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Sarepta Therapeutics, Inc

Pricing in the Tail Risk: An Analysis of Potential Negative Elevidys Catalysts

Key takeaways:
Elevidys, Sarepta Therapeutics' (NASDAQ: SRPT) breakthrough gene therapy for Duchenne Muscular Dystrophy (DMD), once Wall Street's prime example of innovative precision biologics, has been increasingly scrutinized. With FDA accelerated approval in mid-2023 and market expectations of $2.9–3.1B in potential peak sales, Elevidys was hailed as a blockbuster in gene therapy. Sarepta's valuation swelled on the promise of durable treatment, a first-mover advantage, and its broader gene therapy platform.

But this year, share prices have fallen 87% YTD. Reports of non-ambulatory patient deaths, emerging safety concerns, and regulatory uncertainty triggered a market reassessment of Elevidys and the entire gene therapy investment thesis.

This report provides a postmortem on Elevidys, dissecting the key events and structural risks that unraveled investor confidence. I break down:

- The clinical trial design behind Elevidys including its surrogate endpoints, patient population distinctions (ambulatory vs. non-ambulatory), and regulatory pathways.
- The timeline of safety events, including reported patient deaths and liver toxicity concerns.
- The FDA's evolving stance and its broader implications for the company.

I then analyze the financial impact using a hybrid DCF/NPV and SOTP framework to evaluate the current share price. Finally, I'll explore whether SRPT represents a value opportunity or whether the FDA risks and scrutiny will spill over into Sarepta's PMOs and LGMD pipeline.

Background:

Company Overview: Sarepta Therapeutics (NASDAQ: SRPT) is a U.S.-based biotechnology company specializing in the development of precision genetic medicines for rare neuromuscular diseases. The company's foundational assets are based on exon-skipping antisense oligonucleotides (PMOs), including Exondys 51, Vyondys 53, and Amondys 45, which together generate ~$900M in annual revenue treating subsets of Duchenne Muscular Dystrophy (DMD). Over the last several years, Sarepta has pivoted toward gene therapy, investing heavily in AAV-based platforms. This transition culminated in Elevidys, which was granted FDA accelerated approval in June 2023[1]; the first approved gene therapy for DMD in the U.S.

PMO franchise: Together, Exondys 51, Vyondys 53, and Amondys 45 serve roughly 25% of the DMD population.[2,3] Sales have been relatively stable, with ~$900M in 2024 revenue, supported by favorable reimbursement and limited direct competition.

However, the commercial runway is finite. Core U.S. patent exclusivity for Exondys 51 expires in 2028, composition-of-matter patents expire in 2034 and European uptake has been limited.[4] The PMO platform is also being challenged by next-generation Sarepta candidates like SRP-5051, which uses peptide-conjugated PMO (PPMO) technology and aims to improve tissue uptake.[5]

The Opportunity of Elevidys: Elevidys was widely viewed as a transformative product for Sarepta and the DMD field. As a one-time adeno-associated virus (AAV)-mediated gene transfer, it offered the possibility of durable dystrophin expression, a potential functional disease-modifying effect for a devastating condition with limited options.

At launch, Wall Street expectations for Elevidys ranged from $2–3B in peak sales, under the assumption of broad use in both ambulatory and non-ambulatory patients, with global expansion to follow.[6] Sarepta's valuation reached over $10B, largely driven by Elevidys' risk-adjusted net present value (rNPV) and the assumed platform value tied to future gene therapies for LGMD and other indications.

Non-ambulatory Transition: Despite the promise, the regulatory path was complex. Elevidys received accelerated approval based on expression of micro-dystrophin as a surrogate endpoint, without direct evidence of functional benefit.[1] The company initiated confirmatory trials (EMBARK and ENVISION) to fulfill post-marketing requirements. As of 2024, Sarepta was actively seeking label expansion to include non-ambulatory patients, based on preliminary data.[7]

The fragility of Sarepta's valuation became increasingly evident as safety events emerged in 2025, including multiple patient deaths linked to Elevidys treatment. The FDA's scrutiny of both the non-ambulatory population and the gene therapy class as a whole triggered a reassessment of the Elevidys franchise and the company's entire platform strategy.

