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 7, 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 7.01. Regulation FD Disclosure.

On January 7, 2021, Sarepta Therapeutics, Inc. (the "Company") issued a press release and conducted an investor webcast presenting results from part one of Study 102 evaluating SRP-9001, the Company's investigational gene therapy for the treatment of Duchenne muscular dystrophy. Copies of the press release and the presentation are being furnished as Exhibits 99.1 and 99.2, respectively.

The information in this report furnished pursuant to Item 7.01, including Exhibits 99.1 and 99.2 attached hereto, shall not be deemed "filed" for the purposes of Section 18 of the Securities 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 Item 7.01 of this report.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press release dated January 7, 2021: Sarepta Therapeutics Announces Topline Results for Part 1 of Study 102 Evaluating SRP-9001, its Investigational Gene Therapy for the Treatment of Duchenne Muscular Dystrophy
99.2	Presentation dated January 7, 2021: Micro-dystrophin SRP-9001-102 Top-line Clinical Data (Part One)
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 7, 2021

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

Sarepta Therapeutics Announces Top-line Results for Part 1 of Study 102 Evaluating SRP-9001, its Investigational Gene Therapy for the Treatment of Duchenne Muscular Dystrophy

-- Study met the primary biological endpoint of micro-dystrophin protein expression at 12 weeks post-treatment, as measured by western blot, in SRP-9001-treated participants versus placebo --

-- SRP-9001-treated participants showed an increase in NSAA total score compared to placebo at 48 weeks; however, the study did not achieve statistical significance on the primary functional endpoint of improvement in NSAA total score compared to placebo at 48 weeks post-treatment --

-- In the pre-specified analysis by age-group, by which the randomization was stratified, participants aged 4-5 years at time of treatment with SRP-9001 demonstrated a statistically significant improvement in NSAA total score versus the age-matched placebo cohort, achieving a 4.3-point improvement on NSAA at 48 weeks post-treatment from baseline --

-- No new safety signals identified for SRP-9001, reinforcing the favorable safety profile observed to date --

-- Sarepta to host conference call at 4:30 p.m. Eastern time --

CAMBRIDGE, Mass., Jan. 7, 2021 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced top-line results from Part 1 of Study SRP-9001-102 (Study 102), an ongoing, randomized, double-blind, placebo-controlled clinical trial to evaluate the safety, efficacy and tolerability of a single dose of SRP-9001 (rAAVrh74.MHCK7.micro-dystrophin) in 41 patients with Duchenne muscular dystrophy. SRP-9001 is an investigational gene transfer therapy intended to deliver its micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein.

At 12 weeks post-treatment compared to baseline, the study met its primary biological endpoint of micro-dystrophin protein expression ($P<0.0001$). Participants who received SRP-9001 (n=20) had mean micro-dystrophin expression of 28.1%, as measured by western blot. Accompanying secondary biological endpoints including vector genome copies per nucleus, percent positive fibers, intensity, and reduction in creatine kinase (exploratory) were also met.

In the primary functional endpoint, SRP-9001-treated participants showed an increase in NSAA total score compared to placebo at 48 weeks; however, the difference was not statistically significant (P=0.37). At every time point measured, the cohort of SRP-9001 treated participants outperformed the placebo group, and, at 48 weeks, participants in the treatment group demonstrated a statistically significant increase of 1.7 points in NSAA total score compared to baseline (P=0.009), while participants in the placebo group saw an increase of 0.9 points on the NSAA total score compared to baseline, which was not statistically significant (n=21, P=0.1411).

Study randomization was stratified by age group and, in the pre-specified analysis of participants aged 4-5 (n=16) at the time of treatment, the treatment group demonstrated a statistically significant 4.3-point improvement on NSAA total score at 48 weeks post treatment compared to a 1.9-point improvement in the age-matched placebo group (P=0.0172). The functional status at baseline for participants in the 4-5 age group was balanced across the placebo and treatment cohorts. A statistically significant imbalance (P=0.0046) in baseline NSAA total score was present in the cohort of 6-7-year-old participants (n=25), resulting in milder participants in the placebo arm (n=13) than in the treated arm (n=12). The significantly different baseline characteristics between treatment and control groups in the 6-7 age group may have contributed to the inability to observe a treatment effect in the 6-7 age group at the week 48 timepoint in Part 1.

