BIOPHARMA / RARE DISEASES

LEERINK PARTNERS

DMD Market Set to Evolve with Next-Generation Gene Tx. & Skippers; 2025 Primer

April 21, 2025

- **Bottom Line: Following the latest round of updates across the Duchenne muscular dystrophy (DMD) space, we are refreshing our view on the landscape and orienting investors for what's next in the 12+ months.** In similar style to our 2022, 2023, and 2024 deep dives on DMD (here, here, here), we are again taking stock of the treatment and development landscape, which has experienced several significant advancements since our prior installment last year. We have seen important results from several players in the gene therapy space, as well as sponsors who are developing next-generation exon skippers. We also recently learned of the tragic death of an older boy (here) following treatment with Sarepta's (SRPT) Elevidys (SRP-9001), which has caused many in the treatment and investment communities to more carefully evaluate the landscape. Meanwhile, changes at the FDA are top of mind, especially for next-generation gene therapies at Regenxbio (RGNX, OP, Foroohar), Solid Biosciences (SLDB) and Insmed (INSM) which may offer competitive safety and efficacy profiles. As we have outlined previously in conjunction with our gene therapy market model (here), we continue to see a significant opportunity that can support multiple sponsors, even those arriving later to market. Despite the recent safety event for Elevidys, we remain relatively comfortable with our near-term estimates for SRPT in FY25, and believe that the risk/benefit of treatment remains intact for younger boys. We acknowledge that the therapy seems to have sustained a modest reputational hit; however, the event rate remains extremely low (<1.0%) and at this juncture, these boys have no other approved gene therapy options so SRPT can continue to benefit from their first-mover advantage. We expect the market will continue to evolve as new gene therapies and exon skipping treatments come to market, with combination treatment taking hold similar to the spinal muscular atrophy (SMA) treatment paradigm, since gene therapy is unlikely to be a cure. We continue to view the DMD space as an attractive area to invest and see multiple near-, mid-, and long-term opportunities that investors should be watching. ***Continued inside...***

Reason for report:
PROPRIETARY INSIGHTS

S&P 500 Health Care Index:	**1,544.83**
S&P 600 Health Care Index:	**2,780.92**

Companies Highlighted

ARWR, BMRN, EWTX, INSM, JAZZ, KROS, PEPG, PFE, QURE, RGNX, RNA, SLDB, SRPT, WVE

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Ticker	Rating	Price	Price Target Current	Price Target Previous	Mkt. Cap (MM)	Current Rev Est. 2025	Current Rev Est. 2026	Previous Est. 2025	Previous Est. 2026	Current EPS Est. 2025	Current EPS Est. 2026	Previous Est. 2025	Previous Est. 2026
BMRN	OP	$58.65	$105.00	--	11,531	$3,101.1	$3,185.6	--	--	$2.24	$3.53	--	--
EWTX	OP	$13.30	$50.00	--	1,390	0.0	$32.0	--	--	($2.00)	($1.95)	--	--
INSM	OP	$69.51	$100.00	--	12,442	$457.3	$936.3	--	--	($5.65)	($4.75)	--	--
PEPG	OP	$1.52	$20.00	--	50	0.0	0.0	--	--	($3.21)	($2.93)	--	--
RNA	OP	$28.52	$60.00	--	3,665	0.0	$12.8	--	--	($4.69)	($5.32)	--	--
SLDB	OP	$3.03	$20.00	--	280	0.0	0.0	--	$18.4	($1.66)	($1.94)	--	($1.77)
SRPT	OP	$55.23	$150.00	$200.00	5,992	$3,029.1	$3,523.9	--	$4,115.2	$6.93	$14.81	$13.19	$16.72
WVE	OP	$6.12	$26.00	--	988	$66.0	$92.3	--	--	($1.13)	($1.12)	--	--

Source: Company Information and Leerink Partners LLC Research.

- **Model changes:** We are lowering our SRPT PT to $150 (from $200), driven by lower Elevidys sales estimates in FY26 and beyond. However, with shares trading near 52-week lows and at one of the least expensive multiples for the group, we still see a significant value disconnect with the stock. This situation is reminiscent of the setup after the Study 102 miss in 2021 (here) or the EMBARK disappointment in 2023 (here), when shares sold off significantly but recovered over time. Now in 2025, we think this is another situation where you “close your eyes and buy.” We remain Outperform on SRPT. Meanwhile, we are increasing estimates for SLDB's SGT-003, offset by an increase in OpEx assumptions. Our SLDB PT remains $20 and we reiterate Outperform. Other estimates/PTs are unchanged.
- **What's in this note?** In addition to refreshing our DMD gene therapy market model following the Elevidys safety event, we discuss the read-across to other players in the space, including potential near-term entrants RGNX and SLDB. We also touch on the recent reshuffling at the FDA, including the departure of Peter Marks and the potential implications within DMD. SLDB will be one of the first sponsors meeting with the “new” agency, and the company's discussion with the FDA regarding accelerated approval in mid-2025 will be an important event to watch for in gene therapy broadly. In terms of recent data updates, we provide an overview of these within the note, as well as what additional data disclosures to look forward to in 2025.

Death After Elevidys Treatment Sparked Concern for Gene Tx.

- **The death of a patient following Elevidys treatment has made the treatment community and investors more cautious than ever about SRPT.** As disclosed in mid-March 2025 (here), a 16-year-old boy unfortunately passed away following treatment with Elevidys. This patient suffered from acute liver failure, a known possible side effect of Elevidys, as well as other adeno-associated virus/AAV-mediated gene therapies (FDA label here). In addition, testing revealed this patient had a recent cytomegalovirus (CMV) infection which was identified by the treating physician as a possible contributing factor. CMV can infect and damage the liver, a condition known as CMV hepatitis. While details on this event remain sparse, we suspect that given the patient's age, they were on the heavier side, which would require a higher dose of Elevidys, which is dosed by weight. Moreover, we know that older DMD patients have higher morbidity, which may have also played a role. When we spoke with management, they emphasized that over 800 patients have been dosed with Elevidys in clinical trials or commercial product, so they believe that this case is unique. We acknowledge that such severe side effects associated with mortality can certainly be alarming and cause the community to question the risk/benefit of treating older patients; however, we also believe that the very low overall incidence (<0.125% based on aggregate exposure to date) is encouraging. Considering the lack of other approved gene therapy options, it seems unlikely to us that this event will materially change the rate of uptake in younger patients, while use in older/non-ambulatory boys is likely to be slower. We note that the majority of patients treated are ambulatory, with just over 100 non-ambulatory boys treated since June 2024, and our forward assumptions are more heavily weighted to ambulatory patients.
- **MEDACorp KOLs were not that surprised to see this signal pop up.** Following the disclosure of the death with Elevidys, we spoke to several KOLs to get a sense of how the community was reacting. Broadly, the physicians that we talked to were not all that surprised to see this unfortunate event occur, especially considering the patient was on the older side, combined with the high viral load required for dosing heavier patients. The KOLs would all like additional detail on the event, referencing the prior signals seen with Pfizer's (PFE, MP, Risinger) gene therapy included more details. It seems like a viral event could have been complicated by the secondary infection (or a possible reactivation following immunosuppression), but it is hard to know based on the limited information disclosed. One of the physicians we spoke to noted they have not dosed any non-ambulatory patients with Elevidys, while another KOL has dosed patients in both settings. In terms of patient demand, physicians want to do something for these boys as the clock is ticking, but they are also excited about the next-generation therapies (more on this below). These physicians still have a list of patients in line to receive Elevidys and are dosing patients at a rate of 1-2 per month (though the queue is getting smaller as they treat more patients post-approval). This tracks with our prior checks, where we heard that some centers with the necessary infrastructure can infuse 8 patients with a gene therapy (not just Elevidys) per month, while some smaller or less experienced centers are constrained to ~2 infusions/month. Conservatively assuming the ~76 active sites only infuse 1 Elevidys patient per month, that implies ~912 patients are infused during the year, which is above what is required to hit our/consensus estimates. After the initial approval, KOLs were saying that every patient would want Elevidys treatment; however, this has moderated more

recently in older/non-ambulatory patients, while younger patients, who risk losing ambulation, mostly want what is available for them now, versus waiting.

- **Some patients have a bit of time to decide.** While we've heard that most younger patients may opt for the therapy that is available to them now, one of the KOLs also mentioned that some patients have some time to decide. With RGNX aiming to file their Biologics License Application (BLA) under Accelerated Approval in mid-2026, we estimate that this therapy could launch in early 2027. Thus, potentially newly diagnosed patients in 2025 and 2026 may decide to wait for RGX-202, assuming it becomes available in early 2027. This of course will come down to patients and their families, as well as the provider and the team involved (sometimes inclusive of a neurologist, cardiologist, and pulmonologist). SLDB's SGT-003 is moving rapidly, and we now assume it could be approved in 2027 (previously 2026), so we could see some potentially wait for that therapy. As we have heard many times before, DMD is a relentless disease and these patients are losing muscle every day. Thus, the treatment decision is a battle between what's available now, how much muscle the boys have left to save, and how soon other options may become available (and if the benefit of next-generation therapies outweighs the opportunity cost of waiting for them to be available).
- **What does this mean for other gene therapies in development?** Elevidys was the first to arrive, but there are a handful of next-generation gene therapies (**Exhibit 1**) that are expected to hit the market over the next few years (here). These aim to iteratively improve on Elevidys (the construct was originally designed >20 years ago), leveraging different capsids and transgenes, potentially allowing for a lower dose (which will become important for safely dosing older/heavier boys), better efficacy (which we've seen, albeit in small patient numbers), as well manufacturing advantages. Despite the recent safety event with Elevidys, we are detecting increased excitement in the community for the next-generation gene therapies, especially with the potential to differentiate on the safety profile.
  - **RGNX.** RGX-202 is a clinical-stage candidate for DMD, designed to deliver a transgene for a novel microdystrophin that includes the functional elements of the C-Terminal (CT) domain found in naturally occurring dystrophin. According to RGNX, the presence of the CT domain has been shown in preclinical studies to recruit several key proteins to the muscle-cell membrane, leading to improved muscle resistance to contraction-induced muscle damage in dystrophic mice. Additional design features may potentially improve gene expression, increase translational efficiency and reduce immunogenicity. RGX-202 is designed to support the delivery and targeted expression of the transgene throughout skeletal and heart muscle using the NAV AAV8 vector, and a well-characterized muscle-specific promoter (Spc5-12). Several positive data cuts have been disclosed from the Ph.1/2 AFFINITY DUCHENNE study (NCT05693142), with additional functional results expected in the first half of 2025. Meanwhile, the pivotal portion of the trial is enrolling, with a BLA submission expected in mid-2026 (see our colleague's notes here, here, here).
  - **SLDB.** SGT-003 is a next-generation clinical-stage candidate for DMD, utilizing an updated construct, combining SLDB's proprietary microdystrophin containing nNOS with AAV-SLB101, a novel, rationally designed capsid derived from AAV9 and designed for enhanced muscle tropism, reduced liver uptake, and to more selectively deliver the drug to target tissue. Initial data (discussed in more detail below) from the first three DMD patients in the Ph.1/2 INSPIRE DUCHENNE study (NCT06138639) were disclosed in

February 2025 (here). The company plans to enroll and dose more than 10 patients by early 2Q25 and 20 patients by 4Q25. They will also approach the FDA after 10-12 patients have been dosed in mid-2025 (with 90-day data in hand) to discuss their potential path for Accelerated Approval. Considering RGNX's alignment with the FDA on a relatively flexible primary endpoint of proportion of patients ≥10% microdystrophin expression in a single-arm study (here), we assume that a similar path should also be available to SLDB.

- **Genethon (Not Rated).** GNT0004 is an AAV8 based gene therapy, containing a shortened functional dystrophin gene (hMD1) with a Spc5-12 promoter, targeting skeletal and cardiac muscles. While GNT004 is an AAV8 based therapy and shares the same Spc5-12 promoter as RGX-202, it does not contain the CT domain. Genethon shared data from the Ph.1/2 study last year (discussed more below), with plans to launch a pivotal study in Europe in 2Q25, followed by the US. The first patients in the pivotal portion are expected in mid-2025. We note that GNT004 is dosed at 3E13 GC/kg, which is lower than the other programs (which hypothetically could lead to safety and/or manufacturing benefits). The Genethon program has long been in the making, as the company originally received the green light from the French National Agency for Medicines and Health Products Safety (ANSM) in December 2020 for a clinical study, with the first patient being dosed in April 2021. A SUSAR event of immune-mediated myositis occurred in 1st patient with dose 1, which caused the program to pause (resumed at the end of 2022). A prophylaxis regimen of steroids and sirolimus was implemented after this SUSAR event. Recall, SRPT previously had a gene therapy research collaboration with Genethon, which was disclosed in June 2017 (here). However, in May 2024, SRPT and Genethon agreed to terminate the collaboration agreement.
- **INSM.** INS1201 is an intrathecally delivered gene therapy, which received Investigational New Drug (IND) clearance from the FDA in 4Q24. Considering the intrathecal administration directly into the cerebrospinal fluid, INSM believes this approach has the potential to target both skeletal and cardiac muscles at much lower doses, which we believe could be an attractive differentiator for the program. As disclosed at the company's R&D day in 2023 (download the presentation here), consistent functional and histopathology effects in the mdx mouse model were observed with INS1201 at 10-50x lower doses with intrathecal administration versus the competitors (systemic delivery). The Ph.1 ASCEND study (NCT06817382) was listed on clinicaltrials.gov earlier this year and is expected to initiate in the first half of 2025. INS1201 leverages an AAV9-microdystrophin construct and expresses a segment of the dystrophin protein that demonstrates efficacy in the mdx mouse. Nonclinical histopathological and functional efficacy studies with INS1201 demonstrated qualitative improvement in general muscle histology, increased fiber size, decreased inflammation and fibrosis, and quantitative improvement in muscle strength and physiology compared to control animals. Moreover, biodistribution studies in both mice and NHPs demonstrated significant vector genome delivery to muscle groups throughout the body by delivery to the cerebrospinal fluid as well as effective cardiac muscle targeting with limited distribution to the liver compared to systemic dosing.
- **We are also keeping an eye on Kate Therapeutics.** Last year, Novartis (NVS, Not Rated) acquired Kate Therapeutics for a total value of up to $1.1B (PR here). Kate is focused on developing a handful of AAV-based gene

therapies to treat generically defined neuromuscular diseases, including DMD. So far, we've only seen preclinical data with KT-809 (liver de-targeted gene therapy), suggesting high levels of microdystrophin expression in skeletal and cardiac muscles at 3x lower doses than the approved option in non-human primates. Interestingly, Kate's candidate also showed 27x lower vector genomes per nucleus in the liver. It remains to be seen when KT-809 may move forward into the clinic, but we look forward to a future update from NVS.

Despite Safety Event, Elevidys Numbers Look Achievable in 2025

- **SRPT is guiding to net product revenue of $2.9-3.1B, inclusive of the PMO franchise.** On the last earnings call (here), which occurred prior to the safety event, management reiterated net product revenue of $2.9-3.1B, which is inclusive of the PMO franchise (which we expect will do ~$0.9B this year). Based on this guidance and assumptions for the PMO franchise, we assume Elevidys sales of ~$2.1B in 2025, which is in line with consensus estimates. We note that based on how the launch has been trending since the expanded label in June 2024 (here), guidance for the year appears relatively achievable, assuming there is not a drop off in demand. Based on Elevidys sales of ~$384M for 4Q24 (+112% QoQ), the launch is already on a run rate of ~$1.5B per year. Thus, guidance for the year could be met with just ~10% sequential growth QoQ for 2025 (implies ~$2.0B in sales). Based on our KOL discussions, we think it makes sense that utilization among older/non-ambulatory boys may be moderated; however, we emphasize that this patient group was never a major driver to our sales assumptions for Elevidys, especially in near-term years. While this dynamic may make a beat and raise story more unlikely, at this juncture, we remain cautiously optimistic that guidance can be met, even with the dark cloud of safety concerns.
- **We continue to assume FY25 sales of ~$2.1B (Exhibit 2).** Following two quarters of robust growth after the full approval and expanded label (+49% and +112% QoQ in 3Q and 4Q, respectively), we assume that the growth rate tapers off modestly in 2025. Our estimates for the four quarters in 2025 are ~$417M (+9% QoQ), ~$490M (+17% QoQ), ~$557M (+14% QoQ), and ~$619M (+11% QoQ), respectively. This brings total FY25 Elevidys sales to ~$2.1B. Based on our estimate of the net cost per patient ($3.2M wholesale acquisition cost with a 25% gross-to-net), the ~$2.1B figure requires an estimated ~868 patients to be treated throughout the year. While this represents a significant step-up in patients required from FY24, where we estimate that ~342 patients were dosed, the label for Elevidys has been significantly expanded to include essentially all DMD patients (whereas there were only two quarters in 2024 with the broad label). Moreover, as we heard in our KOL checks, many physicians are seeing a run rate of 1-2 Elevidys infusions per month. Based on the ~76 active infusion centers for Elevidys, this implies capacity for of ~912 (on the low end) up to ~1,824 patients (on the high end) for the entire year. We know that not all centers have as robust capacity, while others may be utilizing the drug less. However, we think this exercise highlights the achievability of guidance, with relatively conservative assumptions. Moreover, with ~14k addressable patients (removing those with neutralizing antibodies to AAVrh7), this would require only ~6% penetration. In an even more conservative case, assuming use in only ambulatory patients, the penetration required jumps to ~12%, a figure that we still view as achievable.
- **We don't think Elevidys could be removed from the market now that Peter Marks is gone.** Following the departure of Peter Marks (prior Director of the

Center for Biologics Evaluation and Research/CBER) in late March 2025 (here), who overruled staffers at the FDA during the approval of Elevidys (both the original approval and expanded approval), there has been some conversation regarding the worst-case scenario for SRPT and Elevidys. Now that one of the champions for regulatory flexibility and a key member to Elevidys' approval is gone, could the therapy be removed from the market? We acknowledge that with the increased uncertainty (and recent safety concerns), this is not an entirely outlandish assumption to make. However, we also do not believe there is a real likelihood of this happening. First, we do not view DMD as an area that should draw scrutiny from the new administration, especially considering the high unmet need here. Secondly, we have previously seen this story play out with Exondys 51 (eteplirsen), which was approved controversially, with an FDA Commissioner (Janet Woodcock) pushing the approval along. When Woodcock left the FDA, there was little discussion about having Exondys 51 pulled. Third, and perhaps most importantly, we know how powerful the patient organizations are in DMD. If the agency even hinted at possibly removing the therapy from the market, we imagine the patient organizations would place immense pressure on the FDA to backtrack on this effort. Regardless of how one feels about the controversy of the Elevidys data/approval, we must also remember that these boys have extremely limited options, and the risk/benefit profile remains favorable, in our view.

- **SRPT continues to trade at a discount to peers, as investors debate the tail of Elevidys (Exhibit 3).** SRPT is among a small cohort of biotech stocks who are cash flow positive and not yet mega/large-caps. Given the grouping of names that fit these criteria is limited, we expanded our peer group to include names that are near cash flow breakeven or are commercial with a growing base businesses. SRPT is trading at market cap. of ~$5.4B; with consensus revenue estimates of ~$3.1B for 2025 (includes sales and royalty revenue), the company is trading at a market cap./2025 sales multiple of ~1.8x. When this is compared to the multiples for ~20 peers, SRPT ranks second to last, with Jazz Pharmaceuticals (JAZZ, OP, Goodman) as the only company below them. The average market cap./2025 sales multiple for this cohort of companies is ~6.4x. If SRPT were trading in line with the average, this would imply a valuation of ~$20B or ~$182/share. When we remove the >10.0x multiple names, the average multiple drops to ~3.6x, or roughly a double from where SRPT shares are currently trading. If SRPT were trading at the adjusted average of ~3.6x, this would imply a valuation of ~$11.0B or ~$103/share, which still represents substantial upside from where shares are currently trading. We acknowledge that there remain some questions regarding the durability of the Elevidys franchise, especially with several next-generation therapies coming down the pike. However, as outlined above, the earliest commercialization for these options is likely 2027, thus SRPT still has another ~2 years to capitalize on the opportunity alone. Moreover, SRPT has been entrenched in the DMD space since the launch of Exondys 51 (eteplirsen), almost a decade ago. The other companies would need to build their own commercial infrastructure and counter detail against a product that has been established on the market for years (which will have 4+ years of commercial history by 2027).
- **We wonder if SRPT needs to execute on further BD to entice investors back into the stock.** In November 2024 (here), SRPT entered into a global licensing and collaboration agreement with Arrowhead (ARWR, MP, Foroohar), adding four clinical-stage siRNA assets for rare genetic diseases of the muscle, CNS, and lungs to SRPT's pipeline. The deal closed earlier this year, and we

expect preliminary data from the Ph.1/2 study (NCT06131983) with ARO-DUX4 in facioscapulohumeral muscular dystrophy/FSHD, as well as the Ph.1/2 trial (NCT06138743) for ARO-DM1 (myotonic dystrophy type 1/DM1), in 2H25 (here). At this juncture, the early data disclosed for these programs remains limited, thus it is difficult to assign a probability-of-success (PoS) to these assets (they are not included in our model and represent possible upside). Given the progress that other sponsors have made in the FSHD and DM1 space, including Avidity Biosciences (RNA) and Dyne Therapeutics (DYN, Not Rated), we are cautiously optimistic that SRPT can follow in their footsteps with ARO-DUX4 and ARO-DM1. On the flip side, SRPT's programs are earlier-stage, thus the bar from a competitive standpoint remains high (and may be raised as additional data from others becomes available). As the muscle programs are early stage, and considering the other assets licensed from ARWR are just as early or in an even earlier stage of development (e.g., ARO-MMP7 in idiopathic pulmonary fibrosis/IPF), we wonder if SRPT is thinking about potential business development/BD to supplement the mid-to-late-stage pipeline. While the company does have the limb-girdle muscular dystrophy/LGMD programs, which are in later development, these are gene therapies, an area that many investors have been shying away from. Moreover, the market sizes for the various LGMD subtypes are relatively limited. Thus, with ~$1.5B in cash, cash equivalents, restricted cash, and investments on the balance sheet as of the end of 2024, the recent $600M senior secured revolving credit facility, combined with the cash flow expected to be generated this year, the company has a healthy balance sheet that could support additional BD. We continue to believe that protecting the ~$1.0B exon skipping franchise would be prudent, especially given the discontinuation of vesleteplirsen (SRP-5051) last year (here), while other near-commercial deals could be viewed favorably.

