

NOVEL GENE THERAPY APPROACH TO TREATING LIVER FIBROSIS

Tech ID: 24912 / UC Case 2015-044-0

INVENTION NOVELTY

This invention establishes a new approach to treating liver fibrosis using gene therapy.

VALUE PROPOSITION

Liver fibrosis is a common complication of highly prevalent chronic liver diseases like hepatitis B and C, alcoholic liver disease and fatty liver disease. Liver fibrosis is a scar-like form of wound healing characterized by collagen deposition by myofibroblasts, a cell type absent from the normal liver. Although liver fibrosis progresses slowly, it eventually leads to liver cirrhosis, the main cause of liver failure, portal hypertension and liver cancer worldwide. In the US alone, liver cirrhosis affects approximately 400,000 patients (10 million worldwide) and accounts for more than 35,000 deaths annually. Because liver transplantation is limited by a donor organ shortage and effective drugs are lacking, new approaches to treating liver fibrosis are needed. The inventors developed a gene therapy approach that targets myofibroblasts in the liver and addresses the main outcome—determining factors of liver fibrosis, lack of functional hepatocytes and excess collagen.

This novel invention provides the following advantages:

- Repurposing of myofibroblasts **restores the functional hepatocyte mass** and **reduces liver fibrosis**.
- Potential for rapid clinical translation because of use of **nonintegrating, nontoxic adenoassociated viral vectors**.
- High efficiency and safety because of **specific targeting of hepatic myofibroblasts**.

TECHNOLOGY DESCRIPTION

Liver researchers at UCSF have developed a method for treating liver fibrosis by simple intravenous injection of adenoassociated viral vectors. The technology entails targeting adenoassociated viral vectors to myofibroblasts in the liver for transient expression of a combination of hepatic transcription factors in these cells. The approach generates new hepatocytes and at the same time reduces collagen deposition into the liver, thereby improving liver function and reversing liver fibrosis. Because adenoassociated viral vectors proved to be effective and safe in recent clinical trials of liver-directed gene therapy for hemophilia B, the technology lends itself well to clinical translation.

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OTHER INFORMATION

KEYWORDS

Liver Disease, Liver Fibrosis,

Liver Cirrhosis, Gene

Therapy, Adenoassociated

Viral Vectors, AAV

CATEGORIZED AS

- ▶ [Biotechnology](#)
- ▶ [Health](#)
- ▶ [Medical](#)
- ▶ [Gene Therapy](#)

RELATED CASES

2015-044-0, 2018-158-0

APPLICATION

Therapy for liver fibrosis

LOOKING FOR PARTNERS

To develop and commercialize this technology as a therapy for liver fibrosis

STAGE OF DEVELOPMENT

Preclinical

DATA AVAILABILITY

Under NDA/CDA

INVENTORS PROFILE

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PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Published Application	20210238257	08/05/2021	2018-158
European Patent Office	Published Application	3 784 224	03/03/2021	2018-158
European Patent Office	Published Application			2015-044
Patent Cooperation Treaty	Reference for National Filings	WO 2016/086227	06/02/2016	2015-044

Additional Patents Pending

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