7/1/2020
Vishal Sethi – Stock Pitch

Recommendation: HOLD	Current Price (7/1/2020): $159.18	Target Price (Q1 2022): $195.19

Recommendation

Sarepta Therapeutics (SRPT) is a HOLD with a 23% upside to $195.19/share in July 2020 following positive phase 2/3 approval for SRP-9001 and a potential downside of -42% to $92.56/share following trial failure.

Investment Thesis Summary

- Sarepta's SRP-9001 is on track to be the first gene therapy available for DMD, out-competing all current treatments and applicable to the entire DMD population.
- After a recent licensing agreement with Roche, Sarepta is at a healthy cash position of $2.1B, with $1.7B due in licensing milestones.
- Sarepta has a strong pipeline with multiple indications, partnerships, and cash flow from two approved and one pending exon skipping DMD products.

Company Background

Sarepta Therapeutics (SRPT) is a biopharmaceutical company with a current EV of $11.2B involved in treating neuromuscular disorders, primarily Duchenne Muscular Dystrophy (DMD) and forms of Limb-Girdle Muscular Dystrophy (LGMD). They have two products on the market, Eteplirsen (EXONDYS 51) and Golodirsen (VYONDYS 53), both of which are antisense oligonucleotides which "skip" the mutation in the DNA "reading" process, restore the reading frame, and result in truncated yet partially functional dystrophin production. Sarepta's lead candidate in gene therapy is SRP-9001, an engineered micro-dystrophin delivered via an AAV vector serotype rh74 with a MHCK7 promoter specific to heart and skeletal muscles. This is a first-line therapeutic. I do not know to what extent SRP-9001 will diminish sales of EXONDYS 51 and VYONDYS 53.

Market Breakdown

DMD

Market value of $1.03B[5]. Sarepta is expected to be the first gene therapy available to DMD patients and capture up to 100% of existing treatable cases (boys aged 4-7 & ambulatory) as well as many newly diagnosed patients, adding $4B to their market cap[6]. Duchenne Muscular Dystrophy is a rare x-linked recessive genetic disorder only affecting males, which is characterizes by progressive muscle degeneration. Patients begin experiencing weakness around ages 3-5 and start declining in ambulation around age 7-8. Most are in wheelchairs by age 11, and without ventilator support die due to cardiac complications around 20 years old. With ventilator support, lifespan is extended about 8 years[11]. DMD has a prevalence of 15,000 US patients and 300,000 worldwide, with an incidence rate of 1 in 5000 births[1,2]. Current therapies include corticosteroids (Emflaza), exon-skipping drugs (Eteplirsen), and nonsense mutation drugs (Ataluren). These therapies fall short of being an effective long-term solution and/or may only apply to a subset of the population.

LGMD

Market size not currently assessed**.** LGMD is a type of muscular dystrophy, noted as being rarer than DMD and affecting the hip and shoulder muscles. While multiple companies are interested in LGMD, Sarepta can make quick advancements off SRP-9001s platform, and already have programs for all major mutations. There is a prevalence of 3.3, 3.5, 8.1, 2.0, 5.5 per million for E, D, B, C, and L mutations respectively, and an incidence rate of 1 in 14,500 births (up to 1 in 120,000), affecting both males and females[4].

As SRP-9001 is the primary candidate for Sarepta's near-term success, I will focus on it exclusively, including likelihood of trial success and impact on valuation.

Valuation

DCF

Upon successful completing of Phase II/III DMD trials using single-dose gene therapy, Sarepta Pharmaceuticals will have a 23% upside to $195.19/share. I used assumptions and price estimates based on similar approved products and guidance from Sarepta's CEO, as well as a risk adjusted NPV to derive a total valuation of ~$15.3 billion upon passing their pivotal Ph II/III trial. Market share and cost of market entry assumptions are based on historical first to market research reports, as well as looking at previously approved gene therapies and their market adoption. Assume that in 2021 Sarepta will pursue approval via an NDA submission consisting of multiple data readouts and have either accelerated approval or full approval by 2022. We will consider only U.S. patients as Sarepta has licensed ex-U.S. rights to Roche. Keep in mind that Sarepta is entitled to net sale royalties from Roche as well, so my estimates are conservative[15]. We also must consider that SRP-9001 will need to undergo more study to include a wider age range, patients with existing AAVrh74 antibodies, as well as individuals who are non-ambulatory.