Updating our Gene Therapy Market Model Assumptions

- **We are moderating our non-ambulatory revenue projections for Elevidys.** The DMD patient population is thought to be roughly evenly split between ambulatory and non-ambulatory patients. Prior to the expanded label, we assumed only use in ambulatory patients, especially given we had not seen data in this patient group. We note that the Ph.3 ENVISION study (NCT05881408) is ongoing and has a primary completion date in 2027. However, with the label expanded in June 2024 to also include non-ambulatory patients, we incorporated estimates from this patient group in our SRPT model. Given the safety signal in the older boy, combined with KOL commentary, we think it is prudent at this juncture to incrementally moderate these estimates in the outer years (no change to FY25). We now assume gross peak US sales of ~$2.6B (2026E), down from ~$3.4B (**Exhibit 2**). This change was primarily driven by a tapering of penetration assumptions in non-ambulatory patients. We previously assumed at peak ~500 non-ambulatory boys would be treated with Elevidys; however, now assume this is closer to ~200. Our probability-weighted and royalty-adjusted worldwide sales of Elevidys drops to ~$2.7B (from ~$3.5B). We note that management previously mentioned over 100 non-ambulatory patients have been treated following the expanded label in June 2024. This was mentioned early in 2025, so assuming the same run rate, this implies ~200 non-ambulatory boys could be treated per year. However, that assumes no growth, which we think is unreasonable. The breakdown of use among ambulatory and non-ambulatory boys is something we will continue to monitor as the launch progresses, especially as we have seen less than 12 months launch with this expanded label. In the EU, we continue to

assume use in only ambulatory boys; however, we are tempering our estimates. Partner Roche (RHHBY, Not Rated) expects an EU regulatory decision in 2025; however, this could be delayed incrementally, given the EMA has placed the program on clinical hold until the investigation into the recent death is complete (here). Collectively, these changes lower our PT to $150 (from $200) for SRPT.

- **Following SLDB's solid initial data disclosure, we are raising our numbers (Exhibit 4).** Ahead of the first data readout for SGT-003, we had relatively conservative assumptions, with gross peak sales of ~$0.9B (US + EU + RoW). This required only a few hundred patients to be treated at peak, which we viewed as a reasonable point estimate, given SGT-003 may be the third gene therapy to market. However, after the disclosure of expression data from a handful of patients earlier this year, combined with the potential to accelerate the time to market, we are incrementally raising our estimates. We now assume gross peak sales of just over $0.5B in the US, while estimates in the EU/RoW remain unchanged (gross peak sales across all geographies is now ~$1.1B). We note that our estimates remain relatively conservative versus consensus, which currently assumes ~$1.5B in peak sales in the US alone. We assume SGT-003 is only used in ambulatory patients, which could explain the difference versus consensus. Moreover, redosing represents another avenue for potential upside. Just as we wrote last year for SLDB (here), we view shares as fundamentally undervalued based on the DMD gene therapy program alone. Now with clinical data in hand and additional results expected this year, we continue to see a significant value disconnect with SLDB shares, especially with the several cardiac gene therapy assets (deep dive here). The increase in our SGT-003 estimates is offset by an increase in operating expense assumptions. Thus, our 12-month PT remains unchanged at $20.
- **Putting it all together, we estimate the aggregate opportunity for gene therapy in DMD can grow to almost $3.0B in the US alone (Exhibits 5-6).** Despite the tapering of our Elevidys estimates, which was partially offset by an increase in our SGT-003 numbers, we continue to believe that the DMD gene therapy opportunity can continue to grow towards a potential peak of almost $3.0B in the US alone (we previously estimated a total market of ~$3.5B; here). Our market model includes assumptions for Elevidys, RGX-202, and SGT-003, while earlier stage assets, including those from INSM and Kate, represent potential upside to market growth. For Elevidys, we assume modest growth in 2026 (+27% YoY), with US sales peaking at ~$2.6B. Considering the potential entrance of both RGX-202 and SGT-003 in 2027, we assume sales then begin to modestly decline YoY, as share is lost to competition. For RGX-202, pivotal data are expected in 1H26, thus in our market model we assume a US launch in 2027. Our colleagues assume gross US peak sales of $201M (2029E). Finally, for SGT-003, we assume the company can quickly follow in RGNX's footsteps regarding an Accelerated Approval pathway. We assume a US launch in 2027, with gross peak sales of $540M (2030E). At our combined peak US sales estimate of ~$2.9B in 2029E, we assume this consists of ~78%, ~7% and ~15% share for Elevidys, RGX-202, and SGT-003, respectively. We believe that this highlights how conservative estimates are for next-generation players and the potential upside. Moreover, for next-generation assets, we assume only use in ambulatory patients. Redosing represents potential upside as well.
- **Based on our estimates, what could be left for other players once gaining approval?** With gross peak sales of $2.6B in 2027, we assume that Elevidys can treat a significant chunk of the market before other options make it to the

commercial stage (>3,400 patients treated by the end of 2027). While this seems like a significant portion of the market, we estimate a prevalence of almost 18,000 DMD patients by 2027, 50% of which are ambulatory (and therefore eligible for either RGX-202 or SGT-003, per our currently published model). When RGX-202 and SGT-003 are approved and launched, likely in 2027, our assumptions imply that there will still be almost 7,000 ambulatory DMD patients who have not yet been treated with a gene therapy (**Exhibit 7**). Moreover, with >150 incidence patients per year, that represents another ~$0.3B opportunity per year (i.e., 150 × $1.8M per patient). As we wrote in our prior note (here), given the unique market dynamics of a one-and-done gene therapy, how gene therapy uptake trends in the early years will have a significant impact on uptake in the outer years, for all potential products. Thus, we continue to watch the launch of Elevidys closely, as this will have major implications for what market remains for the other players. It is clear that not every single DMD patient will be treated with Elevidys, even though it is first to market with a significant lead. We believe that this market is large enough for multiple sponsors and with gene therapy pricing, each company can generate significant sales with relatively conservative patient numbers. Moreover, improvements on efficacy and/or safety may entice parents/patients to opt for one gene therapy over another, once there are multiple options approved.

Select Recent Advancements Across the DMD Space

- **RNA.** In March 2025, RNA disclosed positive topline data from the Ph.1/2 EXPLORE 44 study (NCT05670730) with delpacibart zotadirsen (del-zota). Del-zota is an antibody oligonucleotide conjugate (AOC) designed to deliver phosphorodiamidate morpholino oligomers (PMO) to skeletal muscle and heart tissue to specifically skip exon 44 of dystrophin mRNA to enable production of near full-length dystrophin (here). These data built on RNA's first update for del-zota in August of last year (here). The 10mg/kg data disclosed in March 2025 demonstrated consistent results, as compared to the 5mg/kg results last year. In the 10mg/kg cohort (n=10), patients received 3 doses (Q8W). Tissue concentration was ~200nM, in line with 5mg/kg. Exon skipping was also consistent, with a statistically significant 43% increase in exon 44 skipping (up to 67% observed) and statistically significant increase of 26% of normal dystrophin expression (up to 58% was observed). Reductions in creatine kinase (CK) remained above 80% and CK levels continue to be near normal (sustained in the OLE for up to one year). While the exon skipping was slightly and numerically higher with 10 mg/kg, RNA said they were not statistically different and were not sufficient to drive differences in dystrophin expression. The company is moving ahead with 5mg/kg (Q6W) as the registrational dose for the BLA. All patients are transitioning to the 5mg/kg dose and an additional 16 patients were enrolled directly into the OLE (total sample size of 39). Importantly, the dystrophin data generated thus far is sufficient to support a planned BLA submission under Accelerated Approval, expected in the fourth quarter (likely near year-end).
- **Capricor Therapeutics (CAPR, Not Rated).** Following meetings with the FDA last year, CAPR disclosed plans to file a BLA for full approval of deramiocel for the treatment of DMD cardiomyopathy in September 2024 (PR here). Deramiocel is a cell therapy, comprised of cardiosphere-derived cells (CDCs), which are a rare population of cardiac cells isolated from donated cells of healthy human hearts. The company initiated the rolling BLA in October 2024, which was completed in January 2025. In March 2025, the FDA accepted the BLA under priority review, with a PDUFA action target date of August 31, 2025. The filing

was supported by results from the Ph.2 HOPE-2 study (NCT03406780), as well as the HOPE-2 OLE (NCT04428476) trial. Several cuts of data have been shared from the HOPE-2 studies, most recently at the 2025 Muscular Dystrophy Association (MDA) Conference in March (see poster here). Deramiocel is dosed via intravenous infusion every three months; 4 total doses were given in the randomized portion, while the OLE allows for a total of 20 infusions. Over 36 months of treatment, patients treated with deramiocel (n=13) had a modeled yearly decline in Performance of the Upper Limb (PUL) total score of 3.46 points, compared to a mean decline of 7.19 points in the external comparator group (p=0.019); this represents a 52% slowing of disease progression. Deramiocel has also demonstrated improvement in various cardiac measures, including left ventricular ejection fraction, and end systolic/diastolic indexed volumes. Given deramiocel's novel mechanism of action, combined with the focus on cardiomyopathy, it remains to be seen how the treatment option fits into the broader paradigm. But this is another potential entrant that we are watching.

- **Cumberland Pharmaceuticals (CPIX, Not Rated).** In February 2025 (PR here; MDA presentation here), the company disclosed topline results from the Ph.2 FIGHT-DMD study (NCT03340675) with oral ifetroban (Dyscorban). Ifetroban is a selective thromboxane-prostanoid receptor (TPr) antagonist, which is expressed on many cell types, including platelets, immune cells, smooth muscle, and cardiomyocytes. Cumberland is exploring the treatment in DMD, as well as systemic sclerosis (scleroderma) and IPF. The FIGHT-DMD study enrolled 41 DMD patients, who received either 100mg (n=12), 300mg (n=18), or placebo (n=11), once daily. The primary endpoint was the improvement in left ventricular ejection fraction (12 months). Those treated with 300mg ifetroban demonstrated an ejection fraction improvement of +1.8%, compared to 0% for 100mg, and -1.5% for placebo (treatment difference of 3.3%; p=0.108). Cumberland also compared these results to matched natural history controls, which showed a 3.6% decline (treatment difference of 5.4%; p=0.002). It is typically thought that those with DMD experience a 2% annual left ventricular ejection fraction decline. Thus, the 5.4% difference with ifetroban suggests a potentially clinically meaningful effect. Moreover, ifetroban may be able to be used alongside steroids and other DMD treatments (the treatment is also broadly applicable, regardless of mutation type). The company plans to meet with the FDA in an end of Ph.2 meeting.
- **DYN.** In March 2025, DYN disclosed longer-term data from the ongoing Ph.1/2 DELIVER study (NCT05524883) with DYNE-251. These results built incrementally on the several prior data cuts in January (here), May (here), and September (here) 2024; we also saw updated safety/tolerability in January 2025 (here). DYNE-251 consists of a PMO conjugated to a proprietary Fab (a linker and a payload) targeting transferrin receptor 1 (TfR1). See our prior deep dive on the nuances of this approach versus RNA's (It's What's Inside (the Cell) That Counts: Evidence Points Toward Nuclear RNAi). At the selected registrational dose (20mg/kg; Q4W), DMD patients amenable to exon 51 skipping demonstrated a mean absolute dystrophin expression of 8.72% of normal (adjusted for muscle content) at 6 months (3.72% unadjusted). This compares to 7.64% with the 10mg/kg dose. Regarding functional measures, DYNE-251 has demonstrated improvements in Stride Velocity 95th Centile (SV95C), North Star Ambulatory Assessment (NSAA), time rise from the floor, and timed 10-meter walk/run test (out to 18 months for some patients; n=6). The DELIVER registrational expansion cohort of 32 patients

has been fully enrolled, and we expect data in late 2025. The company plans to leverage the Accelerated Approval pathway and file a BLA in early 2026.

- **Genethon.** In April 2024 the company shared initial data from the Ph.1/2 study (here), with another incremental update in November 2024 (here). The study enrolled ambulatory boys aged 6 to 10 years old (stable or in the decline phase of the motor score). These boys had a stable baseline steroids for ≥6 months and received a short term of prophylaxis with sirolimus and add-on steroids. Five patients were treated at one of two dose levels: two at the first and three at the higher level (3E13 GC/kg). For those treated at the higher dose level, up to 85% of muscle fibers expressed microdystrophin (mean 54%; 15%-85%) as measured by immunohistochemistry (8 weeks after treatment). Vector genome copies/muscle fiber nuclei were up to 2.4 (mean 1.2). A reduction in CK was also demonstrated, this was between 50-87% (mean 74%) 12 weeks after treatment and persistent for up to 18 months of follow-up for the first two patients dosed at this level. A comparison with an external control group from the NIH study was conducted to explore GNT004's clinical efficacy, which suggested a stabilization or improvement in functional measures (delta of 4.7-points on NSAA and 0.1m/s for SV95C). Regarding safety, GNT0004 has been well tolerated in all patients receiving the prophylaxis, 5 adverse drug reactions have been reported (including the SUSAR case) and 4 mild events. Genethon plans to start the pivotal phase in more than 60 children in Europe in the second quarter of 2025, followed by the US.
- **Keros Therapeutics (KROS, OP, Smith).** In March 2025, KROS disclosed initial topline data (PR here, presentation here) from the Ph.1 study with KER-065 in healthy volunteers (see our colleague's note here). KER-065 is designed to bind to and inhibit TGF-ß ligands, including myostatin (GDF8) and activin A, which are negative regulators of muscle and bone mass and strength. The Ph.1 study is split into Part 1 (SAD; 1, 2, and 3mg/kg) and Part 2 (MAD; 1.25 and 2mg/kg). Part 1 had a treatment period of 28 days, while Part 2 dosed patients for 85 days. Given the early stage, the focus was primarily on safety and tolerability. KER-065 was generally well-tolerated. All injection-site reactions (expect two Grade 3) were not severe (all resolved without sequelae). All headache AEs (except one Grade 2) were mild (all resolved without sequelae). Increases in hemoglobin were also seen; however, these were asymptomatic and reversible. Interestingly, treatment suggested a potential benefit on bone anabolic activity (simultaneously increasing bone formation, while inhibiting bone resorption). An increase in whole body bone mineral density (BMD) was also seen, alongside decreases in fat mass, and increases in muscle mass. These effects were not clearly dose dependent, which management attributed to the low sample size, wide range of baseline characteristics, and similarly in dose levels between the 1.5 and 2.0mg.kg groups. Looking ahead, KROS plans to engage with regulators on the design of a Ph.2 study in patients with DMD in 3Q25, with a study start planned for 1Q26.
- **RGNX.** In March 2025, RGNX shared incremental data from the Ph.1/2 AFFINITY DUCHENNE study in two additional patients (aged 3 and 7 years old). These results built off the several prior data cuts (see our colleague's notes here, here, here). The two patients were treated with the pivotal dose of RGX-202 (2E14 GC/kg). At 12 weeks, microdystrophin expression was measured to be 122.3% and 31.5% in the three- and seven-year-old, respectively. Across the 8 patients dosed at the pivotal level so far, the mean microdystrophin expression has ranged from 39.7% (n=5) in the 8-11 year olds to 122.3% (n=1) in the 1-3-year-olds (54.3% for the 4-7-year-olds; n=2). For reference, the mean change from baseline in

microdystrophin across the several Elevidys studies was 40.1-51.0% (FDA label here). Regarding functional results, for the two patients with data at 9 months, RGX-202 demonstrated improvements in time to stand, 10-meter walk/run, time to climb, and NSAA. Across all five patients with available functional data, the improvements with RGX-202 exceed external natural history controls. We expect additional interim functional data in 1H25. Regarding safety, RGX-202 has been relatively well-tolerated in patients thus far. The most common drug-related AEs have been nausea, vomiting, and fatigue. We note that the study protocol requires an immunosuppression regimen including Soliris and sirolimus, which does not seem to be appreciated by many investors. Enrollment in the pivotal portion of AFFINITY DUCHENNE is expected to complete in 2H25, followed by topline data in 1H26, and a BLA submission under Accelerated Approval in mid-2026.

- **Satellos Bioscience (MSCLF, Not Rated).** In March 2025, Satellos disclosed initial data from the Ph.1 trial (NCT06565208) with SAT-3247 in healthy volunteers (PR here, presentation here). SAT-3247 has a unique mechanism of action and is designed to rescue the ability of muscle stem cells to drive repair and regeneration. The company screened signaling pathways to identify ways to replace the missing dystrophin and identified AAK1, a protein in the notch signaling pathway (notch signaling is a conserved pathway that regulates cell proliferation, cell fate, and differentiation), as a potential drug target. Following a proof-of-concept study in the canine model of DMD, Satallos moved forward with a clinical program in humans. The Ph.1a study was designed to assess the safety and tolerability of SAT-3247 in 72 healthy volunteers. Individuals were randomized across five SAD cohorts (including one food effect cohort) with single oral doses of up to 400mg and four MAD cohorts with daily oral doses up to 240mg/day for 7 days. Results were shared as of a February 20, 2025 cut-off, with SAT-3247 showing a well tolerated profile. Additionally, the pharmacokinetic data demonstrated consistency with the preclinical studies. Full Ph.1a data are expected in the second quarter, as well as Ph.1b data in the second quarter (likely May 2025). The Ph.1b study will include 10 adults with DMD (safety and pharmacokinetic results at 28 days).
- **SLDB.** In February 2025, SLDB shared initial data from the Ph.1/2 INSPIRE DUCHENNE study from the first three patients, aged 5, 5, and 7 years old at the time of dosing (here). Patients were dosed at a level of 1E14 GC/kg, which is lower than Elevidys (1.33E14 GC/kg) and RGX-202 (2E14 GC/kg). The interim 90-day biopsy results suggest an average microdystrophin expression of 110% (84%, 112%, and 135%). This remains some of the highest levels of microdystrophin expression we have ever seen in DMD. Moreover, dystrophin positive fibers of 70%, 77%, and 88% (mean of 78%) are also the highest we have seen among other gene therapy candidates. Treatment also improved a whole suite of biomarkers, ranging from biomarkers of acute muscle injury to chronic muscle damage and muscle maturation which could very likely translate to impressive clinical improvements. Moreover, there may even be evidence of an early potential cardiac benefit, with improvements in left ventricle ejection fraction. Considering cardiomyopathy is the leading cause of death in DMD, we are intrigued by these results. Regarding safety, SGT-003 treatment has been generally well tolerated in patients thus far (safety database of n=6), with no SAEs, SUSARs, or TMA/aHUS observed. The most common AEs include nausea/vomiting, thrombocytopenia (1 Grade 3), an infusion related hypersensitivity reaction (1 Grade 3), and fever. We note that there has been one episode of mild transient hs-troponin I elevation

(1 Grade 1). Importantly, SGT-003 is given with just prophylactic glucocorticoids alone; no intensive immunomodulation regimen has been required and no changes are being made as a result of the clean safety profile seen to date, which we think are positive attributes that are underappreciated following the patient death following Elevidys and steroid treatment. The company plans to enroll and dose more than 10 patients by early 2Q25 and 20 patients by 4Q25. SLDB will approach the FDA after 10-12 patients have been dosed in mid-2025 (with 90-day data in hand) to discuss their potential path for Accelerated Approval, which we view as an important catalyst for this program to get more credit. Considering RGNX's alignment with the FDA on a relatively flexible primary endpoint of proportion of patients ≥10% microdystrophin expression in a single-arm study, we believe that a similar path should also be available to SLDB. We believe investors remain cautious about the availability of the Accelerated Approval path for DMD gene therapy sponsors beyond SRPT, especially following the recent patient death and Peter Mark's departure from the FDA. However, if safety remains clean we believe the FDA will want to provide patients with more options beyond Elevidys, and we have heard positive commentary from uniQure (QURE, OP) about Dr. Marks' ethos being ingrained throughout CBER more broadly, so we remain optimistic for SLDB's ability to execute their plan to market. In a nutshell, while QURE's AMT-130 gene therapy for Huntington's disease has benefited from Dr. Marks' growth mindset, there was also a very high level of engagement and enthusiasm from another 30+ FDA members in attendance at their in-person Type B multi-disciplinary meeting (which Dr. Marks did not attend, according to management).

- **Wave Biosciences (WVE).** In March 2025, WVE disclosed positive data from the Ph.1/2 FORWARD-53 study (NCT04906460) with WVE-N531 at 48 weeks (here); this follows the interim data announced in September 2024 (here). WVE-N531 is an exon skipping oligonucleotide in development as a treatment for patients with DMD amenable to exon 53 skipping. It is WVE's first splicing candidate incorporating the PN backbone chemistry. Patients were dosed with WVE-N531 at 10mg/kg every two weeks (Q2W). After 48 weeks, mean dystrophin expression was 6.4% (n=8), while mean exon skipping was 54%. Importantly, 88% of the boy were able to achieve greater than 5% average dystrophin expression (associated with the milder Becker phenotype) between 24 and 28 weeks. We note that the dystrophin expression is numerically lower than their prior update, but is within the established 30-35% intra-assay variability for Western blot. WVE also reported significant improvements in multiple indicators of muscle health (e.g., MCP-1, IL-6, CK, and level of muscle fibrosis). Regarding functional assessments, benefits were seen across time to rise and NSAA compared with a matched exon 53 natural history control group. Time to rise showed a statistically significant 3.8-second difference ($p<0.05$) favoring WVE-N531 compared with natural history, while NSAA showed positive trends favoring WVE-N531 relative to natural history (1.2-point improvement; not significant). Following recent feedback from the FDA, WVE intends to file a for Accelerated Approval next year (with data to support monthly dosing at launch).

What to Watch for the Rest of this Year & Beyond

- **BioMarin (BMRN) will have proof-of-concept data with BMN 351 in mid-2025.** Following BMRN's experience with drisapersen, the company went back to the drawing board and developed a true version 2.0 (but stayed with the same phosphorothioate/non-morpholino chemistry), ultimately leading to BMN 351.