LGMD Gene Therapy Programs: Early stage clinical trial results for SRP-9003, SRP-9004, and SRP-9005, utilizing AAVrh74 vectors similar to Elevidys, make up the majority of Sarepta's long-term pipeline optionality.

Clinical Trials; Elevidys

Clinical Development: Elevidys' path to FDA approval was supported by a sequence of clinical trials aimed at evaluating safety, micro-dystrophin expression, and functional outcomes in Duchenne muscular dystrophy patients. These studies ranged from early-stage exploratory work to larger pivotal trials. This section reviews the key trials: ENDEAVOR, EMBARK, and ENVISION. Highlighting their design, timelines, outcomes, and regulatory significance. Together, these studies provide essential context for assessing Elevidys' current market position and the risks tied to its ongoing confirmatory requirements.

Endeavor / Phase Ib/II "Study 103" "ENDEAVOR" open-label n=55 DMD - NCT04626674: The ENDEAVOR trial was central to Elevidys' accelerated approval, enrolling ambulatory boys aged four to seven with Duchenne muscular dystrophy to assess safety and microdystrophin expression. Twelve-week muscle biopsies demonstrated high levels of transgene expression, validating biological activity. As functional outcomes were limited at the time, these data supported the use of microdystrophin as a surrogate endpoint, which the FDA accepted as reasonably predictive of clinical benefit.

ENDEAVOR (Study 101) Key Data[8]:

- Number of cohorts: 7 total
- Primary endpoint: Micro-dystrophin expression at Week 12 muscle biopsy
- Expression results (adjusted by muscle content):
    - High inter-subject variability observed in micro-dystrophin levels
    - Cohort 4 (n=7, 3-year-olds): 99.64% mean protein levels by Western blot[9]
    - Cohort 6 (n=6, Week 12 biopsy):
        - 93.87% mean expression of normal by Western blot
        - 79.9% dystrophin-positive fibers by IF staining
- Safety:
    - Elevated liver enzymes observed in 2 patients; resolved with steroid treatment

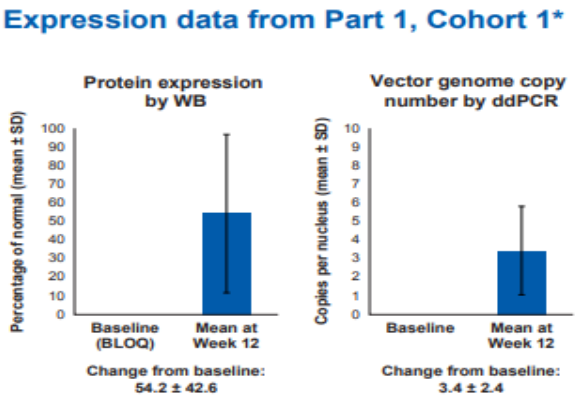


Figure 1 (Expression Data) *Demonstration of srp-9001 dystrophin expression with vector genome copies. N=20; Cohort 1*[8]

The ENDEAVOR trial demonstrated Elevidys's promising biological activity and established micro-dystrophin as a meaningful surrogate marker in ambulatory young DMD patients. This formed the cornerstone of the FDA's accelerated approval, setting the stage for subsequent trials aimed at confirming clinical benefit.

