RaNA Therapeutics

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New Perspectives, New Possibilities

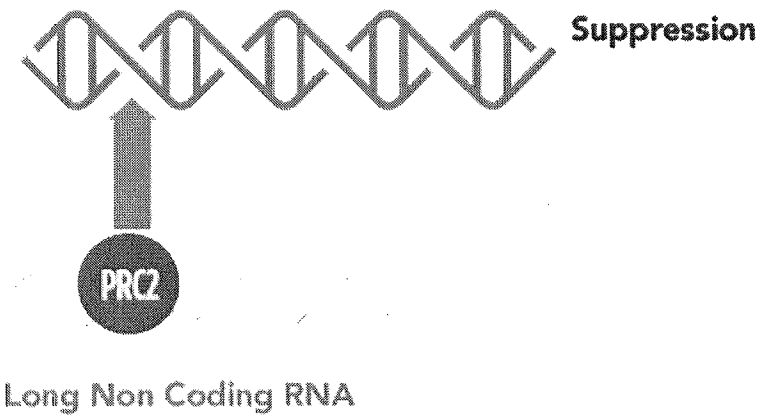
RaNA Therapeutics is pioneering the discovery of a new class of medicines that target RNA to selectively activate protein expression, thereby enabling the body to produce desirable proteins to treat or prevent disease. RaNA's novel therapeutics work by precisely activating the expression of select genes within the patient's own cells, increasing the synthesis of therapeutic proteins. The company's proprietary RNA targeting technology works epigenetically to make it possible, for the first time, to increase the expression of therapeutic proteins with exquisite selectivity.

The ability to precisely upregulate endogenous protein expression represents an exciting new frontier for drug development, opening up a vast number of previously undruggable targets that could pave the way for treating diseases that today have no or few treatment options. RaNA's goal is to generate new, highly-selective treatments in several therapeutic areas, with an initial emphasis in the fields of rare genetic disorders, inflammation, oncology, metabolic diseases and neurodegenerative diseases.

A New Frontier for Drug Development

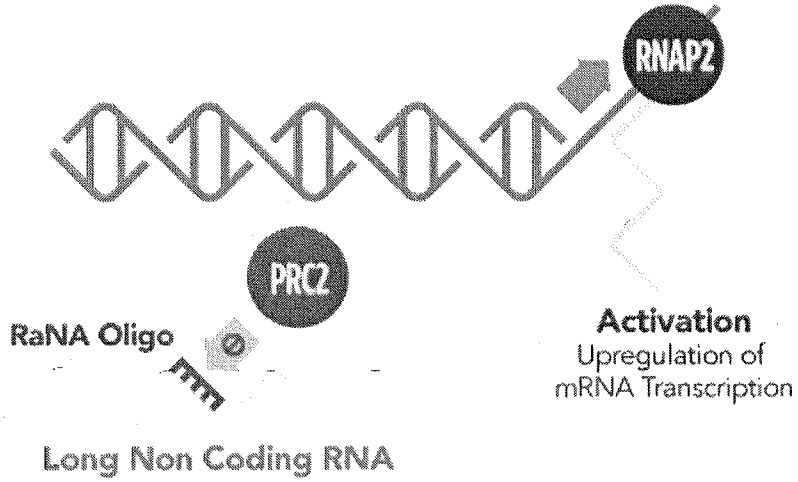
Research by scientists around the world, including RaNA Scientific Founder, Dr. Jeannie Lee,[1] (http://ranarx.com/science/references#ref1) has established the potential of targeting RNA to amplify the expression of beneficial proteins in the body's own cells. In particular, recent discoveries have shown that non-coding RNA perform a variety of critical regulatory functions in controlling gene expression, cell differentiation, and cell development and are increasingly recognized as having diverse regulatory effects on mRNA transcription.[2 (http://ranarx.com/science/references#ref2), 3 (http://ranarx.com/science/references#ref3)] RaNA's approach represents a pioneering step in the field of oligonucleotide therapeutics, targeting specific genes to precisely upregulate the expression of therapeutic proteins.

While the biopharma industry has focused for decades on drugs that inhibit or downregulate their targets, few effective ways exist to increase the amount of a therapeutic protein in the body. Some proteins can be manufactured and injected, but there are many more that are not easy to manufacture, that act intracellularly, or that have poor bioavailability, are unstable, or require complicated delivery methods. RaNA's drug molecules are designed to target RNA in order to selectively increase the expression of the desired protein.



One mechanism by which lncRNA suppress gene expression involves the recruitment of PRC2.

During transcription of the long non-coding RNA, a PRC2 binding domain is revealed, which recruits EZH2 and other epigenetic factors locally. PRC2 activity is then targeted to the nearby gene, suppressing gene transcription.



RaNA's proprietary technology upregulates the expression of desirable genes that can prevent or treat disease. RaNA's approach works epigenetically and reverses the endogenous repression of gene transcription.

Requiring no delivery vehicles, RaNA's therapeutic oligonucleotides can be administered as a subcutaneous injection in saline, and are then taken up by cells in most tissues of the body, crossing the endosome membrane to enter the cytoplasm and nucleus. RaNA's oligonucleotide selectively binds the lncRNA, blocking PRC2 recruitment and allowing for transcription to proceed, resulting in upregulation of the specific desired mRNA.

Learn More About RaNA's Revolutionary Science (http://ranarx.com/science)

Our Management

Based in Cambridge, Massachusetts, RaNA's team leverages aptitude and experience gained through an extensive record of success.

- **Ronald C. Renaud**
  CEO
  Ron has more than 20 years of experience serving in senior management positions in the biotechnology industry.

- **Jim Barsoum, PhD**
  CSO
  Jim has more than 25 years of experience in biotechnology drug discovery and development. Dr. Barsoum has generated protein, small molecule, and gene therapy drug candidates in multiple therapeutic areas.

Our Investors

RaNA received initial seed funding from Atlas Venture in 2011 and raised a Series A financing in January of 2012, which included Atlas Venture, MGH Partners Innovation, Monsanto, MS Ventures, and SR One.

- **Atlas Venture**
  Atlas Venture is a leading early-stage venture capital firm that invests in technology and life sciences companies. Since inception in 1980, Atlas has helped build over 350 companies in more than 16 different countries.

- **Monsanto**
  Monsanto Company is a leading global provider of technology-based solutions and agricultural products that improve farm productivity and food quality.

- **SR One**
  SR One is the independent corporate venture capital arm of GlaxoSmithKline. The firm invests globally in emerging life science companies that are pursuing innovative science which will significantly impact medical care.

- **MGH Partners Innovation**

- **MS Ventures**