241 OCR 7557: MicroRNA-based Therapeutic for NASH

Asset Overview

| Product Type | Nucleic Acid |
|-------------------|-------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| Indication | NASH |
| Current Stage | Lead Identification/optimization |
| Target(MoA) | Inhibition of miR-TA1 |
| Brief Description | Non-alcoholic steatohepatitis (NASH) market across the 7MM* is set to grow from \$138.4M in 2016 to \$18.3bn in 2026. NAFLD is expected to become the most common chronic liver condition globally in relation to the obesity. The healthcare costs associated with NASH could rise up to USD 18 billion by 2030. We have developed a novel miR-TA1 inhibitor that protects against atherosclerosis and steatosis in the mice. Recent studies show that MicroRNAs contribute to pathogenesis of NAFLD/NASH at various levels of disease development and progression. |
| Organization | Yale University |

Differentiation

□ A novel Inhibitor of miR-TA1

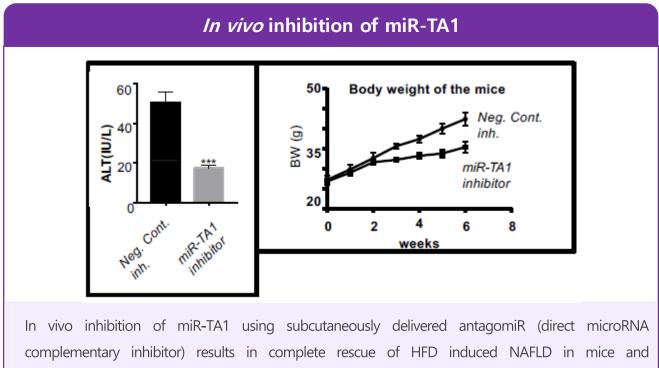
- NASH is associated with metabolic and cardiovascular disease, insulin resistance, dyslipidemia.
 MiR-TA1 promotes vascular inflammation, insulin resistance, obesity and fatty liver
- MiR-TA1 knockout mice are protected against fatty liver. Also, miR-TA1-/-/Apoe-/- mice are protected against atherosclerosis in mice
- The researcher have developed a novel miR-TA1 inhibitor for atherosclerosis and steatosis
- The new miR-TA1 inhibitor prevents accumulation of fat in arteries and in the liver

$\hfill\square$ Advantages of miR-TA1 inhibitor in NASH treatment

- · An inhibitior of miR-TA1 can be delivered by subcutaneously injection
- In vivo inhibition of miR-TA1 using subcutaneously delivered antagomiR (direct microRNA complementary inhibitor) results in complete rescue of HFD induced NAFLD in mice and normalization of ALT

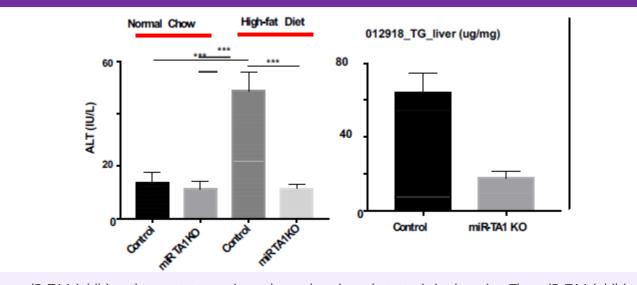
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Key Data



normalization of ALT.

MiR-TA1 KO mice are protected against fatty liver



miR-TA1 inhibitor that protects against atherosclerosis and steatosis in the mice. The miR-TA1 inhibitor prevents accumulation of fat in arteries and in the liver.

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Intellectual Property

| Patent No. | |
|------------------|--|
| Application Date | |
| Status | |
| Country | |

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