UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): January 11, 2021

Sarepta Therapeutics, Inc.
(Exact name of Registrant as Specified in Its Charter)

Delaware	**001-14895**	**93-0797222**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

215 First Street
Suite 415
Cambridge, MA 02142
(Address of principal executive offices, including zip code)

Registrant's Telephone Number, Including Area Code: (617) 274-4000

Not Applicable
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, Par Value $0.0001 per share	SRPT	The Nasdaq Global Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 2.02 Results of Operations and Financial Condition.

On January 11, 2021, Douglas S. Ingram, President and Chief Executive Officer of Sarepta Therapeutics, Inc. (the "Company") disclosed certain preliminary financial information for the year ended December 31, 2020 during the Company's presentation at the 39th Annual J.P. Morgan Healthcare Conference (the "Conference") and in discussions with third parties at the Conference. Specifically, the Company disclosed its cash position of approximately $1.9 billion as of December 31, 2020 and that the Company generated approximately $122.6 million in revenue (unaudited) in the fourth quarter ended December 31, 2020, and approximately $456 million in revenue (unaudited) in the year ended December 31, 2020 from sales of EXONDYS 51® (eteplirsen) Injection and VYONDYS 53® (golodirsen) Injection. A copy of the slide presentation associated with this announcement is furnished as Exhibit 99.1 and is incorporated herein by reference.

The information in this Item 2.02 is unaudited and preliminary, and does not present all information necessary for an understanding of the Company's financial condition as of December 31, 2020 and its results of operations for the three months and year ended December 31, 2020. The audit of the Company's financial statements for the year ended December 31, 2020 is ongoing and could result in changes to the information in this Item 2.02.

Item 7.01. Regulation FD Disclosure.

The disclosure in Item 2.02 above is hereby incorporated by reference into this Item 7.01.

The slides presented by Mr. Ingram at the Conference on January 11, 2021 are furnished with this report as Exhibit 99.1, which is incorporated herein by reference.

The information in this report and Exhibit 99.1 to this report is furnished pursuant to Items 2.02 and 7.01 and shall not be deemed "filed" for the purposes of Section 18 of the Securities and Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section. It may only be incorporated by reference in another filing under the Exchange Act or the Securities Act of 1933, as amended, if such subsequent filing specifically references the information furnished pursuant to Items 2.02 and 7.01 of this report.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Sarepta Therapeutics, Inc. Presentation at the 39th Annual J.P. Morgan Healthcare Conference, dated January 11, 2021
104	The cover page from this Current Report on Form 8-K of Sarepta Therapeutics, Inc., formatted in Inline XBRL and included as Exhibit 101

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Sarepta Therapeutics, Inc.

Date: January 11, 2021

By: /s/ Douglas S. Ingram
Douglas S. Ingram
President and Chief Executive Officer

Exhibit 99.1

Our mission continues.

Armed with the most advanced science in genetic medicine, we are in a daily race to rescue lives otherwise stolen by rare disease.

At Sarepta, every day is another twenty-four hours to stand up for patients, advance technology, challenge convention, and ***drag tomorrow into today***.

DOUGLAS INGRAM
President and Chief Executive Officer

JP Morgan 39th Annual Healthcare Conference 2021
January 11, 2021

SAREPTA THERAPEUTICS

Forward-looking Statements

This presentation contains "forward-looking statements." Any statements that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believe," "anticipate," "plan," "expect," "will," "may," "intend," "prepare," "look," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to our pipeline and the potential of our programs to treat 1.5M patients; the potential benefits of our product candidates, including PPMO's potential to increase cell penetration, lead to more efficient dosing for patients and deliver PMOs to unique muscle types (e.g., heart); the prediction for significantly greater changes as we continue to dose escalate in SRP-5051-201; the estimated prevalence of DMD and potential market opportunities; the potential of our LGMD programs to generate a steady stream of gene therapy candidates in five additional subtypes which together represent more than 70% of all known LGMDs; the PDUFA date of 2/25/21 for AMONDYS 45 and the expected launch of AMONDYS 45; our revenue guidance of $537M – 547M in 2021; and plans and expected milestones, including continuing to advance Part 2 crossover phase of Study 102 and conducting biopsy at week 12 to assess expression and biological markers and longer-term assessments of functional outcomes, reporting biomarker and safety results from Study 103 in Q2 2021, leveraging learnings from Study 102 and Study 103 to inform future clinical development, including Study 301, announcing SRP-5051 data, advancing PPMO rare exons programs and expanding into other disease areas, completing GMP runs for LGMD2E, seeking FDA confirmation of pivotal trial study design for LGMD2E and launching pivotal trial, and advancing our gene editing programs and expanding our gene editing centers of excellence.

