help restore dopamine levels, they often cause a range of side effects and are often not effective in patients with advanced disease. But what if it were possible to replace the lost dopamine-producing cells?

"That idea sounds simple in theory but it's actually quite complicated in practice," said Bob Carter, MD, PhD, Chair of the Department of Neurosurgery at Mass General and the William and Elizabeth Sweet Professor of Neurosurgery at HMS. For several years, he has collaborated with Kwang-Soo Kim, PhD, Director of the Molecular Neurobiology Laboratory at McLean Hospital and Professor of Psychiatry at HMS, and other colleagues at Mass General and Dana-Farber Cancer Institute to devise a potential cell replacement therapy for Parkinson's disease. Various other groups within the MGB system are pursuing similar efforts.

The concept developed by Carter's team involves taking a patch of skin cells from a Parkinson's patient, reprogramming them in the laboratory to become dopamine-producing neurons, and then surgically implanting them into the patient's brain. About four years ago, they tested their approach in a single Parkinson's patient, publishing the results of this "N of 1" study in the *New England Journal of Medicine* last May. Now, they are working to refine their method and preparing to launch a phase 1/2 clinical trial later this year or in early 2022.

"I've dreamed about gene and cell therapy since I was a postdoc," said Carter. "And now it's moving one step closer to the clinic."


He added, "I'm incredibly excited about the potential of this experimental therapy for restoring function to Parkinson's patients. And if the cell therapy platform we're developing proves successful, it could also spur the development of similar therapies for other neurodegenerative disorders." 



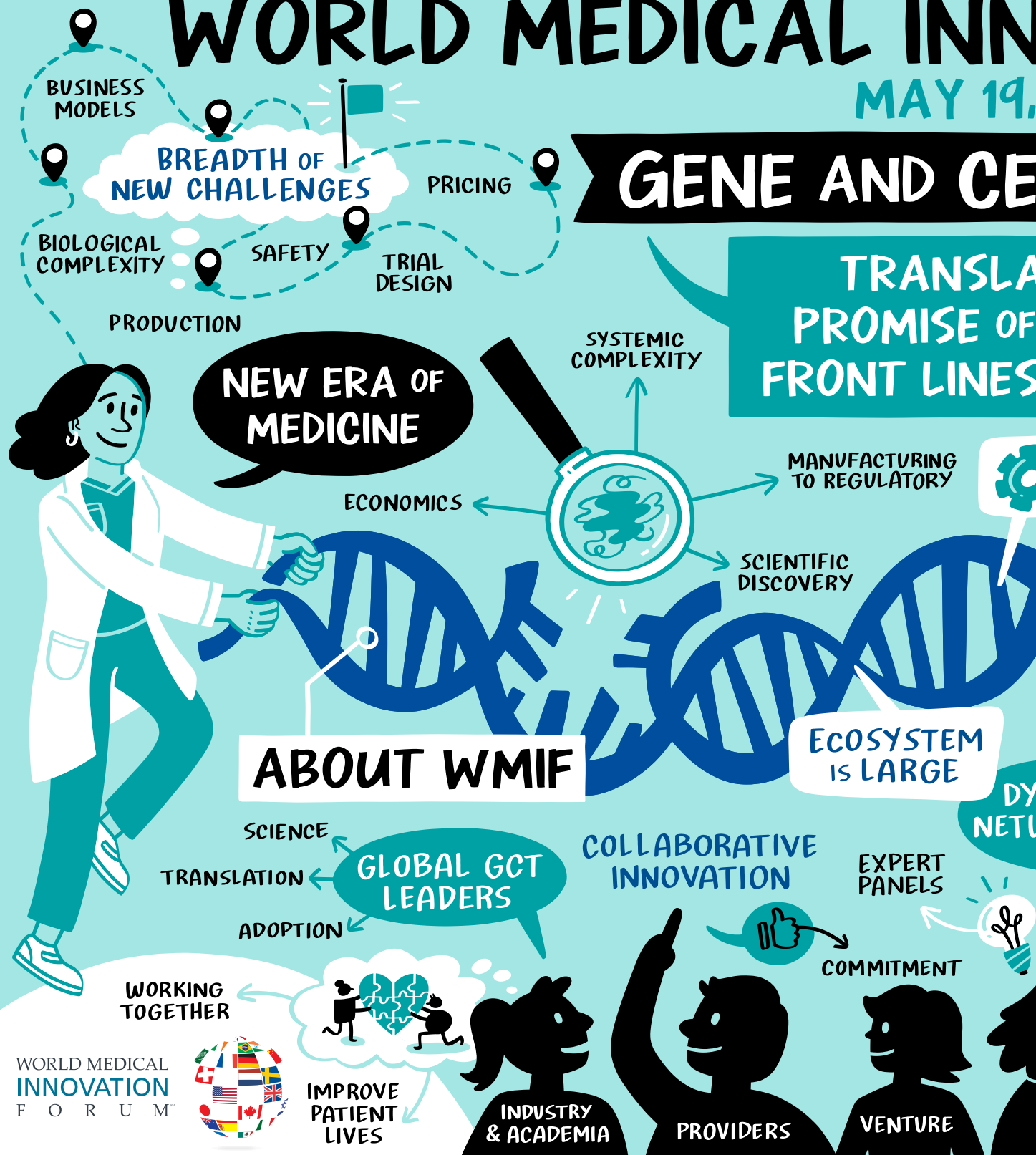
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The Innovation Fellows Program provides short-term, experiential career development opportunities for future leaders in health care focused on accelerating collaborative innovation between science and industry. It facilitates personnel exchanges between Harvard Medical School staff from Mass General Brigham hospitals and participating biopharmaceutical, device, venture capital, digital health, payor and consulting firms. A successful example of open innovation, Fellows and Hosts learn from each other as they collaborate on projects ranging from clinical development to digital health and artificial intelligence to new care delivery models and industry disruption.

