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): May 3, 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 May 3, 2021, Sarepta Therapeutics, Inc. (the "Company") issued a press release and conducted an investor webcast presenting clinical results from Phase 2 MOMENTUM study of SRP-5051 in patients with Duchenne muscular dystrophy who are amenable to exon 51 skipping. 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 May 3, 2021: Sarepta Therapeutics Reports Positive Clinical Results from Phase 2 MOMENTUM Study of SRP-5051 in Patients with Duchenne Muscular Dystrophy Amenable to Skipping Exon 51
99.2	Presentation dated May 3, 2021: Clinical Update: MOMENTUM Multiple- Ascending Dose Study of SRP-5051 for Duchenne Muscular Dystrophy
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: May 3, 2021

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

Sarepta Therapeutics Reports Positive Clinical Results from Phase 2 MOMENTUM Study of SRP-5051 in Patients with Duchenne Muscular Dystrophy Amenable to Skipping Exon 51

– ***Results suggest a highly potent next-generation treatment that could offer greater efficacy with less frequent dosing***

– ***SRP-5051 dosed monthly at 30 mg/kg delivered mean exon skipping of 10.79% and mean dystrophin expression of 6.55%, consistently higher than the other SRP-5051 dosing cohorts at 12 weeks and weekly eteplirsen at 24 weeks***

– ***Sarepta's predictive model indicates that SRP-5051 at 30 mg/kg will achieve greater than 10% dystrophin with monthly chronic dosing***

CAMBRIDGE, Mass., May 3, 2021 (GLOBE NEWSWIRE) – Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced positive results from Part A of the MOMENTUM study (Study 5051-201), a global, Phase 2, multi-ascending dose clinical trial of SRP-5051, its next-generation peptide phosphorodiamidate morpholino oligomer (PPMO) treatment for patients with Duchenne muscular dystrophy who are amenable to exon 51 skipping.

In biopsies taken at a median of 12 weeks and after only three doses, results from Part A of MOMENTUM study found that the 30 mg/kg of SRP-5051 dosed monthly resulted in 18 times the exon skipping and eight times the dystrophin production as eteplirsen, dosed weekly for 24 weeks. Exon-skipping and dystrophin production in the 30 mg/kg cohort were also consistently higher than the 20 mg/kg cohort of MOMENTUM. Hypomagnesemia was identified in patients taking SRP-5051. Cases have resolved with magnesium supplementation and an analysis of all available data indicate that the hypomagnesemia is monitorable and manageable.

"We are pleased to report strong, dose-dependent exon-skipping and dystrophin expression results with monthly dosing of SRP-5051 – in ambulant and non-ambulant patients. Even at an early timepoint of 12 weeks and after as few as only three doses, these data confirm the potential of Sarepta's next-generation PPMO platform to be a step order improvement over our current PMO platform, and to profoundly impact the course of Duchenne. While we saw exceptional expression after only a few initial doses, our models predict that we will exceed dystrophin expression levels of 10% of normal or greater over time with SRP-5051," said Doug Ingram, president and chief executive officer, Sarepta. "We are excited to have chosen our target dose for further development. Part A of MOMENTUM is now complete and Sarepta will work with great urgency to discuss the results with regulatory agencies and gain their insights, including the development path to support an accelerated approval of SRP-5051 in the United States."

Results from the 30 mg/kg dose cohort:

- In biopsies taken at a median of week 12, 30 mg/kg of SRP-5051 dosed monthly resulted in mean exon skipping of 10.79% (n=4). Exon skipping was measured by digital drop polymerase chain reaction (ddPCR).
    - This correlates to >4x increase in exon skipping compared to the 20 mg/kg cohort of SRP-5051 at 12 weeks (mean exon skipping of 2.57%, n=2) and an 18x increase in exon skipping compared a weekly 30 mg/kg dose of eteplirsen at 24 weeks (mean exon skipping of 0.59%, n=16).
- At a median of week 12, 30 mg/kg of SRP-5051 resulted in mean dystrophin production of 6.55% of normal. Dystrophin expression was measured by western blot.
    - This is twice the dystrophin expression compared to the 20 mg/kg cohort at week 12 (mean expression of 3.06%) and eight times that of the eteplirsen comparison group (mean expression of 0.82%).

