Neuroscience [1]

Imagine being able to recreate a piece of your brain in a lab to understand how your brain really works. At the Novartis Institutes for Biomedical Research (NIBR), we are harnessing new technologies to build human models of neurological disease.

“I think this is a unique moment for neuroscience,” says Ricardo Dolmetsch, Global Head of Neuroscience at the Novartis Institutes for BioMedical Research [2] (NIBR). “We have identified many genetic clues about the underlying basis of neurodegenerative and neuropsychiatric diseases and we have developed the technologies to understand them in the lab.”

Psychiatric and neurodegenerative diseases place a heavy physical, emotional and economic burden on patients and their families. This burden will only grow in the coming decades as the population gets older, ¹ and yet we still lack treatments that alter the course of neurodegenerative and psychiatric diseases. Patients with neurodevelopmental diseases also have very few options and need therapies that have better efficacy and fewer side effects.

We believe that we can make new treatments that will modify the course of a disease and
dramatically improve patient lives. In children, we are focusing on autism and intractable epilepsy. In adult psychiatry, we are focusing on addiction, bipolar disorder, depression and schizophrenia.

In neurodegeneration, we are working on Alzheimer’s disease, amyotrophic lateral sclerosis (ALS), frontotemporal dementia and several rare monogenic diseases. We are also trying to improve the treatment of multiple sclerosis. While current drugs do well in preventing disease flare-ups, we are developing therapies aimed at restoring the neurological function that patients lose as the disease progresses.

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Ricardo Dolmetsch, Global Head of Neuroscience, NIBR

Our neuroscience teams have been working hard to tackle these challenges. Many neurological diseases that were intractable can now be attacked thanks to the advent of new technologies that have been helping us fill the gaps in our understanding. Among these advances are platforms for the creation of human neurons in the lab and for manipulating neuronal circuits in preclinical models.

Dolmetsch notes the many of the old preclinical mouse models were useful for making symptomatic drugs but were not effective for developing treatments that could change the course of a disease. “We are now developing models that are more predictive by making them more human,” he says.

For instance, we can now take skin or blood cells from patients and, using our induced pluripotent stem cell platform, convert them into neurons and then into miniature brain-like organoids. This is enabling us to study neurological diseases in human cells from patients with a specific disease.

“For example, we can recreate the neurons of a child with autism or a grandmother with Alzheimer’s disease in the lab and use them to understand how genetic mutations alter the function of their cells as well as to identify new drugs,” says Dolmetsch. “Modelling neurological and psychiatric diseases in single cells can be extremely powerful.”

We are also benefiting from the revolution in DNA sequencing, which has allowed us to link specific genetic mutations to many different neurological diseases. This information now helps guide many of our efforts to identify new therapeutic targets.

In parallel we also have the capacity to manipulate neural circuits using optogenetics — the use of light to control the activity of single neurons. This technology gives us the ability to determine which cells are involved in specific behaviors in a way that was not previously possible.
To succeed, we need to be daring and we need the best people to work on these problems. It won’t be easy or fast, but we believe it’s possible and we are committed to helping patients in need. “The unmet patient needs in neuroscience are huge,” says Dolmetsch. “To be able to improve the lives of the people suffering from psychiatric and neurological diseases would be a huge accomplishment and would mean a lot to us and to our societies.”

Video of A unique moment in neuroscience

Disease burden statistics

- In the United States, neurological illnesses and psychiatric disorders cost more than $760 billion a year.¹
- Alzheimer’s disease affected 35.6 million people worldwide as of 2010, and this number is expected to grow to 115.4 million by 2050.²
- The estimated global cost of Alzheimer’s disease will exceed $1 trillion in 2018.³
- Schizophrenia affects between 0.5 and 1% of the world population and costs about 62 billion dollars a year to treat in the United States.⁴
- The lifetime cost of care for a patient with autism and an intellectual disability is estimated to be $2.4 million in the U.S.⁵
- Depression affects approximately 14.8 million U.S. adults, 6.7% of the population age 18 and older in the United States.⁶

Footnotes:


Selected Publications:

Timothy syndrome is associated with activity-dependent dendritic retraction in rodent and human neurons [3]
Nature Neuroscience, January 2013
Genome-wide association analysis identifies 13 new risk loci for schizophrenia [4]

Nature Genetics, August 2013

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