Impact
Translational Medicine Stories

Novartis
Institutes for Biomedical Research
Tackling the world’s biggest health challenges

At Novartis we are reimagining healthcare; discovering new ways to improve and extend people’s lives with innovative medicines and devices.

Our focus is on doing great science to address areas of unmet medical need.

We approach things in a different way to others. It’s what enables us to make discoveries that take the practice of medicine in new directions.

We look to tomorrow to inspire us today. Never satisfied with the status quo, we anticipate what’s next.

We are passionate about what we do and the impact that we have on patients and societies.

Bridging science and medicine

“There’s always a chance we might uncover a whole new indication.”

Florencia

“As a scientist, I’ve always been driven by acquiring new knowledge and making discoveries that take the science forward. And as a doctor, of course I want to make a difference for patients. Translational research ticks both boxes. I get to bring promising compounds into the clinic for the first time, and then take what we learn back to our basic scientists. There’s always a chance we might uncover a whole new indication, which makes it an exciting space to work.

“When you think about the diseases we’re trying to solve here, they’re very complex problems. That degree of challenge demands that we work as a team and make the most of every single area of expertise.

“Every day, I have hugely enriching conversations with very bright scientists from across disease areas. They open up my thinking in unexpected ways so I can shape a compound’s development path and ultimately help to develop drugs that change people’s lives.”

• Translational Medicine Expert
• Joined NIBR: 2014
• Former jobs: Physician Scientist at Brigham and Women’s Hospital and The Ragon Institute of MGH, MIT and Harvard
The NIBR approach

The Novartis Institutes for BioMedical Research (NIBR) is the company's innovation engine. We have created a way of working that combines the limitless exploration you see in academic research with the entrepreneurial spirit of a biotech and the broad expertise and resources of large pharma.

Pathways to new medicines

Our scientists are given the freedom to be bold in pursuit of breakthroughs; investing long-term focus on scientific understanding of disease mechanisms and then identifying drug targets with high potential. Our approach is to map the complex protein signalling networks inside cells known as molecular pathways. When anything goes awry in a pathway, it can cause disease. Our goal is to develop drugs that will restore normal pathway function and treat disease.

Innovation through collaboration

Collaboration is an essential tool for us as we seek to find answers to many of the most intractable questions in patient health. Through collaboration we share access to the collective knowledge and enthusiasm of thousands of experts. Today, we have more than 100 biotech alliances and 300 academic collaborations.

Within NIBR, collaboration takes place across a network of more than 6,000 scientists and physicians at seven research centers around the world.

• Cambridge, US (NIBR headquarters)
• Basel, Switzerland (Novartis global headquarters)
• East Hanover, US
• Emeryville, US
• La Jolla, US
• Shanghai, China
• City of Singapore, Singapore

Each project is driven by multi-disciplinary teams of pathway scientists, disease area specialists, physicians, bioinformaticians, chemists and more.

How are we doing?

NIBR operates one of the most innovative and productive drug pipelines in the industry. We focus our research on autoimmunity, transplantation and inflammation, cardiovascular and metabolic diseases, infectious diseases, musculoskeletal, neuroscience, oncology, ophthalmology, and respiratory diseases.

We have more than 200 potential new products and indications in development.
The discovery of a treatment for a rare disease can be a platform for a new medicine that may benefit millions

...
Directing research to expand the reach of a niche drug has given Eric the chance of making a difference to millions worldwide.

As a Translational Medicine Expert (TME), Eric has designed and led Proof-of-Concept (PoC) studies to examine the potential for canakinumab, an antibody used since 2009 to treat rare auto-inflammatory syndromes, as a therapy for far more widespread diseases.

On joining NIBR, Eric took over a major project investigating whether canakinumab could help patients with an abdominal aortic aneurysm. If an aneurysm bursts, it is usually fatal. Today, there is no drug to shrink aneurysms. For patients with a large aneurysm, the only effective treatment options are an intra-arterial stent or surgery.

It’s a disorder with particular resonance for Eric. “In my clinic, I had dozens of patients with aortic aneurysms,” he recounts. “I was frustrated I couldn’t do more for them.”

Two members of Eric’s family also had the condition. One opted for surgery and died on the operating table; the other turned down the surgical option but later died when her aneurysm burst.

Eric set up the PoC trial to test the effectiveness of canakinumab in this patient population. He explains: “If canakinumab can reduce inflammation in a way that stops the aneurysm growing or even shrinks it, this could delay or potentially eliminate surgery.”

Eric has also led research into canakinumab’s potential to reduce arterial inflammation in heart attack patients. “I confess I was skeptical about the hypothesis linking inflammation with atherosclerosis,” he recalls. “But what I saw in our initial study got me very excited.”