This is a next-generation oligonucleotide for DMD patients amenable to exon 51 skipping, currently progressing in a Ph.1/2 study (NCT06280209). As we discussed with management last month (here), they have dosed patients in both cohorts (6 and 9mg/kg) and expect to share 26-week data in mid-2025. In terms of the target product profile, management reiterated to us that their goal is 10% dystrophin expression at steady state, which they think will be best in class. When discussing the asset earlier this year at a competitor conference, management mentioned "*we also are eagerly awaiting that 26-week data because we have some data in-house. Now it's very early, but we have two patients with muscle biopsies at 13 weeks and, again, steady state is actually out close to a year or farther. So, this is a very early time point, very small numbers, but from that data, we've been able to determine that, indeed, we can penetrate the muscle, so we're seeing PK there. We can induce skip induction, we see the gene product, and we are seeing measurable levels of functional dystrophin.*" The company plans to share the model for BMN 351 prospectively, so investors can appreciate the expected S-shaped curve (the full effect is expected around 52 weeks). We think this will be important for investors to get comfortable with the BMN 351 program, especially as other players in the space have recently shared results at earlier time points and as we do not expect to see double-digit dystrophin expression with BMN 351 at 26-weeks. Ideally, the model that BMRN shares ahead of time will allow us to extrapolate the early data out to 52 weeks, where that target product profile may be demonstrated. Mechanistically, BMRN is taking a very different approach from all other next-generation exon skippers since BMN 351 leverages different biology (unique splice site) rather than chemistry (conjugation or modifications to backbone chemistry) to drive uptake into muscle and activity, which is unprecedented. Looking ahead, we expect topline data in mid-2025, along with a more detailed presentation at a major medical meeting in 2H25 (potentially the World Muscle Society in October). Since investors are looking for growth drivers beyond Voxzogo (vosoritide) in achondroplasia and other growth stature disorders, BMN 351 could potentially become an important program for BMRN, although we believe its efficacy will need to be very competitive since it is being administered more frequently than other next-generation exon skippers (once-weekly versus once-every 4-6 weeks IV).

- **Edgewise Therapeutics (EWTX) is expected to disclose full LYNX and FOX data in 2Q25.** The focus for EWTX has been squarely on the hypertrophic cardiomyopathy/HCM program with EDG-7500 (our note here). However, some investors seem to forget that the company also has an entire neuromuscular platform as well, centered around sevasemten (EDG-5506) in DMD as well as Becker muscular dystrophy (BMD). We view the approach with sevasemten as particularly intriguing, especially as many competitors in the space are targeting dystrophin, while sevasemten is an orthogonal approach focused on protecting muscle damage inducted by contractions from activities of daily living. Sevasemten is an orally administered, allosteric, selective, fast myofiber (type II) myosin inhibitor that is designed to be inactive against slow myofiber (type I) myosin present in both skeletal muscle and the heart. While sevasemten does not correct the root cause of the disease, we see the potential for sevasemten to be combined with other treatment modalities. For DMD, sevasemten is progressing in the Ph.2 LYNX study (NCT05540860) in children aged 4-9 years old, as well as the Ph.2 FOX study (NCT06100887) in children and adolescents (6-17 years old) who have been previously treated with a gene therapy. As we recently wrote,

some investors may be a bit frustrated that the LYNX/FOX data continue to be pushed out (here), but results are now expected this quarter, which could draw some interest (especially now that HCM data have already been disclosed). For the upcoming DMD readout, please refer to our prior deep dive on the topic (here). We think the upcoming readout could include a robust sample size; at last update, we heard there were ~70 patients across 6 doses in LYNX plus additional "clean" cohorts of patients at 5 and 10 mg and ~30 patients in FOX (prior gene therapy treatment). As for BMD, positive Ph.2 data from the CANYON study (NCT05291091) were disclosed in December 2024 (here). We expect an update on the path to approval (i.e., potential for Accelerated Approval) this quarter.

- **We are watching the regulatory updates for near-commercial exon skippers.** There are three currently approved exon skippers in the US (all from SRPT); Exondys 51 (eteplirsen; FDA label), Vyondys 53 (golodirsen; FDA label), and Amondys 45 (casimersen; FDA label). However, we could start to see a shift in the exon skipping landscape next year, with several new entrants. First up, RNA plans to file a BLA under Accelerated Approval for del-zota (exon 44) around the end of the year, which positions the company for a potential commercial launch in 2026. The exon 44 patient population is relatively small (~900 in the US); however, there are no approved exon skippers for those with this mutation. Next up is DYN, we expect the registrational data from DELIVER (n=32) in late 2025 with DYNE-251, followed by a BLA filing under Accelerated Approval in early 2026, which positions the company for a commercial launch later next year. Assuming approval, this would possibly be the first therapy to compete with Exondys 51, in patients who are amenable to exon 51 skipping (Exondys 51 is dosed once weekly, whereas DYNE-251 is dosed Q4W). SRPT's entire PMO franchise generated sales of almost $1.0B in 2024, with an estimated ~$535M coming from Exondys 51. The Street assumes relatively modest sales for DYNE-251 next year and consensus is currently ~$18M (meanwhile, we assume modest erosion YoY for Exondys 51). Lastly, following recent feedback from the FDA, WVE intends to file for Accelerated Approval with WVE-N531 next year, which positions the company for a potential commercial launch later in 2026. Assuming approval, this would possibly be the first therapy to compete with Vyondys 53, in patients who are amenable to exon 53 skipping (Vyondys 53 is dosed once weekly, whereas we expect WVE-N531 to be dosed Q4W). Vyondys 53 produced an estimated ~ $130M in sales in 2024 (which we expect will erode modestly in 2026). For WVE-N531, consensus currently estimates gross sales of ~$64M next year.
- **PepGen (PEPG) will disclose 10mg/kg data in 3Q25.** Last year, the company reported results from the 5mg/kg cohort in the Ph.2 CONNECT1 study (NCT06079736) of PGN-EDO51 in patients amenable to an exon 51 skipping approach (here). These results narrowly missed PEPG's expectations of 1% or greater dystrophin expression; however, results from the 10mg/kg are expected in the third quarter, which could show higher expression levels. We note that two patients have experienced asymptomatic hypomagnesemia at 10 mg/kg (here), so the results at the higher dose will be important in determining next steps and if the risk/benefit makes sense. The 10mg/kg cohort is fully enrolled with 4 patients. As we discussed with management last month at our Leerink conference (here), PEPG will look at the totality of the data and think that the levels of exon skipping that they have generated so far (which were on par with competitors at a dose that was double what PEPG used and with twice the length of follow-up) should

continue to increase with higher dose and translate to a dystrophin number that could be considerably higher than what some investors are expecting.

- **Keeping a close eye on the continued launch of Elevidys...** As we noted above, given the unique market dynamics of a one-and-done gene therapy, how gene therapy uptake trends in the early years will have a significant impact on uptake in the outer years, for all potential products. Thus, we continue to watch the launch of Elevidys closely, as this will have major implications for what market remains for the other players, when they eventually gain approval. Additionally, for SRPT specifically, we think additional commentary from the company following the patient death will be important, especially as it relates to what the field force is seeing in terms of underlying patient demand pre- and post-event. As we outlined above, we continue to estimate FY25 sales of ~$2.1B (in line with consensus); however, this assumes we do not see a significant drop off in patient demand. Our recent checks have indicated that physicians are infusing patients at a rate of 1-2 per month, which should allow this guidance to be met, but we continue to monitor this. Management last reiterated FY25 guidance on the 4Q24 all in late February 2025, so we are also looking forward to comments on this, likely on the 1Q call.
- **...but also watching the next-generation gene therapy names.** Outside the additional functional data from RGNX in 1H25, we think SLDB's discussion with the FDA regarding accelerated approval in mid-2025 will be an important event to watch for gene therapy broadly. While the Accelerated Approval pathway has been blazed by SRPT, with RGNX also reaching alignment last year on what the path looks like, SLDB will be one of the first sponsors meeting with the FDA under the new administration. We think that it is relatively likely that SLDB will also be able to leverage a similar path (primary endpoint of proportion of patients ≥10% microdystrophin expression in a single-arm study), but given the recent regulatory uncertainty, we sense some nervousness around this event. In parallel, we also think patient advocacy groups will continue to push for additional options as quickly as possible (especially since Elevidys' efficacy leaves room for improvement and cannot treat all patients). Regarding the earlier stage gene therapies, we await the initiation of Genethon's pivotal study in 2Q25 (US to follow thereafter) and any color on regulatory timelines, any forward progress with Kate's KT-809 into the clinic, as well as initial data with INSM's INS1201, likely in 2026.

Exhibit 1. Elevidys was first, but there are a handful of next-generation options

Therapy	Vector/Capsid	Dystrophin Expression	Functional Data	Safety Profile	Status	Milestones
Elevidys	AAVrh74	40.1-51.0%[a]	+2.57-Points (NSAA) -0.27-Second (TTR)[b]	Acute serious liver injury, immune-mediated myositis, myocarditis	Approved	EMA Decision: 2025
RGX-202	AAV8	39.7-122.3%[c]	+5.5-Points (NSAA) -0.5-Second (TTR)[d]	Nausea, vomiting, fatigue[e]	Ph.3	Add'l Functional Data: 1H25
SGT-003	AAV-SLB101	84-135%[f]	--	Nausea, vomiting, thrombocytopenia, one episode of mild transient hs-troponin I elevation	Ph.1/2	Request Meeting to Discuss AA Path: Mid-2025
GNT0004	AAV8	--	+4.7-Points (NSAA)[g] +0.1m/s (SV95C)	One SUSAR. 4 adverse drug events, 4 mild	Ph.1/2	Pivotal Start: 2Q25
INS1201	AAV9	--	--	--	Ph.1/2	Initial Data: 2026
KT-809	MyoAAV-LD 6.1	--	--	--	Preclinical	--

Despite Elevidys' first-to-market status, the Accelerated Approval pathway appears open for other sponsors to take advantage of. Last fall, RGNX disclosed alignment with the FDA on the AFFINITY DUCHENNE pivotal program for Accelerated Approval. The trial will enroll ~30 patients, with a primary endpoint of the proportion of participants whose microdystrophin expression is ≥10% at week 12 (here).

Elevidys (SRP-9001) was granted Accelerated Approval in a subset of patients in June 2023. In June of last year, the approval was expanded to include almost all patients regardless of ambulation status. Behind Elevidys, there are a handful of next-generation gene therapy programs progressing, including RGX-202 (RGNX) and SGT-003 (SLDB).

- **RGX-202:** RGNX has shared several positive data cuts from the Ph.1/2 portion of the AFFINITY DUCHENNE study (NCT05693142). The trial is currently enrolling patients and results from the pivotal portion are expected in 1H26.
- **SGT-003:** SLDB shared initial data from the Ph.1/2 INSPIRE DUCHENNE study (NCT06138639) in three patients early in 2025 (here). The company plans to have >10 patients dosed by early 2Q25. On the regulatory side, SLDB plans to request a meeting with the FDA to discuss an Accelerated Approval path in mid-2025. We assume US approval in 2027.

Source: Leerink Partners Research, FDA Label, Company Presentations (AA; Accelerated Approval)

[a]FDA label (here); mean change from baseline at week 12.

[b]FDA label (here); absolute benefit at week 52.

[c]RGNX April 2025 presentation (here); dose level two, mean expression at week 12.

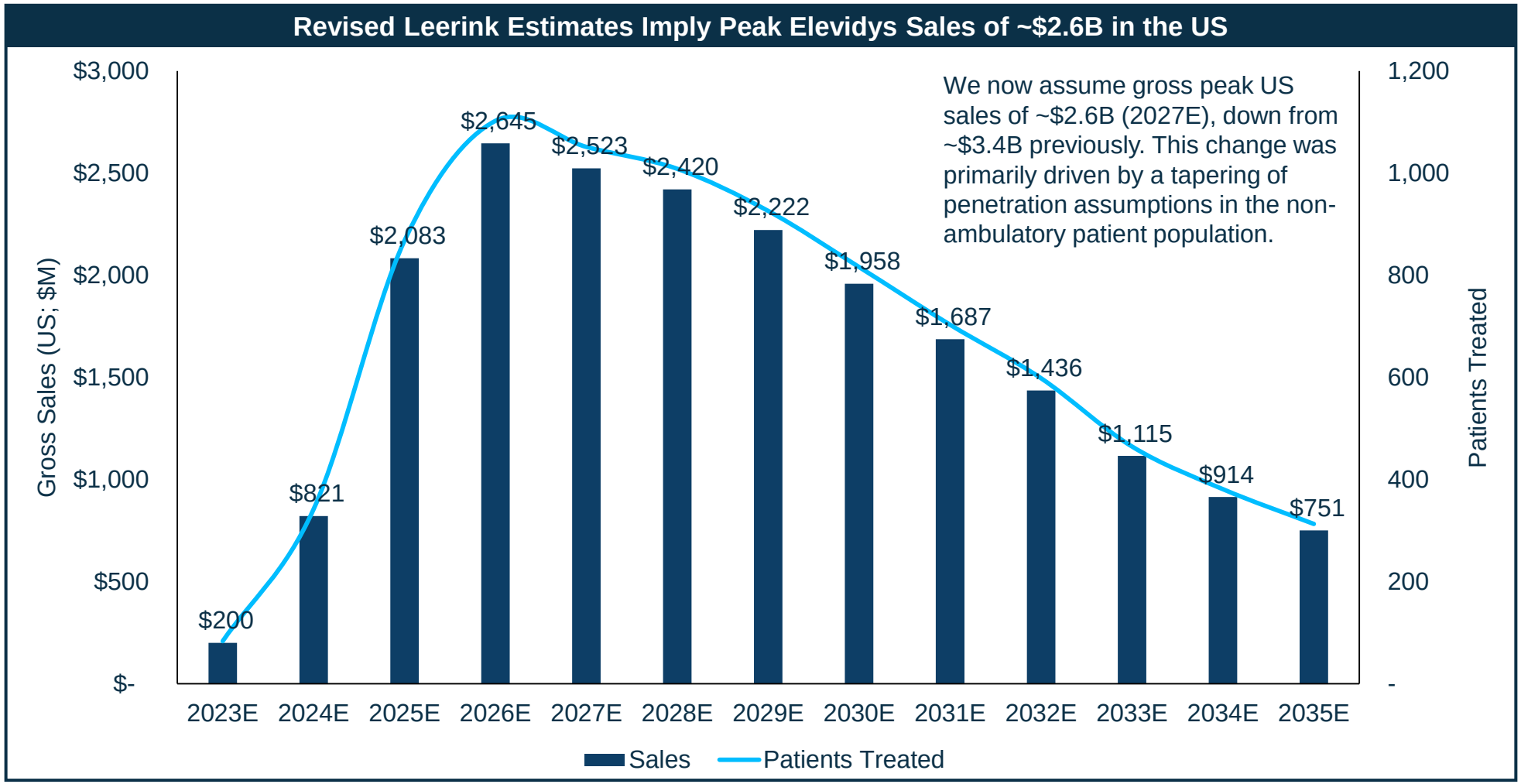
[d]RGNX April 2025 Presentation (here); dose level two, absolute benefit at month 9.

[e]Study requires an immunosuppression regimen including Soliris and sirolimus.

[f]SLDB February 2025 presentation (here); mean change at day 90.

[g]MDA 2025 Abstract (here); 12 months, 4.7-point delta.

Exhibit 2. We remain confident in ’25 numbers, but are tapering ’26+ estimates



We continue to assume Elevidys sales of ~$2.1B in 2025, in line with consensus estimates and aligned with management’s guidance of net product revenue of $2.9-3.1B (inclusive of the PMO franchise). However, we are tapering outer year estimates.

- Considering the safety signal in the older boy, combined with MEDACorp KOL commentary following the death, we think it is prudent at this juncture to incrementally moderate our estimates in the outer years (no change to FY25). Additionally, recent data from RGNX and SLDB suggest that they could be real contenders in the space, thus this trimming of our estimates takes competition into account.

Source: Leerink Partners Research

Exhibit 3. SRPT now trades at one of the cheapest multiples among peers

Ticker	Market Cap. ($B)	2025 Sales Consensus Estimates ($B)	Market Cap. / Sales Multiple
ASND	$9.6	$0.6	16.1x
ALNY	$30.3	$2.2	13.7x
MDGL	$6.5	$0.6	11.4x
KRYS	$4.7	$0.5	10.3x
ARGX	$36.4	$3.6	10.1x
TGTX	$5.8	$0.6	10.1x
AXSM	$5.0	$0.6	8.2x
BPMC	$5.4	$0.7	7.4x
UTHR	$12.8	$3.2	4.0x
NBIX	$10.0	$2.7	3.7x
BMRN	$11.2	$3.1	3.6x
FOLD	$2.1	$0.6	3.3x
ALKS	$4.5	$1.4	3.2x
APLS	$2.3	$0.9	2.6x
INCY	$11.0	$4.7	2.4x
BIIB	$17.1	$9.2	1.9x
SRPT	$5.4	$3.1	1.8x
JAZZ	$6.1	$4.3	1.4x
Average			6.4x

If we remove the outliers above 10.0x, the average for the peer group drops to ~3.6x. Applying this multiple to SRPT implies a PT of ~$103.

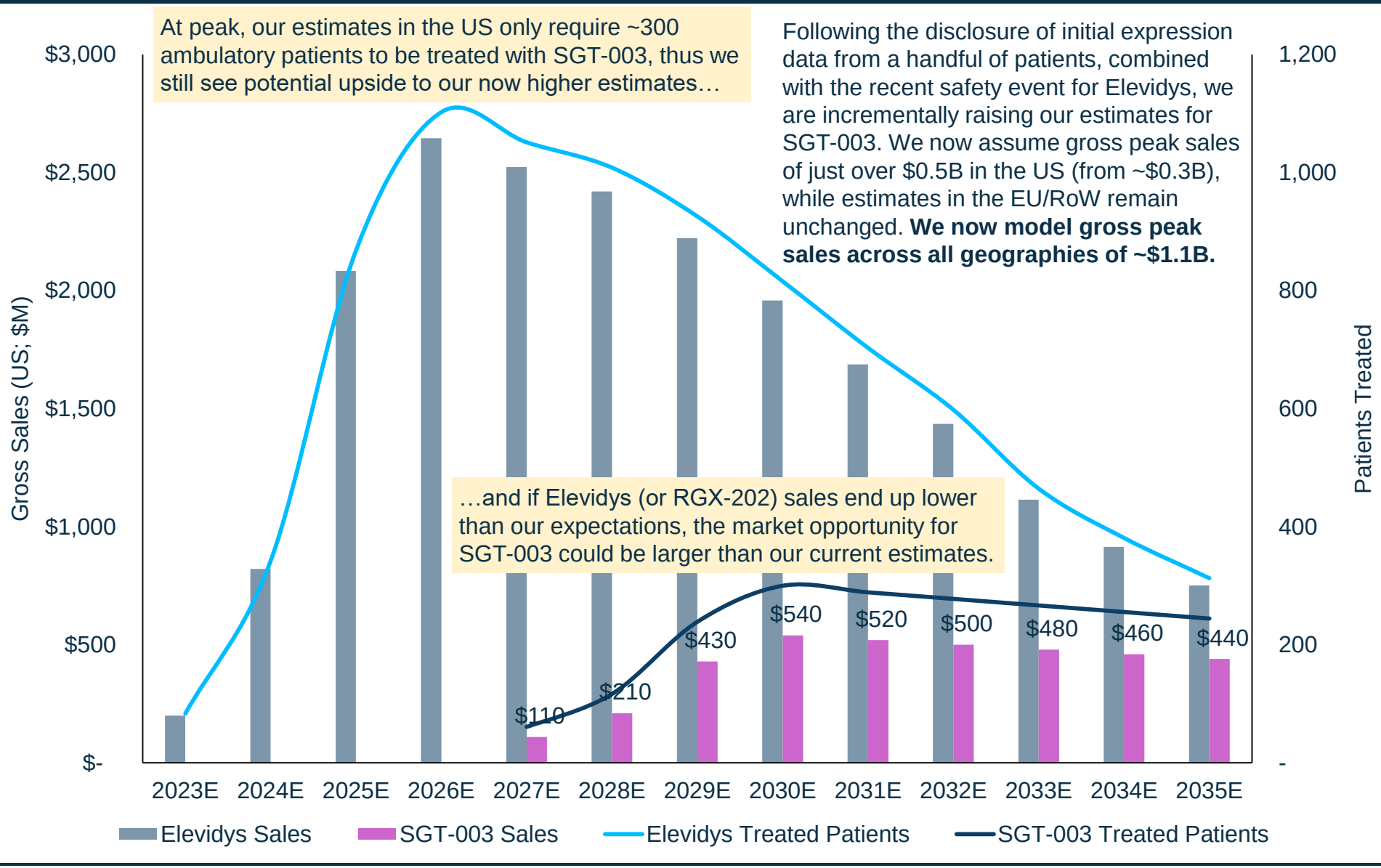
If we look further ahead at 2026 consensus estimates, SRPT is trading at **the lowest multiple** (market cap. to sales) versus these peers (~1.4x) versus the 2026 average of ~6.3x.

If SRPT were trading in line with peers (~6.4x), that would imply a share price of ~$182/share. Our $150 PT represents a ~5.3x multiple.

