RXi Pharmaceuticals Corporation

Investor Fact Sheet

RXi Pharmaceuticals
Next Generation in RNAi

Corporate Overview

RXi Pharmaceuticals is a leader in next generation RNA targeting, including its proprietary rxRNA™ discovery platform, for the treatment of human diseases. RXi is engaged in the discovery, development and commercialization of proprietary therapeutics.

RXi Pharmaceuticals believes it is well positioned to compete successfully in the RNAi therapeutics market with its strong technology platform, broad and early intellectual property position, a management team that is experienced in commercializing products, and accomplished scientific advisors, including Dr. Craig Mello, recipient of the 2006 Nobel Prize for his co-discovery of RNAi.

RNAi Therapeutics

RNAi is a naturally occurring process by which a gene's message in a cell is silenced before it creates a protein. RNAi offers a novel approach to the drug development process, because RNAi compounds can potentially be designed to target any one of the genes in the human genome. Other potential advantages of RNAi therapeutics:

- Rapid development of lead compounds
- Highly selective for the target gene
- Highly potent (low dose)
- Natural mechanism of action (low toxicity)

RXi's Therapeutic Platform

RXi Pharmaceuticals' rxRNA™ compounds are distinct from the siRNA compounds used by many other companies developing RNAi therapeutics and are believed by the Company, based on its internal research, to be up to 100 times more active than conventional siRNA (depending on the target site), nuclease resistant and readily manufactured.

QUICK FACTS

Ticker:
Nasdaq: RXII

Founded:
By Craig Mello, Ph.D
Discoverer of RNAi
2006 Nobel Laureate
and other leaders
in the field

Initial Trading:
March 2008

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Worcester, MA

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Therapeutic Areas

Neurology

Initially, we are pursuing research in ALS (amyotrophic lateral sclerosis, commonly known as Lou Gehrig's Disease). Some forms of ALS are caused by defects in a gene called SOD1. Early preclinical studies conducted by RXi advisors, Dr. Tariq Rana and Dr. Zuoshang Xu at UMMS showed promising results in animals using an RNAi compound to selectively inhibit the SOD1 gene. We are refining and extending this work and, if successful, will move into formal preclinical development. We also intend to leverage our experience related to the delivery of RNAi therapeutics in the central nervous system to explore development of RNAi-based treatments for neurodegenerative diseases other than ALS, including Alzheimer's Disease.

Metabolic Disease

One of our scientific co-founders and scientific advisory board members, Dr. Michael Czech, is a leading metabolic disease researcher. We have in-licensed intellectual property developed by Dr. Czech on genes that appear to be important regulators of metabolism. Studies conducted in Dr. Czech's laboratory at UMMS and by others at Imperial College of London have demonstrated that inactivation of one of these genes, called RIP140, can cause fat cells to metabolize rather than store fat. Mice in these studies that did not express RIP140 remained lean and non-diabetic even when maintained on a high fat diet. We are currently designing RNAi compounds targeting RIP140 as a potential treatment for obesity and obesity-related type 2 diabetes. We also continue to evaluate genes in Dr. Czech's database for candidate targets.

Oncology

We are initiating a program to develop RNAi drugs for use in oncology. This strategy is led by key RXi scientific advisors, Dr. Greg Hannon and Dr. Nicholas Dean, both of whom are leading researchers in targeting oncogene pathways. Additionally, our management team has expertise in developing programs targeting genes involved in cancer. Dr. Pamela Pavco, our Vice President for Pharmaceutical Development, previously managed the pre-clinical programs targeting genes involved with cancer while at Sirna Therapeutics, Inc. (recently acquired by Merck & Co.).

Management and Scientific Advisors

Tod Woolf, Ph.D., President and CEO
- Founded-CEO of Sequitur, an RNAi company acquired by Invitrogen (IVGN)
- Co-invented and commercialized Stealth™ RNAi
- Co-invented two of RPI's (now Sirna-Merck) main RNA technologies

Stephen J. DiPalma, Chief Financial Officer
- Founded and served as President and CEO of Catalyst Oncology
- Formerly CFO at Milkhaus Laboratory, Phytera and Athena Diagnostics
- Successfully turned around publicly traded Aquila Biopharmaceuticals

Pamela Pavco, Ph.D., VP of Pharmaceutical Development
- Brought Sirna's lead RNAi candidate to Phase I in under 12 months
- Three additional RNA drug candidates through IND at Sirna (RPI)
- Managed Sirna's Allergan and Huntington Disease collaborations

Dmitry Samarsky, Ph.D., VP of Technology Development
- Organizer and speaker for dozens of RNAi conferences
- Agreements with over a dozen pharma and biotech companies
- Director of Technology Development at RNAi technology leader Dharmacon

Craig Mello, Ph.D., SAB Chairman
- 2006 Nobel Prize in Medicine for RNAi
- Co-discovered RNAi and invented RNAi therapeutics
- Howard Hughes Medical Institute Investigator at UMMS

Greg Hannon, Ph.D., Founder and SAB Member
- HHMI Investigator at Cold Spring Harbor Laboratory
- Discovered mechanism of RNAi in human cells
- Developed the widely used shRNA

Tariq Rana, Ph.D., Founder and SAB member
- Professor and Founder Program in Chemical Biology, UMMS
- Discovered key parameters to stabilize RNAi
- Developed RXi's Nanotransporter Technology

Michael Czech, Ph.D.
- Professor and Chair, Program in Molecular Medicine, UMMC
- American Diabetes Association's Eli Lilly Award for Diabetes
- Banting Award for scientific achievement