The results from Study 102 reinforce the favorable safety and tolerability profile of SRP-9001 with no new safety signals identified. In line with previously reported clinical data, no clinical complement activation was observed. 85% of participants in the treatment group experienced at least one treatment-related adverse event compared to 43% in the placebo group. Among participants with treatment-related adverse events, 82% were mild or moderate in severity, and 4 participants experienced serious treatment-related adverse events including 3 participants in the treatment group (2 cases of rhabdomyolysis, 2 transaminase elevations) and 1 participant in the placebo (rhabdomyolysis).

Study 102 is ongoing and remains blinded to participants, investigators, site staff and sponsor staff with direct site interaction. All 41 participants have completed their Part 1, 48-week assessment and have entered the Part 2 crossover phase. Participants continue to be monitored for safety and will undergo another biopsy at week 12 in Part 2 to assess expression and biological markers, in addition to longer-term assessments of functional outcomes.

“Study 102 reinforces our confidence in the potentially transformative benefits of SRP-9001, including among other things, the fact that in the Study’s pre-specified analysis, the participants in the 4-5 age group robustly achieved a statistically significant and clinically meaningful improvement in NSAA over placebo, as predicted by our prior Study 101. For the entire population, while we saw separation at every time point between the active and placebo cohorts, Study 102 did not achieve statistical significance on the primary functional endpoint. In this regard, we are very disappointed that the randomization process resulted in a significant imbalance in baseline NSAA scores between the active and placebo cohorts of the participants ages 6-7, making the 6-7 age groups non-comparable and likely substantially contributing to the inability to achieve statistical significance,” said Doug Ingram, president and chief executive officer, Sarepta. “Study 102 remains blinded and we will analyze the functional results for all patients, including cross-over participants, once they have achieved the 48-week timepoint in Part 2. We have already enrolled and dosed 11 participants in Study 103, using our commercial process material, and we will have biomarker and safety results from that cohort in the second quarter. And very importantly, Study 102 has provided us with a wealth of information and insight which we will use to refine and complete the protocol for our upcoming trial using commercial process material. We intend to continue to move forward with diligence and urgency to generate the evidence necessary to bring SRP-9001 to waiting Duchenne patients around the world.”

Sarepta will host an investor webcast and conference call on Thursday, Jan. 7, 2021 at 4:30 pm Eastern Time, to discuss these results. The presentation will be webcast live under the investor relations section of Sarepta's website at https://investorrelations.sarepta.com/events-presentations and slides will be archived there following the call for one year. Please connect to Sarepta's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary. The conference call may be accessed by dialing (844) 534-7313 for domestic callers and (574) 990-1451 for international callers. The passcode for the call is 2538387. Please specify to the operator that you would like to join the "Micro-dystrophin SRP-9001 Study 102 Top-line Results Call."

*The NSAA is a 17-item rating scale that is used to measure functional motor abilities in ambulant children with Duchenne. It is used to monitor the progression of the disease and treatment effects which makes it suitable as an endpoint in clinical trials for Duchenne.

About SRP-9001-102

Study SRP-9001-102 (Study 102) is a double-blind, 1:1 randomized, placebo-controlled clinical trial of SRP-9001 in 41 participants with Duchenne muscular dystrophy between the ages of 4-7. Study 102 uses clinical process SRP-9001 and has two primary endpoints: micro-dystrophin expression at 12 weeks and change in NSAA total score at 48 weeks compared to placebo. Secondary endpoints include certain timed functional tests; micro-dystrophin expression measured by immunofluorescence (IF) fiber intensity; and micro-dystrophin expression measured by IF percent dystrophin positive fibers. In Part 1, results from the treatment and placebo groups are compared through 48 weeks following treatment. In Part 2, the study remains blinded while all participants in the placebo group cross over to active treatment and all participants are followed for another 48 weeks while safety and efficacy continue to be evaluated.