Source: Leerink Partners Research, Factset, Visible Alpha (as of April 21, 2025)

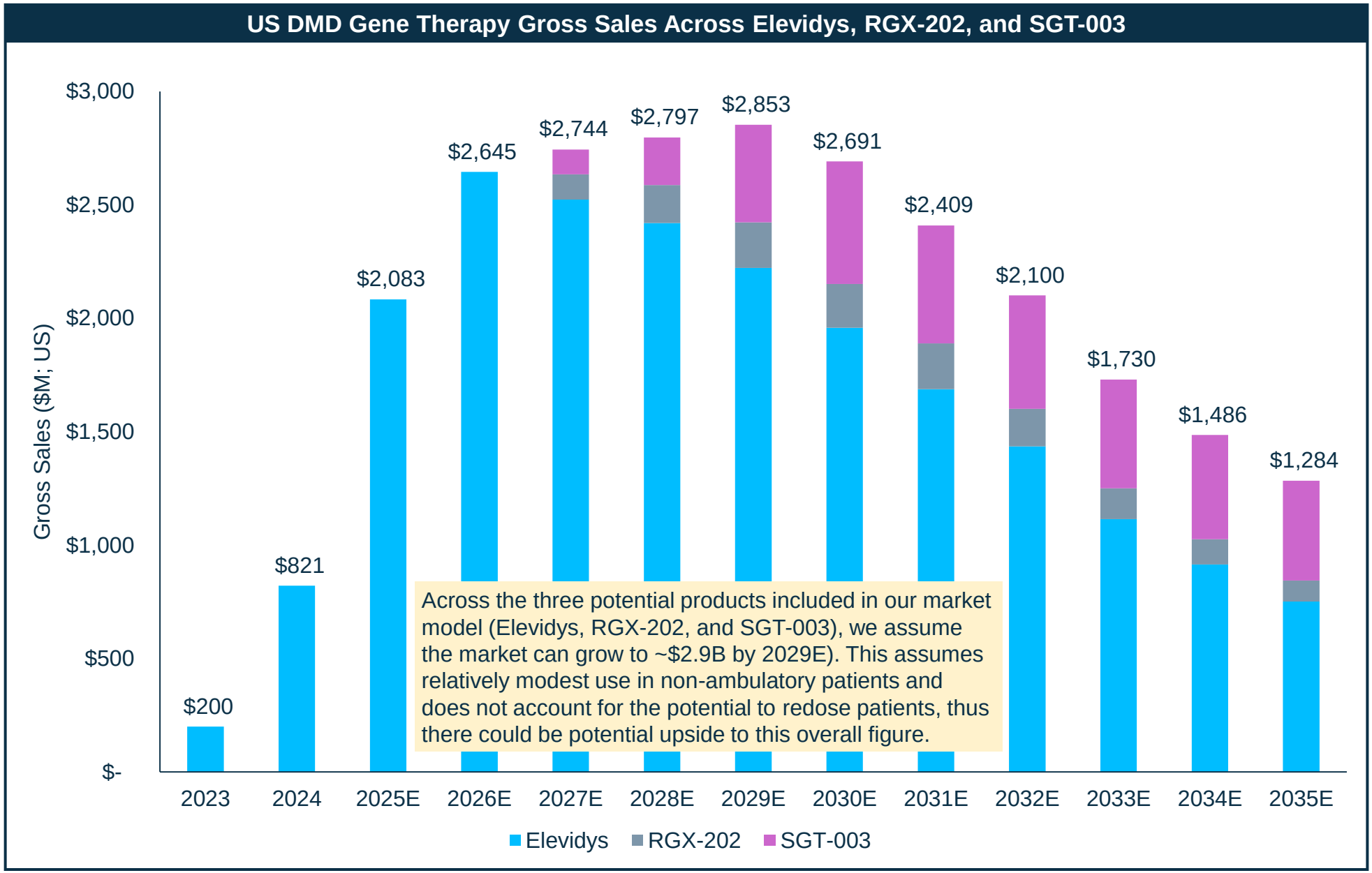
Exhibit 4. Following SLDB's solid data, we are incrementally raising our estimates

Revised Leerink Estimates for Elevidys and SGT-003; More Room for SLDB's Next-Generation Candidate



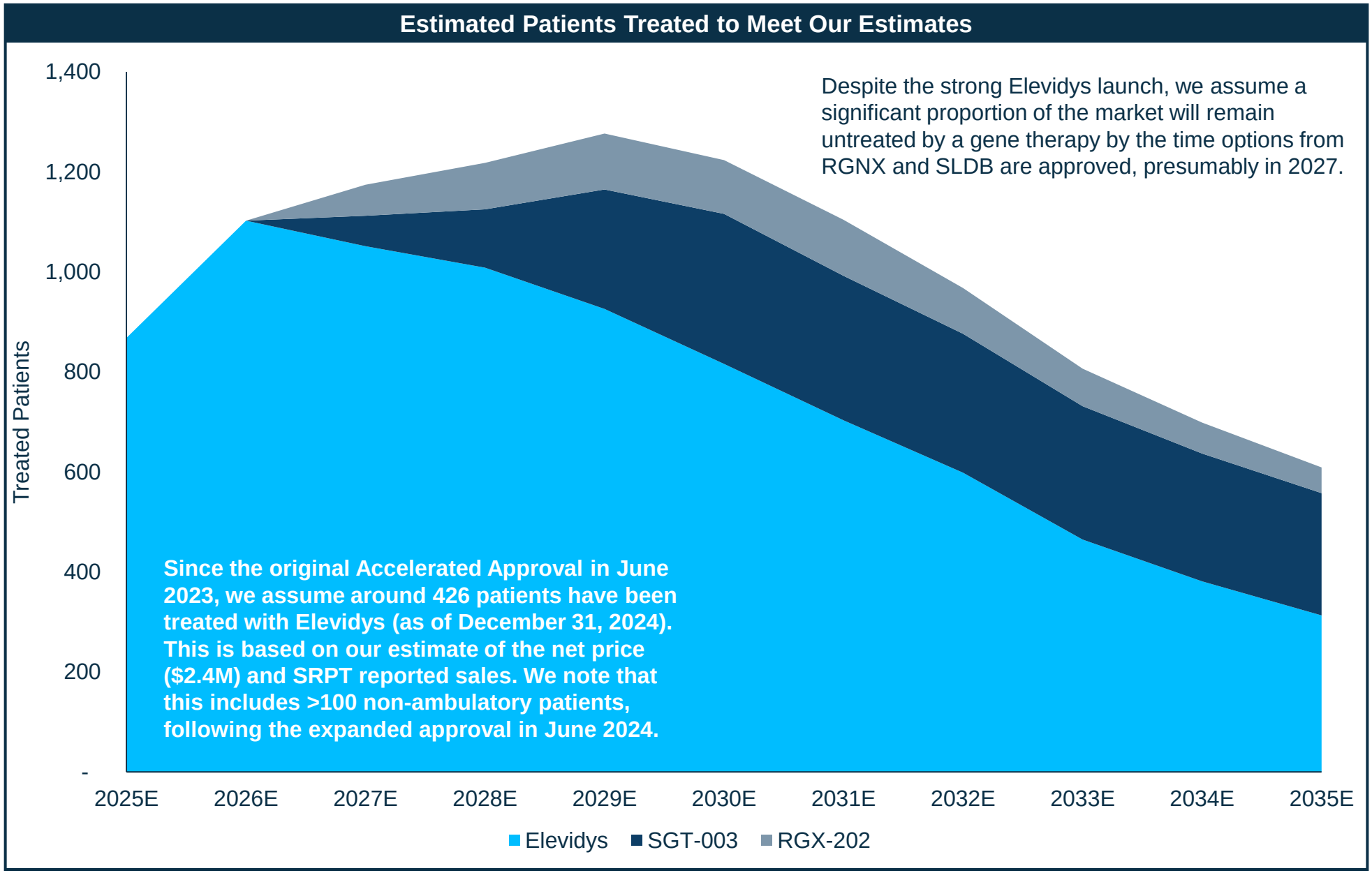
Source: Leerink Partners Research

Exhibit 5. Looking into our crystal ball, how could the landscape evolve? (1/2)



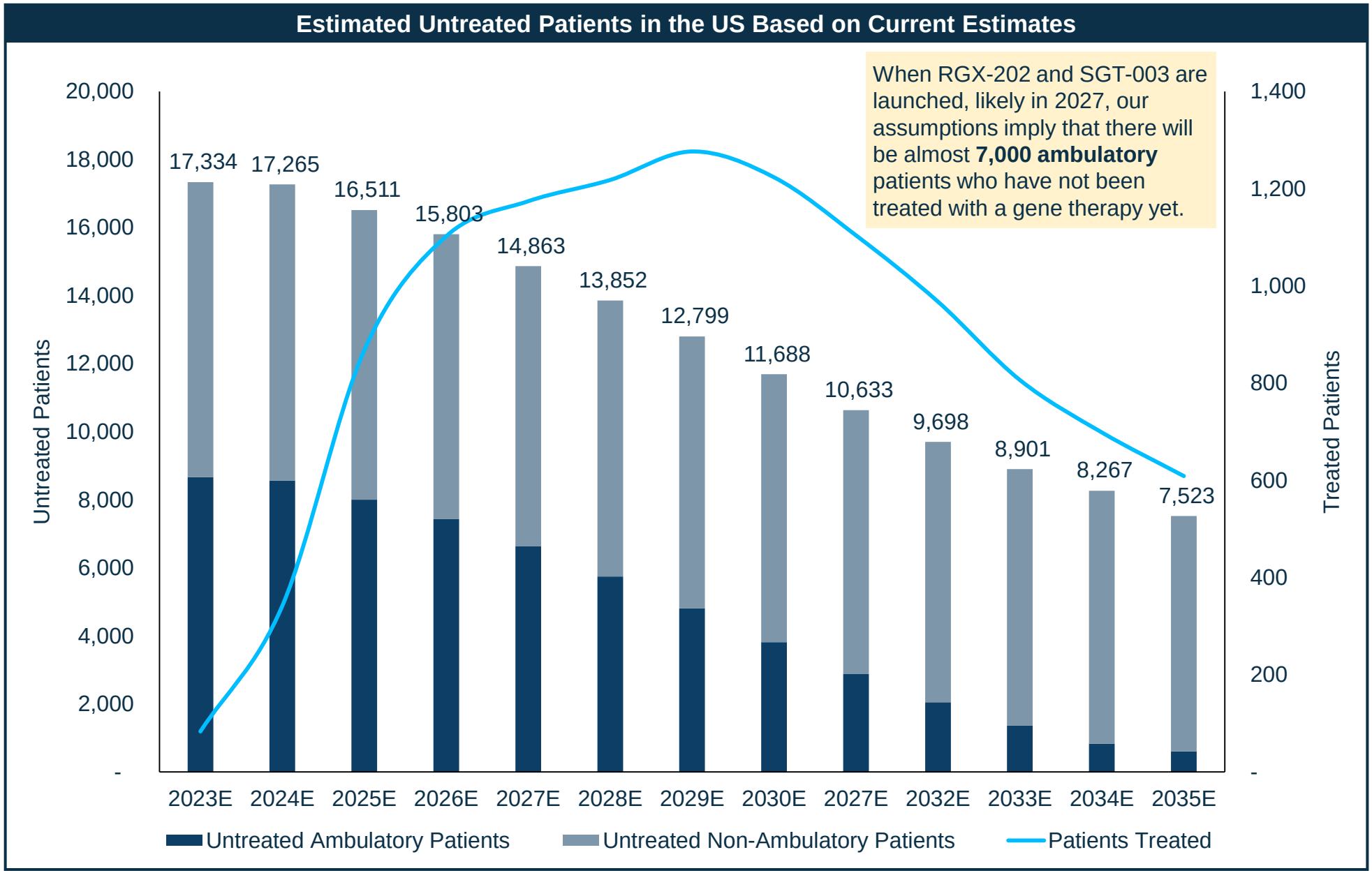
Source: Leerink Partners Research

Exhibit 6. Looking into our crystal ball, how could the landscape evolve? (2/2)



Source: Leerink Partners Research

Exhibit 7. We see a significant market remaining for next-generation options



Source: Leerink Partners Research

BMRN INVESTMENT THESIS

We rate BMRN shares Outperform. BioMarin (BMRN) is a leader in developing and commercializing therapies for rare inherited disorders that lack other treatment options. Aldurazyme is an enzyme replacement therapy (ERT) marketed by SAN FP (OP, Risinger) in a joint venture for the treatment of mucopolysaccharidosis I (MPS I), a progressive and fatal disorder stemming from a single-gene mutation, the sales from which have provided BMRN with a solid royalty stream. BMRN independently markets its own ERTs Naglazyme and Vimizim (GALNS) for patients with MPS VI and Morquio syndrome (MPS IVA) in the US and several other ROW markets. Thus far, Vimizim has tracked ahead of expectations (>2,000 pts. identified), and the robust base business for BMRN surpassed $1.8B in total revenue in 2021. Kuvan is approved for the treatment of phenylketonuria (PKU), a highly prevalent genetic disorder usually identified at birth, although it has gone generic. With Palynziq (formerly pegvaliase/PEG-PAL) approved, adult PKU pts. who are sub-optimal responders to current standard of care or have given up treatment may be able to sufficiently reduce blood phenylalanine (amino acid that accumulates in pts.) in both the US and EU. Voxzogo (achondroplasia) met its primary endpoint in Phase III and is on the market. Regulatory approval of Brineura (CLN2/Batten disease) provided yet another demonstration of BMRN's expertise in treating rare diseases with ERT, while additional products such as Roctavian (formerly BMN270; gene therapy for hemophilia A) could be transformational. The company has faced some setbacks including its antisense oligonucleotide (AON) platform with discontinuation of the Duchenne muscular dystrophy (DMD) drug candidates (Kyndrisa, BMN-044, BMN-045, and BMN-053) and BMN-701, an ERT agent for Pompe disease. Nevertheless, we continue to believe that a solid base business coupled with multiple pipeline opportunities to leverage existing infrastructure supports our positive long-term outlook.

VALUATION

We estimate a $105 fair value in 12 months for BMRN shares which assumes a 9% discount rate for commercial products, 15% discount rate for pipeline products, and 0% terminal growth rate. A sum-of-parts DCF analysis attributes $14/share to Naglazyme, $1/share for Kuvan, $5/share for Aldurazyme, $23/share for Vimizim/GALNS, $0/share for Firdapse, $16 to Palynziq/PEG-PAL, $26 to Voxzogo, $11 to Brineura (BMN-190), $2 to Valrox, and the remainder to potential early pipeline products/BMRN’s platform and net cash.

RISKS TO VALUATION

Risks include the success of marketing initiatives to identify patients for Naglazyme, Kuvan, Brineura, Vimizim, and Voxzogo. BMRN is dependent on SAN FP for the marketing of Aldurazyme. Other programs are at an earlier stage of development, so they entail higher clinical and regulatory development risk.

EWTX INVESTMENT THESIS

Edgewise Therapeutics (NASDAQ: EWTX) is a clinical-stage biopharma company focused on the discovery, development, and commercialization of novel precision medicines for rare muscle disorders. Leveraging expertise in muscle biology and small molecule engineering, EWTX has built a proprietary, muscle-focused drug discovery platform to identify small molecules that regulate key muscle proteins. The company's lead asset EDG-5506 is an oral, fast myofiber (type II) myosin small molecule inhibitor which aims to selectively limit injurious hypercontraction in susceptible fast muscle fibers thereby decreasing muscle damage and preserving muscle function. Focusing initially on Duchenne muscular dystrophy/DMD and Becker muscular dystrophy/BMD, EDG-5506's differentiated MOA could offer an orthogonal and potentially complementary treatment approach to other therapies in the rare muscle space. While initially advancing in DMD and BMD, EDG-5506 could be beneficial in additional inherited myopathies such as limb-girdle muscular dystrophy/LGMD and McArdle disease. Additionally, EWTX is advancing EDG-7500 for development in both obstructive and non-obstructive hypertrophic cardiomyopathy (HCM). EDG-7500 is a novel cardiac sarcomere modulator that has preclinically demonstrated potential to address the abnormal systolic and diastolic function in HCM patients broadly. With multiple data readouts expected over the next few years, we look forward to a string of value-creating catalysts for EWTX which should garner increased investor interest in this precision small molecule-focused company.

VALUATION

We derive a one-year PT of $50 for EWTX based on a DCF analysis using a 14% discount rate and a 2% terminal growth rate, representing an implied ~$4.7B market capitalization. We currently ascribe value to EDG-5506 in Duchenne and Becker Muscular Dystrophy and EDG-7500 in Obstructive (oHCM) and Non-Obstructive HCM (nHCM). In DMD, we project gross peak sales of ~$2.1bn (2035E) for EDG-5506 risk-adjusted to a 45% probability of success (PoS). In BMD, we project gross peak sales of ~$1.3bn (2035E) for EDG-5506 risk-adjusted to a 75% PoS. In HCM, we project gross peak sales of ~$2.5bn for EDG-7500 risk-adjusted to a 65% PoS in oHCM and 25% PoS in nHCM.

RISKS TO VALUATION

As an early clinical-stage company, risks to EWTX include clinical, regulatory, and financial:

- Promising early data may not translate to clinical success in later stages of development.
- Regulatory decisions are unpredictable and may adversely affect commercial potential of EWTX product(s).
- Competition in the neuromuscular space may limit commercial success(es) of EWTX's product(s), if approved.
- Payors' decisions on coverage of new branded drugs could change.

- External circumstances (e.g., pandemic) may negatively affect the company's operational efforts, and development timelines may not be fulfilled on schedule.
- As a non-profitable/non-commercial company, potential dilution risk from follow-on offerings.

INSM INVESTMENT THESIS

We believe INSM shares are poised to appreciate as the company continues to demonstrate that it is a leader in the development of new treatments for pulmonary disorders with high unmet medical need. The company's first commercial product, Arikayce is approved to treat patients with refractory nontuberculous mycobacterial (NTM) lung disease caused by mycobacterium avium complex (MAC). The Arikayce launch exceeded expectations and sales continue to grow robustly despite being on the market for six years. INSM has also generated positive clinical data in the front-line setting for Arikayce which could expand its label and market opportunity significantly. While Arikayce has been the primary driver of sales to date, positive Phase 3 ASPEN data for brensocatib (non-cystic fibrosis bronchiectasis/NCFB) positions the company to launch the first-to-market therapy for this large indication. INSM's third program, treprostinil palmitil inhalation powder/TPIP (pulmonary arterial hypertension/PAH and pulmonary hypertension associated with interstitial lung disease/PH-ILD) has potential to expand the company's respiratory portfolio even further. Following these 3 programs, brensocatib aims to expand into non-pulmonary indications that which could diversify the company's portfolio. Lastly, the company has an array of preclinical programs for genetic diseases which could add value longer term.

VALUATION

We estimate a risk-adjusted per share price target for INSM of $100 in 12 months. We value INSM based on discounted cash flow analysis which uses a 10% discount rate and a 2% terminal growth rate.

RISKS TO VALUATION

Risks include the potential for disappointing clinical data, regulatory setbacks, failure to obtain intellectual property protection abroad, and commercial shortfalls. Since INSM is presently unprofitable and only has one commercial product, any of the possible aforementioned setbacks may impact the stock significantly.

PEPG INVESTMENT THESIS

PepGen (PEPG) is a clinical-stage biotech company developing next-generation oligonucleotide therapeutics through their Enhanced Delivery Oligonucleotide (EDO) platform that we believe has potential to support best-in-class oligonucleotide therapeutics.

- PEPG's proprietary EDO cell-penetrating peptide (CPP) platform was founded on over a decade of research by two of the top researchers in the field. EDOs are optimally designed

to achieve high tissue penetration and intracellular uptake to convey greater clinical activity, a wider therapeutic index and enhanced biodistribution.

- The company's lead clinical-stage asset, PGN-EDO51, is an EDO peptide conjugated to a PMO therapeutic cargo for the treatment of Duchenne Muscular Dystrophy (DMD) patients with mutations amenable to an exon-51 skipping approach, which we view as de-risked from a clinical development and regulatory perspective due to precedent from Exondys 51. Although the DMD landscape is competitive for oligonucleotide therapies, we see PGN-EDO51 as well-positioned to capture meaningful uptake due to its potential to support best-in-class exon skipping and dystrophin expression as seen preclinically. PEPG plans to develop additional EDO programs in DMD patients with exon 53, exon 45 and exon 44 mutations which would provide additional upside to our valuation.
- PEPG's second preclinical-stage asset, PGN-EDODM1, is an EDO peptide conjugated to a steric block antisense oligonucleotide (ASO) that aims to bind toxic CUG repeats in DMPK RNA and block MBNL1 binding to correct the underlying genetic defect in Myotonic Dystrophy Type 1 (DM1) patients. We believe PGN-EDODM1 is well-designed to drive clinical success in this high unmet need space.

VALUATION

We derived a one-year PT of $20 for PEPG based on a DCF analysis using a 14% discount rate and 2% terminal growth rate, representing an implied market cap of ~$650M. We currently ascribe value to the company's lead programs in Duchenne Muscular Dystrophy (DMD) and Myotonic Dystrophy Type 1 (DM1). For PGN-EDO51 in DMD, we model gross peak (2035E) global sales of ~$660M and assign a 45% PoS in the US and 30% PoS in the EU. For PGN-EDODM1, we model gross peak (2035E) global sales of ~$1.8B and assign a 30% PoS. We note that we only model revenue for DMD patients with Exon 51 mutations, so expansion programs into other exon mutation types would provide upside to our valuation.

RISKS TO VALUATION

Risks to PEPG include clinical, regulatory, and financial. Unpredicted issues may arise, including disappointing clinical data, regulatory setbacks, commercial shortfalls, dilutive financing, or other unanticipated complications that could negatively impact the stock.

RNA INVESTMENT THESIS

Avidity Biosciences, Inc. (NASDAQ: RNA) is a platform-based biopharma company pioneering a new class of oligonucleotide-based therapies designed to overcome the current limitations of oligonucleotide therapeutics. RNA's proprietary Antibody Oligonucleotide Conjugate (AOC) platform combines the tissue selectivity of monoclonal antibodies (mAbs) with the precision of oligonucleotide therapies to enhance oligonucleotide delivery in a wide range of tissues and cell types. Preclinically, RNA's AOC platform has shown robust knockdown of target genes in multiple cell types including skeletal muscle, cardiac muscle, liver, and immune cells. Focusing initially on muscle disorders, RNA's muscle disease franchise consists of five programs: myotonic dystrophy type 1 (DM1), muscle atrophy,

Duchenne muscular dystrophy (DMD), facioscapulohumeral muscular dystrophy (FSHD), and Pompe disease. Lead candidate AOC 1001/del-desiran is a potentially disease-modifying treatment for DM1 – a genetic disorder with no currently approved therapies. In our view, DM1's underlying biology – toxic DMPK RNA – is ideally suited for oligonucleotide based therapy. AOC 1001 combines small interfering RNA (siRNA) designed to knock down toxic DMPK RNA linked to an anti-transferrin receptor 1 (TfR1) mAB to target muscle tissue. Preclinically, del-desiran has demonstrated robust and durable knockdown in skeletal, cardiac, and smooth muscle. We believe RNA's AOC approach could deliver on the past promise which went unfulfilled by BIIB/IONS's unconjugated oligonucleotide due to inadequate muscle penetration in DM1 patients. While RNA is initially focused on muscle disorders, its modular and flexible AOC platform may have wide application across multiple disease areas. To this end, RNA has a research collaboration and licensing agreement with Eli Lilly and Company for the discovery, development, and commercialization of AOCs for specific immunology targets and other select indications outside of muscle. We believe this collaboration both validates RNA's AOC platform and illustrates the broad application of RNA's approach. Additional or expanded collaborations in the future could add further upside to our estimates.

