Washington University in St. Louis
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MIRNA MIMICS TO TREAT METABOLIC DISEASE

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Disease indication: metabolic disorders including insulin resistance and diabetic nephropathy

Drug format: chemically modified synthetic microRNA (miRNA)

Drug class: first-in-class for metabolic disease

Research stage and Preliminary data:

- *In vivo mouse model* – Injecting miR-29 mimics into db/db diabetic mice corrected diabetic nephropathy and insulin resistance. This included reduced albuminuria and water intake as well as improved hyperglycemia, glucose tolerance and insulin tolerance
- *Ongoing research* - Developing lead compounds by conjugating miR-29 mimics with carrier molecule

Target: miR-29 mimics can increase the gene expression levels of insulin receptor in peripheral tissues

Background: Defects in the insulin receptor (INSR) gene can cause insulin resistance and type 2 diabetes. This frequently leads to kidney damage (diabetic nephropathy) affecting millions of patients. Diabetic nephropathy has no effective treatment and often requires a kidney transplant.

Keywords: miRNA, microRNA, diabetic nephropathy, diabetic kidney disease, insulin resistance

Mode of action: This technology is based on the discovery that chemically-modified miR-29 mimic molecules can correct insulin resistance and diabetic nephropathy. These molecules increase the gene expression levels of INSR which increases glucose and insulin tolerance while improving kidney function. Compositions of synthetic miR-29 conjugated to carrier molecules can enhance delivery.

Competitive edge: miR-29 mimics could potentially correct the underlying genetic programs that are dysregulated in type 2 diabetes (unlike current therapeutics which offer palliative treatment of symptoms). In addition, miRNA mimics are easy to deliver and have low toxicity.

Patent status: U.S. Patent Application with claims allowed

Web Links: Hou Profile; Hou Lab