There were three serious, treatment-emergent adverse events in two patients in the 30 mg/kg cohort, including two cases of hypomagnesemia. The events were asymptomatic and have resolved with magnesium supplementation. Markers of kidney function have generally been normal and not shown any consistent relationship to the hypomagnesemia.

Predictive modeling for dystrophin accumulation that includes assumptions of known turnover of dystrophin in the muscle and an analysis of data generated with the PPMO platform indicates that SRP-5051 at 30 mg/kg is likely to deliver greater than 10% dystrophin over time with monthly dosing.

Full results will be presented at a future medical meeting.

About MOMENTUM (Study SRP-5051-201)

MOMENTUM is a multi-arm, ascending dose study designed to identify the maximum tolerated dose of SRP-5051, infused monthly. The study will enroll up to 24 patients, both ambulant and non-ambulant, between the ages of 7 to 21 at sites in the U.S., Canada, Australia and European Union. The primary endpoint is safety, and secondary and exploratory endpoints include exon-skipping, dystrophin expression and tissue concentration. More information can be found on www.clinicaltrials.gov.

About SRP-5051

SRP-5051 uses Sarepta's PPMO chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. SRP-5051 is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein. PPMO is Sarepta's next-generation chemistry platform designed around a proprietary cell-penetrating peptide conjugated to the PMO backbone, with the goal of increasing tissue penetration, increasing exon skipping and significantly increasing dystrophin production. Around 13% of DMD patients have mutations which make them amenable to skipping exon 51. If successful, the PPMO offers the potential for improved efficacy and less frequent dosing for patients.

About Duchenne Muscular Dystrophy

DMD is an X-linked rare degenerative neuromuscular disorder causing severe progressive muscle loss and premature death. One of the most common fatal genetic disorders, DMD affects approximately one in every 3,500 - 5,000 male births worldwide. A devastating and incurable muscle-wasting disease, DMD is associated with specific errors in the gene that codes for dystrophin, a protein that plays a key structural role in muscle fiber function. Progressive muscle weakness in the lower limbs spreads to the arms, neck and other areas of the body. The condition is universally fatal, and death usually occurs before the age of 30 due to respiratory or cardiac failure.

About EXONDYS 51

EXONDYS 51 (eteplirsen) uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to bind to exon 51 of dystrophin pre-mRNA, resulting in "skipping" of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein.

EXONDYS 51 is indicated for the treatment of Duchenne muscular dystrophy in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in some patients treated with EXONDYS 51. Continued approval may be contingent upon verification of a clinical benefit in confirmatory trials.

EXONDYS 51 has met the full statutory standards for safety and effectiveness and as such is not considered investigational or experimental.

Important Safety Information About EXONDYS 51

Hypersensitivity reactions, including rash and urticaria, pyrexia, flushing, cough, dyspnea, bronchospasm, and hypotension, have occurred in patients who were treated with EXONDYS 51. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the EXONDYS 51 therapy.

Adverse reactions in DMD patients (N=8) treated with EXONDYS 51 30 mg or 50 mg/kg/week by intravenous (IV) infusion with an incidence of at least 25% more than placebo (N=4) (Study 1, 24 weeks) were (EXONDYS 51, placebo): balance disorder (38%, 0%), vomiting (38%, 0%) and contact dermatitis (25%, 0%). The most common adverse reactions were balance disorder and vomiting. Because of the small numbers of patients, these represent crude frequencies that may not reflect the frequencies observed in practice. The 50 mg/kg once weekly dosing regimen of EXONDYS 51 is not recommended.

In the 88 patients who received ≥30 mg/kg/week of EXONDYS 51 for up to 208 weeks in clinical studies, the following events were reported in ≥10% of patients and occurred more frequently than on the same dose in Study 1: vomiting, contusion, excoriation, arthralgia, rash, catheter site pain, and upper respiratory tract infection.

