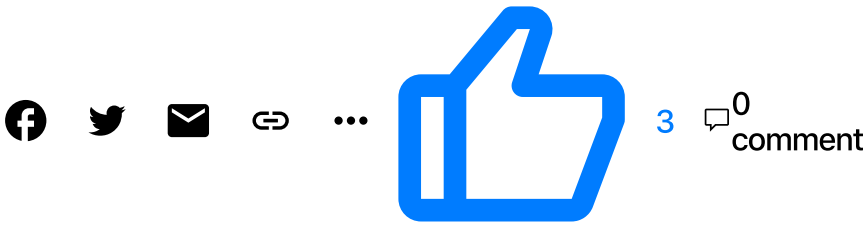
LA Times Piece Glosses Over Gritty Realities Beta Thalassemia Gene Therapy: Bluebird Bio vs. San Rocco Therapeutics



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Recently, a prominent journalist from the Los Angeles Times who launched an inaccurate hit piece against ivermectin last year, Michael Hiltzik, was again reporting part of the actual story involving another drug development scandal. The reporter covered the important, but little-known topic of the approximately 1,500 Americans that suffer from a rare blood disorder called beta-thalassemia, and the recent August 17 regulatory approval of Third Rock Ventures' funded Bluebird Bio's gene therapy called Zynteglo. The U.S. Food and Drug Administration (FDA) recently approved what Hiltzik refers to as the "remarkable" gene therapy, a "life-changing development for those with the inherited condition." These patients face a debilitating situation, one forcing them into bi-weekly blood transfusions. Hiltzik does, however, rightfully

ponder publicly the $2.8 million per treatment price point established by the Cambridge, Massachusetts-based biotech venture Bluebird. The Los Angeles Times scribe reports Zynteglo is but one of many forthcoming high-priced, niche-targeted gene therapies headed to the FDA for authorization in the coming years. The economic and financial implications alone are astounding, but the potential to improve and even save human life are as well. Omitted in the article covering gene therapy-targeting beta-thalassemia patients is the struggle an upstart biotech faces working to deliver critical competition in the form of what the founder of San Rocco Therapeutics suggests is a superior product priced far lower. While SRT's investigational gene therapy product is now ready to go into clinical trials, seemingly treacherous behavior associated with the biotech market continues to inhibit the advancement of what could be a better, more economical gene therapy product. No mention of this important point surfaces in Hiltzik's account.

Before delving into some of the more insidious affairs of Bluebird Bio (Bluebird) along with the competition from San Rocco Therapeutics (SRT), a review of Hiltzik's piece ensues. After correctly describing the plight of those with beta-thalassemia, the reporter raises the multiple issues that "swirl" around Bluebird's Zynteglo, including the economic "sticker shot" associated with the $2.8 million per treatment price point.

Talking to some experts that shine further light on the problem, Hiltzik suggests the prospect of a "national reinsurance program," allowing the government to take

on and cover the prohibitive cost of these niche, gene-based therapies. Yet he acknowledges the limitations associated with such a scheme, noting much of healthcare is owned by the states.

Discussing pricing challenges, such as basing gene therapy pricing based on projected downstream savings generated by the novel therapies, David Rind the chief medical officer of the Institute for Clinical and Economic Review (ICER), the nonprofit that conducts drug pricing analyses, suggests such pricing schemes means "you've become locked into the cycle of paying high prices for new therapies because you were already overpaying for old therapies."

Bluebird Troubles in Europe

By 2019, writes Hiltzik, Bluebird attempted to bring Zynteglo in front of the European Medicines Agency (EMA) at $1.8 million per treatment or according to the writer, "About twice what European regulators anticipated." But the European regulators would have none of it, threatening to limit access to patients, reports Mr. Hiltzik.

Then, Bluebird opted out of the German market, as the rigorous, methodical Germans didn't find the price points rational. At the same time, reports of adverse events associated with the gene therapy didn't help the company's position. But Hiltzik stayed away from the explicit details.

Now America's priciest drug, the gene therapy known as Zynteglo, hasn't led to fortunes for the biotech company

maker—Bluebird, notes the *Los Angeles Times* piece.