DMD	2022	2023	2024	2025	2026	2027	2028	2029	2030	2031
Calendar Year	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6	Year 7	Year 8	Year 9	Year 10
Population boys age 4-7 (U.S.) (m)	8.22	8.22	8.23	8.23	8.23	8.24	8.24	8.25	8.25	8.26
Percent with DMD	0.02%	0.02%	0.02%	0.02%	0.02%	0.02%	0.02%	0.02%	0.02%	0.02%
Prevelent population	1,644	1,479	1,109	721	252	76	15	0	0	0
Market share of prevelent pop.	10%	25%	35%	65%	70%	80%	100%	100%	100%	100%
Treated prevelent pop.	164	370	388	469	177	61	15	0	0	0
Total treated existing cases	164	534	922	1,391	1,568	1,628	1,644	1,644	1,644	1,644
Clinically recognized births (m)	4.00	4.04	4.07	4.11	4.15	4.18	4.22	4.26	4.30	4.34
Addressable patient population (m)	2.04	2.12	2.14	2.16	2.18	2.20	2.22	2.24	2.26	2.28
New DMD cases	408	424	428	431	435	439	443	447	451	455
Market share of new cases	40%	50%	60%	60%	60%	60%	70%	70%	70%	70%
Price per patient (m)	2.50	2.50	2.50	2.50	2.50	2.50	2.50	2.50	2.50	2.50
Total Revenue	819	1,454	1,612	1,819	1,095	810	813	783	790	797
Marketing/Fixed Costs	15%	15%	15%	15%	15%	15%	15%	15%	15%	15%
COGS	15%	15%	15%	15%	10%	10%	10%	10%	10%	10%
Costs	246	436	484	546	274	203	203	196	197	199
EBIT	573	1,018	1,129	1,273	821	608	610	587	592	598
Tax	20%	20%	20%	20%	20%	20%	20%	20%	20%	20%
Net profit after Tax (m)	459	814	903	1019	657	486	488	470	474	478
NPV@10% discount rate (m)	4,001		**If Pass**		**If Fail**			**NPV (current)**		**PPS**
Chance of FDA Approval	70%		% change	**23%**	% change	**-42%**		EXONDYS 51	3,256	**$92.56**
Risk-adjusted NPV (m)	2,801		**If adjusted for $1.7B in milestone payments**					VYONDYS 53	2,009	**Current EV**
Total Shares Outstanding (m)	77.78		NPV@10% discount rate (m)			5,701		Casimersen	1,933	10,955
Additional PPS ($)	36.01		Risk-adjusted NPV (m)			3,991		Total NPV	7,199	**NPV % of EV**
Current PPS ($)	159.18		Additional PPS ($)			51.31	Upside Risk			65%
New PPS ($)	**195.19**		New PPS ($)			**210.49**	**32%**			

DCF Model Assumptions

Population boys age 4-7 (U.S.) – compiled from child pop. by single age data[27]. ~51% of children born under 18 are male. Population growth rate of 0.05% is used[22].

Percent with DMD – studies show roughly 0.02% of the population has DMD (incidence rate of 1/5000)[31].

Market Share of Prevalent Cases – SRP-9001 will be the first DMD gene therapy to market. We account for both patients who have AAVrh74 antibodies and don't undergo plasmapheresis or other procedures to remove existing immunity as well as patients who don't refuse gene therapy. With a target population of age 4-7 ambulatory, we estimate 20% will refuse gene therapy and 20% do not qualify immediately for gene therapy[25,30]. We estimate peak adoption at 70% in year 5 and increment up to 100% within 7 years.

Price – $2.5m based on guidance from Sarepta CEO implying a higher price than Zolgensma, a gene therapy for a similar indication with a similar market size priced at $2.125 million[14].

Clinically recognized births – 4 million births per year growing at 0.09%[11,12]

Market share of newly treated – It is assumed that Sarepta will have a higher adoption rate of newly diagnosed patients as it will likely be the first treatment in the DMD treatment algorithm (for new patients diagnosed around age 4). We begin with a first-year adoption of 40% scaled to 60% in three years.

COGS – Estimated at 15% due to the high production cost of gene therapy ($500,000-$1M)[33]. Estimated from Bay Bridge Bio's analysis of Zolgensma[34]. *More diligence may be needed*

Marketing/Fixed Costs – 15% estimated from general market budget for marketing/fixed costs as well as Bay Bridge Bio's Zolgensma analysis[34]. *More diligence may be needed*

