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Battling baffling brain disease

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Using yeast, stem cells and his impressive brain power, clinician and neuroscientist Vikram Khurana is tracking down the causes of – and treatments for – debilitating brain disorders like Alzheimer's disease. It's all happening at the biotech start-up he co-founded in Cambridge, Massachusetts.

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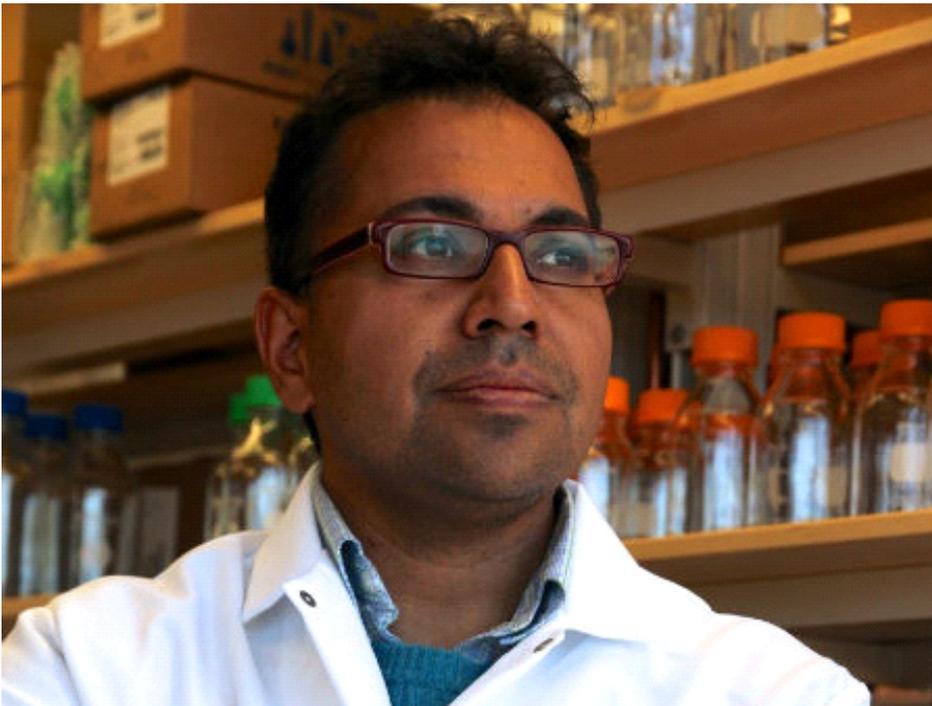
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Vikram Khurana doesn't waste time. Get a medical degree from Sydney University: done. Win a Fulbright scholarship to the US: accomplished. Get a PhD in Neuroscience at Harvard, become a consultant neurologist at Massachusetts General Hospital, and learn stem-cell biology at MIT: check, check, check. Establish a drug discovery start-up in the biotechnology hub of the world, Boston: completed. Oh, and marry your scientific colleague and have two children: yes, and yes.

"I don't like doing things by half" says Khurana, a charmingly gregarious 40-year-old neurologist and neurobiologist. And that includes his professional goal - not just seeing people with devastating neurodegenerative diseases like Alzheimer's, Parkinson's and amyotrophic lateral sclerosis (ALS) in the clinic, but developing new therapies for them.

These are important objectives. An estimated 55 million people worldwide suffer from neurodegenerative diseases, according to Alzheimer's Disease International's World Alzheimer Report 2015 and the Parkinson's Disease Foundation¹. There is not a single strategy that successfully prevents, treats or reverses these diseases and they are not going away. Without treatment, their prevalence will double in 20 years and global costs will exceed US\$1 trillion.²

In sum, neurodegenerative diseases represent one of the world's largest healthcare challenges. "They have devastating personal and economic consequences for patients, caregivers and society," says Khurana – and he knows firsthand.

"When I was growing up, certain family members were afflicted with neurological diseases," he recalls. "My mother's aunt died horrifically of Lou Gehrig's disease." Also known as ALS or motor neuron disease, it's a neurodegenerative disease that affects nerve cells in the brain and the spinal cord.

Khurana's family is studded with medicos. Both his parents are doctors, as was his grandfather. His brother Vini, five years older than Khurana, became a cerebro vascular neurosurgeon, galvanised by the early death from stroke of their grandmother and great aunt. "Obviously, our family experiences influenced us", he says.