While the biotech's stock price spiked by March 2018, *TrialSite's* coverage of this space highlighted the competitive tension with the gene therapy maker founded by Mr. Patrick Girondi, Errant Gene Therapies (EGT), now called San Rocco Therapeutics (SRT), which interestingly, again was completely bypassed by the *Los Angeles Times* article. Importantly, Bluebird Bio's stock traded at 110.59 on March 1, 2018, and trades at 7.14 today.

The Family Man

TrialSite was first introduced to Bluebird Bio, its aggressive, some might even label predatorial investors and the relationship with Memorial Sloan Kettering (MSK) first in the piece titled "*A Familyman's Battle Against the Forefront of Capitalist Medicine: The Case of Errant Gene Therapeutics*," which discussed founder Patrick Girondi, a streetwise musician who grew up on Chicago's gritty working-class Southside neighborhoods before becoming an American success story, thanks to a successful career involving commodity trading. Girondi would eventually become a biotech entrepreneur. The latter transformation occurred quite unexpectedly. When he found out his son Rocco was diagnosed with beta-thalassemia, the unique, charismatic, and hard-charging businessperson went full throttle into the high-pedigree world of biotech.

It was during that process that Girondi discovered the levels of what is the cut-throat, bare-knuckled culture involved at the nexus of finance capital, biotech-

sponsored drug development, and academic medical centers—a ruthless, yet sophisticated world that can lead to amazing cures as well; one that would make the toughness of South Chicago look amateurish.

Driven by a passion and a mission to find a cure for his son, Girondi learned the business and research of biotech on his own—finding experts in the field of gene therapy. Ultimately, Girondi founded ERT, now SRT, securing a license to a competitive gene therapy, while launching studies that would, according to Girondi, showcase a superior gene therapy to that of Bluebird. That led to what became a David vs. Goliath battle, pitting a startup biotech entrepreneur against a cabal involving industry, finance capital, and academia. Court battles have ensued, and Girondi isn't faring too badly so far. Maybe the scions of biotech and private equity underestimate the rigor, passion, and drive of the Family man?

Girondi won a case against MSK involving an undisclosed settlement. Yet as *TrialSite* recently covered, while SRT has the licensure from MSK and the capital and means to now conduct clinical trials, the prestigious New York-based academic medical center isn't releasing the gene therapy vector.

As it turns out, MSK added an improvement, an insulator, that apparently, may make this class of gene therapy safer. As Girondi told *TrialSite's* founder Daniel O'Connor in an interview posted on *TrialSite's* YouTube channel, public dollars were used to fund the enhancement to the same underlying intellectual property that his company has rights to via the settlement agreement. Why won't MSK simply hand over the vector and let SRT clinically

develop the intellectual property? Isn't the whole point to bring better therapies to patients?

On August 24th, Girondi contributed an opinion piece to *TrialSite*, calling out Bluebird and associated investors for what he believes are among other things, "Criminals in Healthcare."

And in his probing of the Bluebird story, why didn't *Los Angeles Times'* Hiltzik cover this very recent and highly relevant set of facts? For example, Hiltzik's piece doesn't even acknowledge that San Rocco Therapeutics and the clash with Bluebird even exists. The first telling sign of a form of censorship by mainstream media.

Why wouldn't a notable journalist do even a scan of the competitive clinical pipeline? Given Hiltzik's purported interest in price gouging, wouldn't he want to note it's public record that SRT's proposed market price of $700,000 is orders of magnitude lower than the now approved $2.8 million associated with Zynteglo?

A Superior Potential Competitor?

The article also fails to mention that in association with the SRT gene therapy vector, since 2012, two out of three patients treated with myelosuppression and the SRT investigational product produced in 2009-2019 have no adverse effects reported and a decrease in transfusions by 43%.

But there is more. While Hiltzik briefly touches on adverse effects associated with the Bluebird gene therapy product now approved, he doesn't delve into any details.