About SRP-9001 (rAAVrh74.MHCK7.micro-dystrophin)

SRP-9001 is an investigational gene transfer therapy intended to deliver the micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein. Sarepta is responsible for global development and manufacturing for SRP-9001 and plans to commercialize SRP-9001 in the United States upon receiving FDA approval. In December 2019, the Company announced a licensing agreement granting Roche the exclusive right to launch and commercialize SRP-9001 outside the United States. Sarepta has exclusive rights to the micro-dystrophin gene therapy program initially developed at the Abigail Wexner Research Institute at Nationwide Children's Hospital.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a rare, fatal neuromuscular genetic disease that occurs in approximately one in every 3,500-5,000 males worldwide. DMD is caused by a change or mutation in the gene that encodes instructions for dystrophin. Symptoms of DMD usually appear in infants and toddlers. Affected children may experience developmental delays such as difficulty in walking, climbing stairs or standing from a sitting position. As the disease progresses, muscle weakness in the lower limbs spreads to the arms, neck and other areas. Most patients require full-time use of a wheelchair in their early teens, and then progressively lose the ability to independently perform activities of daily living such as using the restroom, bathing and feeding. Eventually, increasing difficulty in breathing due to respiratory muscle

dysfunction requires ventilation support, and cardiac dysfunction can lead to heart failure. The condition is universally fatal, and patients usually succumb to the disease in their twenties.

About Sarepta Therapeutics

At Sarepta, we are leading a revolution in precision genetic medicine and every day is an opportunity to change the lives of people living with rare disease. The Company has built an impressive position in Duchenne muscular dystrophy (DMD) and in gene therapies for limb-girdle muscular dystrophies (LGMDs), mucopolysaccharidosis type IIIA, Charcot-Marie-Tooth (CMT), and other CNS-related disorders, with more than 40 programs in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. For more information, please visit www.sarepta.com or follow us on Twitter, LinkedIn, Instagram and Facebook.

Sarepta Forward-Looking Statement

This press release contains "forward-looking statements." Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements regarding the potentially transformative benefits of SRP-9001; our plan to analyze the functional results for all the patients in Study 102 once they have achieved the 48-week timepoint; the expectation to have biomarker and safety results from Study 103 in the second quarter of 2021; our plan to use the information and insight from Study 102 to refine and complete the protocol for our upcoming trial using commercial process material; and our intention to continue to move forward with diligence and urgency to generate the evidence necessary to bring SRP-9001 to waiting Duchenne patients around the world.

These forward-looking statements involve risks and uncertainties that may cause actual results to differ materially from those expressed or implied in the forward-looking statements. Many of these risks and uncertainties are beyond our control. Known risk factors include, among others: 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 release may not be consistent with the final data set and analysis or result in an assessment that SRP-9001 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 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 press release. We undertake no obligation to update forward-looking statements based on events or circumstances after the date of this press release.

Internet Posting of Information

We routinely post information that may be important to investors in the 'For Investors' section of our website at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

Source: Sarepta Therapeutics, Inc.

Sarepta Therapeutics, Inc.

Investors:
Ian Estepan, 617-274-4052
iestepan@sarepta.com
Media:
Tracy Sorrentino, 617-301-8566
tsorrentino@sarepta.com

Exhibit 99.2

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

DOUG INGRAM
President and Chief Executive Officer

LOUISE RODINO-KLAPAC, Ph.D.
Executive Vice President, Chief Scientific Officer

January 7, 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 the potential benefits of SRP-9001 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, and leveraging learnings from Study 102 and Study 103 to inform future clinical development, including Study 301.

These forward-looking statements involve risks and uncertainties that may cause actual results to differ materially from those expressed or implied in the forward-looking statements. Many of these risks and uncertainties are beyond our control. Known risk factors include, among others: 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 SRP-9001 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 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.

