Scribe Therapeutics Achieves Regulatory Clearance to Initiate First-in-Human Clinical Study of STX-1150 for LDL-C Reduction

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ALAMEDA, Calif., May 21, 2026 – Scribe Therapeutics, Inc. (Scribe) a clinical-stage biotechnology company engineering purpose-built *in vivo* CRISPR technologies designed to extend healthy lifespan through disease prevention and durable therapeutic intervention, today announced that it has secured clearance from the Australian Therapeutic Goods Administration (TGA) to initiate a first-in-human clinical study of STX-1150 for the treatment of hypercholesterolemia, a major driver of atherosclerotic cardiovascular disease (ASCVD).

Cardiovascular disease is the leading cause of death worldwide, with elevated low-density lipoprotein cholesterol (LDL-C) recognized as a key causal driver of atherosclerosis and ASCVD. Despite the availability of multiple lipid-lowering therapies, many patients remain above recommended LDL-C levels in real-world settings due to treatment burden, adherence challenges, delayed treatment initiation, and the need for ongoing chronic administration. STX-1150 is a novel, *in vivo* therapy designed to address these limitations by epigenetically silencing PCSK9, a genetically and clinically validated regulator of LDL-C, in the liver. Designed to deliver sustained LDL-C lowering after a single dose without permanently altering the underlying DNA, STX-1150 has the potential to support early and long-lasting ASCVD risk reduction for patients with elevated LDL-C and increased cardiovascular risk.

“Advancing STX-1150 into the clinic marks a pivotal moment for Scribe,” said Benjamin Oakes, Ph.D., co-founder and Chief Executive Officer of Scribe Therapeutics. “Years of iterative engineering to enhance the potency and specificity of our CRISPR technologies has culminated in our first clinical program designed to provide access to the effects of known cardioprotective genetics, potentially freeing patients from decades of treatment burden. This milestone reflects our commitment to developing ultra-long-acting therapies that may redefine the standard of care, reduce adherence challenges, and help make earlier intervention and long-term prevention more practical for patients with chronic cardiometabolic disease.”

The planned Phase 1 study will evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of STX-1150 in adults with elevated LDL-C at increased cardiovascular risk. The study is designed as an open-label, single ascending dose trial followed by a dose expansion phase, with planned enrollment of up to 64 participants across trial sites in Australia and New Zealand. Participants will be monitored for one year after treatment. The first clinical site will be Monash Health’s Victorian Heart Hospital in Clayton, Victoria, Australia. The study will be led by Principal Investigator Stephen Nicholls, MBBS, Ph.D., a world-renowned cardiologist who currently serves as the Director of the Victorian Heart Institute at Monash University and Program Director of the Victorian Heart Hospital at Monash Health.

“The initiation of Scribe’s first-in-human study reflects a critical advance in the development of much-needed, highly durable approaches to LDL-C reduction,” said Dr. Stephen Nicholls. “Targeting PCSK9 with an innovative epigenetic silencing strategy, STX-1150 offers a differentiated approach to long-term lipid management. I look forward to working with Scribe to evaluate this therapy in patients with elevated LDL-C and increased cardiovascular risk.”

Scribe will deliver a late-breaking oral presentation highlighting the preclinical data for STX-1150 at the 94th European Atherosclerosis Society Congress, taking place May 24–27, 2026, in Athens, Greece. Additional details can be found on Scribe’s website.

About STX-1150

STX-1150 is a novel, investigational, epigenetic silencing therapy designed to durably reduce LDL-C by silencing transcription of *PCSK9*, a genetically and clinically validated regulator of cholesterol. The therapy leverages Scribe’s proprietary ELXR technology to enable long-lasting gene silencing in the liver without permanently altering DNA. STX-1150 consists of a messenger RNA encoding a highly engineered ELXR and a single guide RNA that targets the *PCSK9* gene encapsulated by a liver-tropic lipid nanoparticle licensed from Acuitas Therapeutics.