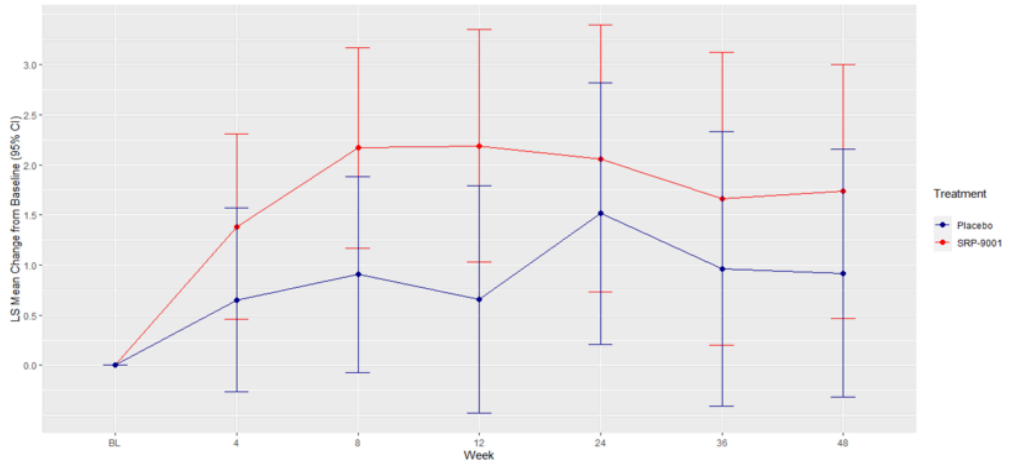
Embark / Phase III n=126 ambulatory RCT ages 4-7yo DMD - NCT05096221: The EMBARK trial was Sarepta's pivotal Phase III study intended to confirm the clinical benefit of Elevidys in ambulatory boys aged four to seven with Duchenne muscular dystrophy. It employed a randomized, double-blind, placebo-controlled crossover design with a 52-week primary analysis. The trial missed its prespecified primary endpoint, change in North Star Ambulatory Assessment (NSAA) score at Week 52, which failed to reach statistical significance ($p = 0.24$). While this marked Sarepta's first major clinical miss in the Elevidys program, the company continued its commercial rollout based on earlier approval via surrogate endpoint and emphasized secondary outcomes.

Despite missing the primary endpoint, Elevidys-treated patients demonstrated statistically significant improvements in key secondary motor function measures, including time to rise and 10-meter walk/run performance. These data were used to support Sarepta's efforts to expand the label to broader populations, including non-ambulatory patients. However, EMBARK's mixed results, combined with subsequent safety concerns, have contributed to growing skepticism around the gene therapy franchise and placed Sarepta in a more precarious regulatory and market position.

EMBARK (Study 301) Key Data:

- Design: Global randomized, double-blind, placebo-controlled crossover, Phase III ($n \approx 126$); 52 weeks per part
- Population: Ambulatory boys aged 4–7 years with genetically confirmed DMD
- Primary endpoint: NSAA total score change at Week 52
    - Elevidys: mean gain +2.6 points; placebo: +1.9 points → difference +0.65 ($p = 0.24$)[10]
- Key secondary endpoints:
    - Time to Rise: −0.64 seconds favoring Elevidys ($p = 0.0025$)
    - 10-m Walk/Run: −0.42 seconds favoring Elevidys ($p = 0.0048$)
    - Functional benefits were more pronounced in children aged 6–7 versus 4–5 years (LSM differences: −0.78 s vs −0.50 s for TTR; −0.52 s vs −0.33 s for 10MWR)[10]
- Safety profile: Consistent with previous trials; no new safety signals observed[11]

Figure 11. LS Mean Change in NSAA Total Score From Baseline Over Time



Source: FDA Statistical Reviewer's analysis
Abbreviation: LS, least squares; NSAA, North Star Ambulatory Assessment.

Figure 2 (LSM estimates). *Shows how the treatment effect evolved in EMBARK; useful for analyzing trajectory and clinical inflection points.*[10] **Note:** *LS mean estimates represent adjusted group means over time, accounting for baseline covariates. Error bars reflect 95% confidence intervals. Data shown are from the full analysis set (n ≈ 125) (1 dropout).*

While NSAA is a widely accepted endpoint for ambulatory DMD trials, it has notable limitations, particularly in a population where disease progression can lead to loss of ambulation over relatively short timeframes (Figure 3).[14] This makes capturing sustained functional improvement inherently difficult. As such, the modest NSAA delta in EMBARK may underrepresent the therapeutic benefit observed in secondary metrics, though regulators still require confirmation of functional impact beyond surrogate markers. These results supported Sarepta's ongoing effort to expand Elevidys' label, and the FDA indicated openness for label expansion based on the totality of evidence.[12,13]

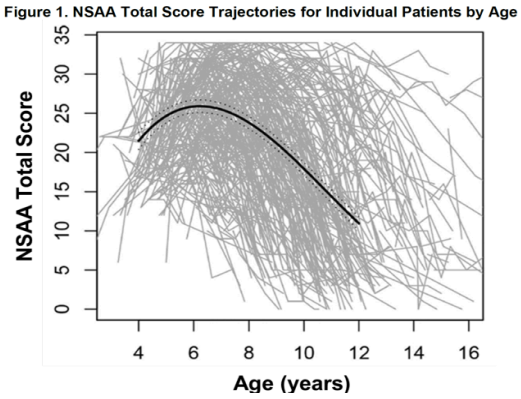


Figure 3. *NSAA total score trajectories for individual untreated Duchenne muscular dystrophy patients by age (in grey) and the fitted mean with 95% confidence interval (in black). Each grey line represents longitudinal NSAA total scores from an individual patient plotted against age, illustrating natural disease progression without treatment. The population mean and its 95% confidence bands are shown in black. NSAA, North Star Ambulatory Assessment. (Muntoni et al., 2019).*[14]

