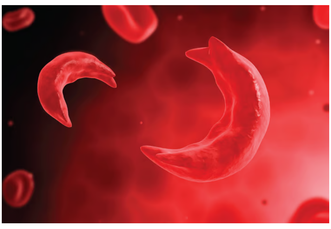
The Cure Is Real. The Bottleneck Isn't Scientific. So Why Is San Rocco Missing From the Story?



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Two years after the U.S. Food and Drug Administration (FDA) first approvals of gene therapies for sickle cell disease, the central question is no longer whether cures are possible. It is who actually gets cured—and at what cost. That paradox sits at the center of a recent Politico investigation: spectacular science constrained by multimillion-dollar price tags, scarce treatment infrastructure, and payer bureaucracy so burdensome that many patients—especially Medicaid patients and those outside major metros—remain stuck in line.

Yet one name remains largely absent from the mainstream conversation: San Rocco Therapeutics, a developer that *TrialSite* has chronicled for years as a would-be "access-first" entrant in sickle cell and thalassemia gene therapy. Although now entering later stage clinical trials, the omission matters, because if the policy debate is truly about affordability and scalability, then ignoring a company explicitly positioning itself as lower-cost is not a neutral editorial choice—it shapes what solutions the public is even allowed to imagine.

What Politico Gets Right—and What It Leaves Out

Politico correctly identifies the primary barriers facing first-generation sickle cell gene therapies:

- **Seven-figure list prices** (CMS cites $2.2M for CASGEVY and $3.1M for LYFGENIA).
- **Delivery complexity**, including intensive conditioning chemotherapy and long, disruptive care journeys.
- **A limited network of qualified treatment sites**, with rural access especially thin.
- **Medicaid budget shock**, even under new payment experiments meant to soften the blow.

It also highlights something politically unusual: a rare zone of overlap where a Trump-era access push, CMS program mechanics, and much of the public-health establishment converge on the same stated goal—getting curative therapies to patients who have historically been underserved.

But the mainstream frame can make today's costs and logistical burdens feel inevitable—as if gene therapy must be unaffordable and geographically gated. That assumption and editorial approach, quietly sidelines challengers whose core pitch is that the bottleneck is solvable—by design.

A Company Built for Access—Before "Access" Became the Headline

San Rocco Therapeutics (formerly Errant Gene Therapeutics/EGT) is not a typical venture-cycle celebrity brand. It is closely associated with patient-driven advocacy, including founder Patrick Girondi, whose family story is tied to beta-thalassemia. *TrialSite* reports that San Rocco's platform is based on a lentiviral gene-addition approach and includes long-term investigational follow-up data from earlier clinical work. Plus Health and Human Services Secretary Robert F. Kennedy Jr. and administration have publicly announced support for San Rocco.

San Rocco's cell therapy product, Minirolu, is now being advanced via updated clinical protocols, intended to improve efficacy while reducing toxicity and cost compared with existing commercial options. Those claims—greater potency in human cells, lower-intensity conditioning, and a target price substantially below $1 million—are consequential if they hold up in regulated clinical outcomes, because they would attack the exact constraints Politico recently described.

If the national thesis is "access is the crisis," then a serious access-focused contender should be part of the national story—whether or not it is already approved.

Why the Silence Persists: Coverage Follows Approval, Capital, and Institutions

Mainstream reporting often defaults to what is easiest to validate on deadline:

- FDA-approved products
- Large public companies, or well-funded VC-backed ventures
- Established treatment centers
- Well-resourced advocacy organizations
- Familiar corporate narratives

This creates a predictable blind spot: non-approved challengers—even those with patient-centered affordability claims and solid if not potentially superior science—can be treated as peripheral until a regulatory milestone forces attention. The result is a public debate trapped inside the boundaries set by current incumbents.

The Backstory Few Want to Touch: Incumbents, Institutions, and Litigation

TrialSite's San Rocco coverage also points to a longer and more contentious history involving bluebird bio, major academic institutions, and disputes that San Rocco supporters argue shaped the commercial landscape long before today's pricing crisis became front-page news. Mainstream outlets tend to avoid this terrain: it is messy, legally fraught, and disruptive to the preferred "heroic innovation" storyline.

But if the U.S. is now building payment models to manage $2–$3 million cures, the public deserves to understand why the market arrived at that price structure—and why lower-cost narratives rarely survive the transition from clinic to commerce. Why is the momentum of San Rocco now as it marches down the clinical trials path in both America and Europe not more widely covered?

Payment Models Help. But They Don't Fix the Root Problem.

CMS's Cell & Gene Therapy Access Model is a real step: it aims to expand Medicaid access using outcomes-based arrangements and standardized agreements with manufacturers, with state participation rolling in from 2025 into 2026.

But payment innovation is not the same as affordability. If therapies remain priced in the millions and require scarce, specialized infrastructure, the system may simply learn to finance scarcity more elegantly—while most patients still wait.

That is why the absence of San Rocco from mainstream coverage is not a small oversight. It is a symptom of a larger pattern: **we debate how to pay for expensive cures more often than we demand cures that are designed to be affordable.**

The Deeper Issue: Who Are These Cures Really For?

Sickle cell disease disproportionately affects Black Americans and many patients rely on Medicaid. A cure that exists but remains inaccessible is not a clean victory—it is a moral contradiction with a policy aftertaste. Politico's reporting captures the lived reality of that contradiction.

San Rocco's supporters pose an uncomfortable question that mainstream coverage rarely engages head-on:

If multiple scientific paths can work, why does the public conversation accept the most expensive path as the default—and treat lower-cost challengers as invisible until approval?

Megan Euker, VP San Rocco Therapeutics responded to a *TrialSite* inquiry on the topic via an email today:

"Cures are locked behind paywalls built by insiders. Families are told that survival of their loved ones has a tall price tag. The media's silence on San Rocco Therapeutics isn't oversight—it's intentional protection of a cowardly system commanded by unbridled greed."

Bottom Line

The cure is real. The science appears to be working. The bottleneck is not biology—it's economics, infrastructure, and public attention.

If the U.S. is serious about democratizing gene therapy for sickle cell disease and thalassemia, the conversation cannot remain limited to financing today's incumbents. It must also scrutinize the contenders claiming they can deliver cures at a price and scale Medicaid—and real families—can actually sustain.

Pharmaceuticals | Cell Therapy | Thalassemia | Sickle Cell | San Rocco Therapeutics

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