Canakinumab is now the subject of a major randomized clinical trial to find out whether the drug lowers the risk of recurrent heart attack and stroke. If so, it could prolong millions of lives. “It’s a great example of how we can use a therapy for a rare disease to pave the way for new treatments with much broader benefits,” says Eric.

“I have the chance to benefit many more people than I could ever treat in a lifetime in the clinic.”

Eric’s path to TME

Eric started out in basic science with a PhD in biological chemistry. A move into medicine followed and Eric completed his medical training before taking up an internal medicine residency. He chose to specialize in cardiology and, in 2001, set up his own lab and clinic, where he led a team that treated heart defects and investigated their genetic causes. He spent more than a decade engaged in this work before being tempted by the offer of a role in Translational Medicine.
"I learn so much from my patients about their disease and what’s important to them."

Chinwe
Ronenn’s path to TME

After gaining his MD, Ronenn spent almost 20 years as a physician and academic researcher in internal medicine, rheumatology, clinical epidemiology, nutrition and immunology. In 2002, he switched to the private sector for stints with two prominent biotech firms. Then in 2009 came an unexpected call from NIBR. “No one in big pharma was doing anything serious in muscles at the time,” Ronenn recounts. “It was exhilarating to be given the chance to lead research in an area that had been neglected.”

On the brink of a breakthrough

“We knew we were onto something very special – potentially transformative.”

Ronenn

The darkest hour came just before the dawn for NIBR researchers investigating the potential of a promising new compound for treating a life-threatening muscle-wasting disease.

The drug bimagrumab was being tested in a small Proof-of-Concept (PoC) trial with 14 patients suffering from sporadic inclusion body myositis (sIBM). A rare and untreatable disorder, sIBM mostly strikes people in their 60s. It weakens their limbs to the point where they may be unable to walk or even feed themselves.

Bimagrumab had performed well in preclinical testing and human muscle samples. But at first glance, the data looked discouraging. Translational Medicine Expert (TME) Ronenn recalls: “We had been so hopeful about bimagrumab and delivered the PoC in record time. But the analysts were glum. They were looking for increased muscle strength and there just wasn’t a big enough signal.” It seemed that the project would fall by the wayside.

But, that wasn’t the end of the story. Ronenn continues: “The usual way neurologists test strength is by having patients push against the doctor. This can lead to different results depending not only on how strong the patient is, but also how strong the doctor is. When we took another look at the results for gait speed – how fast patients can walk four meters – we saw major gains in every one of our subjects.

Gait speed is a huge predictor of survival, and we knew we were onto something potentially transformative.”

The US Food and Drug Administration soon designated bimagrumab a ‘breakthrough therapy’ for sIBM. Since then, NIBR has begun research in larger groups of sIBM patients. Excitingly, trials have begun to explore bimagrumab’s potential for treating more common muscle-wasting conditions, including age-related sarcopenia and chronic obstructive pulmonary disease (COPD).

Says Ronenn: “The chance of making an impact on that scale is why I first left my clinic and my lab. But what I didn’t anticipate is how much fun it would be! In Translational Medicine, we have a free hand to go where the unmet need and biological tractability are. We can take risks to advance the science. It’s exciting.”

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Making a difference

“By the age of five, I’d made up my mind to be a doctor, motivated by a wish to help my grandmother. She was blind from birth and never had the chance to see her parents, children or grandchildren.

“But being a registrar wasn’t enough for me. I found it depressing to tell patients there was nothing we could do for so many diseases of the eye. There just weren’t the therapies out there.

“I then moved into basic research. Going deep into the science was a very rich learning experience. Even so, there wasn’t sufficient scope to translate what I was doing into therapeutic benefit. It all felt too far removed from patients.

“I love that we can bring compounds to patients and see results in one or two years.

“We’re interacting directly with clinics and patients and seeing data in real-time. We can evaluate treatments, gain new insights and accelerate promising compounds. It finally feels like I’m making a difference for patients.”

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“NIBR seemed the right place for me to build on my research. The last few years have been a crazy, wonderful time. I love that we can bring compounds to patients and see results in one or two years.

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When Parisa was a young girl in Iran, it made her sad to see how her blind grandmother struggled with day-to-day tasks. That early influence led Parisa to a career as an ophthalmologist in England.

Parisa
True collaboration sparks new thinking

“Being in a truly collaborative environment has given me new perspectives on the diseases I care about.”

Dan

“In academia, I was getting more and more frustrated with the silo mentality. At NIBR, I became part of a multidisciplinary, global network of experts who are passionate about the science and who share a healthy sense of urgency to get results for patients.

“Being in an open and collaborative environment has given me new perspectives on the diseases I care about and opened my eyes to different ways to attack a research or clinical problem. It means I’m learning from the people around me every day. And it gives us the best chance of making a difference for millions of patients who don’t have access to effective treatment options, which is what really counts.”