VALUATION

We derive a one-year PT of $60 for RNA based on a DCF analysis using a 14% discount rate and a 2% terminal growth rate, representing an implied ~$7.4bn market capitalization. We currently ascribe value to RNA's AOC candidates in development for myotonic dystrophy type 1 (DM1), Facioscapulohumeral Muscular Dystrophy (FSHD) and Duchenne Muscular Dystrophy (DMD) patients with Exon 44 mutations. In DM1, we project gross peak sales of ~ $2bn (2035E) for AOC 1001/del-desiran risk-adjusted to a 60% probability of success (PoS). In FSHD, we project gross peak sales of ~$2bn (2035E) for AOC 1020/del-brax risk-adjusted to a 40% PoS. In DMD, we project gross peak sales of ~$900M (2035E) for AOC 1044/ del-zota risk-adjusted to a 60% PoS in the US and 50% PoS in the EU to account for their different regulatory perspectives to date on dystrophin expression as a surrogate biomarker.

RISKS TO VALUATION

Risks to RNA include clinical, regulatory, and financial. Unpredicted issues may arise, including disappointing clinical data, regulatory setbacks, commercial shortfalls, dilutive financing, or other unanticipated complications that could negatively impact the stock.

SLDB INVESTMENT THESIS

SLDB is a clinical stage biotechnology company focused on advancing a portfolio of gene therapy candidates, including SGT-003 for Duchenne muscular dystrophy (DMD), SGT-212 for Friedreich's ataxia (FA), SGT-501 for catecholaminergic polymorphic ventricular tachycardia (CPVT), and AVB-401 for the treatment of BAG3-mediated dilated cardiomyopathy (BAG3-DCM). We believe that the limitations of exon skipper therapies and traditional standard of care (glucocorticoids) make SLDB a contender to offer a meaningful advancement in DMD vs. competitor SRPT's approved Elevidys. SLDB's lead gene therapy

drug candidate SGT-003 has an optimized microdystrophin construct encapsulated in their novel AAV-SLB101 capsid that can drive enhanced biodistribution to skeletal muscle and diminished biodistribution to the liver relative to AAV9. SGT-003 was preceded by SGT-001, which utilized the same microdystrophin construct encapsulated in an AAV9 capsid. The specialists who developed SGT-001 believe that it is an optimized microdystrophin construct, based on its design and comparative functionality and expression data in animals. SGT-001 and SGT-003's transgene possesses the neuronal nitric oxide synthase (nNOS)-binding domain, which slows muscle damage, delays fibrosis, and reduces muscle function loss in animals, and could translate to clinically meaningful benefits in humans. We believe the promising clinical data previously seen from SGT-001 has positive read through to SGT-003 given they utilize the same nNOS-binding construct and think the early clinical data generated for SGT-003 so far is promising. Following SLDB's acquisition of AavantiBio, they added two additional preclinical-stage gene therapy candidates to their pipeline — AVB-202 for Friedreich's Ataxia (FA) and AVB-401 for BAG3-DCM. SLDB also separately acquired SGT-501 for CPVT. SLDB refined AVB-202 into SGT-212 for FA, which is designed to address both neurological and cardiac aspects of the disease with both intradentate nuclei (IDN) and intravenous (IV) delivery to express frataxin in the CNS and heart. SGT-501 is SLDB's lead cardiac candidate and is designed to address the dominant forms of CPVT (caused by mutations in RYR2 and CASQ2) by overexpressing CASQ2 to restore normal calcium buffering and reduce or even eliminate arrhythmias. SLDB plans to file an IND for SGT-501 in 1H25. AVB-401 is designed to deliver a functional BAG3 gene to prevent DCM. SLDB is currently completing preclinical work for AVB-401, including biodistribution and preclinical studies in mice and NHPs.

VALUATION

We derive our $20 PT using a discounted cash flow (DCF) analysis assuming a 14% discount rate and 2% terminal growth rate. Our model incorporates lead asset SGT-003 in DMD as well as early-stage cardiac gene therapies SGT-501 in CPVT and AVB-401 in BAG3-mediated DCM. We estimate ~$1.1B (2031E) gross peak WW sales for SGT-003 and account for the risks in clinical and regulatory drug development in our probability of success of 75% for SGT-003 (both US and EU). These valuation parameters reflect promising early clinical and preclinical data for SGT-003 and previous de-risking clinical data from its predecessor SGT-001. We estimate ~$2.8B (2035E) gross peak WW sales for SGT-501 and account for the early stage and risks in clinical and regulatory drug development in our probability of success of 10% for SGT-501 (both US and EU). We estimate ~$1.2B (2035E) gross peak WW sales for AVB-401 and account for the early stage and risks in clinical and regulatory drug development in our probability of success of 10% for AVB-401 (both US and EU). Despite our positive view on the programs, we currently do not include SLDB's FA program or any additional preclinical-stage pipeline candidates in our current valuation.

RISKS TO VALUATION

SLDB is developing gene therapy drug candidates for DMD, FA, CPVT, and BAG3-mediated DCM. As with other gene therapy candidates, this platform comes with safety and efficacy risks. Although preclinical and early clinical data seem convincing, SLDB's candidates

are in the early stages of development and clinical/regulatory updates could disappoint. Unpredictable issues may arise, including safety, efficacy, manufacturing, regulatory requirements, market receptiveness, or other unanticipated complications that could impact the stock negatively.

SRPT INVESTMENT THESIS

We rate SRPT shares Outperform. SRPT received FDA Accelerated Approval for eteplirsen (Exondys 51) in September 2016, which addresses ~13% of Duchenne muscular dystrophy (DMD) patients suffering from a mutation in the dystrophin gene amenable to exon 51-skipping. While we estimate that Exondys 51 is only applicable to ~5,000 DMD patients in the U.S. and EU, Sarepta has used the same proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry to develop other exon skipping drugs (e.g., casimersen/Amondys 45 and golodirsen/Vyondys 53) that operate by the same mechanism and may be able to ameliorate the progression of DMD in patients with various disease-causing mutations. We assume Exondys 51 revenues peaked at ~$540M (2023) with an additional ~$400M (2027E) in revenue derived from follow-on candidates Amondys 45 and Vyondys 53. Importantly, SRPT's microdystrophin gene therapy program Elevidys (SRP-9001) has the potential to drive significant clinical and commercial value. SRPT has partnered with Roche to develop Elevidys in the ex-US and maintains sole development rights in the US. We currently model peak (2026E) probability-weighted and royalty-adjusted WW sales of ~$2.7B for Elevidys. We believe Elevidys' strong tolerability profile and full approval offer a competitive advantage to competitor microdystrophin gene therapy programs, including those from SLDB and RGNX. Due to similar attributes, positive microdystrophin progress has a potential halo effect on the company's other gene therapy programs, such as in limb-girdle muscular dystrophy (LGMD).

VALUATION

We estimate a risk-adjusted per share fair value for SRPT of $150 in 12 months. We assume peak Exondys 51 revenues of ~$540M (2023) with an additional ~$400M (2027E) in revenue derived from Amondys 45 and Vyondys 53. Acknowledging SRPT's lead position in DMD gene therapy with Elevidys, we also include peak probability-weighted and royalty-adjusted microdystrophin WW revenues of ~$2.7B (2026E). We use a sum-of-the-parts discounted cash flow (DCF) valuation methodology, attributing ~$31 to Exondys 51, ~$6 to Vyondys 53, ~$13 to Amondys 45, ~$87 to Elevidys, ~$8 to limb-girdle muscular dystrophy (LGMD2E/2D/2C), and the rest to cash. We use a 10% WACC as our discount rate. Over the longer term, we assume a 2% terminal growth rate reflecting multiple growth opportunities as SRPT pursues multiple modalities to address DMD and other neuromuscular diseases (e.g., LGMD). We assign 100% PoS in the US and 60% PoS in the EU/RoW for Elevidys with a market entry of 2025 in the EU. We assign 50% PoS (US/EU) to LGMD 2E following positive cohort 1 data that we believe offer a positive readthrough to other LGMD programs, with a 10% PoS (US/EU) to LGMD2D and LGMD2C programs.

RISKS TO VALUATION

Risks include the potential for disappointing clinical data, regulatory setbacks, and commercial shortfalls. Given a majority of SRPT's revenues come from the US market, setbacks ex-US may raise the issue of pipeline valuation. And although SRPT's partnerships and in-licensed candidates could help solidify the company's leadership in DMD, risks associated with clinical development, novel technology, and cannibalization are potential risks in the future.

WVE INVESTMENT THESIS

We rate WVE Outperform with a $24/share price target. WVE is a clinical-stage biotechnology company focused on developing oligonucleotide-based therapeutics for devastating rare diseases. WVE's proprietary discovery and drug development platform, PRISM, enables the precise design, optimization, and production of novel stereopure oligonucleotides across multiple therapeutic modalities, including RNA editing, splicing, and silencing (with antisense oligonucleotides and RNA interference). WVE has generated preclinical and early clinical data suggesting that stereopure oligonucleotides utilizing their novel and proprietary PN chemistry have improved potency, less immunogenicity, and longer stability than racemic mixtures across the modalities. WVE's current drug candidates include WVE-003 (antisense oligonucleotide selective for mutant huntingtin in Huntington's disease), WVE-N531 (exon 53 skipper for Duchenne muscular dystrophy), WVE-006 (RNA editing for alpha-1-antitrypsin deficiency), and WVE-007 (siRNA targeting INHBE for obesity).

VALUATION

We derive a $24 price target for WVE shares using a discounted cash flow analysis by forecasting cash flows through 2035 and assign a 2% terminal growth rate and 14% discount rate, representing an implied ~$3.2B market capitalization. We currently ascribe value to the company's candidates WVE-003 in Huntington's disease (HD), WVE-N531 in Duchenne muscular dystrophy (DMD), and WVE-006 in alpha-1-antitrypsin deficiency (AATD). In HD, we project gross peak sales of ~$2B (2035E) for WVE-003 risk-adjusted to a 40% probability of success (PoS). In DMD, we project gross peak sales of ~$870M (2035E) for WVE-N531 risk-adjusted to a 70% PoS in the US and 50% PoS in the EU. In AATD, we project gross peak sales of ~$3B (2035E) risk-adjusted to a 25% PoS. For the siRNA targeting INHBE in obesity, we project gross peak sales of $1B (2035) risk-adjusted to a 10% PoS.

RISKS TO VALUATION

As a clinical-stage company, risks to our view, outlook, and valuation for WVE include clinical, regulatory, and financial risks:

• Promising early data may not translate to clinical success in later stages of development.

• Regulatory decisions are unpredictable and may adversely affect commercial potential of WVE product(s).

• Our revenue forecasts for WVE are subject to the risk of better-than-expected market share for competing products in Huntington's disease, Duchenne muscular dystrophy, alpha-1-antitrypsin deficiency and obesity.

• Further dilutive financing may be required in order to support future commercial operations and clinical development of pipeline agents.

• Competition in the HD, DMD, AATD, and obesity spaces may limit the commercial success of WVE's products.

DCF Model

Diluted Shares Out 4Q24	196.6
Net Cash 4Q24	1,059.0
Discount Rate Commercial	9%
Discount Rate Pipeline	15%
Terminal Growth Rate	0%

DCF Valuation	Per share	Val. ($MM)	Proportion
Total	$ 105	20,692	100%
Naglazyme	$ 14	2,827	14%
Kuvan	$ 1	134	1%
Aldurazyme	$ 5	1,001	5%
Vimizim	$ 23	4,485	22%
Firdapse	$ 0	25	0%
Palynziq/PEG-PAL	$ 16	3,210	16%
Voxzogo	$ 26	5,201	25%
Brineura	$ 11	2,214	11%
Roctavian/Valrox	$ 2	386	2%
Eteplirsen royalty	$ -	-	0%
Early Pipeline/Platform	$ 0.8	150	1%
Net Cash	$ 5.39	1,059	5%

Source: Company reports and Leerink

Biomarin Pharmaceuticals P&L

Income Statement - GAAP ($MM)	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
Milestones, Collaboration and Royalties	6.1	6.1	6.0	11.0	9.9	11.9	12.3	45.1	10.0	10.0	10.0	10.0	40.0	40.0
Enzyme Product	1,491.9	1,645.3	1,718.4	448.2	482.2	509.0	488.0	1,927.4	474.4	495.3	514.1	529.0	2,012.8	2,098.8
Naglazyme (MPS VI)	*380.4*	*443.8*	*420.3*	*105.6*	*132.0*	*132.0*	*110.0*	*479.6*	*112.4*	*114.8*	*117.6*	*120.4*	465.3	*470.3*
Aldurazyme (MPS I; Royalty Revenues)	*122.8*	*128.3*	*131.2*	*35.3*	*38.6*	*71.0*	*39.0*	*183.9*	*39.0*	*39.1*	*39.1*	*39.2*	156.4	*158.0*
Vimizim/GALNS (MPS IVA)	*623.3*	*663.9*	*701.1*	*192.6*	*178.0*	*178.0*	*191.0*	*739.6*	*190.2*	*192.8*	*194.8*	*198.5*	776.3	*789.7*
Palynziq (PKU)	*237.5*	*255.0*	*304.0*	*75.7*	*88.3*	*91.0*	*100.0*	*355.0*	*88.7*	*99.9*	*110.9*	*114.8*	414.3	*429.8*
Brineura (CLN2)	*127.9*	*154.3*	*161.8*	*39.0*	*45.3*	*37.0*	*48.0*	*169.3*	*43.9*	*48.7*	*51.6*	*56.2*	200.5	*251.0*
Kuvan (PKU)	285.8	227.5	180.7	35.9	28.6	27.9	28.0	120.4	22.4	17.9	14.3	11.5	66.1	33.1
Firdapse (LEMS)	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Voxzogo (Achondroplasia)	5.9	169.2	469.9	152.9	183.9	190.0	208.0	734.8	215.2	219.3	237.6	250.4	922.4	931.9
Eteplirsen (DMD; Royalty Revenues)	56.6	48.0	32.2	-	-	-	-	-	-	-	-	-	-	-
Valrox (Hemo A)	-	-	3.5	0.8	7.4	7.0	11.0	26.2	10.0	15.4	16.2	18.1	59.7	81.8
Total Revenue	**1,846.3**	**2,096.0**	**2,419.2**	**648.8**	**712.0**	**745.7**	**747.3**	**2,853.9**	**732.0**	**757.9**	**792.3**	**818.9**	**3,101.1**	**3,185.6**
COGS	470.5	483.7	514.9	125.2	130.5	188.5	136.1	580.2	144.4	149.6	156.5	161.8	612.2	629.1
R&D	628.8	649.6	746.8	205.0	183.8	184.9	173.5	747.2	194.9	201.9	211.2	218.4	826.5	786.4
SG&A	759.4	854.0	937.3	225.9	263.0	253.5	266.6	1,009.0	259.9	269.2	281.6	291.2	1,102.0	912.2
Intangible asset charges/(gains)	69.9	67.2	62.2	14.3	14.3	5.0	9.7	43.3	-	-	-	-	-	
Other	-	(108.0)	-	(10.0)				-					-	-
Total Operating Expenses	**1,928.6**	**1,946.5**	**2,261.1**	**560.4**	**591.6**	**631.8**	**585.9**	**2,369.7**	**599.3**	**620.7**	**649.3**	**671.4**	**2,540.7**	**2,327.7**
Operating Income	(82.3)	149.6	158.1	88.5	120.5	113.9	161.4	484.2	132.7	137.1	143.0	147.5	560.4	857.8
Loss on equity investments	-	-	-					-					-	-
Interest Income	10.5	18.0	58.3	19.4	19.8	18.1	17.7	74.9	4.0	4.0	4.0	4.0	16.0	16.0
Interest Expense	(15.3)	(16.0)	(17.3)	(3.5)	(3.6)	(3.0)	(2.6)	(12.7)	-	-	-	-	-	-
Biomarin/Genzyme LLC (MPS I)	(1.3)	-	-	-	-	-	-	-	-	-	-	-	-	-
Other	13.2	(2.1)	(10.5)	1.3	(4.5)	5.5	(6.9)	(4.7)	-	-	-	-	-	-
Total other	7.0	0.0	30.5	17.1	11.7	20.5	8.2	57.5	4.0	4.0	4.0	4.0	16.0	16.0
Pretax income	(75.4)	149.6	188.5	105.5	132.1	134.4	169.6	541.8	136.7	141.1	147.0	151.5	576.4	873.8
Income taxes	(11.3)	8.0	20.9	16.9	25.0	28.4	44.7	114.9	26.0	26.8	27.9	28.8	109.5	166.0
Net Loss Before Extraordinary Items	(64.1)	141.6	167.6	88.7	107.2	106.1	124.9	426.9	110.8	114.3	119.1	122.7	466.9	707.8
Extraordinary Items	-	-	-	-	-	-	-	-	-	-	-	-	-	-
GAAP Net Income (Loss)	**(64.1)**	**141.6**	**167.6**	**88.7**	**107.2**	**106.1**	**124.9**	**426.9**	**110.8**	**114.3**	**119.1**	**122.7**	**466.9**	**707.8**
GAAP EPS (Basic)	**($0.35)**	**$0.76**	**$0.89**	**$0.47**	**$0.56**	**$0.56**	**$0.66**	**$2.25**	**$0.59**	**$0.60**	**$0.63**	**$0.65**	**$2.47**	**$3.72**
GAAP EPS (Diluted)	**($0.35)**	**$0.75**	**$0.87**	**$0.46**	**$0.55**	**$0.55**	**$0.64**	**$2.21**	**$0.46**	**$0.57**	**$0.60**	**$0.61**	**$2.24**	**$3.53**
Reconciliation of GAAP to non-GAAP														
Interest income (expense), net	4.8	(2.0)	-	-	-	-	-	-	-	-	-	-	-	
Provision for (benefit from) income taxes	(11.3)	8.0	(69.6)	(14.9)	(24.1)	(20.0)	(16.0)	(75.0)	-	-	-	-	-	
Depreciation expense	46.1	38.6	-	-	-	-	-	-	-	-	-	-	-	
Amortization expense	61.9	62.8	62.2	14.3	14.3	5.0	10.0	43.6	15.0	15.0	15.0	15.0	60.0	70.0
Stock-based compensation expense	197.4	196.4	207.0	58.2	47.9	43.0	51.0	200.1	54.6	56.5	59.1	61.2	231.4	203.8
Contingent consideration expense	8.0	4.4	-	-	-	-	-	-	-	-	-	-	-	
Severance and reorganization costs	-	23.0	(0.5)	3.4	39.1	44.0	10.0	96.5	25.0	25.0	25.0	25.0	100.0	120.0
Gain on sale of nonfinancial assets, net	-	(108.0)	-	(10.0)	-	-	-	(10.0)	-	-	-	-	-	
Asset impairments			14.0					-					-	
Loss on investment in equity securities			24.5	-	4.5	-	-	4.5	-	-	-	-	-	-
Non-GAAP Net Income	**242.8**	**364.8**	**405.2**	**154.4**	**188.9**	**178.1**	**179.9**	**701.3**	**227.3**	**233.7**	**242.1**	**248.7**	**951.8**	**1,251.7**
Non-GAAP EPS (basic)	**$1.33**	**$1.97**	**$2.16**	**$0.82**	**$0.99**	**$0.94**	**$0.94**	**$3.69**	**$1.20**	**$1.24**	**$1.28**	**$1.31**	**$5.03**	**$6.58**
Non-GAAP EPS (diluted)	**$1.31**	**$1.94**	**$2.10**	**$0.71**	**$0.96**	**$0.91**	**$0.92**	**$3.52**	**$0.71**	**$1.17**	**$1.21**	**$1.24**	**$4.34**	**$6.24**
Wtd. Avg. Shares Outstanding	182.2	185.2	187.8	188.9	190.1	190.4	190.7	190.0	188.9	189.1	189.3	189.5	189.2	190.3
Shares Outstanding (Diluted)	185.3	188.5	193.1	199.3	200.5	197.1	196.6	198.4	199.3	199.5	199.7	199.9	199.6	200.7

Source: Company reports and Leerink

Balance Sheet ($MM)	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
Cash & Equivalents	**1,521.7**	**1,625.4**	**1,684.9**	**1,667.1**	**1,781.4**	**1,492.0**	**1,659.0**	**1,659.0**	**1,824.3**	**1,995.2**	**2,173.4**	**2,357.3**	**2,357.3**	**3,268.9**
Convertible Notes due 2017	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Convertible Notes due 2018	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Convertible Notes due 2020	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Convertible Notes due 2024	495.0	495.0	495.0	495.0	495.0	495.0	-	-	-	-	-	-	-	-
Convertible Notes due 2027	600.0	600.0	600.0	600.0	600.0	600.0	600.0	600.0	600.0	600.0	600.0	600.0	600.0	600.0
Interest Rate Earned on Cash	*0.2%*	*0.3%*	*0.9%*	*1.2%*	*1.1%*	*1.2%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*	*1.1%*
Interest Rate Paid on Convertible Debt	*-0.4%*	*-0.4%*	*-0.4%*	*-0.3%*	*-0.3%*	*-0.3%*	*-0.4%*	*-0.3%*	*-0.3%*	*-0.3%*	*-0.3%*	*-0.3%*	*-0.3%*	*-0.3%*

Cash Flow ($MM)	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
Net Income (Loss)	(64.1)	141.6	167.6	88.7	107.2	106.1	124.9	426.9	110.8	114.3	119.1	122.7	466.9	707.8
Share based comp	166.6	196.3	207.1	58.2	47.9	43.5	51.9	201.6	54.6	56.5	59.1	61.2	231.4	203.8
Equity Issuance	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Convertible Bond Issuance (buyback)	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Other	(195.0)	(200.6)	(345.5)	(98.7)	103.4	(538.2)	73.0	(460.6)	-	-	-	-	-	-
Change in Cash	(92.5)	137.2	29.2	48.2	258.5	(388.7)	249.8	167.8	165.3	170.9	178.2	183.9	698.3	911.6

Margins	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
COGS / Sales	26%	23%	21%	19%	18%	25%	18%	20%	20%	20%	20%	20%	20%	20%
Gross margins	74%	77%	79%	81%	82%	75%	82%	80%	80%	80%	80%	80%	80%	80%
R&D / Sales	34%	31%	31%	32%	26%	25%	23%	26%	27%	27%	27%	27%	27%	25%
SG&A / Sales	41%	41%	39%	35%	37%	34%	36%	35%	36%	36%	36%	36%	36%	29%
Operating Margin	-5%	7%	7%	14%	17%	15%	22%	17%	18%	18%	18%	18%	18%	27%
Taxes	-17%	29%	7%	16%	19%	21%	26%	21%	19%	19%	19%	19%	19%	19%
Net Margin	-4%	7%	7%	14%	15%	14%	17%	15%	15%	15%	15%	15%	15%	22%
Non-GAAP Operating Margin				24%	27%	24%	24%	25%	31%	31%	31%	30%	31%	39%

