

## Who is Envisagenics And What Is SpliceCore?

Some of the information below was copied verbatim from the SMA News Today article and from [envisagenics.com](http://envisagenics.com)

Envisagenics was founded in 2014 as a spin-off company from the Cold Spring Laboratory, with a goal of developing treatments through RNA splicing analytics and artificial intelligence (AI).

Envisagenics announced that they've been given a two-year, \$1.5 million grant from the National Institutes of Health (NIH) to further advance their new biomarker and drug discovery platform called SpliceCore.

A Small Business Innovation Research (SBIR) Phase 2 grant, will help Envisagenics in their efforts to further develop SpliceCore, a cloud-based drug discovery platform that uses machine learning and artificial intelligence to analyze ribonucleic acid (RNA) sequencing data. Such data is important to the discovery of new RNA-targeted treatments.

RNA is the in-between molecule in the process that reads the information in DNA to generate a functional protein. Errors in RNA splicing that affect protein development are a cause of many genetic diseases, including spinal muscular atrophy (SMA).

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Envisagenics was awarded a \$225,000 Phase 1 SBIR grant in 2015, to develop an infrastructure capable of analyzing significant amounts of RNA sequencing data in a scalable manner. That first grant was also used to help build the largest database to date of RNA splicing events, and to develop machine learning algorithms to prioritize splicing errors relevant to diseases like SMA.

The Phase 2 grant will allow the company to expand the knowledge base and predictive functions of its SpliceCore platform, which focuses on RNA splicing.

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Martin Akerman, co-founder and chief technology officer of Envisagenics, said the following in a press release: “The discovery of disease-causing proteins was at the center of pharma innovation for decades, but the new century brought us not only better knowledge of genetic information, but also the computer power to interpret it. RNA splicing treatments that we develop, target the flow of genetic information, so disease-causing proteins cannot be formed in the first place.”



Martin Akerman, PhD

Co-founder & CTO

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Work supported by this SBIR Phase 2 grant will take place at Cold Spring Harbor Laboratory, under the leadership of Dr. Adrian Krainer, a specialist in RNA splicing, and at Rockefeller University under Dr. Thomas Tuschl, professor and head of the laboratory of RNA molecular biology.



Adrian Krainer, PhD

Professor & Program Chair

Cancer & Molecular Biology Cold Spring  
Harbor Laboratory



Thomas Tuschl, PhD

Professor & Head of the Laboratory

RNA Molecular Biology, The Rockefeller  
University

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Maria Luisa Pineda, company co-founder and chief executive officer of Envisagenics, stated the following: “We’re excited for the next stage of growth for the company to leverage the power of Artificial Intelligence and RNA sequencing data, internally and in collaboration with biopharma partners to unlock new treatments.”



Maria Luisa Pineda, PhD

Co-founder & CEO

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## My Opinions Regarding Envisagenic's Drug Platform - SpliceCore

In earlier videos, I told everyone that Cytokinetic's drug Reldesemtiv worked at the muscular level, and Biogen's drug Spinraza worked at the cellular level. Envisagenic's drug platform SpliceCore, if successful, could bridge the gap between Cytokinetics and Biogen. Biogen is working to get the SMN2 gene to either produce a full protein, or to allow this gene to produce more protein than it's currently producing. In my opinion, if Envisagenics can successfully accomplish their goals, and be able to correct the errors in RNA splicing, the "one-two" punch that Cytokinetics and Biogen had, may become a three-way party.

It seems as if every time we blink our eyes or turn around, new medical techniques and advanced procedures regarding medical testing, are not only getting those of us with SMA excited, it's providing another glimmer of hope that all SMA patients can cling to. With all of the pharmaceutical companies, clinical trials and new drugs that are being brought to market, how can those of us with SMA not be excited about our futures.