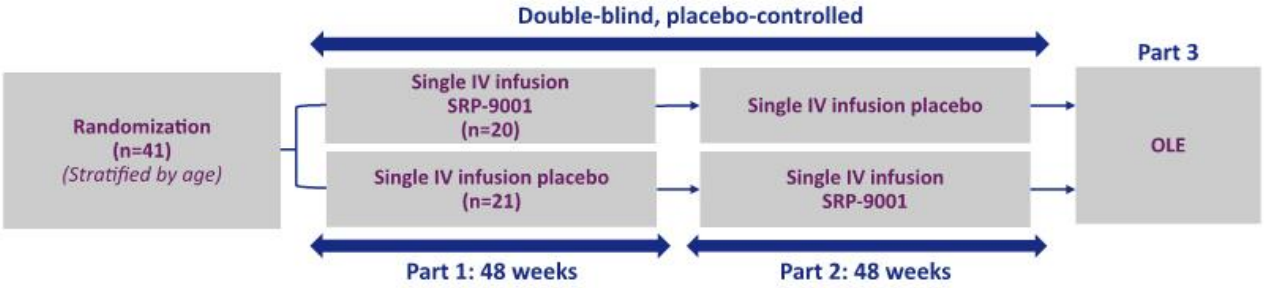
These forward-looking statements involve risks and uncertainties, many of which are beyond our control and are based on our current beliefs, expectations and assumptions regarding our business. Actual results and financial condition could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties and could materially and adversely affect our business, results of operations and trading price. Known risk factors include, among others: we may not be able to meet expectations with respect to sales of our products; success in preclinical and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; the data presented in this presentation may not be consistent with the final data set and analysis or result in an assessment that any of our product candidates provides a safe or effective treatment benefit; different methodologies or assumptions than we utilize to assess particular safety or efficacy parameters may yield different statistical results, and, even if we believe the data collected from clinical trials are positive, the data may not be sufficient to support approval by the FDA or foreign regulatory authorities; we may not be able to comply with all FDA post-approval commitments and requirements with respect to EXONDYS 51 and VYONDY 53 in a timely manner or at all; our data for our different programs, including casimersen, PPMO and gene therapy-based product candidates, may not be sufficient for obtaining regulatory approval; if the actual number of patients suffering from DMD and LGMD is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; various factors may decrease the market size of our product and product candidates, including the severity of the disease, patient demographics and the response of patients' immune systems to our product candidates; our dependence on our manufacturers to fulfill our needs for our clinical trials and commercial supply, including any failure on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet product demand, may impair the availability of products to successfully support various programs, including research and development and the potential commercialization of our gene therapy product candidates; we may not be able to execute on our business plans and goals, including meeting our expected or planned regulatory milestones and timelines, clinical development plans, and bringing our product candidates to market, due to a variety of reasons, many of which are outside of our control, including possible limitations on company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover our product candidates; the impact of the COVID-19 pandemic; and those risks identified under the heading "Risk Factors" in our most recent Annual Report on Form 10-K for the year ended December 31, 2019, and most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings we make, which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect the Company's business, results of operations and the trading price of Sarepta's common stock. For a detailed description of risks and uncertainties we face, we encourage you to review our SEC filings. We caution investors not to place considerable reliance on the forward-looking statements contained in this presentation. We undertake no obligation to update forward-looking statements based on events or circumstances after the date of this presentation.

Micro-dystrophin SRP-9001-102
Top-line Clinical Data (Part 1)

SRP-9001-102 Study Design: Parts 1 and 2

A randomized, double-blind, placebo-controlled clinical trial to evaluate the safety, efficacy and tolerability of a single dose of SRP-9001 compared to placebo, in boys with DMD aged 4–7 years old; Study is ongoing and remains blinded, functional results for all patients will be analyzed at 48 week timepoint



Primary endpoints

- Micro-dystrophin protein expression, from Baseline to Week 12, as measured by western blot
- Change in NSAA total score from Baseline to Week 48

Statistical Analysis Plan

- Stratified by age cohort (4-5 age group vs. 6-7 age group)
- Pre-specified analysis for age cohorts

Micro-dystrophin Protein Expression and Vector Genome Copies per Nucleus Achieved Endpoints in Part 1 of Study 102 (n=20, Week 12)

Micro-dystrophin Expression (Western Blot)

	Percentage of Normal
Mean (n=20)	28.1%

Micro-dystrophin Expression (IF)

	Intensity (% Normal)	Percentage of Dystrophin-positive Fibers
Mean (n=20)	63.7%	33.0%

Vector Genome Copy Number

	Copies per Nucleus
Mean (n=20)	1.56

Study 102 Placebo Crossover Patients Showed Similar Expression Results to Patients in Study 101

Micro-dystrophin Protein Expression Results: Study 102 Placebo Crossover Patients (n=11)

Micro-dystrophin Expression (Western Blot)

	% of Normal Expression Study 101 Mean (n=3*)	% of Normal Expression Study 102 – Part 1 Mean (n=20)	% of Normal Expression Study 102 – Part 2 Placebo Crossover Patients Mean (n=11)
	53.7%	28.1%	51.7%
Mean age at time of biopsy	5.79	6.58	7.14

