

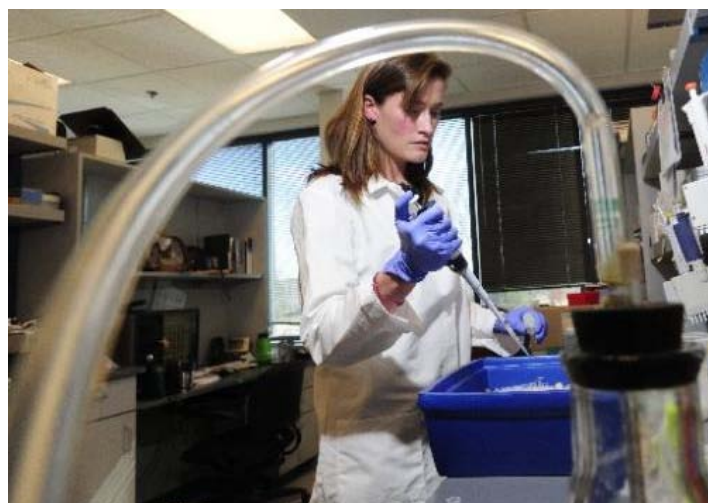


## Boulder's miRagen moving forward on microRNAs

Local biotech recently receives orphan drug designation for compound

By **Alicia Wallace** Camera Business Writer

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Katie Robinson, an associate scientist at MiRagen Therapeutics, works in the molecular biology lab last month. ( MARTY CAIVANO )

MiRagen Therapeutics Inc.

Founded: 2007

CEO: William Marshall, Ph.D.

Address: 6200 Lookout Road,  
Suite 100, Boulder

Phone:  
303-531-5952

Website:  
[miragentherapeutics.com](http://miragentherapeutics.com)

In a few years' time, a burgeoning Boulder biotech is positioning itself to shine a big light on a relatively young scientific find that's viewed as the industry's "dark matter."

Boulder-based miRagen Therapeutics specializes in therapeutics based on microRNAs, small ribonucleic acid molecules. In recent years, studies have shown that microRNAs play a significant role in gene expression and also are responsible for many diseases.

By targeting these molecules that respond to cellular stress, miRagen has high hopes of developing therapeutics for cardiovascular and muscle disease.

"Overall, our studies both on the heart failure side and the myeloproliferative (bone marrow diseases) have progressed nicely," said William Marshall, miRagen's president, chief executive officer and co-founder. "... That's what drives everything, is good data."

MiRagen was founded four years ago by some of the top researchers in the biotech and RNA fields including Michael Bristow, co-director of the University of Colorado Cardiovascular Institute in Denver; Marvin Caruthers, a co-founder of biotech giant Amgen; and Eric Olson, founder of the Department of Molecular Biology at the University of Texas Southwestern Medical Center.

The 20-person company that also was incubated in Boulder Ventures is banking on further quality studies to make its therapeutics a reality, Marshall said, noting recent research that showed a mouse without miR-208 -- a microRNA in the heart region -- showed some resistance to cardiac stress, he said.

Considering the industry's

relatively young age -- analysts peg it at about a decade old -- Marshall said he realizes there may be some hurdles to overcome on the regulatory, investment and partnership fronts.

"We're still science fiction as far as drug discovery is concerned," he said.

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In the past six years, there has been a surge in the amount of microRNAs that have been discovered, said Christi Bird, an industry analyst for Frost & Sullivan. As a result, the research sector of the microRNA industry has grown to about \$20 million, she said.

"That market is continuing to grow," she said.

Other arms of the industry include diagnostics and therapeutics, she said, adding that most of the therapeutics firms are in the beginning stages and building up their pipelines. A lot of companies also are "jumping on the bandwagon" of microRNAs because of the potential, she said.

While delivery issues appear to be the biggest challenge on the horizon for these companies, Bird said miRagen appears to be somewhat uniquely positioned.

"(MiRagen is) taking on cardiovascular," she said. "Most of these companies are targeting cancer."

As far as Marshall is concerned, miRagen appears poised to make believers out of many in the industry. He noted a move made last month by the U.S. Food and Drug Administration to grant orphan drug designation to one of the company's compounds. The FDA grants the status to products that show promise for diagnosing or treating rare diseases.

Some incentives -- including tax credits and a seven-year marketing exclusivity -- come with the designation.

MiRagen's newly orphan compound, MGN-4893, targets microRNA-451, which studies have shown to have an effect on the regulation of blood cell development. By targeting microRNA-451, miRagen's compound could serve as a treatment for polycythemia vera, a rare blood disorder that could lead to leukemia; myelofibrosis and thrombosis, blood clotting that can cause strokes or heart attacks.

MiRagen hopes to begin dosing MGN-4893 in humans in the first half of 2012, Marshall said.

MGN-4893's progression and the furthering of an extensive pipeline have Marshall's optimism climbing about the future of his Boulder company and its potential to land a partnership or additional funding. MiRagen has raised \$12 million since its founding.

"We've had very productive partnering and investor discussions," he said. "We just continue to move in the right direction."

Contact Camera Business Writer Alicia Wallace at 303-473-1332 or [wallacea@dailycamera.com](mailto:wallacea@dailycamera.com).



Bill Marshall is president and CEO at MiRagen Therapeutics in Gunbarrel. (MARTY CAIVANO)

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