MyCardioAdvocate™

Gene Editing & CV Medicine

The therapies that could change everything

Why This Matters

Gene editing represents a fundamental shift in how we approach cardiovascular disease. Rather than managing cholesterol for a lifetime with daily medications, CRISPR and base editing technologies offer the potential to correct the underlying genetic defects that drive conditions like familial hypercholesterolemia (FH) and elevated lipoprotein(a). VERVE Therapeutics and others are advancing therapies that could become one-time treatments—editing your genes once and then stepping back. This is not distant science fiction; human trials are underway now.

Why Gene Editing Flies Under the Radar

Gene editing doesn't fit into traditional medicine's framework. There's no annual prescription renewal, no chronic medication market. The pharmaceutical industry is built around repeating revenue—pills you take forever. A one-time curative treatment upends that model. Additionally, gene therapy sits at the intersection of science, ethics, and regulation. Fear of adverse effects, long-term unknowns, and legitimate ethical questions (who gets access? What about off-target effects?) keep these therapies in the headlines but out of most doctors' treatment algorithms. Most cardiologists have not yet integrated gene editing into their mental model of how to manage familial disease.

MyCardioAdvocate™ Checklist

1. Understand what gene editing is (and what it isn't)

CRISPR cuts DNA at a targeted site; base editing makes single-letter changes without a double-strand break. These are not the same as gene therapy (which adds a new gene) or the Hollywood version of designer babies. PCSK9 gene silencing (turning down the protein that pulls LDL receptors from cell surfaces) is the first cardiovascular target in human trials.

2. Know which clinical trials are active now

VERVE-101 (intravenous base editing of PCSK9 in FH) is enrolling. If you have severe FH with inadequate LDL control, ask your doctor about eligibility. These trials have strict inclusion criteria and rigorous safety monitoring. Access is limited and selective.

3. Grapple with ethical and practical questions

One-time treatments raise questions about long-term durability, off-target effects, cost, equity, and access. What happens if the edit fails after 20 years? Who pays for a $2M treatment? These are real questions your team should be thinking through, not reasons to dismiss the technology.

On the Horizon

- VERVE-101 base editing trial results for PCSK9 silencing in FH
- Next-generation approaches targeting Lp(a) genes directly
- Advancement of in vivo (in-body) vs. ex vivo (cell extraction) editing methods

Key Takeaways

- **Gene editing is real and actively in trials.** Familial hypercholesterolemia is the first target, but Lp(a) is next.
- **One-time treatment changes the equation.** Lifestyle, adherence, and cost-per-lifetime shift dramatically vs. lifelong therapy.
- **This is a shared decision.** Understand the trial design, safety data, and your eligibility before committing to an experimental therapy.

Next Steps & Related Content

- Ask your cardiologist: Is there a gene editing trial I might qualify for?
- Review VERVE Therapeutics' clinical trial criteria and safety updates
- Understand your genetic risk: Do you have FH? Is Lp(a) elevated?