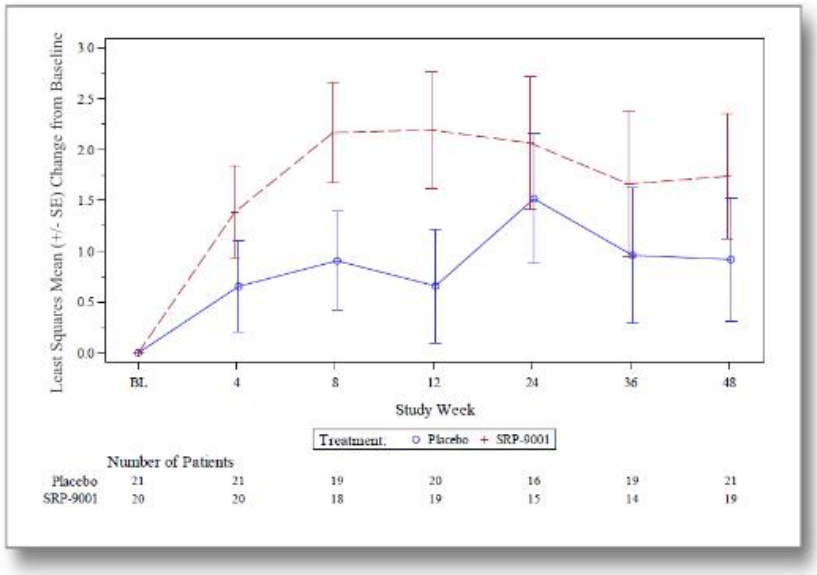
Vector Genome Copy Number

	Study 101 Mean (n=3*)	Study 102 Mean (n=20)	Study 102 Placebo Crossover Patients Mean (n=11)
	1.63	1.56	2.62

* Does not include 4th patient deemed to be an outlier

NSAA Primary Functional Endpoint: Treated Patients Outperformed Placebo Patients at All Time Points

NSAA change from baseline of +1.7 in SRP-9001 treated group vs. +0.9 in placebo group, which is not statistically different (p= 0.37)



- Separation shown at every timepoint between SRP-9001 and placebo groups
- Baseline analysis at 48 weeks:
  - Treatment group showed 1.7-point increase compared to baseline (P=0.0090)
  - Placebo group showed 0.9-point increase compared to baseline (P=0.1411)

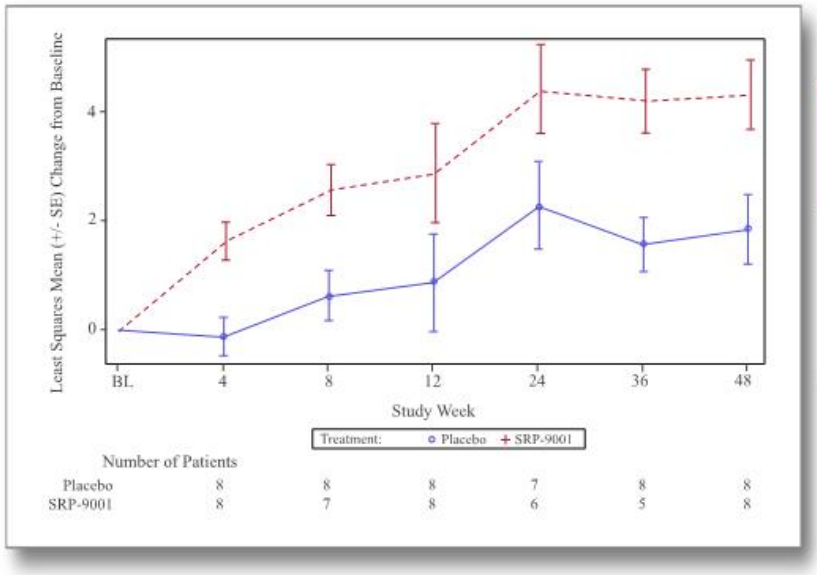
Functional Measures Well Matched at Baseline (4-5 Year Old Group)

Characteristic	Statistics	SRP-9001 Age 4-5 (n=8)	Placebo Age 4-5 (n=8)
NSAA	Mean *P-value(vs Placebo)*	20.1 ***0.8318***	20.4
100 meter (seconds)	Mean *P-value(vs Placebo)*	58.76 ***0.7925***	59.79
Ascend 4 Steps (seconds)	Mean *P-value(vs Placebo)*	3.46 ***0.9822***	3.48
Time to Rise (seconds)	Mean *P-value(vs Placebo)*	3.89 ***0.7421***	3.76
10 meter (seconds)	Mean *P-value(vs Placebo)*	5.01 ***0.5832***	5.24

Pre-specified NSAA Subgroup Analysis (Ages 4-5): Reached Statistical Significance

The 4- to 5-year old group had a statistically significant improvement in NSAA vs. placebo group at week 48

NSAA change from baseline of +4.3 in SRP-9001 treated 4–5-year-olds vs. 1.9 in placebo (p= 0.0172); age was a stratification factor at randomization



Measure	Treatment		Age 4-5 yrs	
		Baseline	LSM Change (SE)	P-value
NSAA	SRP-9001	20.1	***4.3 (0.6)***	<0.0001
	PBO	20.4	1.9 (0.6)	0.0126
	SRP-9001 vs PBO		***2.5 (0.9)***	***0.0172***

Functional Measures Not Well Matched at Baseline (6-7 Year Old Group)