For further information, please see the full **Prescribing Information**.

About Sarepta Therapeutics

Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold leadership positions in Duchenne muscular dystrophy (DMD) and limb-girdle muscular dystrophies (LGMDs), and we currently have more than 40 programs in various stages of development. Our vast pipeline is driven by our multi-platform Precision Genetic Medicine Engine in gene therapy, RNA and gene editing. For more information, please visit www.sarepta.com or follow us on Twitter, LinkedIn, Instagram and Facebook.

Forward-Looking Statements

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 market opportunities; the potential benefits of PPMO, including offering greater efficacy with less frequent dosing, being a step order improvement over our current PMO platform, and profoundly impacting the course of Duchenne; our prediction that SRP-5051 at 30 mg/kg will achieve greater than 10% dystrophin over time with monthly dosing; and our plans, including to work with great urgency to discuss the results with regulatory agencies and gain their insights, including the development path to support an accelerated approval of SRP-5051 in the United States and to present full results at a future medical meeting.

These forward-looking statements involve risks and uncertainties, many of which 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 thereof or result in a safe or effective treatment benefit; different methodologies, assumptions and applications 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 of our product candidates are positive, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities; if the actual number of patients suffering from DMD is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; 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, some of which may be outside of our control, including possible limitations of 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 and the COVID-19 pandemic; and even if Sarepta's programs result in new commercialized products, Sarepta may not achieve the expected revenues from the sale of such products; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K for the year ended December 31, 2020, as well as other Securities and Exchange Commission (SEC) filings made by the Company 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 Sarepta faces, you are encouraged to review the SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

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.

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iestepan@sarepta.com

Media Contact:
Tracy Sorrentino, 617-301-8566
tsorrentino@sarepta.com

EXHIBIT 99.2

Clinical Update: Results from 30 mg/kg Cohort of MOMENTUM Study of SRP-5051 for Duchenne Muscular Dystrophy

DOUG INGRAM
President and CEO

GILMORE O'NEILL, MB, MMSC
Executive Vice President, R&D and Chief Medical Officer

May 3, 2021
8:30 a.m. ET

SAREPTA THERAPEUTICS

Welcome and Introduction

Doug Ingram
President and CEO

SAREPTA THERAPEUTICS

Forward-Looking Statements

This presentation contains "forward-looking statements." Any statements contained in this presentation 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 potential market opportunities for SRP-5051; the potential benefits of PMO, including specificity, stability, versatility and safety; the potential benefits of PPMO, including enhanced PMO tissue penetration leading to greater exon skipping and dystrophin production and more efficient dosing; the predicted dystrophin trajectory of >10% expression over time with monthly dosing of SRP-5051; the expectation that expression driven by SRP-5051 will deliver clinically better changes in patient outcomes; and plans and milestones for SRP-5051 and other PPMO product candidates, including initiating Part B of MOMENTUM, our goal that Part B will be a pivotal trial, supporting an accelerated approval in the United States, plans regarding engagement with regulatory agencies, and plans regarding the development of future PPMOs for other exons in Duchenne and other indications.

These forward-looking statements involve risks and uncertainties, many of which 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 thereof or result in a safe or effective treatment benefit; different methodologies, assumptions and applications 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 of our product candidates are positive, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities; if the actual number of patients suffering from DMD is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; 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, some of which may be outside of our control, including possible limitations of 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 and the COVID-19 pandemic; and even if Sarepta's programs result in new commercialized products, Sarepta may not achieve the expected revenues from the sale of such products; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K for the year ended December 31, 2020, and most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company 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 Sarepta faces, you are encouraged to review the SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained in this presentation. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

Clinical Update: MOMENTUM Multiple-Ascending Dose Study of SRP-5051 for Duchenne Muscular Dystrophy