We welcome interested Fellow candidates and potential host organizations to learn more at: innovation.massgeneralbrigham.org/about/special-programs/innovation-fellows-program

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Omid Farokhzad, MD

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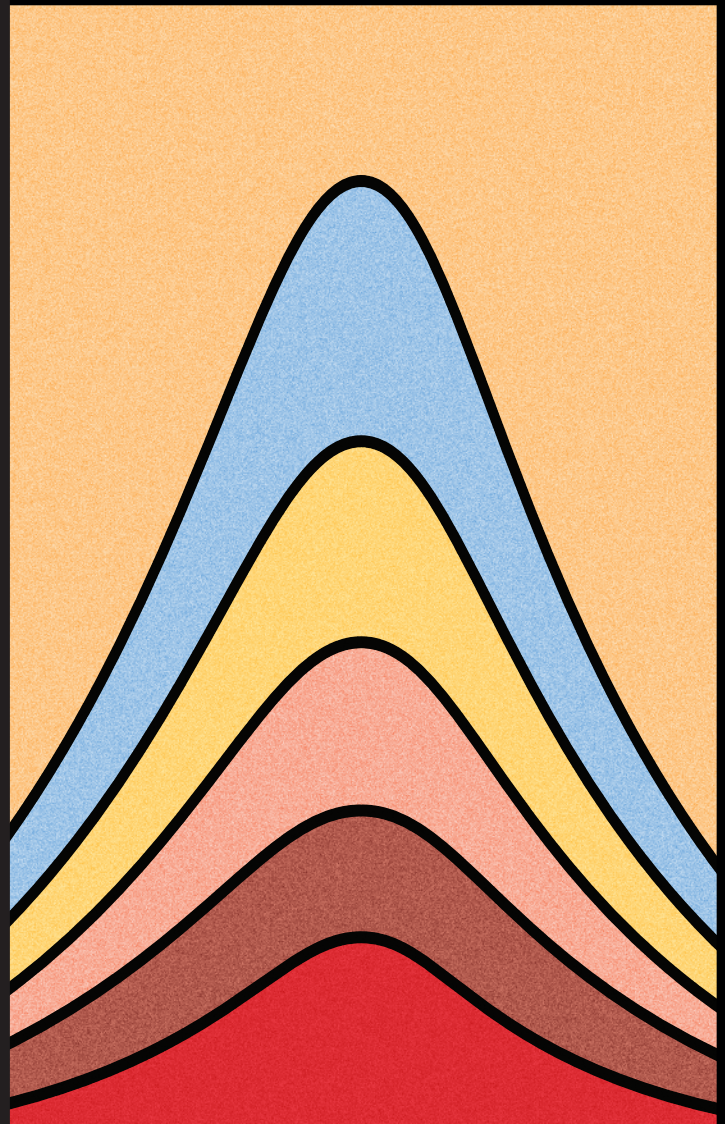
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A special thanks to Innovation's Planning Committee and Event Team for their unstinting commitment over the last 18 months to create the 2021 World Medical Innovation Forum.

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