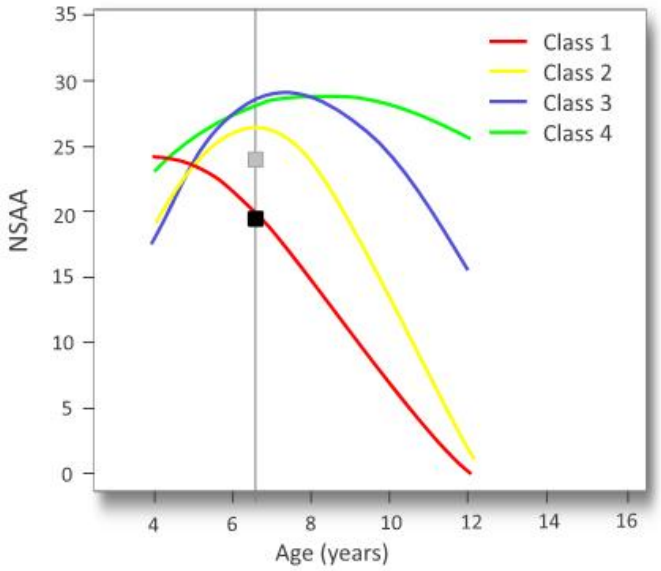
Patients in the treated group had significantly lower NSAA scores at baseline

Characteristic	Statistics	SRP-9001 Age 6-7 (n=12)	Placebo Age 6-7 (n=13)	Difference (from Placebo)
NSAA	Mean ***P-value(vs Placebo)***	19.6 ***0.0046***	24.0	***- 4.4***
100 meter (seconds)	Mean ***P-value(vs Placebo)***	62.56 ***0.0219***	50.21	***+ 12.35***
Ascend 4 Steps (seconds)	Mean ***P-value(vs Placebo)***	3.83 ***0.0958***	2.86	***+ 0.97***
Time to Rise (seconds)	Mean ***P-value(vs Placebo)***	5.91 ***0.0053***	3.44	***+ 2.47***
10 meter (seconds)	Mean ***P-value(vs Placebo)***	5.58 ***0.0313***	4.58	***+ 1.00***

Note that an imbalance in NSAA and timed tests exist in the older (6–7-year-olds) between the two groups with the treated group worse than placebo.

Differences in Baseline NSAA Scores Have a Significant Impact on Disease Progression

NSAA Natural History Data



Age and baseline NSAA are predictors of disease progression

■ Mean Baseline NSAA Score for 9001 Treated Patients (6-7 year old group)

■ Mean Baseline NSAA Score for Placebo Patients (6-7 year old group)

Source: Muntoni F, Domingos J, Manzur AY, et al. Categorising trajectories and individual item changes of the North Star Ambulatory Assessment in patients with Duchenne muscular dystrophy. PLoS ONE. September 2019.14(9): e0221097.

Safety Summary

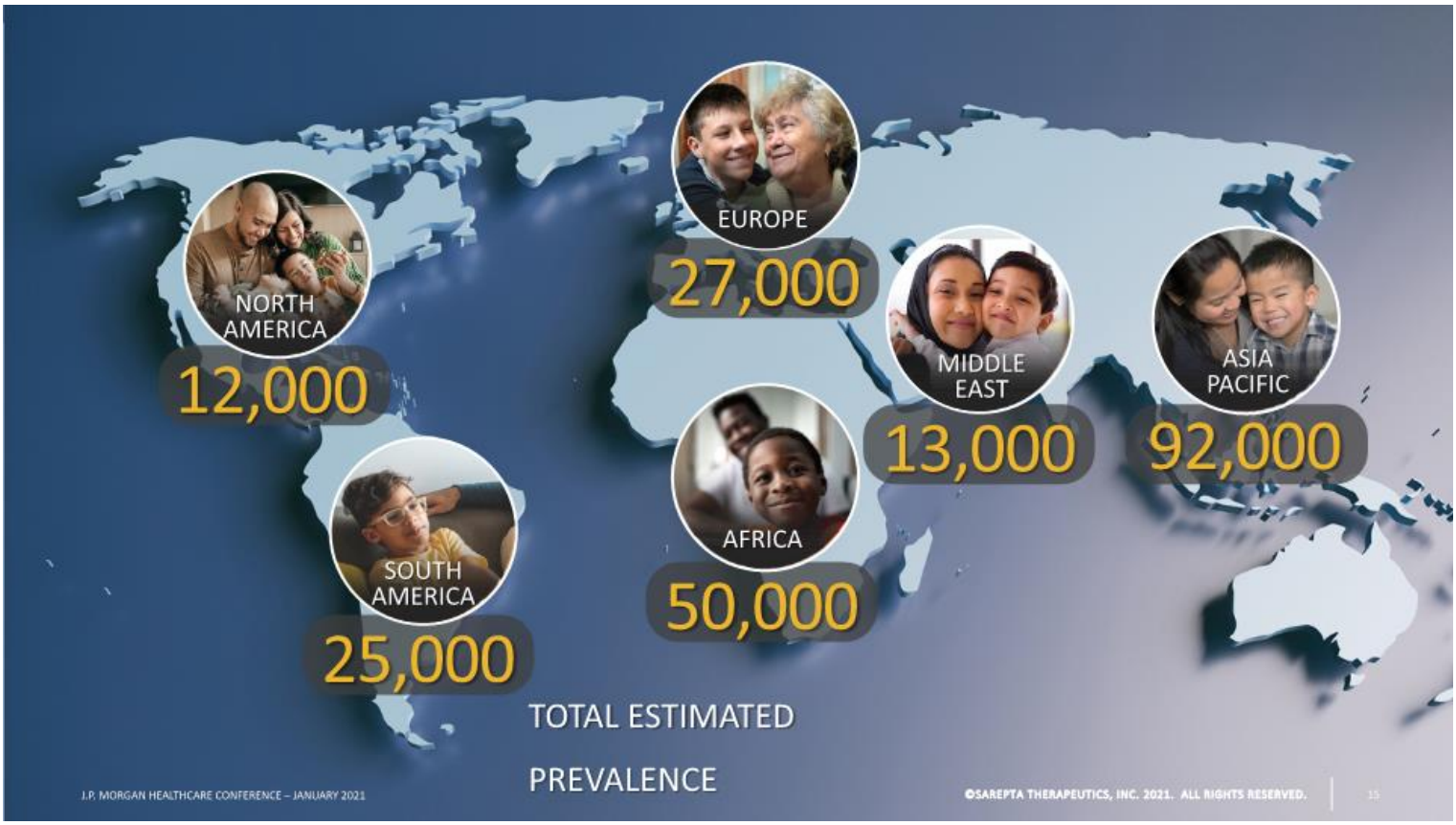
- No new safety signals
- Safe and well tolerated; consistent with previous studies
- 85% of the treated group had treatment related adverse events vs. 43% in the placebo group
  - The most common treatment related adverse event was vomiting
    - 60% (12/20) in treatment group vs. 19% (4/21) in placebo group
- Among patients with treatment-related AEs 82% were mild or moderate in severity
- Total of 4 patients with 5 treatment related SAEs
  - 4 SAEs in the treated group and 1 in the placebo group
    - Musculoskeletal: 3 rhabdomyolysis (2 in 9001 group and 1 in placebo)
    - Hepatobiliary/Investigations: 2 transaminases increased in 9001 group
- No adverse event related discontinuations and no deaths
- No clinical complement activation observed

