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# 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.

## CONTACT

Gemma E. Rooney  
[Gemma.Rooney@ucsf.edu](mailto:Gemma.Rooney@ucsf.edu)  
tel: [415-625-9093](tel:415-625-9093).



## 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

Holger Willenbring, MD, PhD; <http://profiles.ucsf.edu/holger.willenbring>

PATENT STATUS

Country	Type	Number	Dated	Case
Austria	Issued Patent	3224362	09/25/2024	2015-044
Belgium	Issued Patent	3224362	09/25/2024	2015-044
Bulgaria	Issued Patent	3224362	09/25/2024	2015-044
Switzerland	Issued Patent	3224362	09/25/2024	2015-044
Germany	Issued Patent	3224362	09/25/2024	2015-044
Denmark	Issued Patent	3224362	09/25/2024	2015-044
Estonia	Issued Patent	3224362	09/25/2024	2015-044
European Patent Office	Issued Patent	3224362	09/25/2024	2015-044
Finland	Issued Patent	3224362	09/25/2024	2015-044
France	Issued Patent	3224362	09/25/2024	2015-044
United Kingdom	Issued Patent	3224362	09/25/2024	2015-044
Ireland	Issued Patent	3224362	09/25/2024	2015-044
Italy	Issued Patent	3224362	09/25/2024	2015-044
Lithuania	Issued Patent	3224362	09/25/2024	2015-044
Luxembourg	Issued Patent	3224362	09/25/2024	2015-044
Latvia	Issued Patent	3224362	09/25/2024	2015-044
Malta	Issued Patent	3224362	09/25/2024	2015-044
Netherlands (Holland)	Issued Patent	3224362	09/25/2024	2015-044
Portugal	Issued Patent	3224362	09/25/2024	2015-044
Romania	Issued Patent	3224362	09/25/2024	2015-044
Sweden	Issued Patent	3224362	09/25/2024	2015-044
Slovenia	Issued Patent	3224362	09/25/2024	2015-044
Unitary Patent	Issued Patent	3224362	09/25/2024	2015-044
United States Of America	Published Application	<a href="#">20210238257</a>	08/05/2021	2018-158
European Patent Office	Published Application	3 784 224	03/03/2021	2018-158

Additional Patents Pending

ADDRESS

UCSF

Innovation Ventures

600 16th St, Genentech Hall, S-272,  
San Francisco,CA 94158

CONTACT

Tel:  
innovation@ucsf.edu  
https://innovation.ucsf.edu  
Fax:

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