ENVISION / Phase III, n = 148 Non-Ambulatory DMD – NCT05881408: The ENVISION study is Sarepta's ongoing Phase III trial designed to evaluate the safety and efficacy of Elevidys in non-ambulatory patients with DMD. Unlike ENDEAVOR and EMBARK, which focused on ambulatory boys aged 4 to 7, ENVISION targets an older and more progressed patient population.

The primary endpoint is the change in Performance of the Upper Limb (PUL) v2.0 score from baseline to Week 72, reflecting the clinical shift from lower to upper extremity function assessment in non-ambulatory individuals. Secondary outcomes include pulmonary function (FVC%, PEF%), patient-reported outcome measures (PROMIS), micro-dystrophin expression, and exploratory cardiac imaging parameters. A subset of patients (Cohort 2) will also be assessed on the NSAA, allowing comparisons to EMBARK in partially ambulatory participants.[27]

ENVISION is being closely watched as the trial that could enable full label expansion of Elevidys to include non-ambulatory DMD patients. If successful, this would significantly broaden the addressable market and reinforce Elevidys' value proposition. However, given prior safety signals in this population, the trial is also viewed as a key regulatory and reputational test for Sarepta's platform.

ENVISION (Study 303) Key Details:

- Design: Ongoing, randomized, double-blind, placebo-controlled Phase III trial; n=148
- Population: Non-ambulatory DMD patients aged 4–18 years with confirmed genetic diagnosis
- Primary endpoint:
    - Change in PUL v2.0 total score from baseline to Week 72 (Part 1)
- Key secondary endpoints:[27,28]
    - Change in FVC% predicted and PEF% predicted
    - Micro-dystrophin expression via Western blot at Week 12
    - Change in PROMIS Upper Extremity Function
    - Cohort 2 only: Change in NSAA total score
    - Cardiac strain measured by cMRI
    - Comprehensive safety assessment including TEAEs, SAEs, labs, ECGs, ECHOs

As of August 10th, 2025, no clinical readout has been released. The study remains active and recruiting, with initial data likely to emerge in late 2025 or 2026. Until then, ENVISION represents a commercial catalyst and a risk. Success would revive optimism, but further safety concerns or failure to meet functional endpoints could deepen scrutiny.

FDA Scrutiny & Fallout Timeline

Accelerated Approval, June 2023: The FDA granted Elevidys accelerated approval for ambulatory DMD patients aged 4–7, based on micro-dystrophin expression as a surrogate endpoint. While this regulatory flexibility was seen as a major win, it came with a caveat: confirmatory functional data from the EMBARK trial would be required to retain long-term approval and support broader label expansion.[1]

Embark Disappointment, October 30 2023: Sarepta reports that the EMBARK Phase III trial failed to meet its primary endpoint; change in NSAA score at 52 weeks ($p = 0.24$). Although secondary endpoints (e.g., time to rise and 10-meter walk) showed statistical significance, the miss raised serious doubts about Elevidys' functional efficacy and triggered renewed FDA skepticism, weakening the case for non-ambulatory use.[10]

Label Expansion Effort, December 2023: Sarepta submits a supplemental biologics license application (sBLA) to the FDA seeking label expansion of Elevidys to include non-ambulatory patients. This filing was based on preliminary, non-public data from the ongoing ENVISION trial, though full efficacy and safety data have yet to be disclosed.[15]