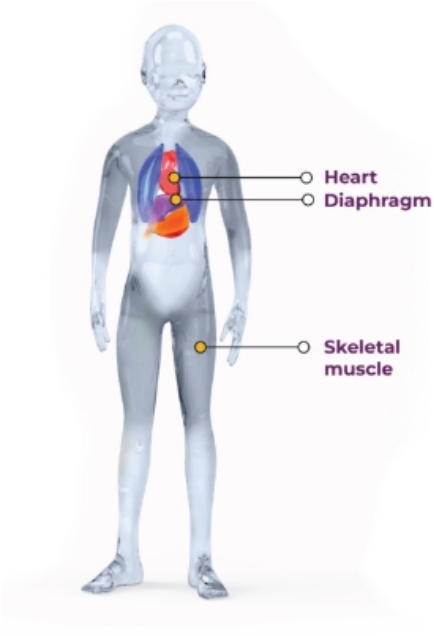
Gilmore O'Neill, MB, MMSC
Executive Vice President, R&D and Chief Medical Officer

SAREPTA THERAPEUTICS

Duchenne Muscular Dystrophy (DMD)

DMD affects approximately 1 in 3,500-5,000 males worldwide[1]

- DMD is a rare, fatal neuromuscular genetic disease inherited in an X-linked recessive pattern[2]
- Muscle weakness becomes increasingly noticeable by age 3 to 5, and most patients use a wheelchair by the time they are 11[2]
- During adolescence, cardiac and respiratory muscle deterioration lead to serious, life-threatening complications[3]



1. National Institutes of Health. Genetics Home Reference. Duchenne and Becker muscular dystrophy. https://ghr.nlm.nih.gov/condition/duchenne-and-becker-muscular-dystrophy. Accessed Jan 2020.
2. Hoffman EP, Brown RH, et al. Dystrophin: the protein product of the Duchenne muscular dystrophy locus. Cell. 1987;51:919-928.
3. Passamano L, Taglia A, et al. Improvement of survival in Duchenne Muscular Dystrophy: retrospective analysis of 835 patients. Acta Myologica. 2012;31(1): 121-125.

Sarepta's Proprietary PMO Technology

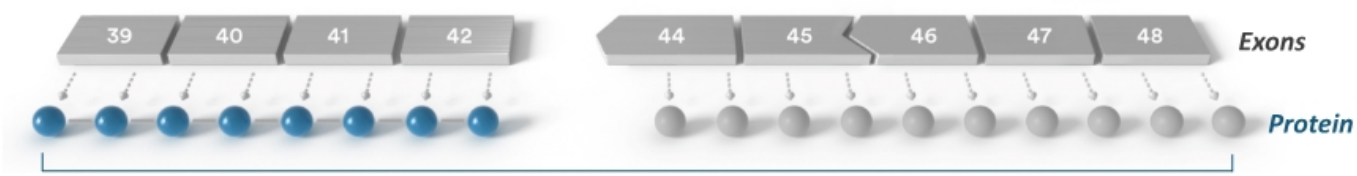
Phosphorodiamidate morpholino oligomer (PMO) technology

Specificity: Enhanced affinity for targeting pre-mRNA for precise binding to the selected RNA target

Stability: Highly resistant to degradation by enzymes

Versatility: Ability to rapidly design and construct drug candidates that are specific for human or pathogen RNA; and target specific tissues

Safety: Built upon a charge-neutral backbone, which may be reflected in tolerability



The PMO directs the splicing machinery to skip an exon when processing the pre-mRNA. As a result, the alternate mRNA allows for the production of a shortened, functional dystrophin protein.