Conclusions

- No new safety signals observed
- Primary biological endpoint (micro-dystrophin expression at 12 weeks post-treatment) achieved
- Total NSAA score of treated patients vs. placebo demonstrated a positive increase at all post-treatment time points
  - The study did not achieve a statistical significance on the primary functional endpoint of improvement in total NSAA score compared to placebo at 48 weeks post-treatment
- Pre-specified analysis in the 4- to 5-year old group showed a significant improvement in NSAA vs. placebo group at 48 weeks
- Imbalance in baseline functional characteristics in the 6-to 7-year old group contributed to the lack of statistical significance on the functional endpoint
- Data support future clinical development plans

Next Steps

- Continue to advance Part 2 crossover phase; conduct biopsy at week 12 to assess expression and biological markers and longer-term assessments of functional outcomes
- Enrolled and dosed 11 patients in Study 103 using commercial process material
  - Report biomarker and safety results in Q2 2021
- Leverage learnings from Study 102 and Study 103 to inform future clinical development, including Study 301


NORTH AMERICA
12,000
EUROPE
27,000
MIDDLE EAST
13,000
ASIA PACIFIC
92,000
SOUTH AMERICA
25,000
AFRICA
50,000
TOTAL ESTIMATED
PREVALENCE
J.P. MORGAN HEALTHCARE CONFERENCE – JANUARY 2021
©SAREPTA THERAPEUTICS, INC. 2021. ALL RIGHTS RESERVED.
15


EUROPE
NORTH AMERICA
MIDDLE EAST
ASIA PACIFIC
AFRICA
SOUTH AMERICA
PATIENTS LIVING WITH DMD TODAY*: >200,000 globally
* Estimated global prevalence
J.P. MORGAN HEALTHCARE CONFERENCE – JANUARY 2021
©SAREPTA THERAPEUTICS, INC. 2021. ALL RIGHTS RESERVED.