In turn, Vini influenced young Vikram to attend his alma mater, the University of Sydney. There, as a budding neurologist Khurana recognised that diagnosing disorders like Alzheimer's without having therapies to offer wasn't enough. "I was incredibly frustrated", he says.

Gaining laboratory experience was essential. After medical school in 2001, Khurana moved to the US with a

Harvard doctorate and neurology training on his 'to-do-list'. Persistence opened doors. "I'm very grateful for the help and support I've had," says Khurana, citing the Fulbright Commission, the American Australian Association, the Harvard Neurodiscovery Center and his many mentors on both sides of the Pacific as pivotal supporters of his training. And he wants to give back. Since those early days, building a healthy biomedical bridge between the US and Australia has always been on his mind.

Fast forward to 2014 and the launch of Yumanity Therapeutics. This private company develops treatments for illnesses with critical unmet medical needs. Currently, Yumanity focuses on neurodegenerative diseases.

Khurana co-founded Yumanity, along with two other scientists he collaborated with at MIT's Whitehead Institute - Daniel Tardiff and Khurana's wife, Chee Yeun Chung – and their mentor, renowned MIT biologist Susan Lindquist. Biotech industry veterans Tony Coles and Ken Rhodes also joined. "I can't imagine working with a better team", says Khurana.

Under Coles' leadership, cancer biopharmaceutical company Onyx was acquired in 2013 for US\$10.5 billion. And Rhodes, formerly Biogen, had a track record of getting neurodrugs through preclinical development and into clinical trials.

Khurana and Chung met at Harvard and married in 2006. "We took a chance and decided to work together," says Khurana. He confesses, "It's not easy" working while raising two daughters, Jaya, 5, and Mira, 20 months. "I think we sustain each other and somehow land on our feet", he says.

Yumanity is growing fast, with twenty employees on the payroll in Cambridge, part of the Boston metropolitan area, and research partnerships with companies around the world including China. Khurana anticipates Yumanity growing by around 20 more employees in the next few months. The firm is already following up many promising leads for new therapies for Alzheimer's, Parkinson's and ALS.

What makes Yumanity's development of treatments surprisingly swift is that it goes to the causative heart of the debilitating disorders - protein misfolding. "Misfolding is an ancient problem and we exploit that fact at Yumanity," says Khurana.

He explains that proteins are molecules packed inside cells. They do key jobs and are required for cell structure, function and regulation. To achieve so many functions, they need to fold precisely into distinct shapes. When folding goes awry, critical functions are lost. Worse, renegade proteins can set off cascades of destruction, causing brain cells to malfunction and die.

Khurana, Chung and Tardiff - showed that these renegade proteins commit crimes in ways that are conserved across a billion years of evolution. They used-cutting edge stem cell techniques to make brain cells 'in a dish' from patients with neurodegenerative disease, and then found that drug screening and genetic wizardry in really simple cells – yeast cells better known for leavening dough and brewing beer – provided clues on ways to find and reverse abnormalities in the brain cells. Their back-to-back papers in the journal *Science*³ caused quite a stir in the scientific community⁴ and gave birth to Yumanity.

So why call the company Yumanity? "It's Yeast to Man," replies Khurana. "It was coined by Sue."

Today, Khurana has a string of publications, patents, honours and awards on his completed list. Thanks to an Australian National Health & Medical Research grant, he's also collaborating with University of New South Wales neuroscientist Glenda Halliday – developing stem cell models for a disease related to Parkinson's called Multiple System Atrophy.

Keeping to his 'no half measures' dictum, Khurana is about to take up a professorship at Harvard Medical School and the Ann Romney Center for Neurologic Disease in the Department of Neurology at Brigham and Women's Hospital. "There's a new neuroscience institute being built where I'll be able to see patients and

perform research in the same building,” he says excitedly.

It's a good thing that Vikram Khurana can get by on little sleep... and support from his life and scientific partner Chee Yeun.

-END-

1. <http://www.alz.co.uk/research/world-report-2015>; http://www.pdf.prg/en/parkinson_statistics

2. Ibid.

3. <http://science.sciencemag.org/content/342/6161/983>; <http://science.sciencemag.org/content/342/6161/979.full>

4. <http://directorsblog.nih.gov/2013/11/05/yeast-reveals-new-drug-target-for-parkinsons/>



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