Kole R, Lepper BJ. Discovery Medicine, Volume 14, Number 74, July 2012

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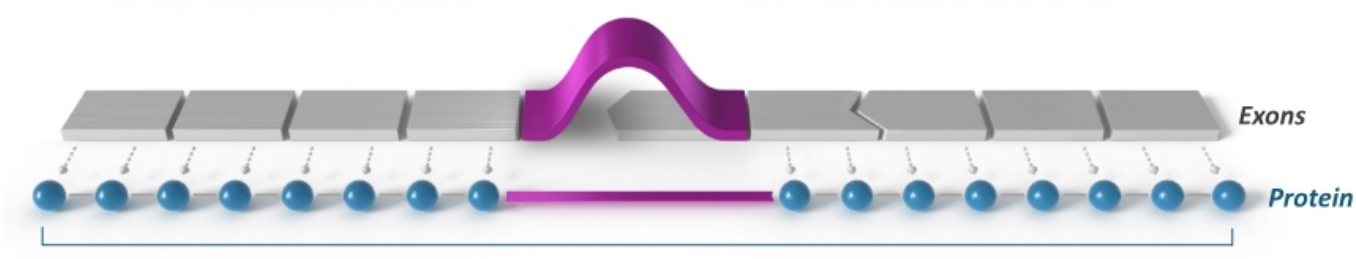

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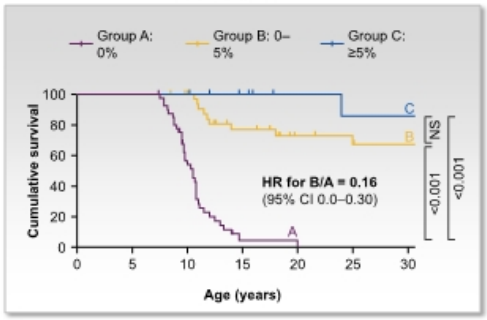
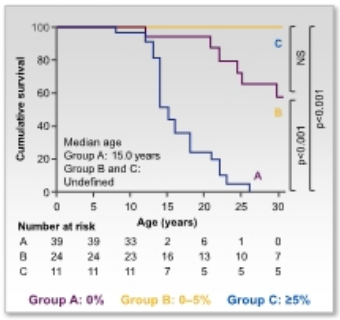
Natural History Genotype/Phenotype Correlations Predict that Even Low Dystrophin Quantities can Significantly Mitigate Duchenne Phenotype

- Integrated study of 100 patients with Xp21 linked muscular dystrophy using clinical, genetic, immunochemical, and histopathological data. Part 1. Trends across the clinical groups. Nicholson LV et al. J Med Genet 1993;30:728–736.
- Differentiation of Duchenne and Becker muscular dystrophy phenotypes with amino- and carboxy-terminal antisera specific for dystrophin. Bulman DE et al. Am J Hum Genet 1991;48:295.
- Very Low Residual Dystrophin Quantity Is Associated with Milder Dystrophinopathy. de Feraudy et al. Ann Neurol 2021; 89:280-292.
  - "Residual dystrophin of 0 to 5% in group B was associated with delayed LoA compared to group A, and ambulation was almost completely conserved in group C (Fig 3A; $p < 0.001$)"
  - "data showed that disease severity correlates with dystrophin quantities even at very low levels, as observed in patient groups with 0 to 0.5% and 0.5 to 5% dystrophin when compared to 0% dystrophin (group A) and ≥5% dystrophin"

LOSS OF AMBULATION
(Groups A,B,C)



VC <50%



Patients with residual dystrophin protein expression >0% demonstrated a delay in loss of ambulation and improved respiratory function compared to those with no residual dystrophin[1]

Delay in onset first symptoms, LOA, time of spinal surgery and improvement in survival