Commercial Monitoring and Trial Progress, 2024: Throughout 2024, Sarepta focused on commercializing Elevidys for ambulatory DMD patients, supported by accelerated approval and ongoing reimbursement efforts. Market uptake was steady but cautious; emerging uncertainty following the EMBARK trial miss in late 2023. No significant safety concerns or patient deaths were publicly reported during this period, though pharmacovigilance and safety monitoring continued as part of routine post-marketing obligations. Meanwhile, Sarepta advanced enrollment and data collection in the ENVISION trial, preparing the groundwork for the planned label expansion to non-ambulatory patients. Positive investor sentiment during this time lifted Sarepta's stock price by ~30%, with market capitalization reaching ~$12B.[16]

Revenue Guidance Reduction, March 2025: During the Q1 earnings call, Sarepta lowered its full-year 2025 revenue guidance from $2.9—3.1 billion to $2.4—2.6 billion, attributing the revision to slower Elevidys uptake and regulatory uncertainties.[16]

First Reported Patient Death, March 2025: Sarepta discloses that a 16-year-old non-ambulatory patient had died from acute liver failure following treatment with Elevidys. This was the first fatality linked to the gene therapy. Sarepta reported the event to the FDA on June 20, 2025, and updated the prescribing information to include liver toxicity warnings.[17]

Leadership Changes at FDA, May 2025: Vinay Prasad was appointed Director of the FDA's Center for Biologics Evaluation and Research (CBER). During his tenure, regulatory scrutiny on Sarepta's Elevidys and related gene therapies intensified, including shipment holds and delayed label expansion decisions.[18]

Second Reported Patient Death, June 2025: Sarepta reported a second patient death due to acute liver failure following Elevidys treatment. This patient, also non-ambulatory, died within two months of receiving the therapy. Sarepta reported the event to the FDA on June 18, 2025, via the FDA's postmarketing electronic database, FAERS.[19,20]

FDA Delays Label Expansion Decision, June 2025: The FDA had delayed decisions on the ENVISION trial's label expansion request, citing the need for additional data to address safety and efficacy concerns.[21]

Platform-Wide Regulatory Scrutiny, July 2025: The FDA increased its oversight of Sarepta's gene therapy pipeline, including LGMD programs (e.g., SRP-9003), leading to postponed trial milestones and ongoing safety assessments.[22]

Shipping Holds Initiated, July 2025: The FDA issued a hold on Elevidys shipments, suspending distribution for all patients due to emerging safety concerns. From January 1 to July 27, 2025, Sarepta (NASDAQ: SRPT) shares had fallen by ~89%.[16,23]

FDA Lifts Shipment Hold for Ambulatory Patients, July 2025: The FDA officially rescinded its shipment hold for ambulatory patients, permitting Elevidys' distribution to this group to resume. However, the shipment hold for non-ambulatory patients remains in effect pending further data and review. Prasad resigned on July 30, 2025, amid political pressures, adding uncertainty to the FDA's regulatory trajectory.[24,25]

Prasad Reappointed, August 2025: The FDA announces Dr. Prasad's reappointment as Director of CBER. The move has shocked the biopharma industry and makes his leadership the single most critical variable in assessing Sarepta's future.[26]

Footnote: Sources: Sarepta press release (February 2023 — July 2025), U.S. Food & Drug Administration. *Drugs@FDA: FDA-Approved Drugs*. Sarepta Therapeutics. *Form 8-K: Revision of FY2025 Revenue Guidance and Safety Update on Elevidys*. March 6, 2025.

Regulatory Risk ≠ Clinical Failure: Despite mounting scrutiny, Elevidys remains an FDA-approved gene therapy with a growing commercial footprint. While regulatory setbacks have cast a shadow over Sarepta's near-term trajectory, it is important to remember that Elevidys continues to generate revenue, has demonstrated biologic activity via micro-dystrophin expression, and retains FDA approval for ambulatory patients. The scrutiny Sarepta faces reflects the broader regulatory climate surrounding AAV-based gene therapies rather than a unique failure of Elevidys itself. Although Sarepta may currently be in the agency's "doghouse", if the company can successfully navigate safety concerns and demonstrate long-term efficacy, the market may be exaggerating the risks and undervaluing the upside.

Valuation Scenarios

The valuation of Sarepta Therapeutics (SRPT) hinges heavily on the trajectory of Elevidys, the company's pioneering gene therapy for Duchenne Muscular Dystrophy (DMD), alongside the performance of its PMO exon-skipping franchise and the promise of its LGMD pipeline. Given the significant clinical and regulatory uncertainties surrounding Elevidys, it is prudent to explore multiple valuation scenarios reflecting different outcomes for Elevidys and the broader assets.