RNA Franchise

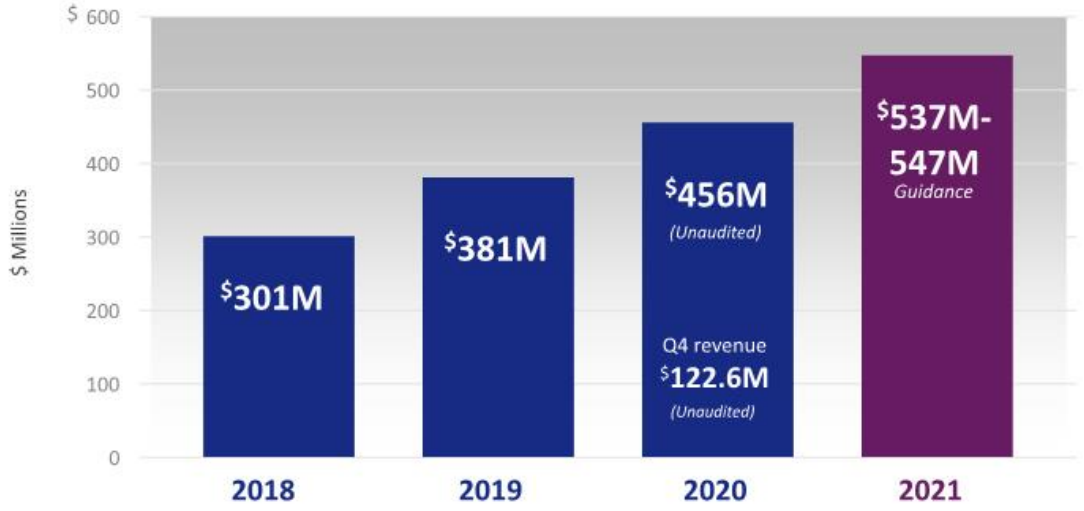
PMO and PPMO for Duchenne

PMO Franchise Revenue and Guidance

EXONDYS 51 (eteplirsen) Injection

VYONDYS 53 (golodirsen) Injection

AMONDYS 45 (casimersen) Injection | PDUFA DATE: FEBRUARY 2021



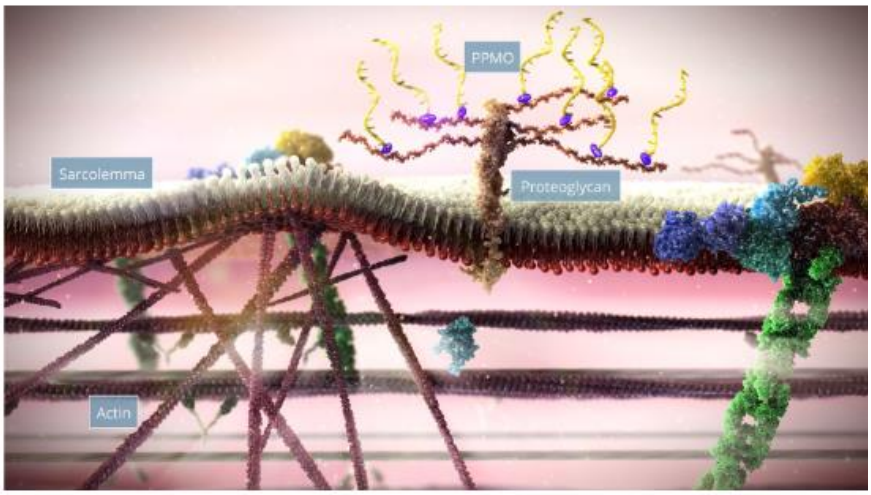
$1.9B in cash on 12/31/20

Sarepta's PPMO Next-generation Technology

Peptide phosphorodiamidate morpholino oligomer (PPMO) technology

Enhances PMO

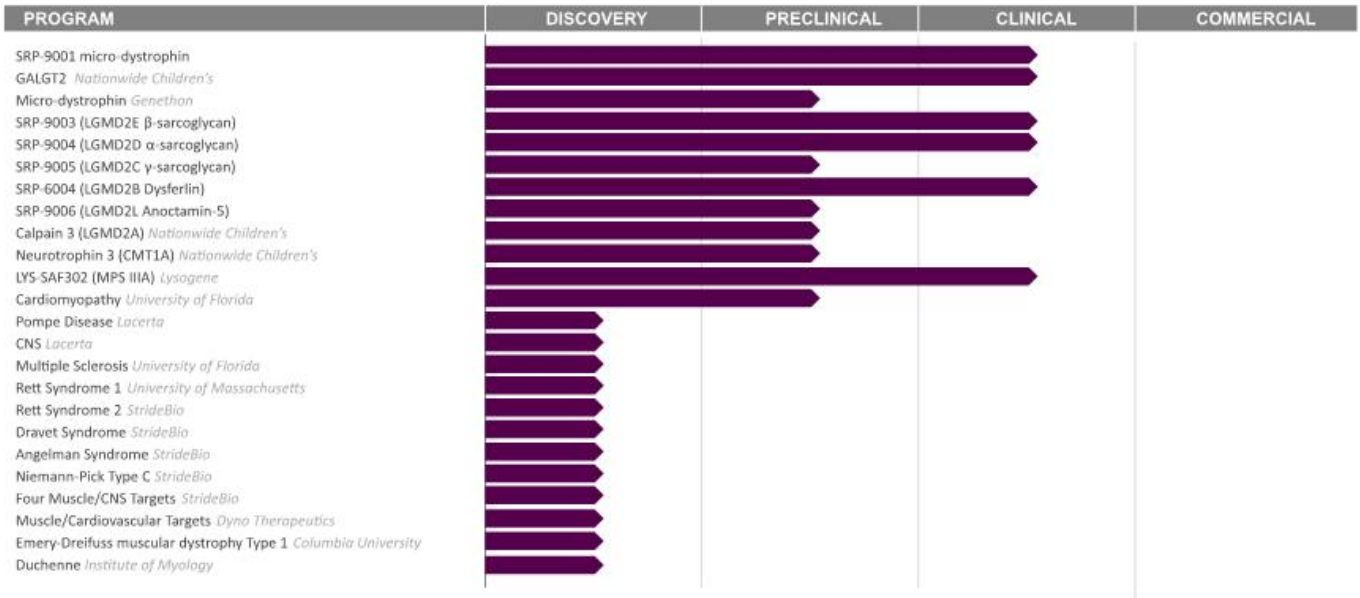
- Conjugated peptide greatly increases cell penetration
- Could potentially lead to more efficient dosing for patients
- Able to deliver PMOs to unique muscle types (e.g., heart)



