

Rumi Scientific, Inc.

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Point of Contact

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Management Team

CEO, Ilona Nemeth, MBA

CSO, Fred Etoc, PhD

Industry: Biotechnology, Drug Discovery & Development, CNS

Core Business

To date, the development of drugs for neurological (CNS) diseases has been mostly unsuccessful. Due to the profession's lack of knowledge of the underlying disease biology, most, if not all of these disorders have no cures.

Thus, the front end of the drug discovery pipeline is risky and expensive, especially in neuroscience. Rumi Scientific's approach changes the usual sequence: we start with standardized human tissues in micro-version.

Rumi has licensed core technology from our co-founders at The Rockefeller University and then further developed it. Rumi has proven a transformative process for screening and testing therapeutic candidates for CNS diseases – faster and more effectively than ever before. Rumi's unique platform can model neural differentiation from an embryonic stage, with the goal to identify compounds reversing the disease state at the cellular and tissue levels in large scale screenings. The accurate genetic modeling and cellular context results in novel and better targets, assisted by our inhouse machine-learning algorithm.

We work on CNS disorders with unmet medical needs - we have identified an early candidate molecule able to reverse disease phenotypes associated with Huntington's Disease, a fatal monogenic disease. We are currently pursuing development of this candidate, as well as validating the associated novel target which works through an innovative mechanism of action. Rumi Scientific's platform is broadly applicable, and we will build a pipeline centered around neurodegeneration (eg Huntington's Disease) and neurodevelopmental (eg our work with a leading autism center currently).

The combined market value for neurodegenerative diseases and neurodevelopmental disorders is approximately \$9 billion. Tangentially, the drug discovery CRO market currently has a market size of approximately \$5 billion.

Our platform allows the first realization of large-scale drug discovery campaigns at the level of standardized synthetic human tissue, and results in a powerful engine for the discovery of novel therapeutics: for virtually any disorder, tens of thousands of compounds can now be screened, and differences between diseased and healthy tissues can be measured quite precisely, allowing better early prediction of the therapeutic success of a drug.

Company Milestones

Scientific:

- Built the foundations for our platform technology (2017-2019)
- Demonstrated phenotype assays for Huntington's disease (2018-2019)
- Established AI analysis capability (2019)
- Demonstrated the ability of our platform to perform a drug discover campaign and generate new therapeutic leads (2019)

- Discovered target for Huntington’s disease (2019)
- Established phenotypes for neurodevelopmental disorders (2020)
- *in vivo* mice model work (2021)

Business:

- Licensed core technologies from the co-founder’s labs (2017-2019)
- Established initial lab (2017)
- Received \$2M total in grant funding from CIRM, NSF, NIH, and DOD to help build our platform technology and start screening programs (2017-2020)
- Filed a mechanism of action patent for Huntington’s disease (2019)
- Filed patent for machine learning (2019)
- Filed patent for neuroloid structures (2020)
- Obtained two commercial contracts (2019, 2020). A follow-up revenue-generating collaboration has already resulted. (2021-2022).
- Goal: Huntington’s disease IND (2024-2025)
- Goal: 3 additional CNS lead candidates in the pipeline (2024-2025)

Financial Overview

Funds Raised to Date: \$2.8M in grants and contracts

Fundraising: \$22-32M to complete IND for Huntington’s Disease within 4 years, plus 3 additional lead compounds for other CNS disorders.

Intellectual Property:

Rumi Scientific holds an exclusive license for core technology components that underlie and differentiate our platform.

Additionally, Rumi has filed 3 patent applications as outlined above.

Product Development and Regulatory Strategy:

For each disease area, our development strategy consists of 1) using our platform to develop synthetic human models of the disease, 2) screening to identify hit compounds, 3) optimizing hit compounds, 4) performing *in vivo* studies, and 5) filing an IND. When novel targets are identified during this process, we patent the novel target. For each disease area, we may pursue partnership, sale or out-licensing of the therapeutic candidate and/or the ability to use our platform in a specific indication.

Commercialization Strategy:

Rumi generates incremental revenue by completing predictive assays for partners and customers in biopharma and at foundations. Longterm, we primarily plan to generate income by partnering or out-licensing therapeutic candidates discovered in-house using our platform technology.

Scientific Co-Founders:

Dr Ali Brivanlou, Ph.D: Professor and Lab Head, Human Embryology and Stem Cell Biology, Synthetic Embryology, at The Rockefeller University.

Dr Eric Siggia, Ph.D: Professor and Lab Head, Laboratory of Theoretical Condensed Matter Physics, at The Rockefeller University. National Academy of Sciences. Biophysics.

Scientific Advisory Board:

Rumi’s SAB is a stellar panel of leading stem cell researchers:

- Dr Kristen Brennand, Ph.D: Yale School of Medicine; previously at the Icahn School of Medicine at Mt Sinai. Studies the impact of patient-specific variants across and between the cell types of the brain.
- Dr Stephen Chang, Ph.D: over 30 years of commercial experience in life sciences in areas of stem cells, cell and gene therapy and drug development. Serial entrepreneur. NYSCF. EIR at Rutgers and NYU.
- Dr Steven Goldman, MD, Ph.D: co-Director of Rochester's Center for Translational Neuromedicine. He has a concurrent appointment as Professor of Neuroscience at the University of Copenhagen in Denmark, and as co-director of its sister Center for Translational Neuromedicine. He also serves as Senior Vice President and Head of CNS Therapy at Sana Biotechnology.
- Dr Insoo Hyun, Ph.D: Co-Director, Undergraduate Bioethics and Medical Humanities Program, Case Western University School of Medicine. Faculty Member, Center for Bioethics, Harvard Medical School. Fulbright Scholar. Hastings Center Fellow. Leader in ethical and policy issues in stem cell research and new biotechnologies.
- Dr Lorenz Studer, MD: Founder and director of the Center for Stem Cell Biology at Memorial Sloan Kettering in NYC. 2015 MacArthur Fellowship (aka the "Genius Grant") for his innovative work on stem cell and Parkinson's disease research. Scientific co-founder of BlueRock Therapeutics (acquired by Bayer).