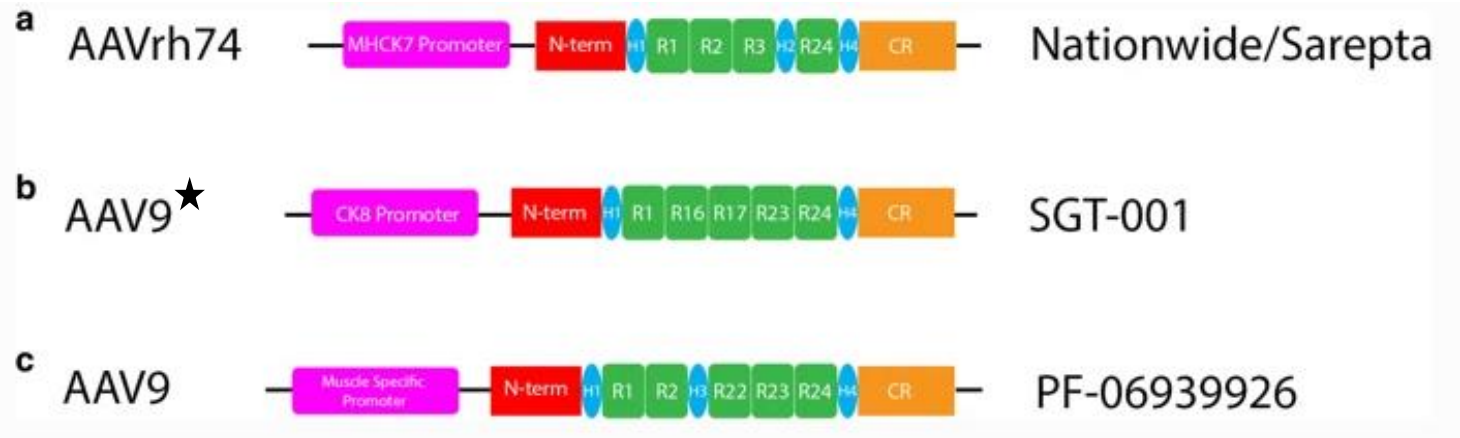
Risk-adjusted NPV – No literature on gene therapy probability of success; large molecule drugs have a 65.8% chance of Phase I to II[15]. Based on guidance from Sarepta's CEO, a combination of safety/efficacy (Study 101), Ph II top line data for functional benefits (Study 102), and expression results for a subset of Study 301 patients will lead to an NDA. Since their Ph III need not be finished before submission, we assume the FDA is more willing to grant approval as seen in Zolgensma. I've chosen a reasonable 70%.

Upside/Downside Risk –I calculated Sarepta's current NPV, consisting of their three exon skipping drugs. Assuming similar growth rates between the three drugs, their current NPV is roughly ~$7 billion, implying ~$5 billion of baked-in value for SRP-9001, or 35% of Sarepta's valuation.

Forecast Period – There is an international patent for SRP-9001 which was filed in 2017[35]. They are assumed to have 20 years of exclusivity from their IP. I have forecasted for 10 years.

Please note that while my numbers seem conservative (roughly ~4B NPV) we are ignoring $1.7B in milestone payments from Roche as well as net sale royalties from ex-U.S. sales, thus increasing the total NPV. If we add the additional $1.7B, my risk adjusted NPV is $4B, imputing a new PPS of $210.49, a 32% upside and 42% downside risk. This is not including net sale royalties from Roche OR the time value of money in terms to the $1.7B in milestone payments.

Comparable Company Analysis



**NOTE: SGT-001 no longer uses an AAV9 vector, but rather AAV-SLB101, a novel vector developed in-house*.

There are two other companies currently with gene therapies for DMD: Pfizer with PF-06939926 and Solid Biosciences with SGT-001. As seen above, the micro-dystrophin is different primarily in Solid's protein, as well as the vector.

Solid is currently are on clinical hold after a SAE (decreased RBCs/platelets/complement activation). The reason for this SAE is likely due to using the AAV9 capsid, as a patient in Pfizer's trial experienced similar complement activation, thus de-risking SRP-9001's competition. With Solid's new capsid, they still must lift their clinical hold in Q320 after submitting more info.

Pfizer recently announced data from their Ph Ib gene therapy drug in May 2020, and they are currently on hold from recruiting for their Ph III due to COVID-19, as the trial was supposed to begin in May 2020. However, this product will not be competing for market share for multiple reasons. First, it is much earlier in development than SRP-9001. Second, using the AAV9 capsid is disadvantageous to both Sarepta's and Solid's capsid, due to more seroprevalence in the population as well as a greater likelihood of side effects (Pfizer reported an individual with completement activation, like Solid Bio).