Source: Company reports and Leerink

Leerink Partners Catalyst Tracker

Stock (Ticker Symbol)	Lateral Impact (Other companies /stocks)	Drug (Brand or chemical name) / Instrument / Area	Indication / Product Class	Type of Event	Event or Trial Details	Expected Timing	Specific Event Date if known or specified	Impact: H(igh) > 9% M(edium) 3 - 9% L(ow) < 2%	Estimated Stock Up/Down % on Best/Worst Outcomes	Leerink Partners View of Expected Outcome
BMRN		Voxzogo	Hypochondroplasia	Phase 3 Trial Full Enrollment		1H25		L		Positive
BMRN		Voxzogo	Hypochondroplasia	Phase 3 Results Announcement		2026		H		Positive
BMRN		BMN 351	Duchenne muscular dystrophy	Phase 2 Data Announcement	Phase 1/2 POC data	2H25		M		
BMRN		BMN333	Achondroplasia and hypochondroplasia	Phase 1 Data Presentation	Initial PK data	4Q25	YE25	M		
BMRN		BMN333	Achondroplasia and hypochondroplasia	Phase 1 Data Presentation	Detailed PK data at a scientific conference	1H26		M		
BMRN		BMN370	von Willebrand disease	Phase 1 Trial Initiation		2026		L		
BMRN		BMN390	PKU	Phase 1 Trial Initiation		1H26		L		
BMRN		Palynziq	PKU adolescents ages 12-17	sNDA/sBLA/sMAA Filing		2H25		L		

Source: Leerink Partners LLC Equity Research and Company Filings

Edgewise Therapeutics P&L (USD, MM)	2019	2020	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
Revenues																
EDG-5506 - BMD & DMD	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	32.0
EDG-7500 - Obstructive & Non-obstructive HCM	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Other	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Total revenue	**-**	**-**	**-**	**-**	-	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**32.0**
Operating expenses																
COGS	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	4.8
Gross Profit	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	27.2
R&D	8.6	15.0	32.2	54.0	90.9	27.7	30.7	32.2	36.4	127.0	38.2	40.1	42.1	44.2	164.6	181.1
SG&A	1.3	2.2	11.0	17.6	23.5	7.1	7.4	8.2	9.2	31.9	10.1	11.1	12.2	13.4	46.8	53.8
Total expenses	**9.9**	**17.2**	**43.2**	**71.7**	**114.4**	**34.8**	**38.1**	**40.4**	**45.5**	**158.8**	**48.3**	**51.2**	**54.3**	**57.6**	**211.4**	**234.9**
EBIT (loss)	**(9.9)**	**(17.2)**	**(43.2)**	**(71.7)**	**(114.4)**	**(34.8)**	**(38.1)**	**(40.4)**	**(45.5)**	**(158.8)**	**(48.3)**	**(51.2)**	**(54.3)**	**(57.6)**	**(211.4)**	**(202.9)**
Interest Income	0.2	0.1	0.4	4.0	14.2	6.2	6.6	6.3	5.9	25.0	6.2	0.4	0.4	0.5	7.5	1.8
Interest Expense	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
EBT (loss)	**(9.7)**	**(17.1)**	**(42.8)**	**(67.6)**	**(100.2)**	**(28.5)**	**(31.5)**	**(34.1)**	**(39.7)**	**(133.8)**	**(42.0)**	**(50.8)**	**(53.9)**	**(57.2)**	**(203.9)**	**(201.1)**
Taxation	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Other income/(loss)	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Net Income (loss)	**(9.7)**	**(17.1)**	**(42.8)**	**(67.6)**	**(100.2)**	**(28.5)**	**(31.5)**	**(34.1)**	**(39.7)**	**(133.8)**	**(42.0)**	**(50.8)**	**(53.9)**	**(57.2)**	**(203.9)**	**(201.1)**
EPS (basic)	**(13.57)**	**(12.24)**	**(1.14)**	**(1.26)**	**(1.57)**	**(0.33)**	**(0.34)**	**(0.36)**	**(0.42)**	**(1.45)**	**(0.44)**	**(0.49)**	**(0.51)**	**(0.55)**	**(2.00)**	**(1.95)**
EPS (diluted)	**(13.57)**	**(12.24)**	**(1.14)**	**(1.26)**	**(1.57)**	**(0.33)**	**(0.34)**	**(0.36)**	**(0.42)**	**(1.45)**	**(0.44)**	**(0.49)**	**(0.51)**	**(0.55)**	**(2.00)**	**(1.95)**
Shares outstanding (basic)	0.7	1.4	37.5	53.6	63.7	87.6	93.5	93.8	94.7	92.4	94.5	104.5	104.6	104.7	102.1	103.1
Shares outstanding (diluted)	0.7	1.4	37.5	53.6	63.7	87.6	93.5	93.8	94.7	92.4	94.5	104.5	104.6	104.7	102.1	103.1

BS & Cash Flow (USD, MM)	2019	2020	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
Net cash	**23.7**	**129.1**	**280.8**	**351.9**	**318.4**	**532.8**	**511.8**	**492.5**	**470.2**	**470.2**	**428.2**	**563.5**	**507.6**	**448.4**	**448.4**	**227.6**
Cash and equivalents	23.7	129.1	280.8	351.9	318.4	532.8	511.8	492.5	470.2	470.2	428.2	563.5	507.6	448.4	448.4	227.6
Debt	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Change in cash (USD, MM)	**15.4**	**81.3**	**(89.4)**	**77.2**	**64.1**	**(55.8)**	**13.2**	**1.3**	**(3.1)**	**(44.4)**	**(41.9)**	**135.2**	**(55.9)**	**(59.2)**	**(21.8)**	**(220.8)**
Cash Flow - Operating (USD,MM)	**(9.2)**	**(14.6)**	**(33.5)**	**(56.3)**	**(91.9)**	**(28.6)**	**(26.1)**	**(27.4)**	**(27.0)**	**(109.0)**	**(41.6)**	**(50.2)**	**(53.2)**	**(56.3)**	**(201.3)**	**(197.3)**
Cash Flow - Investing (USD, MM)	**(0.2)**	**(24.4)**	**(242.2)**	**3.4**	**102.9**	**(268.3)**	**37.4**	**25.4**	**20.9**	**(184.7)**	**(0.3)**	**(2.6)**	**(2.7)**	**(2.9)**	**(8.5)**	**(23.5)**
Cash Flow - Financing (USD, MM)	**24.8**	**120.3**	**186.4**	**130.1**	**53.2**	**241.1**	**2.0**	**3.3**	**2.9**	**249.3**	**-**	**188.0**	**-**	**-**	**188.0**	**-**

Sources: Company filings, Factset, Leerink Partners LLC Equity Research

Edgewise Therapeutics DCF (USD, MM)	2019	2020	2021	2022	2023	2024	2025E	2026E	2027E	4Q22	2029E	2030E	2031E	2032E	2033E	2034E	2035E	TV
CF - Operating	(9)	(15)	(34)	(56)	(92)	(109)	(201)	(197)	(133)	90	291	484	705	933	1,123	1,277	1,360	
CF - Investing	(0)	(24)	(242)	3	103	(185)	(8)	(23)	(29)	(47)	(75)	(95)	(111)	(124)	(137)	(143)	(151)	
FCFE	(9)	(39)	(276)	(53)	11	(294)	(210)	(221)	(162)	43	216	389	594	809	986	1,135	1,209	10,275
Discount Periods	-	-	-	-	-	-	-	0.50	1.50	2.50	3.50	4.50	5.50	6.50	7.50	8.50	9.50	
FCFE NPV	-	-	-	-	-	-	(105)	(207)	(134)	31	137	216	289	345	369	373	348	2,959

Sum FCFE NPV (MM DCF)	$	4,622
Net Cash (2Q25E) (MM USD)	$	563
Implied Market Cap (M USD)	**$**	**5,185**
PT	**$**	**50**

Diluted Shares Outstanding (M) (2Q25E)	104.5
Discount Rate	14%
Terminal Growth Rate	2%

Sources: Company filings, Leerink Partners LLC Equity Research

Leerink Partners Catalyst Tracker

Stock (Ticker Symbol)	Lateral Impact (Other companies/stocks)	Drug (Brand or chemical name) / Instrument / Area	Indication / Product Class	Type of Event	Event or Trial Details	Expected Timing	Specific Event Date if known or specified	Impact: H(igh) > 9% M(edium) 3 - 9% L(ow) < 2%	Estimated Stock Up/Down % on Best/Worst Outcomes	Leerink Partners View of Expected Outcome
EWTX		EDG-5506 / sevasemten	DMD	Phase 2 Data Announcement	Ph.2 LYNX/FOX Data	2Q25		H		Positive
EWTX		EDG-5506 / sevasemten	BMD	Other Event	Regulatory Feedback on CANYON	1H25		M		Positive
EWTX		EDG-5506 / sevasemten	DMD	Phase 3 Trial Initiation	Ph.3 Trial Initiation	2H25		L		Neutral
EWTX		EDG-7500	HCM	Phase 2 Data Announcement	12-week oHCM and nHCM Data	4Q25		M		Positive
EWTX			Cardiometabolic	Other Event	In vivo Proof-of-Concept Data	2H25		L		Neutral
EWTX		EDG-CV	Heart Failure	IND Submission	IND for EDG-CV in HF	2025		M		Positive
EWTX		EDG-5506 / sevasemten	BMD	Phase 3 Results Announcement	GRAND CANYON Data	4Q26		M		Positive
EWTX		EDG-7500	HCM	Phase 3 Trial Initiation	Phase 3 Initiations in oHCM and nHCM	1H26		M		Positive
EWTX		EDG-CV	Heart Failure	Phase 2 Data Announcement	Phase 2 Data in HV/HF patients	1H26		M		Positive

Source: Leerink Partners LLC Equity Research and Company Filings

INSM P&L ($MM)	2018	2019	2020	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
Arikayce (Refractory MAC)	9.8	136.5	164.4	188.5	245.4	305.2	75.5	90.3	93.4	104.4	363.7	95.9	103.2	107.1	111.0	417.2	461.8
Arikayce (Front-Line MAC)		-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	10.7
Brensocatib (Non-CF Bronchiectasis)	-	-	-	-	-	-	-	-	-	-	-	-	-	6.7	33.5	40.2	463.8
TPIP (PAH and PH-ILD)	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Total Revenue	**9.8**	**136.5**	**164.4**	**188.5**	**245.4**	**305.2**	**75.5**	**90.3**	**93.4**	**104.4**	**363.7**	**95.9**	**103.2**	**113.8**	**144.5**	**457.3**	**936.3**
COGS	(2.4)	(24.2)	(39.9)	(44.2)	(55.1)	(65.6)	(17.5)	(21.0)	(21.2)	(26.2)	(85.7)	(21.1)	(22.7)	(25.0)	(31.8)	(100.6)	(206.0)
R&D	(145.3)	(131.7)	(181.2)	(272.7)	(397.5)	(571.0)	(121.1)	(146.7)	(150.8)	(179.7)	(598.4)	(180.3)	(183.7)	(184.3)	(186.4)	(734.6)	(842.7)
SG&A	(168.2)	(210.8)	(203.6)	(234.3)	(265.8)	(344.5)	(93.1)	(106.6)	(118.9)	(142.5)	(461.1)	(143.8)	(144.5)	(146.8)	(147.4)	(582.4)	(702.2)
Amortization of Intangible Assets	(1.2)	(5.0)	(5.0)	(5.1)	(5.1)	(5.1)	(1.3)	(1.3)	(1.3)	(1.3)	(5.1)	-	-	-	-	-	
Change in Fair Value of Deferred and Contingent Consideration Liabilities				(7.3)	20.8	(28.7)	11.9	(103.7)	(14.7)	14.8	(91.7)	-	-	-	-	-	
Operating Expenses	(317.2)	(371.7)	(429.6)	(563.6)	(702.7)	(1,014.8)	(221.0)	(379.2)	(306.9)	(334.9)	(1,242.0)	(345.2)	(350.8)	(356.1)	(365.5)	(1,417.6)	(1,750.9)
Operating Income	**(307.3)**	**(235.2)**	**(265.2)**	**(375.1)**	**(457.3)**	**(709.6)**	**(145.5)**	**(288.9)**	**(213.4)**	**(230.4)**	**(878.3)**	**(249.3)**	**(247.6)**	**(242.3)**	**(221.1)**	**(960.3)**	**(814.6)**
Interest Income	10.3	9.9	1.7	0.2	11.1	42.1	8.8	10.3	17.0	17.3	53.3	6.0	4.9	3.8	2.7	2.7	1.6
Interest Expense	(25.5)	(27.7)	(29.6)	(40.5)	(26.4)	(81.7)	(21.0)	(21.3)	(21.1)	(21.6)	(84.9)	(17.3)	(17.6)	(17.8)	(18.1)	(35.8)	(68.4)
Other Income (Expense)	(1.6)	(0.5)	0.4	(21.0)	(7.5)	2.2	1.3	0.1	(2.0)	0.4	(0.2)	-	-	-	-	-	-
Pretax Income (Loss)	(324.1)	(253.6)	(292.7)	(436.4)	(480.2)	(747.0)	(156.5)	(299.8)	(219.5)	(234.3)	(910.1)	(260.5)	(260.3)	(256.3)	(236.5)	(1,013.6)	(881.4)
Tax Expense (Benefit)	(0.2)	(0.8)	(1.4)	2.2	(1.4)	(2.6)	(0.6)	(0.8)	(1.0)	(1.3)	(3.7)	-	-	-	-	-	-
Net Income (Loss)	**(324.3)**	**(254.3)**	**(294.1)**	**(434.2)**	**(481.5)**	**(749.6)**	**(157.1)**	**(300.6)**	**(220.5)**	**(235.5)**	**(913.8)**	**(260.5)**	**(260.3)**	**(256.3)**	**(236.5)**	**(1,013.6)**	**(881.4)**
Diluted EPS	**(4.22)**	**$ (3.01)**	**(3.01)**	**$ (3.88)**	**$ (3.91)**	**$ (5.34)**	**$ (1.06)**	**$ (1.94)**	**$ (1.27)**	**$ (1.32)**	**$ (5.57)**	**$ (1.45)**	**$ (1.45)**	**$ (1.43)**	**$ (1.32)**	**$ (5.65)**	**$ (4.75)**
Basic Shares Outstanding	76.9	84.5	97.6	112.0	123.0	140.4	148.5	154.7	173.7	179.0	164.0	179.1	179.2	179.3	179.4	179.3	185.4
Diluted Shares Outstanding	76.9	84.5	97.6	112.0	123.0	140.4	148.5	154.7	173.7	179.0	164.0	179.1	179.2	179.3	179.4	179.3	185.4

Source: Company Reports, Leerink

INSM Balance Sheet & Cash Flow ($MM)	2018	2019	2020	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
Net Cash	**45.1**	**37.4**	**82.8**	**(83.2)**	**(1.7)**	**(369.6)**	**(554.3)**	**96.8**	**317.9**	**508.8**	**508.8**	**280.7**	**53.2**	**(170.0)**	**(386.1)**	**(386.1)**	**(613.0)**
Cash & Cash Equivalents	**495.1**	**487.4**	**532.8**	**716.8**	**1,148.3**	**780.4**	**595.7**	**1,246.8**	**1,467.9**	**1,433.8**	**1,433.8**	**1,205.7**	**978.2**	**755.0**	**538.9**	**538.9**	**312.0**
Total Debt	**450.0**	**450.0**	**450.0**	**800.0**	**1,150.0**	**1,150.0**	**1,150.0**	**1,150.0**	**1,150.0**	**925.0**	**925.0**	**925.0**	**925.0**	**925.0**	**925.0**	**925.0**	**925.0**
Long-Term Debt	450.0	450.0	450.0	800.0	1,150.0	1,150.0	1,150.0	1,150.0	1,150.0	925.0	925.0	925.0	925.0	925.0	925.0	925.0	925.0
Change in Cash	**114.0**	**(7.6)**	**35.7**	**185.4**	**358.3**	**(591.4)**	**114.3**	**651.8**	**(787.2)**	**95.1**	**74.0**	**(228.1)**	**(227.5)**	**(223.2)**	**(216.1)**	**(894.9)**	**(226.9)**
Operating Activities	**(258.0)**	**(250.6)**	**(228.5)**	**(330.3)**	**(400.4)**	**(536.3)**	**(184.0)**	**(122.9)**	**(180.9)**	**(196.0)**	**(683.9)**	**(213.1)**	**(212.5)**	**(208.2)**	**(201.1)**	**(834.9)**	**(666.9)**
Net Income	(324.3)	(254.3)	(294.1)	(434.2)	(481.5)	(749.6)	(157.1)	(300.6)	(220.5)	(235.5)	(913.8)	(260.5)	(260.3)	(256.3)	(236.5)	(1,013.6)	(881.4)
SOE	26.2	27.0	36.2	46.0	57.7	74.8	21.5	23.3	25.5	26.6	96.8	32.4	32.8	33.1	33.4	131.7	154.5
Other	40.1	(23.3)	29.4	57.9	23.4	138.5	(48.4)	154.4	14.1	13.0	133.1	15.0	15.0	15.0	2.0	47.0	60.0
Investing Activities	**(14.8)**	**(42.3)**	**(6.8)**	**(64.3)**	**(34.6)**	**(223.6)**	**295.3**	**(6.8)**	**(1,003.5)**	**131.8**	**(583.2)**	**(15.0)**	**(15.0)**	**(15.0)**	**(15.0)**	**(60.0)**	**(60.0)**
Financing Activities	**386.7**	**285.3**	**271.0**	**612.5**	**793.3**	**168.4**	**3.0**	**781.5**	**397.2**	**159.3**	**1,341.0**	**-**	**-**	**-**	**-**	**-**	**500.0**
Debt Issue (Payment)	377.9	-	-	350.0	350.0	-	-		-	-	-	-	-	-	-	-	-
Equity Issue (Buyback)	-	261.1	245.9	269.9	292.2	152.5	-	713.2	371.0	(0.0)	1,084.1	-	-	-	-	-	500.0
Other	8.8	24.2	25.1	(7.3)	151.1	16.0	3.0	68.3	26.2	159.3	256.9	-	-	-	-	-	-

Source: Company Reports, Leerink Partners

INSM DCF Valuation ($MM)	2020	2021	2022	2023	2024	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E	TV
Operating Cash Flow	(228)	(330)	(400)	(536)	(684)	(835)	(667)	(488)	(117)	425	1,072	1,441	2,071	2,697	
CFI+Net Borrowing	(7)	(64)	(35)	(224)	(583)	(60)	(60)	(60)	(60)	(60)	(60)	(60)	(60)	(60)	
FCFE	(235)	(395)	(435)	(760)	(1,267)	(895)	(727)	(548)	(177)	365	1,012	1,381	2,011	2,637	
NPV	-	-	-	-	-	(895)	(661)	(453)	-133	249	628	779	1,032	1,230	15,687
Discount periods	-	-	-	-	-	-	1.00	2.00	3.00	4.00	5.00	6.00	7.00	8.00	

Diluted Shares Outstanding 4Q24	179.0
Net Cash 4Q24	508.8
Probability Weighted Value/Share	**100**
Implied Market Cap	17,973

Assumptions	
WACC	10%
Terminal Growth Rate	2%

Source: Leerink Partners, Company Filings

Leerink Partners Catalyst Tracker

Stock (Ticker Symbol)	Lateral Impact (Other companies/stocks)	Drug (Brand or chemical name) / Instrument / Area	Indication / Product Class	Type of Event	Event or Trial Details	Expected Timing	Specific Event Date if known or specified	Impact: H(igh) > 9% M(edium) 3 - 9% L(ow) < 2%	Estimated Stock Up/Down % on Best/Worst Outcomes	Leerink Partners View of Expected Outcome
INSM		Arikayce	MAC (Front-Line)	Phase 3 Results Announcement	Registrational ENCORE Study Data	1Q26		H		Positive
INSM		TPIP	PAH	Phase 2 Data Announcement	Ph.2b Topline Data	3Q25	Mid-2025	H		Positive
INSM		Brensocatib	Chronic rhinosinusitis without nasal polyps (CRSsNP)	Phase 2 Data Announcement	Ph.2b BiRCh data	2025	YE2025	H		Positive
INSM		TPIP	PH-ILD	Phase 3 Trial Initiation	Ph.3 initiation	2H25		L		Positive
INSM		Brensocatib	Non-cystic fibrosis bronchiectasis (NCFB)	FDA Approval	PDUFA	3Q25	8/12/2025	H		Positive
INSM		INS1201	Duchenne muscular dystrophy (DMD)	Phase 2 Trial Initiation		1H25		L		
INSM		INS1201	Duchenne muscular dystrophy (DMD)	Phase 2 Data Announcement		4Q25	Late 2025/2026	H		
INSM		Arikayce	MAC (Front-Line)	sNDA/sBLA/sMAA Filing		2026	Later in 2026	L		Positive

