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Australia leads first-in-human trial of cholesterol-lowering genetic therapy

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Phase 1 trial will evaluate STX-1150 in adults with elevated LDL cholesterol and increased cardiovascular risk

The Victorian Heart Hospital and the Victorian Heart Institute will lead a first-in-human clinical trial of a new investigational genetic therapy that aims to lower cholesterol in people at increased risk of cardiovascular disease.

The Victorian Heart Hospital, operated by Monash Health in partnership with Monash University, Australia, will be the first clinical trial site globally to begin testing the investigational therapy STX-1150, developed by Scribe Therapeutics.

The therapy is designed to reduce LDL (‘bad’) cholesterol by targeting a gene in the liver called PCSK9, a well-established regulator of cholesterol levels and cardiovascular risk. Elevated LDL cholesterol is a major cause of atherosclerotic cardiovascular disease, including heart attacks and strokes.

STX-1150 uses a next-generation CRISPR-based approach known as epigenetic silencing. Delivered as a one-time infusion, the therapy is designed to reduce cholesterol levels for an extended period without permanently altering a person’s DNA.

The Phase 1 study will assess the safety, tolerability and biological effects of STX-1150 in adults with elevated LDL cholesterol who are at increased cardiovascular risk.

The trial plans to enrol up to 64 participants across sites in Australia and New Zealand, with participants monitored for one year following treatment.

The study follows regulatory clearance from Australia’s Therapeutic Goods Administration and builds on growing international interest in genetic approaches to cardiovascular disease prevention.