Therapies Currently in Development

- Italfarmaco is an Italian-based company working on a Histone deacetylase (HDAC) inhibitor for DMD. HDAC inhibitors are thought to promote muscle regeneration. It is currently recruiting for their Phase III.
- Catabasis Pharmaceuticals is developing Edasenolexent to slow disease progression by inhibiting NF-kB, a driver in inflammation and fibrosis exacerbated by muscle stress. They are currently due for a Ph III readout in June 2020.
- PTC Therapeutics currently markets Emflaza, a glucocorticoid used for DMD, as well as Ataluren/Translarna, a drug allowing read-through of stop-codons for treating nonsense mutations.
- Santhera Pharmaceuticals is seeking approval for two drugs dosed orally, Idebenone and Vamorolone, both designed to boost respiratory function through boosting ATP production in muscle cells.
- Roche abandoned their myostatin inhibitor drug after Ph III trials failed to meet their primary endpoint.
- Pfizer abandoned Domagrozumab (myostatin inhibitor as well) after failing a Ph II primary endpoint.

As seen above, other than two gene therapies being produced, there are no current therapies being developed to be "curative" of DMD but rather focused on treating symptoms or slowing disease progression. Gene therapies are an emerging and validated field of therapy, supported by currently approved drugs Luxterna by Spark and Zolgensma by Novartis/Avexis, among many others.

Near Term Catalysts

SRP-9001 lead gene therapy candidate Ph II due Q121:

Pivotal Readout: March 2021

NDA submission: 2H21

Commercial Supply Study: Commencing Mid 2020

Casimersen accelerated approval by end of 2020 (NDA submitted June 26th, 2020)

SRP-5051 data 2H20

Predicting

We will assess in this pitch the most recent report of both safety and efficacy data published in JAMA Neurology. Due to the nature of this disease and sentiment of the FDA, this is the "pivotal" trial as Study 103 will not be finished before their NDA submission. Keep in mind that this is contingent on the fact that Sarepta's CMC data comparing clinical to commercial supply must be accepted by the FDA as well.

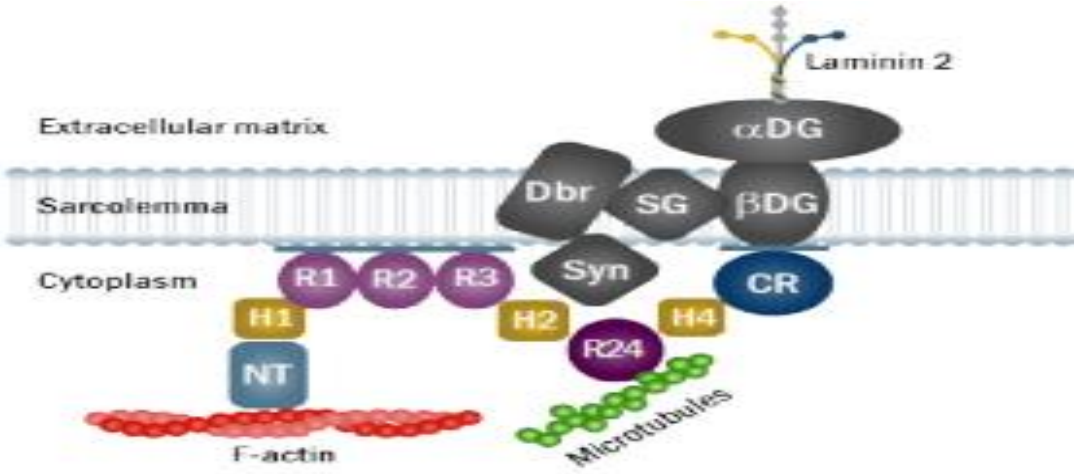
A combination of primarily safety and efficacy of Study 102, their Ph I/II study, and an interim update of Study 301, their multi-center (compared to Study 102's two-center) commercial study, along with expression data, functional data, and safety data, is enough to support an NDA submission by mid-2021 with a decision in Q421/Q122.