Source: Leerink Partners LLC Equity Research and Company Filings

PEPG P&L ($MM)	2020	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
PGN-EDO51	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
PGN-EDODM1	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Total Revenue	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**
COGS	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
R&D	1.0	19.0	54.1	68.1	14.7	25.1	17.7	19.0	76.5	19.9	20.9	21.9	23.0	85.8	94.4
SG&A	0.9	8.1	14.2	16.6	5.1	5.4	5.4	5.4	21.3	5.6	5.8	6.1	6.4	23.8	28.6
Total OpEx	**1.9**	**27.1**	**68.3**	**84.8**	**19.8**	**30.4**	**23.2**	**24.3**	**97.7**	**25.5**	**26.7**	**28.0**	**29.4**	**109.6**	**123.0**
EBIT (loss)	**(1.9)**	**(27.1)**	**(68.3)**	**(84.8)**	**(19.8)**	**(30.4)**	**(23.2)**	**(24.3)**	**(97.7)**	**(25.5)**	**(26.7)**	**(28.0)**	**(29.4)**	**(109.6)**	**(123.0)**
Interest income	0.0	-	2.8	6.4	1.7	2.1	1.8	1.5	7.1	0.9	0.7	0.6	0.5	2.7	0.6
Other income (expense)	(0.0)	(0.2)	0.1	(0.2)	0.0	(0.0)	(0.0)	0.0	(0.0)	(0.0)	(0.0)	(0.0)	(0.0)	(0.2)	-
Total other income (expense)	**(0.0)**	**(0.2)**	**2.9**	**6.2**	**1.8**	**2.1**	**1.8**	**1.5**	**7.1**	**0.8**	**0.7**	**0.6**	**0.4**	**2.5**	**0.6**
EBT	(1.9)	(27.3)	(65.4)	(78.6)	(18.0)	(28.3)	(21.4)	(22.9)	(90.6)	(24.7)	(26.0)	(27.4)	(29.0)	(107.1)	(122.4)
Tax expense / (benefit)	-	-	3.7	-	-	-	-	(0.6)	(0.6)	-	-	-	-	-	-
Net income (loss)	**(1.9)**	**(27.3)**	**(69.1)**	**(78.6)**	**(18.0)**	**(28.3)**	**(21.4)**	**(22.2)**	**(90.0)**	**(24.7)**	**(26.0)**	**(27.4)**	**(29.0)**	**(107.1)**	**(122.4)**
EPS (Basic)	**(4.61)**	**(29.74)**	**(4.42)**	**(3.30)**	**(0.63)**	**(0.87)**	**(0.66)**	**(0.68)**	**(2.85)**	**(0.75)**	**(0.78)**	**(0.82)**	**(0.86)**	**(3.21)**	**(2.93)**
EPS (Diluted)	**(4.61)**	**(29.74)**	**(4.42)**	**(3.30)**	**(0.63)**	**(0.87)**	**(0.66)**	**(0.68)**	**(2.85)**	**(0.75)**	**(0.78)**	**(0.82)**	**(0.86)**	**(3.21)**	**(2.93)**
Shares Outstanding (Basic)	0.9	0.9	15.6	23.8	28.7	32.5	32.6	32.6	31.6	32.9	33.2	33.5	33.8	33.4	41.7
Shares Outstanding (Diluted)	0.9	0.9	15.6	23.8	28.7	32.5	32.6	32.6	31.6	32.9	33.2	33.5	33.8	33.4	41.7

Sources: PEPG Filings; Leerink Partners LLC Equity Research

PEPG Balance Sheet and Cash Flow ($MM)	2020	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
Net Cash	**9.8**	**132.9**	**181.8**	**110.4**	**175.2**	**161.3**	**138.9**	**120.2**	**120.2**	**175.2**	**149.2**	**121.8**	**93.0**	**93.0**	**119.6**
Cash & Cash Equivalents	9.8	132.9	181.8	110.4	175.2	161.3	138.9	120.2	120.2	175.2	149.2	121.8	93.0	93.0	119.6
Debt	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Change in Cash	**6.3**	**124.6**	**49.2**	**(101.2)**	**53.1**	**(67.8)**	**(23.8)**	**7.2**	**(31.3)**	**(24.3)**	**(26.0)**	**(27.4)**	**(28.8)**	**(106.4)**	**26.6**
Cash Flow from Operations	**(1.7)**	**(22.6)**	**(59.3)**	**(69.0)**	**(22.5)**	**(16.4)**	**(24.0)**	**(19.5)**	**(82.4)**	**(22.3)**	**(24.0)**	**(25.4)**	**(26.8)**	**(98.4)**	**(113.4)**
Cash Flow from Investing	**(0.0)**	**(0.5)**	**(3.8)**	**(32.0)**	**(11.3)**	**(52.7)**	**(0.1)**	**26.5**	**(37.7)**	**(2.0)**	**(2.0)**	**(2.0)**	**(2.0)**	**(8.0)**	**(10.0)**
Cash Flow from Financing	**8.0**	**147.7**	**112.2**	**(0.2)**	**87.0**	**1.3**	**0.3**	**0.1**	**88.7**	**-**	**-**	**-**	**-**	**0.0**	**150.0**

Sources: PEPG Filings; Leerink Partners LLC Equity Research

PEPG DCF Analysis ($MM)	2020	2021	2022	2023	2024	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E	2034E	2035E	TV
CF - Operating	(1.7)	(22.6)	(59.3)	(69.0)	(82.4)	(98.4)	(113.4)	(127.7)	(117.3)	(101.4)	(45.8)	37.7	119.4	212.3	283.8	334.9	
CF - Investing	(0.0)	(0.5)	(3.8)	(32.0)	(37.7)	(8.0)	(10.0)	(10.0)	(10.0)	(10.0)	(10.0)	(10.0)	(10.0)	(10.0)	(10.0)	(10.0)	
FCFE	(1.7)	(23.1)	(63.0)	(101.0)	(120.0)	(106.4)	(123.4)	(137.7)	(127.3)	(111.4)	(55.8)	27.7	109.4	202.3	273.8	324.9	2,761.3
Discount Periods	-	-	-	-	-	-	1.00	2.00	3.00	4.00	5.00	6.00	7.00	8.00	9.00	10.00	
FCFE NPV	-	-	-	-	-	(106.4)	(108.3)	(105.9)	(86.0)	(65.9)	(29.0)	12.6	43.7	70.9	84.2	87.6	744.8

Total FCFE NPV ($MM)	$ 542
Net Cash (4Q24) ($MM)	$ 120
Implied Mkt. Cap ($MM)	$ 663
PEPG PT	**$ 20**

Shares Outstanding (4Q24)	32.6
Net Cash Per Share	$ 3.69
Discount Rate	14%
Terminal Growth Rate	2%

Sources: PEPG Filings; Leerink Partners LLC Equity Research

Leerink Partners Catalyst Tracker

Stock (Ticker Symbol)	Lateral Impact (Other companies/stocks)	Drug (Brand or chemical name) / Instrument / Area	Indication / Product Class	Type of Event	Event or Trial Details	Expected Timing	Specific Event Date if known or specified	Impact: H(igh) > 9% M(edium) 3 - 9% L(ow) < 2%	Estimated Stock Up/Down % on Best/Worst Outcomes	Leerink Partners View of Expected Outcome
PEPG	RNA, DYN, TRDA, DSGN	PGN-EDODM1	Myotonic Dystrophy Type 1	Phase 1 Data Announcement	Ph.1 Safety, Splicing Correction, and Functional Outcomes Data from 15 mg/kg Cohort	2H25		H		Positive
PEPG	SRPT, RNA, DYN, TRDA, WVE	PGN-EDO51	Duchenne Muscular Dystrophy	Phase 2 Data Announcement	Ph.2 CONNECT1-EDO51 Open-label MAD Safety, Exon Skipping, and Dystrophin Expression Data for the 10 mg/kg Cohort	3Q25		H		Positive
PEPG	RNA, DYN, TRDA, DSGN	PGN-EDODM1	Myotonic Dystrophy Type 1	Phase 2 Data Announcement	Ph.2 Safety, Splicing Correction, and Functional Outcomes Data from 5 mg/kg Cohorts	1Q26		H		Positive

Source: Leerink Partners LLC Equity Research and Company Filings

Avidity Biosciences Inc. P&L (USD, MM)	2019	2020	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
Revenues																
AOC-1001: DM1	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
AOC-1020: FSHD	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	4.0
AOC-1044: DMD Exon 44 mutations	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	8.8
Collaboration & Contract Research	2.3	6.8	9.3	9.2	9.6	3.5	2.0	2.3	3.0	10.9	-	-	-	-	-	-
Total Revenue	**2.3**	**6.8**	**9.3**	**9.2**	**9.6**	**3.5**	**2.0**	**2.3**	**3.0**	**10.9**	**-**	**-**	**-**	**-**	**-**	**12.8**
Operating Expenses																
COGS	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	11.5
Gross Profit	2.3	6.8	9.3	9.2	9.6	3.5	2.0	2.3	3.0	10.9	-	-	-	-	-	1.3
R&D	14.5	37.6	101.2	150.4	191.0	66.8	63.9	77.2	95.6	303.6	102.3	109.5	117.1	125.3	454.3	522.4
SG&A	5.1	13.5	26.2	37.7	54.2	13.9	20.7	23.3	28.3	86.2	31.7	35.5	39.8	44.6	151.7	182.0
Total Expenses	**19.7**	**51.1**	**127.4**	**188.1**	**245.2**	**80.7**	**84.7**	**100.5**	**124.0**	**389.8**	**134.1**	**145.0**	**157.0**	**169.9**	**606.0**	**704.5**
EBIT (Loss)	**(17.3)**	**(44.3)**	**(118.1)**	**(178.9)**	**(235.6)**	**(77.2)**	**(82.6)**	**(98.1)**	**(121.0)**	**(378.9)**	**(134.1)**	**(145.0)**	**(157.0)**	**(169.9)**	**(606.0)**	**(691.7)**
Interest Income	-	0.2	0.0	2.2	24.0	8.4	11.9	18.0	18.5	56.9	0.2	0.2	0.2	0.2	0.8	0.2
Interest Expense	(7.4)	(0.2)	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Other	(0.0)	(0.1)	0.0	2.7	(0.6)	(0.1)	(0.1)	(0.2)	0.2	(0.2)	-	-	-	-	-	
EBT (Loss)	**(24.7)**	**(44.4)**	**(118.0)**	**(174.0)**	**(212.2)**	**(68.9)**	**(70.8)**	**(80.4)**	**(102.3)**	**(322.3)**	**(133.9)**	**(144.8)**	**(156.8)**	**(169.7)**	**(605.2)**	**(691.5)**
Taxation	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Other Income/(Loss)	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Net Income (Loss)	**(24.7)**	**(44.4)**	**(118.0)**	**(174.0)**	**(212.2)**	**(68.9)**	**(70.8)**	**(80.4)**	**(102.3)**	**(322.3)**	**(133.9)**	**(144.8)**	**(156.8)**	**(169.7)**	**(605.2)**	**(691.5)**
EPS (Basic)	**(9.12)**	**(2.05)**	**(2.85)**	**(3.34)**	**(2.91)**	**(0.79)**	**(0.66)**	**(0.65)**	**(0.80)**	**(2.89)**	**(1.04)**	**(1.12)**	**(1.21)**	**(1.31)**	**(4.69)**	**(5.32)**
EPS (Diluted)	**(9.12)**	**(2.05)**	**(2.85)**	**(3.34)**	**(2.91)**	**(0.79)**	**(0.66)**	**(0.65)**	**(0.80)**	**(2.89)**	**(1.04)**	**(1.12)**	**(1.21)**	**(1.31)**	**(4.69)**	**(5.32)**
Shares Outstanding (Basic)	2.7	21.7	41.4	52.2	73.0	87.2	106.9	123.4	128.5	111.5	128.7	128.9	129.1	129.3	129.0	130.0
Shares Outstanding (Diluted)	2.7	21.7	41.4	52.2	73.0	87.2	106.9	123.4	128.5	111.5	128.7	128.9	129.1	129.3	129.0	130.0

Avidity Biosciences, Inc. DCF (USD, MM)	2018	2019	2020	2021	2022	2023	2024	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E	2034E	2035E	TV
CF - Operating	(10)	2	(37)	(95)	(136)	(119)	(301)	(526)	(598)	(412)	(73)	330	711	1,185	1,346	1,520	1,725	1,897	
CF - Investing	(0)	(0)	(8)	(83)	(190)	(130)	(854)	-	-	-	-	-	-	-	-	-	-	-	
FCFE	(10)	2	(45)	(177)	(326)	(249)	(1,155)	(526)	(598)	(412)	(73)	330	711	1,185	1,346	1,520	1,725	1,897	16,127
Discount Periods	-	-	-	-	-	-	-	-	1.00	2.00	3.00	4.00	5.00	6.00	7.00	8.00	9.00	10.00	
FCFE NPV	-	-	-	-	-	-	-	(526)	(524)	(317)	(49)	196	369	540	538	533	531	512	4,350

Sum FCFE NPV (MM DCF)	$ 6,152
Net Cash (4Q24) (MM USD)	$ 1,501
Implied Market Cap. (M USD)	**$ 7,653**
Per Share Value	**$ 60**

Shares Oustanding (M) (4Q24)	128.5
Discount Rate	14%
Terminal Growth Rate	2%

Sources: Company filings, Leerink Partners LLC Equity Research

Leerink Partners Catalyst Tracker

Stock (Ticker Symbol)	Lateral Impact (Other companies/stocks)	Drug (Brand or chemical name) / Instrument / Area	Indication / Product Class	Type of Event	Event or Trial Details	Expected Timing	Specific Event Date if known or specified	Impact: H(igh) > 9% M(edium) 3 - 9% L(ow) < 2%	Estimated Stock Up/Down % on Best/Worst Outcomes	Leerink Partners View of Expected Outcome
RNA		Delpacibart Braxlosiran	Facioscapulohumeral Muscular Dystrophy (FSHD)	Other Legal/Regulatory	Alignment on Ph.3 Trial Design	2Q25		M		Positive
RNA		Delpacibart Braxlosiran	FSHD	Other Legal/Regulatory	Alignment on Accelerated Path to Approval	2Q25		H		Positive
RNA		Delpacibart Braxlosiran	FSHD	Phase 3 Trial Full Enrollment	Completion of Enrollment in FORTITUDE (Biomarker Cohort)	2Q25		M		Positive
RNA		Delpacibart Braxlosiran	FSHD	Phase 3 Results Announcement	Presentation of Topline Data from FORTITUDE	2Q25		H		Positive
RNA		Delpacibart Braxlosiran	FSHD	Phase 3 Trial Initiation	Initiation of Registrational Study	2Q25		L		Positive
RNA		Delpacibart Etedesiran	DM1	Phase 3 Trial Full Enrollment	Completition of Enrollment in HARBOR Study	2025	Mid-2025	L		Positive
RNA		Delpacibart Zotadirsen	DMD	Phase 2 Data Announcement	Topline Data from EXPLORE44-OLE	4Q25		M		Positive
RNA		Delpacibart Etedesiran	DM1	Phase 2 Data Announcement	Update from MARINA-OLE	4Q25		M		Positive
RNA		Delpacibart Zotadirsen	DMD	NDA/BLA Filing	Under Accelerated Approval	2025	YE25	M		Positive
RNA		Delpacibart Etedesiran	Myotonic Dystrophy Type 1 (DM1)	Phase 2 Data Announcement	Publication of Additional Analyses from MARINA	2025		L		Positive
RNA		Delpacibart Etedesiran	DM1	NDA/BLA Filing	BLA Filing	2026		H		Positive

Source: Leerink Partners LLC Equity Research and Company Filings

SLDB P&L ($MM)	2020	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
SGT-003 (DMD)	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
SGT-501 (CPVT)	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
AVB-401 (BAG3)	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Amortization of milestone payments			-	-	-	-	-	-	-	-	-	-	-	-	-
Collaboration revenue		13.6	8.1	-	-	-	-	-	-	-	-	-	-	-	-
Total Revenue	**-**	**13.6**	**8.1**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**	**-**
COGS	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
R&D	64.9	58.7	78.4	76.6	18.9	19.5	27.3	30.8	96.4	30.8	30.8	30.9	30.9	123.4	133.3
SG&A	21.6	27.1	28.9	27.8	8.0	8.3	7.9	9.1	33.3	9.2	9.3	9.4	9.5	37.4	48.7
Restructuring charges	1.9		7.2	(0.1)	-	-	-	-	-	-	-	-	-	-	-
Total OpEx	**88.4**	**85.9**	**114.5**	**104.3**	**26.9**	**27.8**	**35.2**	**39.9**	**129.7**	**40.0**	**40.1**	**40.3**	**40.4**	**160.8**	**181.9**
Operating Income (loss)	(88.4)	(72.3)	(106.5)	(104.3)	(26.9)	(27.8)	(35.2)	(39.9)	(129.7)	(40.0)	(40.1)	(40.3)	(40.4)	(160.8)	(181.9)
Total Other Income (loss)	0.1	0.1	20.5	8.2	2.6	2.7	2.5	(2.7)	5.0	2.1	1.9	1.6	1.4	7.0	0.17
EBT	(88.3)	(72.2)	(86.0)	(96.0)	(24.3)	(25.1)	(32.7)	(42.6)	(124.7)	(37.9)	(38.3)	(38.6)	(39.0)	(153.8)	(181.7)
Income Tax (benefit)	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Net income (loss)	**(88.3)**	**(72.2)**	**(86.0)**	**(96.0)**	**(24.3)**	**(25.1)**	**(32.7)**	**(42.6)**	**(124.7)**	**(37.9)**	**(38.3)**	**(38.6)**	**(39.0)**	**(153.8)**	**(181.7)**
EPS GAAP	**(1.70)**	**(0.68)**	**(10.10)**	**(4.84)**	**(0.64)**	**(0.61)**	**(0.79)**	**(1.00)**	**(3.06)**	**(0.41)**	**(0.41)**	**(0.42)**	**(0.42)**	**(1.66)**	**(1.94)**
Basic Shares Outstanding	51.9	106.8	8.5	19.8	38.2	40.9	41.4	42.7	40.8	92.3	92.5	92.7	93.7	92.8	93.8
Diluted Shares Outstanding	51.9	106.8	8.5	19.8	38.2	40.9	41.4	42.7	40.8	92.3	92.5	92.7	93.7	92.8	93.8

Source: Company Filings; Leerink Partners LLC Equity Research

SLDB Balance Sheet & Cash Flow ($MM)	2020	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
Net Cash	**154.7**	**207.8**	**213.7**	**123.6**	**206.1**	**190.3**	**171.1**	**148.9**	**148.9**	**302.7**	**268.2**	**233.3**	**198.0**	**198.0**	**33.0**
Cash & Cash Equivalents	154.7	207.8	213.7	123.6	206.1	190.3	171.1	148.9	148.9	302.7	268.2	233.3	198.0	198.0	33.0
Debt	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Change in Cash	**78.7**	**(33.9)**	**36.0**	**(81.4)**	**43.6**	**(21.6)**	**(31.4)**	**15.9**	**6.3**	**153.8**	**(34.6)**	**(34.9)**	**(35.3)**	**49.1**	**(165.0)**
Cash Flow From Operations	(56.6)	(77.8)	(98.0)	(94.2)	(25.2)	(21.5)	(23.7)	(29.6)	(100.0)	(34.0)	(34.4)	(34.7)	(35.1)	(138.1)	(164.2)
Cash Flow From Investing	6.6	(91.1)	59.2	9.7	(38.5)	(5.2)	(11.0)	38.6	(16.1)	(0.2)	(0.2)	(0.2)	(0.2)	(0.8)	(0.8)
Cash Flow From Financing	128.7	135.0	74.8	3.1	107.2	5.0	3.3	6.9	122.4	188.0	-	-	-	188.0	-

Source: SLDB Filings; Leerink Partners LLC Equity Research

DCF Analysis ($MM)	2021	2022	2023	2024	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E	2034E	2035E	TV
Cash Flow From Operations	(77.8)	(98.0)	(94.2)	(100.0)	(138.1)	(164.2)	(117.1)	(72.7)	172.5	343.0	400.9	434.8	409.2	385.0	382.8	
Cash Flow From Investing	(91.1)	59.2	9.7	(16.1)	(0.8)	(0.8)	(0.8)	(0.8)	(0.8)	(0.8)	(0.8)	(0.8)	(0.8)	(0.8)	(0.8)	
FCFE	(168.9)	(38.8)	(84.5)	(116.1)	(138.9)	(165.0)	(117.9)	(73.5)	171.7	342.2	400.1	434.0	408.4	384.2	382.1	3,247.5
Discount Periods	-	-	-	-	-	0.75	1.75	2.75	3.75	4.75	5.75	6.75	7.75	8.75	9.75	
NPV FCFE	-	-	-	-	(104.2)	(149.5)	(93.7)	(51.3)	105.0	183.6	188.3	179.2	147.9	122.1	106.5	905.2

Total NPV FCFE ($MM)	$	1,539.1
Net Cash 1Q25E ($MM)	$	302.7
Implied Market Cap ($MM)	$	1,841.9
Per Share Value	**$**	**20**

Discount Rate		14%
Terminal Growth Rate		2%
Shares Outstanding (1Q25E)		92.3
Net Cash/Share	$	3.28

Source: Company Reports and Leerink Partners LLC Equity Research

Leerink Partners Catalyst Tracker

Stock (Ticker Symbol)	Lateral Impact (Other companies/ stocks)	Drug (Brand or chemical name) / Instrument / Area	Indication / Product Class	Type of Event	Event or Trial Details	Expected Timing	Specific Event Date if known or specified	Impact: H(igh) > 9% M(edium) 3 - 9% L(ow) < 2%	Estimated Stock Up/Down % on Best/Worst Outcomes	Leerink Partners View of Expected Outcome
SLDB		SGT-003	DMD	Other Event	>10 patients dosed	2Q25	Early 2Q25	H		Positive
SLDB		SGT-501	CPVT	IND Submission	IND submission for RYR2	1H25		M		Positive
SLDB		SGT-003	DMD	Other Event	20 patients dosed	4Q25		H		Positive
SLDB		Capsid Library	Cardiac	Lead Candidate Selection	Final capsid selection from first cardiac capsid library	4Q25				
SLDB		SGT-003	DMD	Other Legal/Regulatory	Meet with the FDA to discuss potential accelerated approval path	2H25	Mid-2025	H		Positive
SLDB		SGT-212	FA	Phase 1 Trial Initiation	Phase 1b initiation	2H25		L		Positive
SLDB		SGT-601	TNNT2	IND Submission	IND submission	2H26		L		Positive