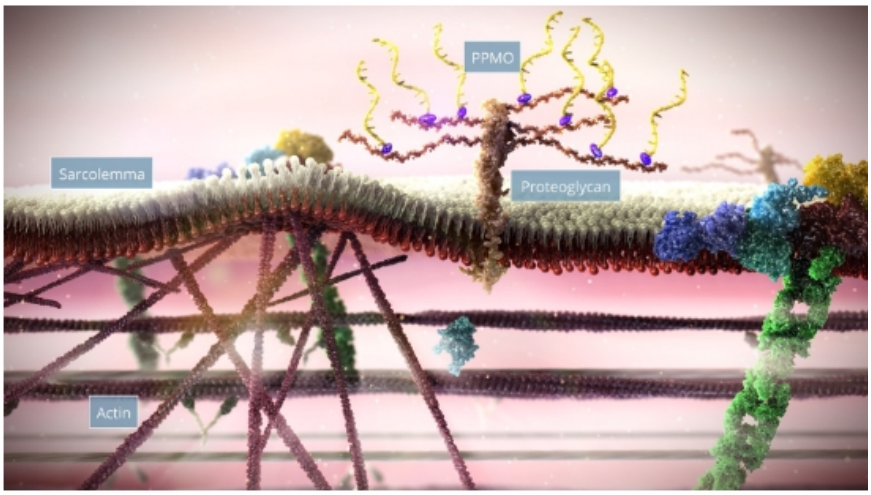
Peptide-conjugated PMO (PPMO): Next-generation Technology for Enhanced PMO Tissue Penetration Leading to Greater Exon Skipping and Dystrophin Production

Enhances PMO

- Conjugated peptide greatly increases cell penetration
- Could potentially lead to more efficient dosing and greater benefit for patients
- Non-clinical data demonstrates delivery of PPMOs to unique muscle types (e.g., heart)

Lead PPMO candidate: SRP-5051

- Designed to skip exon 51
- In clinical development to treat patients with DMD amenable to exon 51 skipping



Clinical Update: SRP-5051 MOMENTUM Multiple-Ascending Dose Study, Results from 30 mg/kg Cohort

SRP-5051-201 MOMENTUM PART A: Designed to Assess Safety and PK/PD of Multiple Doses of SRP-5051 in Plasma and Muscle of Duchenne Patients

- **Primary Outcome Measure**
  - Safety
- **Secondary Outcome Measures Including:**
  - PK plasma concentration of SRP-5051
  - Change from baseline at 12 weeks:
    - Muscle concentration of SRP-5051
    - Muscle exon-skipping measured by ddPCR
    - Muscle dystrophin protein measured by western blot
- **Inclusion criteria**
  - Confirmed DMD mutation amenable to exon 51-skipping
  - Stable dose of oral corticosteroids for at least 12 weeks prior to study drug administration, or no corticosteroids for at least 12 weeks prior to study drug administration.
  - Part A accepts ambulatory and non-ambulatory patients ages 7 to 21 years

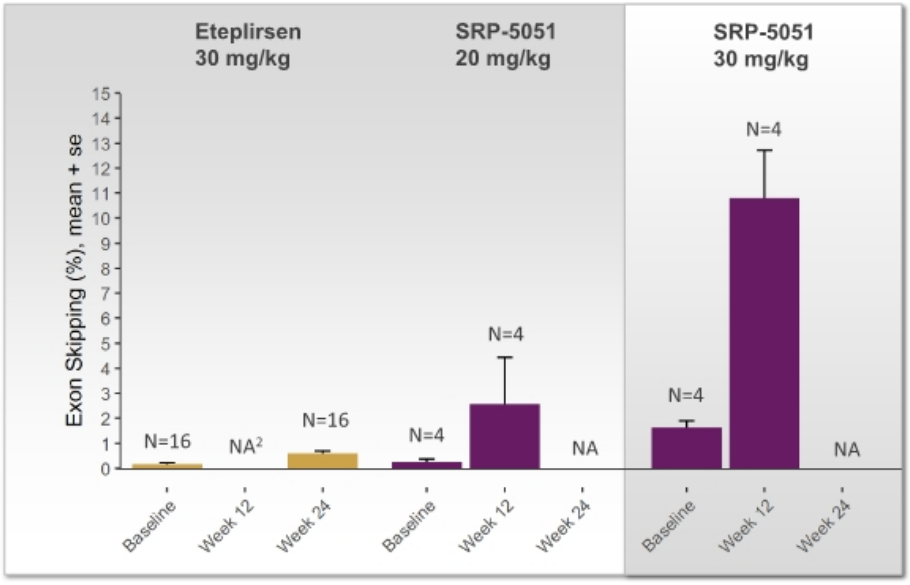
ClinicalTrials.gov Identifier: NCT04004065.
SRP-5051 PPMO is investigational and has not been reviewed or approved by any regulatory authority .

