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Business

Cambridge startup CAMP4 Therapeutics raises $100 million for RNA therapies

CAMP4 will develop drugs for genetic epilepsies and liver diseases

By Ryan Cross
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CAMP4 Therapeutics announced Wednesday that it raised $100 million in series B financing to develop a novel class of RNA-based therapies. The Cambridge startup's experimental approach will allow it to dial up the output of genes to treat genetic diseases, with an initial focus on a severe form of epilepsy and life-threatening liver diseases.

The new funds will help the company grow its Cambridge staff by about 50 percent over the next year, get its first two therapies into clinical trials, and expand its scope to develop therapies for muscle and heart diseases. "It's more money than we've ever had, and we're truly excited about that," said Josh Mandel-Brehm, the startup's chief executive.

CAMP4 was founded in 2016 based on the research of its founder Richard Young, a biologist at MIT and the Whitehead Institute. The company initially tried to make small molecules drugs — ones usually taken as pills — to control how genes are turned on. CAMP4 formed and ended partnerships with two larger Cambridge firms, Alnylam Pharmaceuticals and Biogen, before changing course in 2020.

"We essentially started fresh as a new company," Mandel-Brehm said. His startup is now developing synthetic RNA molecules to control genes and either increase their output of proteins essential for health or decrease the production of proteins linked to disease.

The idea comes from discoveries that scientists such as Young have made over the past 10 to 15 years when studying what some have called the "dark matter" of the genome or "junk DNA" — large swathes of genetic code once thought to have no function, explained David Bumcrot, CAMP4's chief scientific officer.

CAMP4 THERAPEUTICS

CAMP4 Therapeutics CEO Josh Mandel-Brehm.

It turns out that some regions of so-called junk DNA are actually used to make molecules called regulatory RNAs, which, as their name implies, control other genes. CAMP4 is developing drugs that control these regulatory RNAs. If it works, it will give scientists a new way to fine-tune how much a particular gene is dialed up or down.

Mandel-Brehm believes the approach could be applied to treating as many as 1,000 genetic diseases, but CAMP4 is starting with programs for two.

The company's first test of its technology will be for a debilitating form of epilepsy called Dravet syndrome, which is caused when a child inherits a broken copy of a gene crucial for brain cell signaling. Although these children still have a working copy of a gene from the other parent, it's not enough to keep them healthy.

CAMP4 plans to treat the condition by turning up the volume on the working gene, essentially coaxing it to do the work of two genes. The company will submit its plans for a clinical trial to regulators by the middle of next year.

Later, in 2023, the company will ask regulators for permission to begin a clinical trial for genetic liver diseases called urea cycle disorders, where the toxic buildup of improperly metabolized molecules can cause cognitive delays, seizures, and comas in children. The company has earlier stage programs for other liver diseases as well as a brain disease called frontotemporal dementia.

CAMP4 has about 50 employees in its labs and offices at the One Kendall Square campus in Cambridge. It will use the funds to hire more than 25 employees over the next year. It has also opened a small lab in Boulder, Colorado where a team of chemists will design the synthetic RNA molecules used in its therapies, and new strategies for delivering the drugs into the body.

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