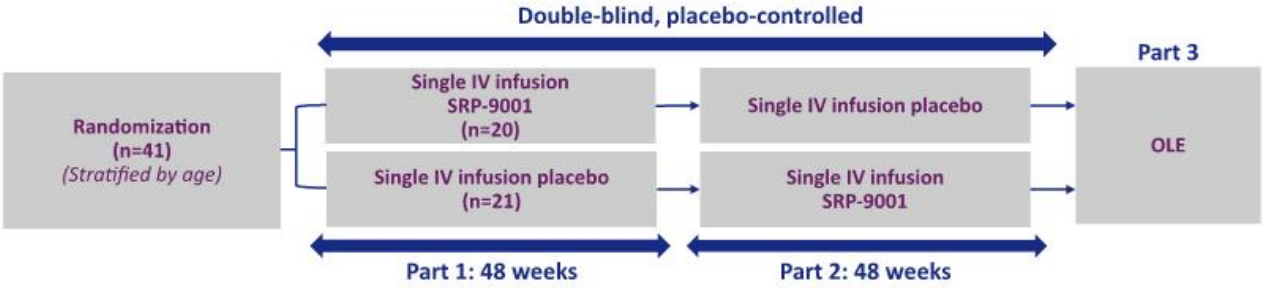
Welcome and Introduction

Doug Ingram
President and CEO

SAREPTA THERAPEUTICS

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

Secondary endpoints

- Micro-dystrophin protein expression measured by immunofluorescence (IF) and percent positive fibers
- Other timed function tests

Micro-dystrophin Protein Expression and Vector Genome Copies per Nucleus Achieved Endpoints (n=20, Week 12)

Micro-dystrophin Expression (Western Blot)

	Percentage of Normal
Mean (n=20)	28.1%

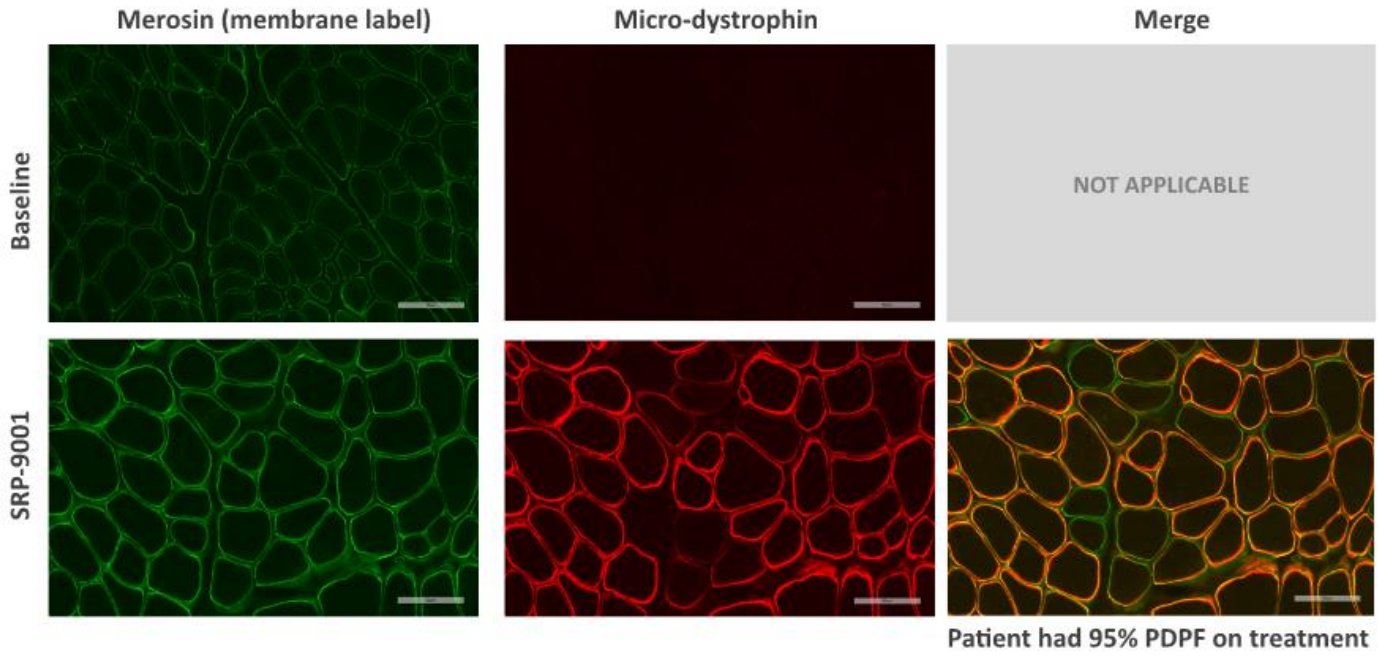
Micro-dystrophin Expression (IF)