Methods

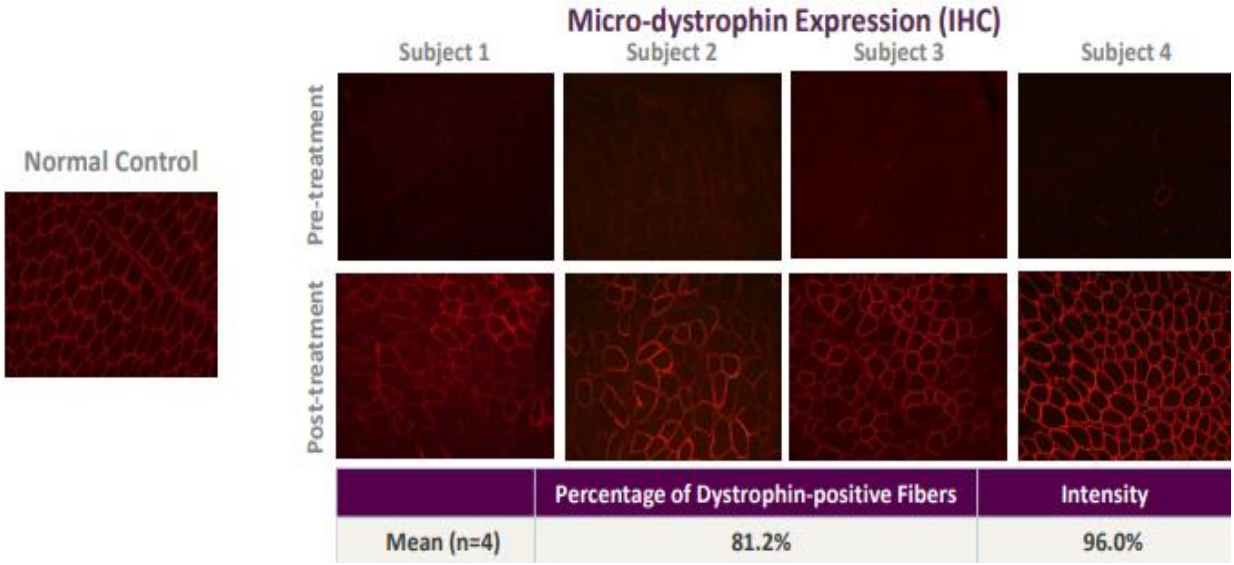
We will be using Novartis' Zolgensma (AVXS-101) when necessary, a recently approved gene therapy for spinal muscular atrophy (SMA), as reference for comparison due to multiple similarities. Both are spinal/muscular based drugs, aim to produce protein based on a transgene in vivo, use an AAV capsid, and have a similar market size.

