



RNA THERAPEUTICS FOR LIVER DISEASE

Restore liver function.  
Reopen the treatment  
options.

REGEN<sub>001</sub>

REGEN001 is designed to let a damaged liver heal  
itself again.



SEED ROUND 2026

# A billion people, one disease



1. WHO, Obesity and overweight, 2024.  
2. Teng et al., Global incidence and prevalence of NAFLD, 2023.  
3. Luo et al., Global burden of liver cirrhosis, 2025.  
4. Li et al., Global epidemiology of liver cancer 2022, 2024.

# Where liver cancer patients get stuck

Child-Pugh A

Score 5–6

**Treatment eligible**

Curative intent

Child-Pugh B

Score 7–9

**Eligibility narrows**

Palliative pathway

**Tumours under 3 cm** Removable — and liver function decides what can be offered

**Median survival** 6–13 months

**\$150k+ per patient** Wasted in palliative loops

A removable tumour, and a liver without the reserve that treatment requires. The barrier is hepatic reserve — not tumour stage, not anatomy.

HCC = hepatocellular carcinoma. The size of this subgroup is not established by the current evidence base and is not represented here as a fixed proportion of all HCC.

REGENo01 is designed to restore liver reserve, and reopen the approved treatment options

TODAY

Low liver  
reserve

8-12  
weeks

AFTER REGENo01

Reserve  
restored

REGENo01 is intended to restore the function that surgery, ablation, chemoembolisation and immunotherapy all require, so those options can open again.

# One gene, switched down



REGEN001 is a **GalNAc-conjugated siRNA** that **silences C/EBP $\beta$**  — a gene that drives lipotoxic injury in the liver. GalNAc carries it to liver cells specifically, so the drug acts where the disease is and nowhere else.

1

## Delivered

GalNAc conjugate targets hepatocytes

2

## Silenced

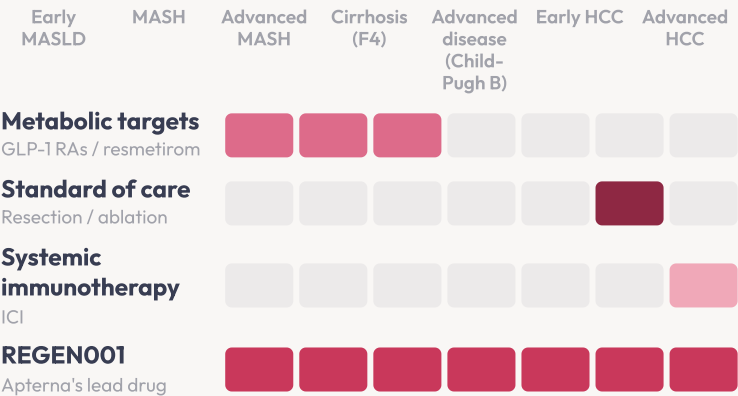
C/EBP $\beta$  expression is reduced

3

## Restored

The liver rebuilds its own functional reserve

# Designed to act at every stage



A single target with the potential to act at every stage: intended to reduce inflammation, preserve liver function, and support the liver's own repair capacity.

# A potential major market in liver disease

## 1 Eligibility unlock initial focus (SOM)

**100k–200k patients | £500M–£1.5B peak annual revenue**

Worldwide. Our realistic near-term target.

## 2 Cirrhosis & transplant avoidance adjacency

**120k–160k patients | £1.2B–£2.24B transplant market proxy**

Combines transplant avoidance and preserved liver function.

## 3 Fatty liver & obesity GLP-1 adjacency

**2.1B–2.8B patients | £5.9B today, £23.9B–£69.5B projected**

Largest layer, adjacent to the fastest growing drug class.

**Total addressable market £11.2B–£14.9B today, over £37B by 2035**

The large figures are the full category ceiling. The realistic near-term opportunity is the £500M–£1.5B treatment-eligibility unlock — where accelerated approval in a high-need orphan indication can bring the right to sell by year three, years ahead of a standard registration.

# What the investment funds

SEED ROUND 2026

Lead candidate optimisation and in vitro / ex vivo  
proof of concept

28-day GLP toxicology (rodent and NHP)

GMP manufacturing and analytical development

CTA / IND-enabling regulatory work and scientific  
advice

At the end of the seed round REGENoor is IND-ready,  
with the clinical trial application filed. Round size and  
allocation are available on request.

# They have built and sold this before

**Nagy Habib** MD, ChM, FRCS

**CO-FOUNDER, CHAIR & CEO**

- Professor of HPB Surgery and Head of the Department of Liver Surgery, Imperial College London
- Founded EMcision, acquired by Boston Scientific in 2018
- Co-founded MiNA Therapeutics — over \$100M raised through licence deals with Eli Lilly (\$1.25bn), Boehringer Ingelheim, Servier and BioMarin

**Declan Doogan** MD, FRCP

**CHIEF MEDICAL OFFICER**

- Former Head of Global Drug Development, Pfizer
- Co-founder and Chair of Biohaven, acquired by Pfizer for over \$11bn

**John Rossi** PhD

**CO-FOUNDER · DISCOVERY AND DEVELOPMENT**

- Co-founder of Dicerna, acquired for \$3.3bn by Novo Nordisk
- Chairman and Professor, Beckman Research Institute of City of Hope

Joanna Nicholls — Chief Operations Officer, co-founder of EMcision and MiNA Therapeutics

Kai-Wen Huang, MD, PhD — in vivo oncology, National Taiwan University



A proven platform, a clear  
unmet need,  
and a defined path

**A defined path from here to the clinic.**



Prof. Nagy Habib

**[nagy@apterna.com](mailto:nagy@apterna.com)**

---

**Apterna Ltd**

London, United Kingdom