Valuation Methodology: My valuation is based on a Hybrid Framework that utilizes two distinct methodologies to determine the market capitalization of Sarepta Therapeutics across different scenarios.

For my Bear Case, I perform a fundamental Unlevered Discounted Cash Flow (DCF) Analysis on the entire company. This model, detailed in Appendix A, projects the company's unlevered free cash flows through 2035 and discounts them to arrive at a Net Present Value (NPV), which represents a core, intrinsic value for the enterprise. This DCF-driven approach provides a credible "floor valuation" in a near-worst-case scenario.

For my Base and Bull Case price targets, I utilize a Sum-of-the-Parts (SOTP) Analysis. This methodology values Sarepta's key assets (Elevidys, the PMO franchise, and pipeline assets) separately using market-based revenue multiples (Appendix C). This approach is better suited for these more optimistic scenarios as it captures the potential for a "re-rating" of the stock, where an improvement in the company's outlook would lead investors to apply higher multiples to its revenue streams.

This hybrid approach allows me to anchor my thesis in a fundamental valuation while using a market-based methodology to set price targets that reflect potential changes in investor sentiment. **Note:** See Appendix for a summary of key assumptions.

Bear Case: DCF-Driven Floor Valuation (~$15.00/share)

Key Assumptions:

- This scenario models a commercial failure for Elevidys, with revenues collapsing after 2025 following the initial crisis.
- The PMO franchise remains a stable cash-flow generator.
- R&D spending is not eliminated but drops to a sustainable maintenance rate of 12% of revenue from 2026 onwards to support the remaining commercial assets.

The Bear Case valuation is derived directly from the comprehensive, 12-year Unlevered DCF model detailed in Appendix A. This fundamental analysis provides what I believe is the most credible "floor valuation" for the company in a near-worst-case scenario. The DCF calculates the value of the entire enterprise, including all its operational assets (like PP&E) and accounting for the costs of its research programs (like the LGMD pipeline).

- My DCF model, which projects unlevered free cash flows through 2035 and discounts them back to today at a 10% rate, yields an Enterprise Value of $1,797M.
- To determine the value attributable solely to shareholders, I adjust this Enterprise Value for the company's net debt position. Subtracting *Net Debt of 305M*(~$3.14/share) results in an Implied Equity Value of $1,492M.
- Dividing this Implied Equity Value by the 97 million diluted shares outstanding results in a fundamental Bear Case value of **~$15.00 per share.**

Base Case: A Permanently Impaired Franchise (~$34.00/share)

Key Assumptions:

- The Elevidys launch is permanently impaired by the clinical and regulatory setbacks. Peak sales reach $1.6 billion, significantly below original expectations. Applying a 1.0x revenue multiple to reflect high risk and a no-growth outlook for the asset.
- The PMO franchise remains a stable cash generator with $900 million in annual revenue, valued at a conservative 1.7x multiple to reflect possible spill-over.
- The company's tangible assets (PP&E) and early-stage pipeline (LGMD) are included at sharply discounted values of $300M and $200M, respectively, to reflect heightened risk.

This scenario, which now forms the foundation of my more conservative price target, assumes the company stabilizes but at a level far below its former potential. It reflects a new reality where Elevidys is a niche product rather than a blockbuster franchise.

- Elevidys: 1.6B(PeakSales)x1.0= 1,600M
- PMO Franchise: 900M(AnnualSales)x1.7= 1,530M
- LGMD Pipeline: $200M
- PP&E: $300M
- Total Enterprise Value: $3,630M

Net Debt: 305M (~$3/share)
Implied Equity Value: $3,325M
Shares Outstanding (Diluted):97M

- Total Implied Base Case Value: **~$34.00/share**

Bull Case: Accelerated Recovery and Sentiment Reversal: (~$62/share)

Key Assumptions:

- The commercial launch of Elevidys successfully shrugs off regulatory hits, but launch expectations were never truly met, and no label expansion to non-ambulatory patients occurs. Commercial execution allows Elevidys to reach the 2025 year guidance of $2.4 in peak sales y/y.
- Market sentiment recovers, assigning a healthier, though still conservative, 1.5x revenue multiple to Elevidys.
- The PMO franchise is valued as a stable cash generator with a 2.0x revenue multiple on its ~$900 million in annual sales.
- As the gene therapy platform is de-risked, the value of the LGMD pipeline is revised upward to $400M. And PP&E revised to $500M.