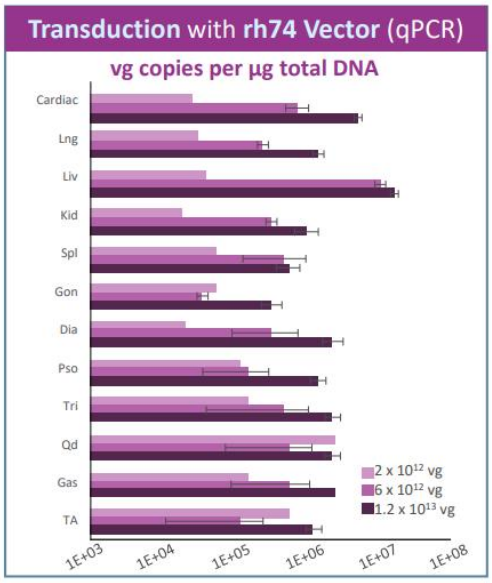
Clinical Profiles and Pharmacology

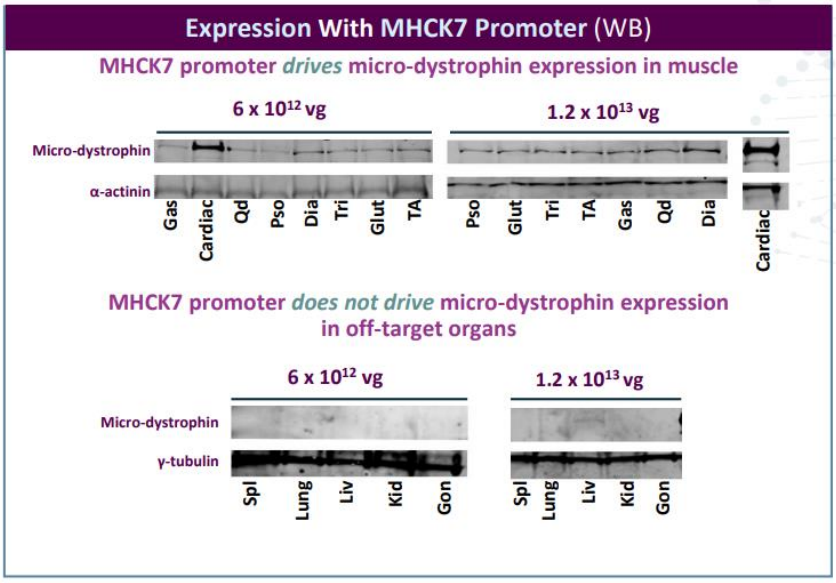
Structure



SRP-9001 consists of a transgene (micro-dystrophin), a promoter (MHC7 selective for skeletal and cardiac muscle), and an AAV vector (rh74). The reason for constructing a micro-dystrophin is that with AAV vector transmission, the AAV vector can only hold roughly ~4.7 kb of DND[21]. A full-length dystrophin is the biggest gene in the human body at 14 kb[21]. As such, micro-dystrophin allows for the essential components from the protein to be used and still fit into the AAV vector. The above picture illustrates how it supports the dystrophin-associated protein complex (DAPC) and mimics full-length dystrophin. The AAV vector is validated in SMA by Biogen (antisense oligonucleotide data) and Zolgensma by Novartis (uses an AAV9 capsid, SMN protein transgene, and chicken-β-actin hybrid promoter. SMN protein is small enough to fit into AAV9 without the need for truncation). The micro-dystrophin was constructed based on determining which hinges, subunits, and domains are necessary for good binding and expression. At 3.3 viral genomes per nucleus and a dose of 2x10^14 vg/kg, compared to the 1.12x10^14 vg/kg of Zolgensma[19], and ignoring fibrosis and fat tissue, there is an 81.2% in dystrophin-positive fibers. Keep in mind that it remains a challenge to accurately determine downstream dystrophin production in methods other than immunofluorescence staining. As well, dystrophin only comprises 0.002% of total muscle tissue[20], thus large amounts of protein need not be produced.







Potency/Toxicity

As seen in the image above, at double the dose there is still extra expression but a marginal amount. No toxicity was seen at any dose. We conclude that in reference to Zolgensma's dose (which was 2.2-fold lower than the toxicity level), Sarepta chose a dose at least 2x below their respective tox level[19]. Zolgensma saw more cardiac toxicity in doses of 7. 9x10^13 vg/kg and 1.5x10^14 vg/kg.

PK/PD

In attempt to analyze the PK/PD (or rather, Cellular Kinetics), we reference analysis done on Zolgensma. PK data showed vector shedding declined to undetectable levels within weeks with a spike in DNA found in stool in 1-2 weeks before declining. With an AAV9 capsid targeting the CNS, vector expression was found throughout the body. We can assume that for SRP-9001, viral shedding will be minimal. Preclinical studies showed systemic delivery of the drug as reduction correlated to dose-dependency, as well as no toxicity concerns, and up to 95% cardiac expression 6mo post-injection, which may alleviate concerns of biodistribution. Regarding human safety, proof-of-concept was shown in Study 101/102 via safe and efficacious data from four patients (9mo and 1yr data). As well, the data above shows that transduction with rh74 is effective in targeting muscle tissue and does not express in off-target organs at high doses.

Trial Overview

Sarepta has one trial completed, one trial commencing in March 2021, and one trial slated for mid-2020, denoted Study 101, 102, and 103 respectively. See below for comparison among competing gene therapy trials. Sarepta has not commenced their commercial supply trial yet, which is slated to be an age 4-7 multi-center, multi-country RCT. There is also another study planned for older + non-ambulatory patients to commence in 2020 as well.

Evaluating Sarepta's Study 102, top-line data shows good safety and tolerability, as well as secondary outcomes of expression. Common AEs included vomiting as well as elevated liver enzymes, helped by corticosteroids. Coupled with decreased CK levels (signaling reduced muscle damage) and functional improvement in NSAA scores (common benchmark for function outcomes)[8], SRP-9001 is very promising.

Sarepta's studies overall are cleaner. They don't seem to have issues with dosing during COVID-19 as mentioned during the BOFA Napa Health Conference in June 2020.

Solid Biosciences had a clinical hold due to a SAE of a patient having reduction in RBCs, transient renal impairment, and complement activation. Treated with steroids and Soliris (an immunosuppressive drug designed to treat other

indications), the patient recovered yet the IGNITE DMD study remains on hold. Earlier in the year, there was another patient with a GI infection coupled with elevated liver enzymes, bilirubin, and decreased platelets. Pfizer also had one of their first six patients suffered from complement activation, requiring an admit to the ICU and released after hemodialysis and dosed with a complement inhibitor. These SAEs pose hurdles for both Pfizer and Solid.

