CICEP | INNOVATION AND ECONOMIC PROSPERITY UNIVERSITIES AWARDS PROGRAM

CASE STUDY 2013

HocusLocus, LLC., the University at Albany and the College of Nanoscale Science and Engineering

In partnership with the University at Albany, HocusLocus, LLC. developed a trans-RNA switching mechanism called structurally interacting RNA or "sxRNA" that uses unique microRNA expression profiles to express desired proteins in a cell specific manner. sxRNA technology enables targeted expression of any chosen protein in cells of specific tissues, disease states, and developmental stages by coupling post- transcriptional regulation processes with unique microRNA signatures. The ability to target and control expression of a protein in-vivo using RNA, rather than DNA, opens up new possibilities for molecular tools, therapeutics, vaccines, and imaging applications.

Stem cells have enormous scientific and therapeutic potential, but are presently hindered by the difficulty of maintaining and ensuring their homogeneity in culture either in an undifferentiated state or as a uniform, definable, differentiated population. sxRNA could be used to negatively cull out differentiated cells while allowing undifferentiated cells to continue growing, or vice versa. Likewise, the ability to positively select for specific miRNA expressing cells would be valuable for directing stem cell differentiation to homogeneity.

The market for stem cells was $21.5 billion in 2010 and is expected to grow at a compound annual rate of 24%. Research tools supplied to this market is expected to reach $1.4 billion in size in 2013. There currently is no product on the market that allows for quality control at the cellular level.

This innovative approach for harnessing the power of gene expression has the potential to rival the expanding miRNA/siRNA/shRNA fields. While there are numerous methods to quantify miRNAs in vitro, quantifying miRNAs in vivo is difficult and tedious. We believe that our sxRNA based kits would be unique in the marketplace.

Beyond molecular tools, there are over 900 conditions resulting from the improper production of a protein including Cystic Fibrosis, Niemann-Pick disease, and Spinal Muscular Atrophy. sxRNA offers a way to target select cell types for corrected expression of proteins that are lacking. In addition to genetic disorders, many types of cancer have already been shown to have unique miRNA expression profiles. In some cases, miRNAs typically only expressed in embryonic cells are also expressed in metastasizing cancer cells, for example miR-373 and miR-520c. sxRNA could be used to target these cells with a death- gene, killing cancer cells selectively.

The sxRNA mechanism as a naturally occurring post-transcriptional regulatory mechanism was originally identified by Dr. Scott Tenenbaum and his lab at the State University of New York University at Albany College of Nanoscale Sciences and Engineering. The Tenenbaum lab has pioneered methods for immunoprecipitating RBP-RNA complexes and received a $2.5MM NIH grant as part of the Human Genome Project to study RBP activity. While studying the potential for miRNA/RBP cross-talk, the Tenenbaum lab identified several naturally occurring miRNAs that appear to stabilize or disrupt RBP binding sites using sxRNA principles. The ability to rationally design sxRNA switches to control RBP binding was patented by the University at Albany and exclusively licensed by HocusLocus, LLC.

HocusLocus has received a Phase I STTR from the National Institutes of Health to develop molecular tool applications using sxRNA and received an investment from the Eastern New York Angels.

The President of HocusLocus, LLC., Ted Eveleth, began his relationship with Dr. Scott Tenenbaum and SUNY in 2008 when he was asked to be the business lead on a team of individuals evaluating the commercialization potential of University Albany technology during a Pre-Seed Workshop. Initially skeptical of the commercial potential, over the 2 ½ day workshop, Ted became more enthusiastic of the technology and stayed in contact with Dr. Scott Tenenbaum, the inventor and now CNSE faculty member. U Albany filed patent applications and provided funding to further support the development of the technology. Then Ted was recruited as the first Technology Entrepreneur in Residence in the Albany Small Business Development Center. In that capacity he began providing one-on-one consulting and advice to Dr. Tenenbaum, which eventually led to Ted forming HocusLocus LLC. and licensing the technology into the company. The combination of Dr. Tenenbaum research and Ted's business savvy led to HocusLocus successfully receiving very competitive, STTR funding from the federal government, Dr. Tenenbaum receiving 2 rounds of SUNY TAF funding to support further research and development, and most recently led to HocusLocus receiving an investment from a local angel investment community. This is all to support the development and commercialization of a technology developed by at the University at Albany. None of this would have ever been realized if it was not for the partnership the University at Albany created with Ted Eveleth.