SRP-5051-201 MOMENTUM: 30 mg/kg Patient Demographics at Baseline

SRP-5051 *30 mg/kg/month X4*	AGE (YEARS)	WEIGHT (KG)	Doses at the time of biopsy
Patient 1	18	57.9	5
Patient 2	7	25	3
Patient 3	15	51	3
Patient 4	16	52.5	3

SRP-5051-201 MOMENTUM: SRP-5051 30 mg/kg Arm at 12 Weeks Drove 18x Increase in Exon Skipping vs. Eteplirsen at 24 Weeks [1]

>4x increase in exon skipping in the 30mg/kg arm vs. 20 mg/kg at 12 weeks

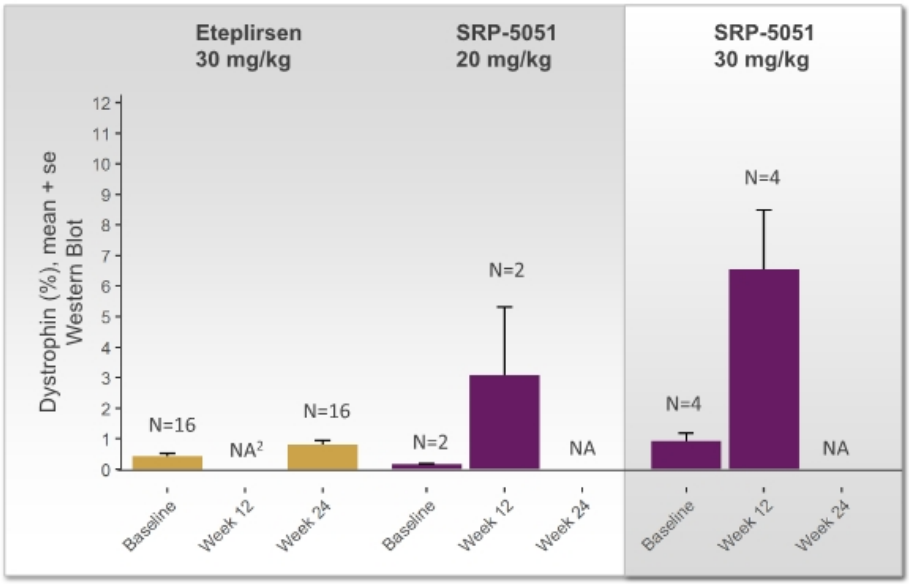


	Exon Skipping (%) mean
Eteplirsen (week 24)	0.59
SRP-5051 20 mg/kg (week 12)	2.57
SRP-5051 30 mg/kg (week 12)	10.79

[1] Comparative data produced with the same analytical methods using biopsies obtained from Part A of Study 5051-201 MOMENTUM and Study 4658-202 PROMOVI.
[2] NA = Not Applicable, data not collected at these time points
*Target dose was 12 weeks. Patient 1 had 5 doses. 19 weeks from baseline to biopsy.

SRP-5051-201 MOMENTUM: SRP-5051 30 mg/kg Arm at 12 Weeks Drove 8x Increase of Dystrophin vs. Eteplirsen at 24 Weeks [1]

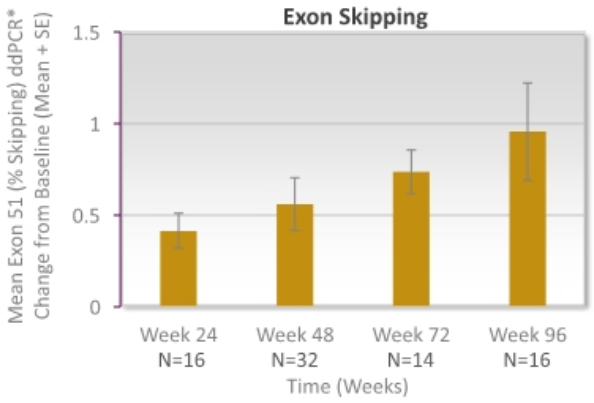
~2x increase in dystrophin in the 30 mg/kg arm vs. 20 mg/kg at 12 weeks