Actually, Zynteglo is associated with adverse events, including patients with Clonal Dominance (a precursor to cancer), Myelodysplastic Syndrome, and Leukemia. By December 2021, *Fierce Pharma's* Angus Liu wrote about yet again another FDA hold against the gene therapy as the company acknowledged in a press release. Why didn't Hiltzik discuss these incidents?

Hiltzik didn't cover any of the legal skirmishes involving the gene therapy's intellectual property status. SRT secured the IP from MSK. Now, at least some experts at MSK and elsewhere suggest that lentiviral vectors are safer with an insulator. See the piece in Cell on this topic.

Interestingly, as reported recently by *TrialSite,* while San Rocco Therapeutics (SRT) secured a settlement with MSK and the rights to the lentivector gene therapy for beta-thalassemia, the academic medical center continues to refuse access to the vector, despite its obligation as part of a legal settlement. Is it because MSK added an insulator (thanks to public funds)which improves the investigational product? Are they holding back for other reasons?

Why wouldn't they follow through on what was settled? Who are they aligned with behind the scenes? Why would the *Los Angeles Times* totally bypass such a related story?

According to a recent discussion with San Rocco Therapeutic CEO Patrick Girondi:

"*Bluebird misappropriated SRT technology to attempt to fix their faulty vector which uses a mutant gene. We are*

currently in a lawsuit for patent infringement—as a result of Bluebird buying the wrong product, the misappropriation, sabotage, and cost investors billions. The stock fell from over 200 with a $12b market capitalization to under a million. But there is a bigger victim in all of this—the entire country. People lose faith in our system, and we all suffer greatly."

TrialSite has again previously chronicled the ongoing "David vs. Goliath" battle of SRT against Bluebird and Third Rock Ventures, a battle needing more transparency in mainstream media. *TrialSite's* founder Daniel O'Connor shared, "More of these stories need to make it in front of each and every American."

Big Money & Big Pharma Don't Necessarily Equal Patient Benefits

While *TrialSite* has chronicled the pharmaceutical industry's use of misinformation, promulgated and backed by various lobbying and donations to politicians, not to mention the externalization of costs of drug development along with the regular usurpation of revenues/profits (the pandemic is an extreme edge case), this media notes that while pharmaceutical incentivization is important in the high risk, high-stakes world of drug development, raw profiteering isn't a flattering look and will lead to a severe societal pushback.

That lobbyists favor Big Pharma shouldn't be a surprise. According to one analysis, over 60% of pharmaceutical industry, lobbyists were at one-time government officials while industry pumps billions into political funds. A *STAT* analysis revealed that over two-thirds of

Congress had cashed a check from the pharmaceutical industry ahead of the 2020 election.

On the other hand, some Senators such as the controversial Elizabeth Warren, Amy Klobuchar, and even Senator Bernie Sanders are but a few politicians, as well as Dr. Lucio Luzzatto and Professor Eugene McCarthy represent a small group of folks fighting for each and every patient. A courageous artist and SRT's Project Manager, Megan Euker, demonstrated at the International Museum of Surgical Science in Chicago the allegations of criminal acts ("The Cure," 2019-2020) associated with the business of drug development.

Back to the Courts

Now, SRT is back in courts trying to ensure it can access the gene therapy plus the bolt-on insulator to test in humans. With desperate patients waiting, MSK has a batch of gene therapy vectors ready to go in their freezers. Moreover, an investigational new drug (IND) application has been authored. SRT and MSK had already settled a lawsuit affording the former's access to the latter's gene therapy via existing licensure. What could very well be the best pathway for patients is unfortunately in limbo due to MSK's refusal to relinquish the vector to SRT, the established licensee, while MSK doesn't offer any alternative funding scenarios.

Something is truly rotten in the state of healthcare while mainstream media offers a slice of the reality, but most definitely not an all-encompassing access to truth. Perhaps that truth is too inconvenient.

Call to Action: Taxpayers, patients, and family members/loved ones should consider writing state Attorney Generals, the SEC, and elected officials about situations such as this. It's time to back taxpayers, patients, and families over industry lobbies for matters of urgent public health.

Blood Drug Development Gene Therapy

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