Source: Leerink Partners LLC Equity Research and Company Filings

SRPT P&L ($MM)	2019	2020	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
Total Product Revenue	**380.8**	**455.9**	**612.4**	**843.8**	**1,144.9**	**359.5**	**360.5**	**429.8**	**638.2**	**1,788.0**	**637.3**	**715.6**	**789.7**	**858.0**	**3,000.6**	**3,495.3**
PMO	380.8	455.9	612.4	843.8	944.5	225.5	238.8	248.8	254.0	967.1	220.2	225.6	232.9	238.9	917.5	799.4
Elevidys	-	-	-	-	200.4	133.9	121.7	181.0	384.2	820.8	417.2	490.0	556.8	619.1	2,083.0	2,645.3
Limb-Girdle	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	50.6
Collaboration/Royalty Revenue		**84.2**	**89.5**	**89.2**	**98.5**	**54.0**	**2.4**	**37.4**	**20.3**	**114.0**	**3.0**	**4.8**	**7.2**	**13.6**	**28.5**	**28.6**
Total Revenue	**380.8**	**540.1**	**701.9**	**933.0**	**1,243.3**	**413.5**	**362.9**	**467.2**	**658.4**	**1,902.0**	**640.3**	**720.3**	**796.9**	**871.6**	**3,029.1**	**3,523.9**
COGS	(56.6)	(63.4)	(97.0)	(140.0)	(150.3)	(50.6)	(44.5)	(91.7)	(132.3)	(319.1)	(114.7)	(128.8)	(142.2)	(154.4)	(540.1)	(629.2)
R&D	(560.9)	(722.3)	(771.2)	(877.1)	(877.4)	(200.4)	(179.7)	(224.5)	(200.0)	(804.5)	(752.0)	(200.2)	(200.4)	(200.6)	(1,353.0)	(734.0)
SG&A	(284.8)	(317.9)	(282.7)	(451.4)	(481.9)	(127.0)	(138.8)	(128.2)	(163.9)	(557.9)	(165.5)	(167.2)	(168.8)	(170.5)	(672.0)	(594.2)
Other	(174.1)	(0.7)	(10.7)	(0.7)	(1.6)	(0.6)	(0.6)	(0.6)	(0.6)	(2.4)	-	-	-	-	-	-
Operating Expenses:	(1,086.4)	(1,104.3)	(1,161.6)	(1,469.2)	(1,511.2)	(378.6)	(363.6)	(445.0)	(496.7)	(1,683.9)	(1,032.2)	(496.1)	(511.3)	(525.5)	(2,565.2)	(1,957.4)
Operating Income (Loss)	**(705.6)**	**(564.2)**	**(459.7)**	**(536.2)**	**(267.8)**	**34.9**	**(0.7)**	**22.2**	**161.7**	**218.1**	**(391.9)**	**224.2**	**285.6**	**346.1**	**464.0**	**1,566.5**
Loss on Debt Extinguishment			-	-	(387.3)	-	-	-	-	-	-	-	-	-	-	-
Interest Income (Expense)	-	-	-	-	-	-	-	-	-	-	(10.5)	(10.5)	(10.5)	(10.5)	(41.8)	(32.0)
Other Income (Expense)	(8.3)	11.1	40.8	(153.8)	135.1	6.5	14.3	11.8	10.1	42.7	34.4	218.6	38.6	46.3	338.0	203.7
Pretax Income	(713.9)	(553.1)	(418.9)	(690.0)	(520.1)	41.4	13.6	34.0	171.7	260.8	(367.9)	432.4	313.7	381.9	760.2	1,738.3
Tax (Expense) / Benefit	(1.2)	(1.1)	0.2	(13.5)	(15.9)	(5.3)	(7.1)	(0.4)	(12.7)	(25.5)	-	-	-	-	-	(83.6)
Net Income (Loss) - GAAP	**(715.1)**	**(554.1)**	**(418.8)**	**(703.5)**	**(536.0)**	**36.1**	**6.5**	**33.6**	**159.0**	**235.2**	**(367.9)**	**432.4**	**313.7**	**381.9**	**760.2**	**1,654.7**
Basic EPS - GAAP	**(9.71)**	**(7.11)**	**(5.15)**	**(8.03)**	**(5.80)**	**0.38**	**0.07**	**0.35**	**1.65**	**2.47**	**(3.80)**	**4.44**	**3.21**	**3.89**	**7.79**	**16.62**
Diluted EPS - GAAP	**(9.71)**	**(7.11)**	**(5.15)**	**(8.03)**	**(5.80)**	**0.36**	**0.07**	**0.33**	**1.47**	**2.31**	**(3.38)**	**3.95**	**2.85**	**3.46**	**6.93**	**14.81**
Diluted EPS - Non-GAAP						**0.76**	**0.44**	**0.64**	**1.90**	**3.69**	**(2.21)**	**4.36**	**3.26**	**3.87**	**9.32**	**16.31**
Basic Shares Outstanding	73.6	78.0	81.3	87.6	92.4	94.0	94.6	95.4	96.3	95.1	96.8	97.3	97.8	98.3	97.5	99.5
Diluted Shares Outstanding	73.6	78.0	81.3	87.6	92.4	99.1	99.1	100.4	108.5	101.8	109.0	109.5	110.0	110.5	109.7	111.7

SRPT BS & CFS	2019	2020	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
Net Cash	**304.7**	**946.1**	**1,019.0**	**445.1**	**543.7**	**257.1**	**341.3**	**259.8**	**366.4**	**366.4**	**99.0**	**582.4**	**947.3**	**1,380.6**	**1,380.6**	**3,239.4**
Cash, Cash Eq, ST	**1,124.7**	**1,938.6**	**2,115.9**	**1,989.4**	**1,676.3**	**1,390.7**	**1,476.1**	**1,395.8**	**1,503.5**	**1,503.5**	**1,238.2**	**1,723.6**	**2,090.6**	**2,525.9**	**2,525.9**	**4,386.8**
Cash & Equivalents	1,124.7	1,502.6	2,115.9	1,989.4	1,676.3	1,390.7	1,476.1	1,395.8	1,503.5	1,503.5	1,238.2	1,723.6	2,090.6	2,525.9	2,525.9	4,386.8
Total Debt			**-**													
Long Term Debt	820.0	992.5	1,096.9	1,544.3	1,132.5	1,133.7	1,134.8	1,136.0	1,137.1	1,137.1	1,139.2	1,141.2	1,143.3	1,145.3	1,145.3	1,147.4
Change in Cash	**472.8**	**668.1**	**613.8**	**(1,139.7)**	**(541.8)**	**(1.1)**	**(43.7)**	**(185.7)**	**905.1**	**674.6**	**(265.3)**	**485.5**	**367.0**	**435.3**	**1,022.4**	**1,860.9**
Operating Activities	**(456.5)**	**107.5**	**(443.2)**	**(325.3)**	**(501.0)**	**(242.1)**	**14.9**	**(70.7)**	**92.0**	**(205.8)**	**(210.3)**	**507.5**	**389.1**	**457.6**	**1,143.9**	**1,933.9**
Net Income	(715.1)	(554.1)	(418.8)	(703.5)	(536.0)	36.1	6.5	33.6	159.0	235.2	(367.9)	432.4	313.7	381.9	760.2	1,654.7
SOE	78.6	108.1	113.9	233.0	182.5	40.7	50.5	43.5	49.7	184.3	137.6	55.1	55.4	55.7	303.8	199.2
Other	180.0	**553.5**	**(138.3)**	145.1	(147.5)	(318.9)	(42.0)	(147.8)	(116.7)	(625.3)	20.0	20.0	20.0	20.0	80.0	80.0
Investing Activities	**286.7**	**(121.7)**	**495.4**	**(1,046.9)**	**(165.8)**	**218.8**	**(98.5)**	**(128.8)**	**764.1**	**755.6**	**(55.0)**	**(22.0)**	**(22.2)**	**(22.3)**	**(121.5)**	**(73.1)**
Financing Activities	**642.6**	**682.3**	**561.6**	**232.5**	**125.0**	**22.1**	**39.9**	**13.8**	**49.0**	**124.8**	**-**	**-**	**-**	**-**	**-**	**-**
Other	32.3	375.1	13.0	30.0	44.4	22.1	39.9	13.8	49.0	124.8	-	-	-	-	-	-
Debt Issue (Payment)	244.9	-	-	202.5	80.6	-	-	-	-	-	-	-	-	-	-	-
Equity Issue (Buyback)	365.4	307.3	548.5	-	-	-	-	-	-	-	-	-	-	-	-	-

Source: Leerink Partners Research and Company Reports

Valuation	2020	2021	2022	2023	2024	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E	2034E	2035E	TV
CF Operating	107	(443)	(325)	(501)	(206)	1,144	1,934	2,014	1,908	1,882	2,002	1,658	1,535	1,368	1,212	1,083	
CF Investing + Net borrowing	(122)	495	(844)	(85)	756	(122)	(73)	(1,203)	(49)	(27)	(23)	(21)	(12)	(9)	(4)	(3)	
FCFE	(14)	52	(1,170)	(586)	550	1,022	1,861	812	1,860	1,856	1,979	1,637	1,523	1,358	1,208	1,080	
Discount Periods	-	-	-	-	-	-	1.00	2.00	3.00	4.00	5.00	6.00	7.00	8.00	9.00	10.00	
PV FCF	-	-	-	-	-	1,022	1,692	671	1,397	1,267	1,229	924	781	634	512	416	5,309

NPV	15,855
NPV/Share	$ 146

Cost of Equity	10%
Terminal Growth Rate	2%
Diluted Shares Outstanding 4Q24	108.5

DCF Breakdown	Value/Share
Exondys 51 (Eteplirsen)	$ 31
Vyondys 53 (Golodirsen)	$ 6
Amondys 45 (Casimersen)	$ 13
Elevidys (SRP-9001 Microdystrophin)	$ 87
Limb-Girdle Muscular Dystrophy	$ 8
Net Cash/Diluted Share 4Q24	$ 3
Per Share Valuation	**$ 150**
Implied Market Cap ($MM)	**$ 16,222**

US Scenario Map	Probability
Exondys 51 (Eteplirsen) Approval	100%
Vyondys 53 (Golodirsen) Approval	100%
Amondys 45 (Casimersen) Approval	100%
Elevidys (SRP-9001 Microdystrophin) Approval	100%
SRP-9003 (LGMD2E) Approval	50%
SRP-9004 (LGMD2D) Approval	10%
SRP-9005 (LGMD2C) Approval	10%

EU Scenario Map	Probability
Exondys 51 (Eteplirsen) Approval	0%
Vyondys 53 (Golodirsen) Approval	0%
Amondys 45 (Casimersen) Approval	0%
Elevidys (SRP-9001 Microdystrophin) Approval	60%
SRP-9003 (LGMD2E) Approval	50%
SRP-9004 (LGMD2D) Approval	10%
SRP-9005 (LGMD2C) Approval	10%

Source: Leerink Partners Research and Company Reports

Leerink Partners
Catalyst Tracker

Stock (Ticker Symbol)	Lateral Impact (Other companies/ stocks)	Drug (Brand or chemical name) / Instrument / Area	Indication / Product Class	Type of Event	Event or Trial Details	Expected Timing	Specific Event Date if known or specified	Impact: H(igh) > 9% M(edium) 3 - 9% L(ow) < 2%	Estimated Stock Up/Down % on Best/Worst Outcomes	Leerink Partners View of Expected Outcome
SRPT		Elevidys	DMD	Phase 3 Trial Full Enrollment	ENVISION Full Enrollment	1H25		L		Neutral
SRPT		SRP-9003	LGMD Type 2E	Phase 3 Results Announcement	EMERGENE Ph.3 Expression Data	Mid-2025		M		Positive
SRPT		Elevidys	DMD	Phase 1 Data Announcement	Study 104 (Imlifidase) Expression Data	2H25		M		Neutral
SRPT		Elevidys	DMD	Phase 1 Data Announcement	Study 105 (Plasmapheresis) Expression Data	2H25		M		Neutral
SRPT		Elevidys	DMD	Phase 1 Data Announcement	ENDEAVOR Cohort 6 Expression Data (Ambulatory; ≥2 to <3 years of age)	2H25		L		Neutral
SRPT		Elevidys	DMD	NDA/BLA Filing	sBLA Filing for <4 Years Old	2H25		L		Neutral
SRPT	RNA, DYN	ARO-DUX4	FSHD	Phase 1 Data Announcement	Preliminary Results from Ph.1 Study	2H25		H		Neutral
SRPT	RNA, DYN, PEPG	ARO-DM1	DM1	Phase 1 Data Announcement	Preliminary Results from Ph.1 Study	2H25		H		Neutral
SRPT		-	LGMD	Other Event	JOURNEY Data in Sarcoglycanopathies (Natural History)	2H25		L		Neutral
SRPT		SRP-9003	LGMD Type 2E	NDA/BLA Filing	BLA Filing	2H25		M		Neutral
SRPT		SRP-9003	LGMD Type 2E	Phase 1 Data Announcement	VOYAGENE Ph.1 Data (Expression, Function, Safety)	2H25		M		Neutral
SRPT		SRP-9010	LGMD Type 2A	IND Submission		2H25		L		Neutral
SRPT		ARO-HTT	Huntington's Disease	IND Submission		2H25		L		Neutral
SRPT		-	-	Other Event	R&D Day	2025		M		Neutral
SRPT		Casimersen and Golodirsen	DMD	Phase 3 Results Announcement	Confirmatory ESSENCE Data	2026	Early 2026	H		Neutral

Source: Leerink Partners LLC Equity Research and Company Filings

WVE P&L ($MM)	2018	2019	2020	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
WVE-003	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
WVE-N531	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	84.8
WVE-006	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
INHBE	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Other revenue	14.4	16.0	20.1	41.0	3.6	113.3	12.5	19.7	(7.7)	83.7	108.3	16.5	16.5	16.5	16.5	66.0	7.5
Total revenue	**14.4**	**16.0**	**20.1**	**41.0**	**3.6**	**113.3**	**12.5**	**19.7**	**(7.7)**	**83.7**	**108.3**	**16.5**	**16.5**	**16.5**	**16.5**	**66.0**	**92.3**
COGS	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	23.7
R&D	134.4	175.4	130.9	121.9	115.9	130.0	33.4	40.4	41.2	44.6	159.7	45.1	45.5	46.0	46.5	183.1	186.8
SG&A	39.5	48.9	42.5	46.1	50.5	51.3	13.5	14.3	15.0	16.1	59.0	16.3	16.5	16.6	16.8	66.2	66.8
Other OpEx	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Total operating expense	**173.9**	**224.3**	**173.5**	**168.0**	**166.4**	**181.3**	**47.0**	**54.7**	**56.2**	**60.8**	**218.7**	**61.4**	**62.0**	**62.6**	**63.2**	**249.3**	**277.3**
Operating income (loss)	**(159.5)**	**(208.3)**	**(153.4)**	**(127.0)**	**(162.7)**	**(68.0)**	**(34.5)**	**(35.0)**	**(63.9)**	**23.0**	**(110.4)**	**(44.9)**	**(45.5)**	**(46.1)**	**(46.7)**	**(183.3)**	**(185.0)**
Dividend income and interest income, net	3.4	4.9	0.6	0.0	1.6	7.9	2.5	2.1	1.8	3.7	10.2	-	-	-	-	-	-
Other income, net	9.5	9.7	2.1	4.5	0.0	1.9	0.4	(0.0)	0.3	2.5	3.2	-	-	-	-	-	-
Total other income, net	**12.9**	**14.7**	**2.6**	**4.6**	**1.6**	**9.8**	**2.9**	**2.1**	**2.1**	**6.3**	**13.4**	**-**	**-**	**-**	**-**	**-**	**-**
Net income (loss) before taxes	**(146.6)**	**(193.6)**	**(150.8)**	**(122.4)**	**(161.1)**	**(58.2)**	**(31.6)**	**(32.9)**	**(61.8)**	**29.3**	**(97.0)**	**(44.9)**	**(45.5)**	**(46.1)**	**(46.7)**	**(183.3)**	**(185.0)**
Tax expense (benefit)	0.1	-	(0.8)	(0.2)	0.7	(0.7)	-	-	-	-	-	-	-	-	-	-	
Tax rate											*0%*					*0%*	*0%*
Net income (loss)	**(146.7)**	**(193.6)**	**(149.9)**	**(122.2)**	**(161.8)**	**(57.5)**	**(31.6)**	**(32.9)**	**(61.8)**	**29.3**	**(97.0)**	**(44.9)**	**(45.5)**	**(46.1)**	**(46.7)**	**(183.3)**	**(185.0)**
EPS GAAP	**$ (5.06)**	**$ (5.72)**	**$ (3.82)**	**$ (2.36)**	**$ (2.05)**	**$ (0.54)**	**$ (0.24)**	**$ (0.25)**	**$ (0.47)**	**$ 0.18**	**$ (0.70)**	**$ (0.28)**	**$ (0.28)**	**$ (0.28)**	**$ (0.29)**	**$ (1.13)**	**$ (1.12)**
Basic shares outstanding (MM)	29.0	33.9	39.2	51.8	78.9	106.1	129.3	129.5	132.6	161.5	138.2	161.7	161.9	162.1	162.3	162.0	165.9
Diluted shares outstanding (MM)	29.0	33.9	39.2	51.8	78.9	106.1	129.3	129.5	132.6	161.5	138.2	161.7	161.9	162.1	162.3	162.0	165.9

Source: Leerink Partners LLC Equity Research and Company Filings

WVE BS & CFS ($MM) GAAP	2018	2019	2020E	2021	2022	2023	1Q24	2Q24	3Q24	4Q24	2024	1Q25E	2Q25E	3Q25E	4Q25E	2025E	2026E
Net Cash	**174.8**	**147.2**	**184.5**	**150.6**	**88.5**	**200.4**	**180.9**	**154.0**	**310.9**	**302.1**	**302.1**	**266.4**	**230.4**	**193.6**	**156.5**	**156.5**	**103.7**
Cash & equivalents	174.8	147.2	184.5	150.6	88.5	200.4	180.9	154.0	310.9	302.1	302.1	266.4	230.4	193.6	156.5	156.5	103.7
Debt	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-	-
Change in Cash	**32.3**	**(27.6)**	**37.3**	**(33.9)**	**(62.1)**	**112.0**	**(17.2)**	**(29.0)**	**156.9**	**(8.8)**	**101.9**	**(35.7)**	**(36.0)**	**(36.8)**	**(37.1)**	**(145.6)**	**(52.8)**
Operating Cash Flow	**(22.9)**	**(188.2)**	**(116.0)**	**(89.0)**	**(127.8)**	**(19.4)**	**(31.3)**	**(29.6)**	**(46.5)**	**(43.6)**	**(151.0)**	**(35.3)**	**(35.9)**	**(36.4)**	**(37.0)**	**(144.6)**	**(145.8)**
Investing Cash Flow	**(9.9)**	**(3.9)**	**(1.3)**	**(0.6)**	**(1.3)**	**(1.1)**	**(0.4)**	**(0.1)**	**(0.4)**	**(0.1)**	**(0.9)**	**(0.4)**	**(0.1)**	**(0.4)**	**(0.1)**	**(1.0)**	**(1.0)**
Financing Cash Flow	**65.1**	**164.5**	**154.7**	**55.6**	**67.0**	**132.5**	**14.5**	**0.7**	**203.7**	**35.0**	**253.9**	**-**	**-**	**-**	**-**	**-**	**94.0**

Source: Leerink Partners LLC Equity Research and Company Filings

WVE DCF Analysis	2019	2020	2021	2022	2023	2024	2025E	2026E	2027E	2028E	2029E	2030E	2031E	2032E	2033E	2034E	2035E	TV
Cash Flow From Operations ($M)	(188.2)	(116.0)	(89.0)	(127.8)	(19.4)	(151.0)	(144.6)	(145.8)	5.6	116.2	232.7	360.7	493.5	622.7	753.6	898.2	1,053.3	
Cash Flow From Investing ($M)	(3.9)	(1.3)	(0.1)	(0.3)	(0.1)	(0.0)	(0.6)	(1.3)	(1.1)	(0.9)	(1.0)	(1.0)	(1.0)	(1.0)	(1.0)	(1.0)	(1.0)	
Free Cash Flow ($M)	(192.1)	(117.3)	(89.1)	(128.1)	(19.5)	(151.0)	(145.1)	(147.1)	4.5	115.3	231.7	359.7	492.5	621.7	752.6	897.2	1,052.3	8,944.8
Discount Periods	-	-	-	-	-	-	0.00	1.00	2.00	3.00	4.00	5.00	6.00	7.00	8.00	9.00	10.00	
NPV FCF ($M)	-	-	-	-	-	-	(145.1)	(129.0)	3.5	77.8	137.2	186.8	224.4	248.5	263.8	275.9	283.9	2,412.8

Sum NPV FCF ($M)	$3,840
Net Cash (4Q24)	$302
Implied WVE Mkt Cap ($M)	**$4,143**
WVE Per Share Value	**$26**

Cost of Equity	14%
Terminal Growth Rate	2%
Diluted Shares Outstanding (4Q24)	161.5

Source: Leerink Partners LLC Equity Research

Leerink Partners Catalyst Tracker

Stock (Ticker Symbol)	Lateral Impact (Other companies/ stocks)	Drug (Brand or chemical name) / Instrument / Area	Indication / Product Class	Type of Event	Event or Trial Details	Expected Timing	Specific Event Date if known or specified	Impact: H(igh) > 9% M(edium) 3 - 9% L(ow) < 2%	Estimated Stock Up/Down % on Best/Worst Outcomes	Leerink Partners View of Expected Outcome
WVE		WVE-003	Huntington's disease (HD)	IND Submission	Submit an IND for the potentially registrational study	2H25		M		Positive
WVE		WVE-007	Obesity	Phase 1 Data Announcement	Proof-of-concept data	2H25		H		Positive
WVE		WVE-006	Alpha-1-Antitrypsin Deficiency (AATD)	Phase 1 Data Announcement	Multiple dose data in AATD patients	2025		M		Positive
WVE			Liver disease / familial hypercholesterolemia	Lead Candidate Selection	Select RNA editing candidates to target PNPLA3 in liver disease and LDLR and APOB in familial hypercholesterolemia	2025		M		Positive
WVE			Liver disease / familial hypercholesterolemia	Other Event	Initiate clinical development for PNPLA3 in liver disease and LDLR and APOB in familial hypercholesterolemia	2026		M		Positive
WVE		WVE-N531	Duchenne muscular dystrophy (DMD)	NDA/BLA Filing	NDA filing for accelerated approval	2026		M		Positive
WVE			Duchenne muscular dystrophy (DMD)	NDA/BLA Filing	Multiple CTAs for additional exons (52, 51, 45, and 44)	2026		M		Positive

Source: Leerink Partners LLC Equity Research and Company Filings

DISCLOSURE APPENDIX

Completion: April 21, 2025 21:19 P.M. EDT.
Distribution: April 21, 2025 21:19 P.M. EDT.

Analyst Certification

I, Joseph P. Schwartz, certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

Distribution of Ratings/Investment Banking Services (IB) as of 03/31/25			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	219	73.2	104	47.5
HOLD [MP]	79	26.4	18	22.8
SELL [UP]	1	0.3	0	0

Explanation of Ratings

Outperform (Buy): We expect this stock to outperform its benchmark over the next 12 months.

Market Perform (Hold/Neutral): We expect this stock to perform in line with its benchmark over the next 12 months.

Underperform (Sell): We expect this stock to underperform its benchmark over the next 12 months.

The degree of outperformance or underperformance required to warrant an Outperform or an Underperform rating should be commensurate with the risk profile of the company.

For the purposes of these definitions the relevant benchmark for "Leerink Partners" branded healthcare and life sciences equity research will be the S&P 600® Health Care Index for issuers with a market capitalization of less than $2 billion and the S&P 500® Health Care Index for issuers with a market capitalization over $2 billion.

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