Gene Therapy

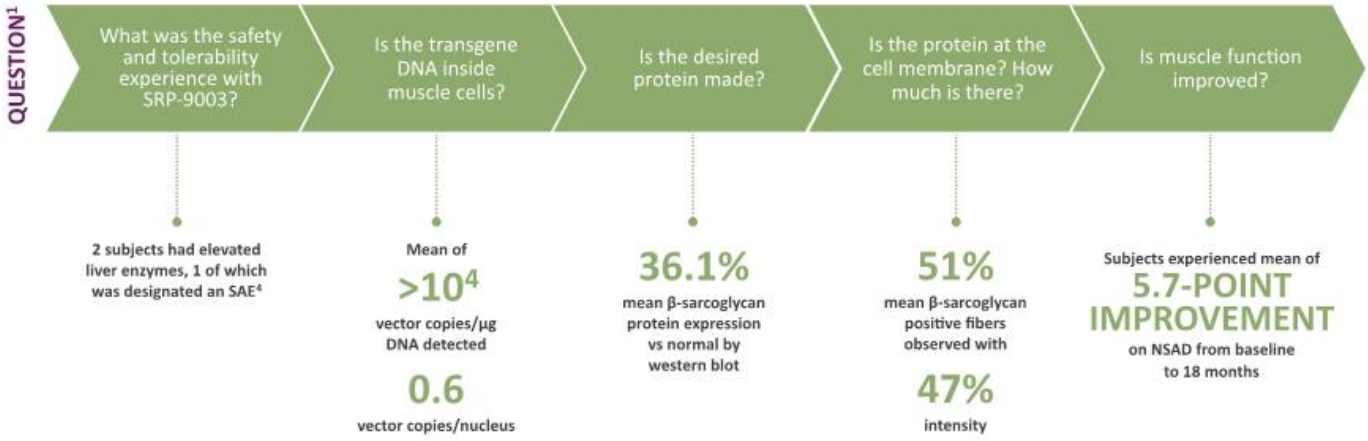
LGMD Portfolio

Sarepta's Gene Therapy Pipeline



All pipeline compounds are undergoing clinical trial investigation.

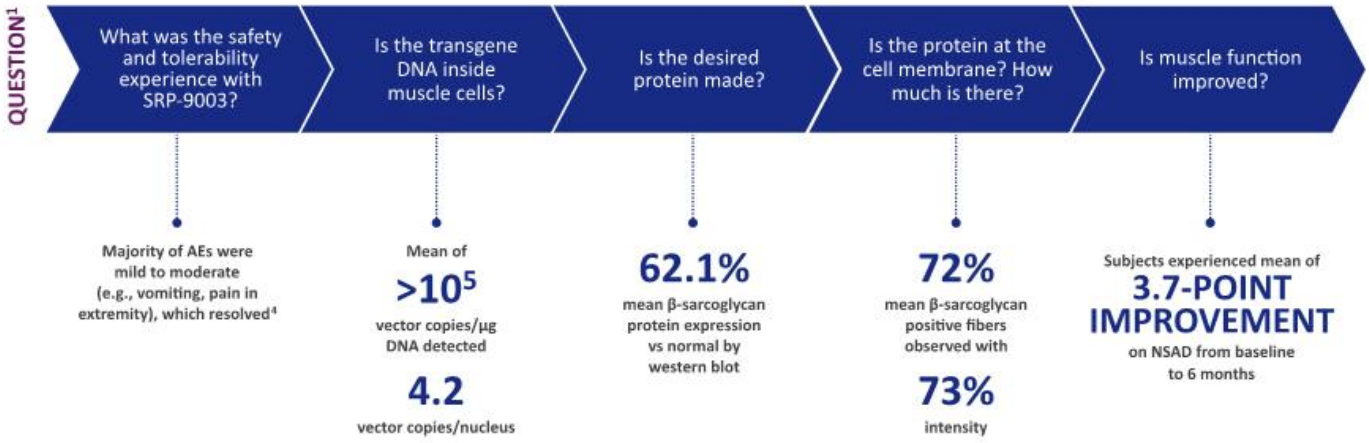
Clinical Results to Date: SRP-9003-101 (N=3)*, Cohort 1 (0.5 x 10^{14} vg/kg)[2,3]



*ClinicalTrials.gov Identifier: NCT03652259.

1. Asher DR, et al. Expert Opin Biol Ther. 2020;20(3):263-274.
2. September 28, 2020. https://investorrelations.sarepta.com/news-releases/news-release-details/sarepta-therapeutics-investigational-gene-therapy-srp-9003 .
3. Rodino-Klapac L, et al. Systemic Gene Transfer with rAAVrh74.MHCK7.SGCB Increased β-sarcoglycan Expression in Patients with Limb Girdle Muscular Dystrophy Type 2E (LGMD2E). WMS 2020.
4. Safety:
   - 1 patient experienced mild vomiting, which resolved 1 day without treatment
   - No other clinically significant laboratory findings
     - No decreases in platelet counts observed outside of the normal range
     - No clinical sequelae associated with complement activation

Clinical Results to Date: SRP-9003-101 (N=3)*, Cohort 2 (2.0 x 10^{14} vg/kg)[2,3]



*ClinicalTrials.gov Identifier: NCT03652259.