	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

Representative Micro-dystrophin Images

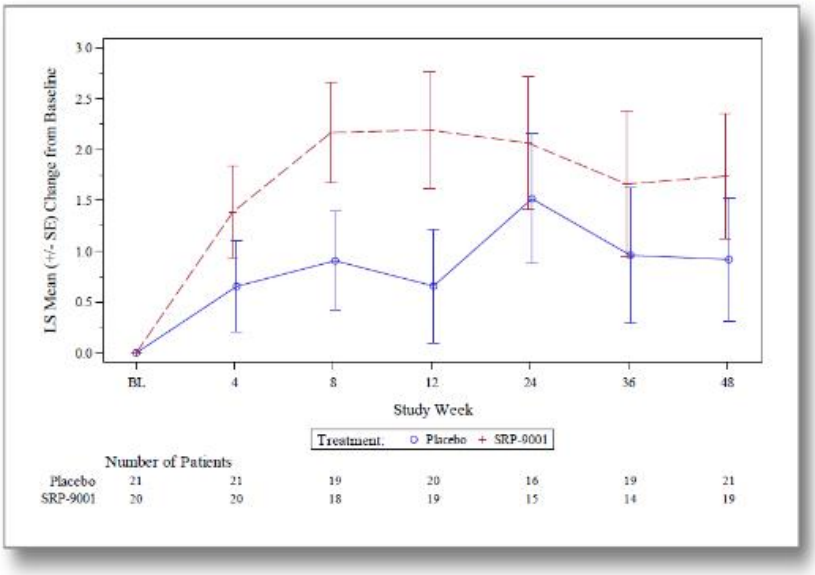


Patient had 95% PDPF on treatment

Functional Results

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)

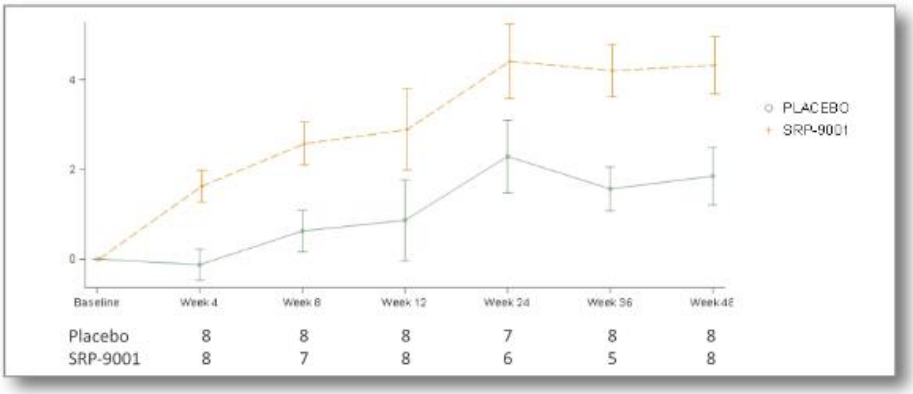


- Separation shown at every timepoint between SRP-9001 and placebo groups
- Baseline analysis at 48 weeks:
  - Treatment group showed 1.7-point increase compared to baseline (P=0.0090)
  - Placebo group showed 0.9-point increase compared to baseline (P=0.1411)

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

In a pre-specified analysis, 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 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

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.

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

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

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

Q&A