

BioCentury

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EMERGING COMPANY PROFILE

CEREVANCE'S HUMAN ELEMENT

BY ALLISON JOHNSON, STAFF WRITER

Cerevance Inc. plans to treat neurological diseases by rebalancing disrupted neuronal circuits. The company is identifying circuit-specific neurological targets via its NETSeq RNA profiling platform, and thinks its ability to start with human tissue will translate better to the clinic than platforms based on preclinical animal models.

NETSeq has yielded eight programs in the last two years. Lead program CVN058 is in Phase Ib testing to treat cognitive impairment in schizophrenia, and Cerevance plans to submit an IND next month on symptomatic Parkinson's disease therapy candidate CVN424, which has greater circuit-specificity than SOC and may reduce the dyskinesia side effect. Preclinical programs include additional compounds for PD, neuroinflammation in Alzheimer's disease, Rett syndrome, and anxiety and addiction.

Neuronal circuits comprise a mixture of cell types that relay messages within the brain to control homeostatic, motor or other biological functions, but are disrupted in various neurological diseases.

To restore damaged circuits, one strategy is to develop therapeutics that replace the functionality of a depleted cell type, such as using dopamine agonists to address lost dopaminergic neurons in PD.

In contrast, Cerevance's strategy relies on the idea that "if you change the activity of another cell type in the circuit, you may be able to normalize function," said co-founder and CEO Brad Margus.

Cerevance's platform utilizes its expanding tissue bank of more than 2,000 matched clinical and phenotype data from diseased patients and healthy donors whose ages span five decades.

NETSeq profiles gene expression of glial and neuronal cell populations within circuits of interest in human post-mortem brain tissue by isolating and sorting nucleic RNA using cell-specific markers.

The method pools transcripts from multiple cells of the same type, allowing it to find transcripts expressed at low levels that may be missed by techniques like single cell analysis.

CEREVANCE INC.

Boston, Mass.

Technology: NETSeq target discovery platform utilizing post-mortem human brain tissue

Disease focus: Neurology

Clinical status: Phase I

Founded: 2016 by Brad Margus and Nathaniel Heintz

University collaborators: Rockefeller University

Corporate partners: None

Number of employees: 31

Funds raised: \$29 million

Investors: Dementia Discovery Fund, Lightstone Ventures, Takeda Pharmaceutical Co. Ltd.

CEO: Brad Margus

Patents: One patent family issued covering CVN058

Cerevance has not disclosed the targets of its development programs.

By starting with human tissues, Margus said NETSeq may bypass problems caused by the lack of predictive CNS animal models. "Companies advancing CNS therapies can't draw as much on preclinical *in vivo* proof-of-concept data before investing in human trials as companies working in other disease areas," he said.

Margus added the data generated by NETSeq could help define disease subtypes and patient populations, enabling biomarker identification and informing preclinical model design.

According to BioCentury's BCIQ database, at least two other companies have created human-based neurology target identification platforms: BlackThorn Therapeutics Inc. and Verge Genomics Inc.

BlackThorn links quantitative behavioral readouts to functional brain imaging data to identify neuroanatomical circuits and targets dysregulated in psychiatric diseases.

Verge is identifying disease-driving molecular networks using genetic, epigenetic, RNA and protein readouts housed in non-public academic databases.

Both companies complement Cerevance's target identification strategy, said Margus. NETSeq could be used to understand the expression profiles of circuits identified by BlackThorn, and Verge's data-mining capabilities could be applied to Cerevance's data sets.

NETSeq was developed by Cerevance co-founder Nathaniel Heintz, a professor of molecular biology at The Rockefeller University. Cerevance has exclusive, worldwide rights to the technology and has further developed it in-house.

Cerevance has raised \$43.7 million, including \$14.7 million in non-dilutive funding, which gives the company two to three years of runway. Margus said Cerevance is open to raising capital sooner, whether through partnerships or a venture round, to expand the number of NETSeq targets it can pursue. 

COMPANIES AND INSTITUTIONS MENTIONED

BlackThorn Therapeutics Inc., South San Francisco, Calif.

Cerevance Inc., Boston, Mass.

The Rockefeller University, New York, N.Y.

Verge Genomics Inc., San Francisco, Calif.

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