1. Asher DR, et al. Expert Opin Biol Ther. 2020;20(3):263-274.
2. September 28, 2020, https://investorrelations.sarepta.com/news-releases/news-release-details/sarepta-therapeutics-investigational-gene-therapy-srp-9003
3. Rodino-Klapac L, et al. Systemic Gene Transfer with rAAVrh74.MHCK7.SGCB Increased β-sarcoglycan Expression in Patients with Limb Girdle Muscular Dystrophy Type 2E (LGMD2E). WMS 2020.
4. Safety:
   - 1 treatment-related SAE observed
   - 1 patient had mildly elevated GGT
   - No stopping/discontinuation rules were triggered by AEs
   - No other clinically significant laboratory findings
     - No decreases in platelet counts observed outside of the normal range
     - No clinical sequelae associated with complement activation

Next Indications: LGMD Types 2C and 2D

OPPORTUNITY TO GENERATE A STEADY STREAM OF GENE THERAPY CANDIDATES IN FIVE ADDITIONAL SUBTYPES WHICH TOGETHER REPRESENT MORE THAN 70% OF ALL KNOWN LGMDS[1]

LGMD2D **LGMD2C** LGMD2B LGMD2L LGMD2A*

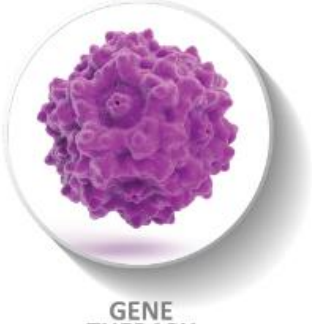
*This program is in collaboration with Dr. Zarife Sahenk at Nationwide Children's Hospital.

1. Taghizadeh E, Rezaee M et al. Prevalence, pathological mechanisms, and genetic basis of limb-girdle muscular dystrophies: A review. J Cell Physiol. 2019;234(6):7874-7884.

Sarepta's Pipeline

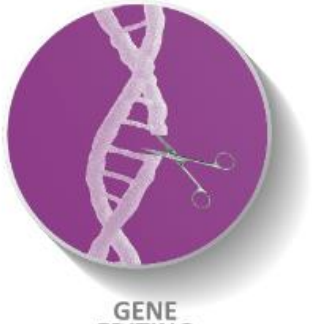
RNA

14
PROGRAMS



GENE THERAPY

27
PROGRAMS



GENE EDITING

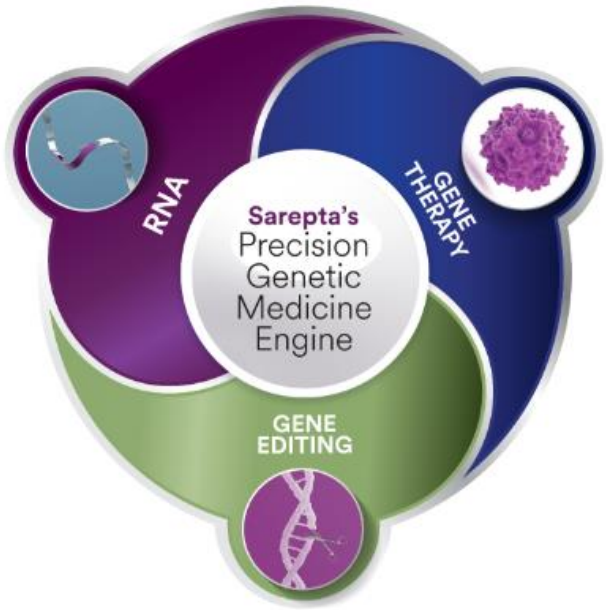
2
PROGRAMS

More than 40 programs in all

Potential to treat 1.5M patients*

*Based on published epidemiology.

Sarepta's Multi-Platform Genetic Medicine Engine – Upcoming Milestones



GENE THERAPY

Duchenne (SRP-9001)

- Continue to advance Part 2 crossover phase; conduct biopsy at week 12 to assess expression and biological markers and longer-term assessments of functional outcomes
- Enrolled and dosed 11 patients in Study 103 using commercial process material
  - Report biomarker and safety results in Q2 2021
- Leverage learnings from Study 102 and Study 103 to inform future clinical development, including Study 301

Limb-girdle muscular dystrophy (LGMD)

- SRP-9003 Type 2E
  - Complete GMP runs
  - Seek FDA confirmation - pivotal trial study design
  - Launch pivotal trial
- LGMDs portfolio path forward

RNA

Duchenne (PMO)

- EXONDYS 51® and VYONDYS 53® revenue growth
- AMONDYS 45™ approval (PDUFA date: 2/25/21) and launch

Duchenne (PPMO)

- Announce SRP-5051 data
- Advance rare exons programs
- Expand into other disease areas

GENE EDITING

- Advance programs
- Expand Centers of Excellence

#DraggingTomorrowIntoToday