Trial Comparison

Company	Sarepta	Pfizer	Solid Biosciences
Study Name	Study 102	N/A	IGNITE DMD
Indication	DMD	DMD	DMD
Compound + Formulation	SRP-9001	PF-06939926	SGT-001
Trial Design	RCT, double-blind	RCT, double-blind	random, OLE, SAD
Phase	2	3	1|2
Start	12/22/2018	5/13/2020	12/6/2017
End	10/10/2022	4/13/2022	3/1/2023
NCT#	NCT03769116	NCT04281485	NCT03368742
Sample Size (n)	41	99	16
Primary Outcome	Incidence of SAEs, TEAEs, Change from Baseline in Quantity of Micro-dystrophin protein expression messed by western blot	Change from Baseline in NSAA	Change in baseline of micro-dystrophin in active treatment groups, AEs, abnormalities
Secondary Outcome	Change from Baseline in NSAA/Time to Rise from Floor/Ascend 4 steps/10m/100m	Change from Baseline in micro-dystrophin expression, distribution, CK, 10m, Rise from floor, PODCI	N/A
Exploratory/Functional Outcomes	N/A	N/A	N/A
Inclusion Criteria	Confirmed DMD, symptomatic, ability to do motor testing, on steroids for 12wk	Confirmed DMD, Steroids 3mo prior to screen, ambulatory	Confirmed DMD, confirmed absence of dystrophin, Anti-AAV9 antibodies below threshold, stable function, non-ambulatory by criteria, daily corticosteroids
Exclusion Criteria	impaired cardio function on ECHO, ongoing medical condition, exposure to another medication/gene therapy, abnormal liver/renal	Nabs to AAV9, exon-skipping/nonsense mutation suppression for 6mo, prior gene therapy, abnormalities	Ongoing abnormalities (labs or otherwise), impaired cardiovascular or respiratory function, BMI >95%, previous
Number of Sites/Countries	2; California, Ohio	N/A	2; Florida, Massachusetts
Results	1-year safety/tolerance published in JAMA Neurology	N/A	N/A
Interpretation	safe/tolerable; no SAEs on concern. Exploratory outcomes showed very impressive micro-dystrophin expression	N/A	N/A
Notes	later end date due to 96-wk extension; data will readout Q121	Two Cohorts; 2/3 get gene therapy for 1 yr. then placebo for 1 yr. 1/3 get reverse treatment.	control arm dropped after 4 patients; had two clinical holds
Clinicaltrials.gov Link	link	link	link

Comparison with Pfizer's and Solid Bio's gene therapy candidates

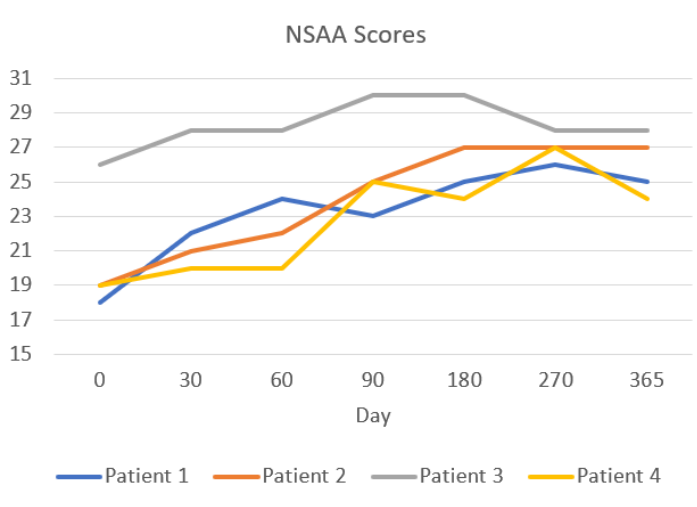
Reviewing the Science, we see that Solid includes the nNOS binding domain in their micro-dystrophin, which contains Hinge 1 and 4, and R1, R16/R17, and R23/24, leading to a much shorter protein than SRP-9001, which is more focused on force contraction and production. In a case study, a 61yo patient with Becker Muscular Dystrophy (BMD) had no nNOS domain yet remained ambulatory, validating the hypothesis that the nNOS domain is not necessary. Besides, it seems to be useful for high intensity exercise, which for DMD patients is only secondary to remaining ambulatory and fighting off muscle fibrosis. As well, R2/R3 are critical for force production, while R16/17 are not necessary as seen in 61yo man with BMD with R16/17 deletion. With Pfizer's gene therapy and Solid, use of AAV9 results in decreased platelets/RBCs and more importantly complement activation, thus Solid is exploring alternate capsids. However, note that Solid's western blots have absolutely no liver targeting (as shown on western blot), but inferior transgene expression even at high doses compared to SRP-9001. Solid doses at 2x10^14 vg/kg.

