130 miR-494 inhibitor, for the treatment of retinal disease

Asset Overview

Product Type	Gene therapy
Indication	Retinal disease
Current Stage	Lead optimization
Target(MoA)	miR-494-3p
Brief Description	Epiretinal membranes (ERMs) are conditions where a very thin layer of scar tissue forms on the surface of the retina in an area that is responsible for our sharpest vision. A team of Stanford researchers have developed a non-surgical therapeutic strategy for treating or preventing epiretinal membranes or other eye diseases by inhibiting microRNA (miRNA). MicroRNAs are short (20-24 nt) non-coding RNAs that are involved in post-transcriptional regulation of gene expression in multicellular organisms by affecting both the stability and translation of mRNAs. The inventors have validated miR-494-3p as the only miRNA expressed at significantly greater levels in ERM tissue compared to controls.
Organization	Stanford University

Differentiation

□ **miR-494-3p as a target that can be used for the treatment of epiretinal membranes (ERMs)**

- Traditionally, epiretinal membranes (ERMs) or other eye diseases has been corrected through surgery with no noninvasive alternative
- Treating or preventing ERMs with a targeted therapeutic agent administered through an eye drop or intravitreal injection instead of surgery could lower the costs and the risk to patients
- This technology identifies miR-494-3p as a target that can be used for that non-invasive treatment to counteract the cellular transformation associated with ERM pathophysiology
- Pilot data shows miR-494-3p is selectively expressed in epiretinal membranes and that inhibiting this miRNA potentially offers an easier, safer and less expensive option for preventing, treating or reversing this type of ophthalmologic condition

□ **Easy and non-invasive ERM treatment**

- Compared with current ERM treatment (surgery), an miRNA inhibitor is likely to be administered via eye drops, resulting in:

  1. lower cost
  2. lower risk for patients (surgery poses risk of cataract and rhegmatogenous retinal detachment)

- The inventors have begun additional studies on the effects of a locked nucleic acid inhibitor of miR-494

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Key Data

miR-494-3p

MIR494 microRNA 494 [*Homo sapiens* (human)]

Gene ID: 574452, updated on 28-Oct-2019

Summary

Official Symbol	MIR494 provided by HGNC
Official Full Name	microRNA 494 provided by HGNC
Primary source	HGNC:HGNC:32084
See related	Ensembl:ENSG00000194717 MIM:616036; miRBase:MI0003134
Gene type	ncRNA
RefSeq status	PROVISIONAL
Organism	Homo sapiens
Lineage	Eukaryota; Metazoa; Chordata; Craniata; Vertebrata; Euteleostomi; Mammalia; Eutheria; Euarchontoglires; Primates; Haplorrhini; Catarrhini; Hominidae; Homo
Also known as	MIRN494; mir-494; hsa-mir-494

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Mature sequence hsa-miR-494-3p

Accession number	MIMAT0002816
ID	hsa-miR-494-3p
Stem-Loop	hsa-mir-494
Sequence	ugaaacauacacgggaaaccuc

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Intellectual Property

Patent No.	PCT-US2019-024090
Application Date	2019.03.26
Status	Application Pending
Country	

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