About Scribe’s Epigenetic Long-Term X-Repressor (ELXR)

ELXR is an epigenetic silencing technology designed to durably yet reversibly silence gene expression without altering the underlying DNA sequence. Built on a nuclease-inactivated, highly engineered CasX-derived CRISPR protein fused to epigenetic effector domains, ELXR installs targeted histone and DNA methylation marks to enable long-term silencing while maintaining genomic integrity. ELXR also incorporates allosteric regulation designed to enhance both specificity and on-target activity, differentiating it from other epigenetic editors used in the field or in the clinic.

Epigenetic silencing technologies such as ELXR may represent the next evolution of mRNA-reducing therapies, potentially overcoming the durability and adherence limitations of existing modalities such as siRNAs and ASOs. Unlike therapies that require

chronic dosing every few weeks to months, ELXR is designed to enable sustained, clinically meaningful mRNA reduction for years following a single administration, without permanently altering the underlying DNA. The durability, reversibility, and specificity inherent to epigenetic silencing may make ELXRs particularly well-suited for applications where permanent genome modification is less desirable, and long-term adherence and consistent target suppression are critical determinants of outcomes.

About PCSK9 and its Role in Elevated LDL-C

Elevated low-density lipoprotein cholesterol (LDL-C), often referred to as “bad” cholesterol, is a primary contributor to ASCVD. Inhibition of PCSK9 is among the most effective known mechanisms to reduce LDL-C, complementing or outperforming existing therapies such as statins, bempedoic acid, ezetimibe, and emerging CETP inhibitors. Individuals born with loss-of-function variants in the *PCSK9* gene live with meaningfully lower baseline LDL-C and experience up to 88% lower risk for coronary heart disease without any distinguishable adverse effects from lifetime lower LDL-C levels.

About ASCVD and the Need for an Improved Treatment Paradigm

Despite a wide variety of approved therapeutics, cardiovascular disease remains the leading cause of death worldwide and impacts over 120 million individuals in the United States alone. Every 40 seconds, someone in the United States suffers a heart attack.

Elevated levels of low-density lipoprotein cholesterol (LDL-C), lipoprotein(a), and triglycerides are key lipid drivers of atherosclerosis and ASCVD. Hypercholesterolemia, an excess of LDL-C in the bloodstream, promotes plaque formation in the arterial walls, restricting blood flow and increasing the risk of heart attacks and strokes. Despite major advances in the development of new classes of lipid-lowering therapies, today’s standard of care for ASCVD treatment and prevention is insufficient. Existing treatments struggle to demonstrate broad impact as they suffer from significant lack of durability, diminishing levels of adherence-adjusted efficacy, and onerous treatment burdens including well-documented side effects. All of this leads to poor uptake, low adherence, and limited real-world effectiveness. Moreover, treatment is often initiated only after decades of substantial silent cumulative arterial injury or an acute cardiovascular event. These limitations underscore the importance of developing durable therapies that can be administered safely earlier in the course of disease. Scribe’s goal is to develop CRISPR-based therapeutics that are safe, effective, durable, and scalable enough to preemptively reduce lifetime cardiovascular risk with the aim to reduce the global burden of ASCVD.

About Scribe Therapeutics

Scribe Therapeutics is a clinical-stage biotechnology company engineering CRISPR-based technologies into purpose-built *in vivo* genetic medicines designed to become standard of care treatments for patients suffering from highly prevalent diseases, starting with cardiometabolic disease. Leveraging its CRISPR by Design™ approach and nature’s blueprint for improved cardiovascular health, Scribe’s initial programs focus on addressing the key lipid drivers of ASCVD such as elevated LDL-C, lipoprotein(a), and triglycerides. The company’s lead candidate, STX-1150, is a novel liver-targeted therapy designed to epigenetically silence the *PCSK9* gene and reduce LDL-C levels without inducing permanent DNA changes. To broaden and accelerate the impact of its engineered CRISPR technologies for patients, Scribe has formed strategic collaborations with world-leading pharmaceutical companies including Sanofi and Eli Lilly. Co-founded by Nobel Prize winner Jennifer Doudna and backed by leading life sciences investors, Scribe is advancing scalable, transformative, and preventative genetic medicines with the goal of improving outcomes and democratizing access to the protective effects of beneficial human genetics. To learn more, visit www.scribetx.com.