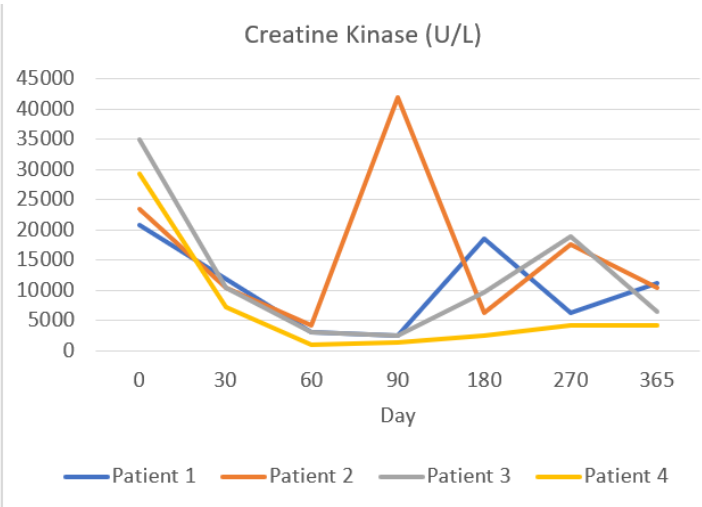
Functional Outcomes Assessment

As the only paper/study with published data is the result of Study 102, we will assess the functional outcomes of rAAVrh74.MHCK7.micro-dystrophin and see if it is effective[36]. In the study, there were four patients with a mean age of 4.8 (SD of 1.0), with a mean NSAA of 20.5 (SD of 3.7) and creatine kinase (CK) level of 27064.3 (SD of 6340.5) units/liter (U/L). For other baseline demographics, please refer to the paper[34]. We know there was a significant increase in dystrophin-positive fibers expressed as seen in immunofluorescence-stained images and on Western Blots, but this is meaningless if there is no improvement in functional outcomes which directly affect Quality-of-Life (QOL).

After one year, there was a 7 point, 8 point, 2 point, and 5 point increase in patients 1-4 NSAA scores respectively. Patient 3 was the oldest patient (at 6yo), and would have been expected to decline, thus their benefit is predicted to be greater than 2 points[34]. CK levels dropped to a mean 8035 U/L (for reference, normal reference values are 39-308 U/L[37]). In the paper, the authors noted the questionable reliability of measuring CK levels, as they vary greatly based on activity level and on days of assessment.

These charts show a general upward resulting in statistically significant improvements for NSAA scores and a downward trend in CK levels over time. Note that on Day 90, patient 2 had elevated CK levels (40,000+) but did not myoglobinuria, a result of elevated CK levels and muscle damage. The question remains that if these results will last, and more importantly how will this therapy affect non-ambulatory patients. If SRP-9001 can lengthen lifespan, delay changes in ambulation into the early or late teens, then there is clear tangible value in this therapy. Unfortunately, other functional outcomes like the 6-minute walk test (6MWT) were not reported, and it is likely Study 103 will focus on these outcomes as they most directly affect QoL for DMD patients.





Risks

One note to keep in mind is that this therapy only works in people with no previously existed antibodies to AAVrh74. In clinical trials, it is known that no test subject has existing AAVrh74 antibodies. However, due to seroprevalence in the population, albeit a lower percentage in kids aged 2-7, this is an issue that must be considered[17]. However, recently plasmapheresis has been studied to show that anti-AAV antibodies can be removed from human IgG pools, allowing vector administration without depleting the entire immunoglobulin makeup (in mice)[18]. As well, Genethon has a patent approved for a technique of removing patient's blood, attaching affinity ligands to all existing antibodies, and perfusing it back into the blood allowing for vector administration. This seems to be how Sarepta is managing this issue, as they are in a partnership with Genethon, the filing company. As well, Sarepta found 83% of test subjects seronegative for AAVrh74 in their LGMD and DMD populations[21], partly because of a nonhuman serotype. We will see how they address this issue in the coming future. Another risk is whether they can scale up their commercial supply up to GMP and meet their demand, considering both the current pandemic and manufacturing concerns. However, we de-risk this concern with their operational manufacturing plant and preparation, as well as their partnership with Thermo Fisher to produce at scale.

Other risks to consider include

- The ongoing discussion with payers regarding the expected cost of SRP-9001; Zolgensma worked out numerous negotiations and plans to facilitate payment with customers and we anticipate Sarepta will as well, given that their therapy is likely to cost more.

In this report, we did not assess

- the market size and potential of Sarepta's other pipeline programs, namely their LGMD programs. Although it is a smaller market-size, proof-of-concept with SRP-9001 will heavily boost the likelihood of their platform and its capability to treat other conditions (similar vector, promoter, delivery system, etc.)
- gross to net discount of SRP-9001 list price
- sensitivity analysis on DCF assumptions; variable costs as well as prevalent market captured % heavily influence the generated NPV. More diligence is needed.

Conclusion

I believe that SRPT is in the most advantageous position for bringing an effective and safe gene therapy to the DMD market first and will attain majority market share and capture most revenue. The micro-dystrophin cassette is better than Solid Biosciences, and their capsid better than Pfizer, as well as having cleaner toxicity profiles and clinical trials. However, the upside is not as favored as initially theorized, thus Sarepta is a recommended HOLD. Please note that all their LGMD gene therapy programs will be validated by their DMD success, so with additional read-through, Sarepta's success hinges on their ability to prove efficacy in a large population and execute on their distribution plan.

Appendix

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General Guidance on market model provided by Kenny Chan, PharmD.