This scenario models a strong recovery driven by successful commercial execution and a normalization of investor sentiment across all of the company's assets.

- Elevidys: 2.4B(PeakSales)x1.5= 3,600M
- PMO Franchise: 900M(AnnualSales)x2.0= 1,800M
- LGMD Pipeline: $400M
- PP&E: $500M
- Total Enterprise Value: $6,300M

Net Debt: 305M (~$3/share)
Implied Equity Value: $5,995M
Shares Outstanding (Diluted): 97M

- Total Implied Bull Case Value: **~$62/share**

Investment Thesis

I am initiating coverage of Sarepta Therapeutics with a Buy rating and a 12-month price target of ~$34.00, derived from my Base Case valuation scenario. Following a catastrophic series of clinical and regulatory setbacks, Sarepta now stands at a critical inflection point. My analysis suggests the current share price fairly reflects a "value trap" scenario: the stable, cash-generating PMO franchise and net cash provide a hard valuation floor, but significant upside remains capped by the immense uncertainty surrounding Elevidys and the unpredictable regulatory

environment. The single greatest overhang is the renewed leadership of CBER Director Vinay Prasad. Until the FDA provides clear guidance on the path forward for Elevidys in the crucial non-ambulatory population, where the fatal adverse events occurred, I see a lack of near-term catalysts powerful enough to warrant a fundamental re-rating of the stock. I would become more constructive upon a clean resolution of the partial clinical hold or tangible evidence of a stabilizing commercial launch in the approved ambulatory population.

Key Catalysts

- **FDA Guidance on Non-Ambulatory Hold:** Any communication from the FDA regarding the partial clinical hold on Elevidys is the most significant near-term catalyst.
- **Regulatory Interaction on Label Expansion:** Any FDA meeting minutes or briefing documents signaling a potential Elevidys label expansion could materially shift sentiment.
- **Q3/Q4 2025 Elevidys Revenue:** Quarterly earnings will provide the first concrete data on the stability and trajectory of the commercial launch in the ambulatory-only population post-crisis.
- **ENVISION Topline Data**: The full data readout from the Phase III ENVISION trial in non-ambulatory patients remains a pivotal, albeit high-risk, clinical event.
- **LGMD Pipeline Updates:** Data from SRP-9003 or other limb-girdle muscular dystrophy programs could remind investors of the platform's long-term value beyond DMD.
- **Competitive Readouts & Market Dynamics:** Data from Pfizer's *Fordadistrogene movaparvovec* (PF-06939926), or other competitors' DMD gene therapy programs, could affect Sarepta's market positioning.

Conclusion

The dramatic collapse of Sarepta's valuation is the direct result of a collision between the immense scientific promise of Elevidys and a brutal clinical and regulatory reality. The past year has demonstrated the fragility of a valuation built on optimism, especially when confronted with patient deaths and an unpredictable FDA. The risks moving forward remain profound; the commercial relaunch is fragile, the path for the crucial non-ambulatory population is blocked, and the future of gene therapy regulation under CBER and the FDA is a critical, unknowable variable.

And yet, my analysis suggests that these severe risks are more than fully priced into the stock. At these depressed levels, the market is pricing in a high probability of continued failure, while ascribing little value to the IP & clinical assets that exist within Elevidys and the company's broader pipeline.

I therefore set a **~$34 price target**. Investors are not buying a flawless asset, but rather a deeply discounted one at a point of maximum pessimism, with a stable PMO franchise providing a tangible valuation floor. I am confident in this thesis and would be eager to discuss my DCF + SOTP model and underlying assumptions in greater detail.

Disclaimers

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I, Justin Marciano, hereby certify that all of the views expressed in this research report accurately reflect my personal views about the subject, security, and company. I also certify that no part of my compensation was, is, or will be, directly or indirectly, related to the specific recommendations or views expressed in this research report.

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Company-Specific Disclosures:

- As of the date of this report, August 10, 2025, I do not hold a financial interest or any shares in Sarepta Therapeutics (NASDAQ: SRPT).
- I do not have any Investment Banking or other business relationships with Sarepta Therapeutics, Inc.

