

Florida company hunts cure for Alzheimer's disease

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More than three decades ago, when Dr. Kiminobu Sugaya was a young scientist in Japan, he made a calculation: If he became a neurosurgeon, treated as many as 200 patients each year and practiced for 20 years, he would improve the lives of about 4,000 people. Only 4,000 people, he thought.

"I didn't like that. So I moved to research," he said recently in his office at the University of Central Florida's new Lake Nona Life Science Incubator.

Since then, Sugaya has been searching for a cure for <u>neurodegenerative</u> <u>diseases</u>, such as Alzheimer's and Parkinson's, and he said that today, more than ever, he's close to finding a cure and potentially helping millions of people.

His young company SynapCyte is working on developing a proprietary <u>drug</u> that targets the <u>stem cells</u> in the brain and triggers them to multiply and create new neurons, replacing the ones lost to <u>disease</u>.

"This drug is totally different from the previous ones," said Sugaya, a neurobiologist and professor of medicine at UCF. "We're re-creating your brain. That's the difference. We're not dealing with symptoms."

Sugaya's research is still in the pre-clinical stage, and all the results are based on animal studies, but he said so far the data are promising.



The idea that the nervous system can't regenerate was disproven in the late 1990s when researchers found that stem cells existed in the adult brain, although there's still debate as to exactly how many cells there are, where they are and their rate of regeneration, said Dr. Michael Valenzuela, professor of <u>regenerative medicine</u> at Brain and Mind Centre at the University of Sydney in Australia.

Sugaya is one of several researchers studying stem cells for neurodegenerative diseases. Some, like Sugaya, are trying to activate existing stem cells, while others are trying to make the right kind of stem cells in the lab and introduce them to parts of the brain that are under stress.

"Stem cells are relatively new technology and are believed to hold much promise," Dr. Kelly Shepard, a senior science officer at California Institute for Regenerative Medicine, said in an email. "It will take time to understand them, their capabilities and their limitations—but there is much to look forward to."

Almost 6 million Americans live with Alzheimer's disease. Another million have Parkinson's. But researchers have yet to find an effective treatment for the diseases.

Five drugs have been approved to treat the symptoms of Alzheimer's, helping with memory and thinking problems, according to the Alzheimer's Association. But none reverses the effects of the disease.

Many drug trials aiming to slow down or treat the disease have failed, mainly because how the disease starts and progresses is poorly understood, Shepard said.

Some promising news came a few months later from preliminary data of another experimental drug developed by Biogen and Eisai, which slowed



the progression of Alzheimer's disease. But it must be tested further.

Sugaya is banking on harnessing the power of stem cells already in the brain and goes as far as saying that his drug may one day cure Alzheimer's and Parkinson's.

Some experts are skeptical.

"It would be a grave mistake to get too excited by some isolated rodent experiments," Valenzuela said. "Recall that we have 'cured' Alzheimer's disease at least 50 times over in different mouse models, and each of those treatments has failed when tested in humans," he wrote in an email.

And there are some caveats.

Shepard pointed out that Parkinson's and Alzheimer's affect more cell types than just neurons.

"We don't know enough about (Alzheimer's disease) to say what would be a cure," Kelly wrote. "(Neural stem <u>cells</u>) might be a tool of study to discover new treatment options. And one day they might be used to regenerate lost neurons. But this is a long way off and there is much more that needs to be understood."

Sugaya arrived in the U.S. 31 years ago. After research stints at University of Illinois in Chicago and Mayo Clinic in Jacksonville, he came to UCF 14 years ago. Along the way, he founded a biotech company, Progenicyte, licensing more than 60 patents and patents pending, including some for a drug that stimulates <u>neural stem cells</u> in the brain.

To develop the drug, he formed a UCF spin-off company called



SynapCyte in late 2016. The company is one of the first startups to move to UCF's new Lake Nona Life Science Incubator, which opened in May.

SynapCyte expects to present the drug's pre-clinical results to FDA within the year and with an approval, start the Phase I human trials to test the safety of the therapy and potentially get a hint at the effectiveness of the drug. If successful, the drug will then move to Phase II clinical trials. All this could be done in the next three years, said company CEO Bob Hering.

SynapCyte could then license the drug to a major pharmaceutical company.

"I didn't want to go to big pharma with this drug," Sugaya said. "When they license it, they'll put in on the shelf because they have other drugs in the pipeline or want to treat the patients forever."

For now, SynapCyte's main objective is to raise money for the startup, and it's a challenge.

"When you approach someone and say, 'We have the cure for Alzheimer's disease and Parkinson's disease, while all the major (pharmaceutical companies) have failed,' they ask, 'Why should they fund us?'"

But Sugaya and Hering are confident they are developing a cure.

"It's a bold statement, isn't it?" Hering said. "We think we're onto something really special."

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