The best of both worlds

“When I came out of medical school, I had a very simple notion of medicine. It meant knowing your textbook, understanding your patients and treating diseases.

“When I got into research and clinical academia, that broadened my horizons because I could look into the mechanisms of complex diseases. I found that fascinating over many years.

“And then I got the chance to come to NIBR and do translational medicine. Initially, I had a very vague idea of what that meant, but I soon learned what an exciting field it is. It allows me to bridge the two worlds of research and clinical practice to help NIBR exploit our understanding of disease pathways and find new indications.

“The best of both worlds has made me a more complete physician. I still run a clinic for half a day every week, and now I can give patients far better information about their disease as well as current and potential treatment options.”

Stephan

“Working in the translational space has made me a more complete physician.”
Fighting disease on the front line

“We could be looking at game-changing drugs in the fight against drug-resistant malaria.”

Joel

When Joel climbed into the back of a truck at a remote clinic on the Thai-Burmese border and started his stopwatch, he was helping Novartis take a step closer to the next generation of malaria treatments.

A Translational Medicine Expert (TME) with the Novartis Institute for Tropical Diseases (NITD) in Singapore, Joel was visiting one of the proposed sites for studies of a potent new antimalarial drug candidate. As the truck jolted along the dirt road, he was timing the trip to the city hospital to make sure patients would have ready access to intensive care if they experienced side-effects.

That hands-on involvement is typical of Joel’s work to bring promising drug candidates to the clinic. “It’s all about getting things done for the communities who need new treatments,” says Joel. “We’re often working in places where the infrastructure is limited and we need to find pragmatic ways to push development forward.”

The compound in question is the first antimalarial drug candidate with a novel mechanism of action to achieve positive Proof-of-Concept for more than 20 years. Crucially, it may become a therapy able to beat drug-resistant malaria. It’s welcome news now that the mosquito-borne parasites that carry malaria are showing early signs of resistance to artemisinins, the key compound class in today’s standard treatments.

More recently, Joel has also been leading the initial work on a second highly promising antimalarial medicine, steering it from initial discovery in the lab to successful early clinical trials. Both compounds are now in full development. “These have the potential to save countless lives. If they make it through to approval, we could be looking at game-changing drugs in the fight against drug-resistant malaria,” says Joel.

Joel’s path to TME

Visiting remote clinics in Southeast Asia is a far cry from Joel’s early career. After medical school and specialty training in Australia, Joel moved to the UK for a clinical lectureship at Oxford University. A move into industry followed as medical director of an Oxford University spinout company. He combined this with a parallel visiting professorship at the University of California, Los Angeles. In 2007, Joel joined NITD in Singapore to help tackle neglected tropical diseases. “These are diseases people should not be dying from,” he says.
“It’s like being in a biotech bubble, but with access to the resources and scientific strength of a global pharma”

Joan

“One of the questions that always fascinated me as an academic researcher and physician was whether we could intervene chemically in the biological pathways involved in aging. But I’d never found anyone to support any serious research.

“I got to Novartis and said to my boss: ‘How about we work on aging?’ A lot of people have talked about how great it would be if someone did a trial, but no one had the courage to do it. I was given the resources to do one of the first clinical trials ever to see if we could impact the rate of aging in humans.

“We’re not limited to working on predetermined compounds or targets here. Instead, it’s up to us to make a compelling case for pursuing research where unmet medical needs intersect with deep understanding of disease biology. That’s true even in indications other companies have avoided because of the perceived high risk of failure.

“It’s like being in a biotech bubble, but with access to the resources and scientific strength of a global pharma. There’s a lot of freedom you are given to push boundaries and do the most innovative research you can. That’s very rare.”

• Translational Medicine Expert
• Joined NIBR: 2010
• Former job: Medical Director, Genzyme
I consider myself lucky. Everyday, I get to work with brilliant, highly-motivated physician-scientists and clinical-scientists doing extraordinary, cutting-edge work.

Their expertise in all three disciplines - basic science research, medical practice and clinical research - means that they are uniquely equipped to work at the intersection of molecular pathways and clinical application, bringing potential drugs all the way from the lab bench to the patient.

The success of their work owes much to a laser-like focus on patients. Each project is born out of just a few questions: Where are the patient groups in desperate need of innovative medicines? How do we more fully understand their disease? How do we get breakthrough medicines to them as quickly as possible?

And I know from speaking to my colleagues - as is reflected in the stories shared in this publication - it is often the patients that first brought us to work in the field of translational medicine. When you see your patients suffering without an effective therapy, it gives you a powerful appetite to join the fight to develop one.

We work in a unique culture here at NIBR. We are given freedom to pursue the science in a way that not only transforms our ability to innovate; it also makes work fun! But ultimately the reward comes from the opportunity to make a difference - a significant, life-changing, life-prolonging difference to millions of people.

Evan Beckman  
Global Head of Translational Medicine