Editor's Note: This article is part of a biotech valuation series I'm publishing to demonstrate my industry knowledge and investment approach. If you're a hiring manager or recruiter, feel free to reach out. I'm looking for roles in [Equity Research / Corporate Strategy / Biotech & Biopharma Investing / Investment Banking].

Appendix

Appendix A: Unlevered DCF Model (Bear Case)

Note: All figures in millions USD, unless otherwise noted.

Fiscal Year	2024	2025	2026	2027	2028	2029	2030	2031	2032	2033	2034	2035
Total Revenue	1,788	1,818	1,011	1,031	1,062	1,083	1,103	1,124	1,130	1,146	1,137	1,128
Gross Margin %	82%	77%	85%	85%	85%	85%	85%	85%	85%	85%	85%	85%
Gross Profit	1,469	1,403	859	877	903	920	938	955	960	974	966	959
R&D Expense	(780)	(450)	(121)	(124)	(127)	(130)	(132)	(135)	(136)	(137)	(136)	(135)
SG&A Expense	(558)	(558)	(503)	(452)	(407)	(366)	(330)	(297)	(267)	(240)	(216)	(195)
Operating Income (EBIT)	130	394	235	301	368	424	476	524	558	596	614	629
Taxes (at 21%)	(27)	(83)	(49)	(63)	(77)	(89)	(100)	(110)	(117)	(125)	(129)	(132)
NOPAT	103	311	186	238	291	335	376	414	441	471	485	497

Appendix B: Bear Case DCF Valuation Summary

Metric	Value
Present Value of FCF (NPV)[1]	$1,797M
Discount Rate (WACC)	10.0%
Implied Enterprise Value	$1,797M
Less: Net Debt	($305M)
Implied Equity Value	$1,492M
Shares Outstanding (Diluted)	97M
Implied Equity Value per Share	~$15.38

Analyst Notes: The Net Present Value (NPV) of $1,797M is provided from the model's output. It represents the sum of all future unlevered free cash flows, discounted back to today. For this Bear Case model, UFCF is calculated starting with NOPAT and adjusting for non-cash items and investments. I have assumed that Depreciation & Amortization is fully offset by maintenance Capital Expenditures, and that the Change in Net Working Capital is negligible. Under this common assumption for a stable, low-growth business, UFCF is equal to NOPAT. R&D expenses from 2026 onward are modeled at a maintenance rate of 12% of revenue.

Appendix C: Comparable Company Analysis

To provide a quantitative anchor for the Sum-of-the-Parts valuation, I analyzed a peer group representing three key archetypes: a premium platform leader (Vertex), a stable and diversified rare disease franchise (BioMarin), and a distressed gene therapy peer facing significant headwinds and a declining revenue (Sangamo).

Comparable Company Analysis (Data as of August 10, 2025)

Company	Ticker	Market Cap ($B)	Enterprise Value ($B)	EV / NTM Revenue	NTM Rev. Growth
Premium Platform					
Vertex Pharmaceuticals	VRTX	$96.0	$100.2	8.7x	13%
Stable Rare Disease					
BioMarin Pharmaceutical	BMRN	$6.7	$10.4	3.3x	11%
Distressed Gene Therapy Peer					
Sangamo Therapeutics.	SGMO	$0.13	$0.10	1.6x	-67%

Analyst Notes:

This analysis reveals various valuation spectrums. Vertex, as a dominant, diverse, and highly profitable leader, commands a premium multiple of 8.7x Next Twelve Months (NTM) revenue. BioMarin, a mature and diversified company, trades at a more moderate 3.3x. Critically, Sangamo Therapeutics, which has faced significant clinical and regulatory setbacks, trades at a deeply discounted 1.6x.

This peer set provides a compelling and highly defensible framework for my Sarepta valuation. The Base Case multiple of 1.7x for the stable PMO franchise is positioned directly in line with the distressed peer Sangamo, reflecting the risk of Sarepta's company-specific crisis onto its otherwise stable assets. The 1.0x multiple for the embattled Elevidys franchise represents a substantial ~38% discount to even the distressed peer, a valuation that appropriately quantifies the extreme and unique regulatory and safety overhang specific to Sarepta Therapeutics, Inc.

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