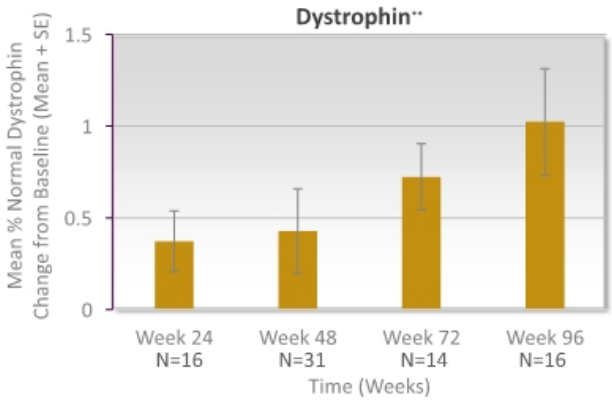


	Dystrophin (%) mean
Eteplirsen (week 24)	0.82
SRP-5051 20 mg/kg (week 12)	3.06
SRP-5051 30 mg/kg (week 12)	6.55

[1] Comparative data produced with the same analytical methods using biopsies obtained from Part A of Study 5051-201 MOMENTUM and Study 4658-202 PROMOVI.
[2] NA = Not Applicable, data not collected at these time points
*Target dose was 12 weeks. Patient 1 had 5 doses. 19 weeks from baseline to biopsy.

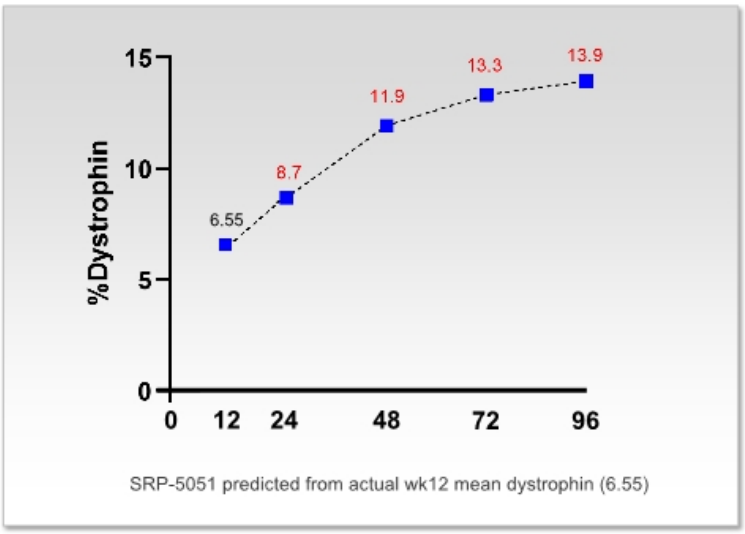
Strong Evidence that Exon Skipping and Dystrophin Increase Over Time, as Seen in PROMOVI with Eteplirsen





A quantitative ddPCR assay was used to measure % exon skipping, providing precise and accurate measurements
*** As measured by western blot, adjusted for muscle content*

Predicted Dystrophin Trajectory of >10% Expression is Achievable Over Time with Monthly Dosing of SRP-5051



SRP-5051 predicted from actual wk12 mean dystrophin (6.55)

- Dystrophin levels from 30mg/kg 5051-201 data
- Red numbers are predicted values

- Model assumes that trajectory of PPMO will be similar to the increase of dystrophin that we observed in PROMOVI longitudinal study
- Predictions are based on dystrophin turnover model using PROMOVI and SRP-5051 data at 30 mg/kg and predicting dystrophin production over time

Reference: Dystrophin estimated half life in the turnover model

136 days based on PROMOVI data

60 days in skeletal muscle PPMO in mdx mice.
Wu et al Am J Pathol 2012 181:392-400

103 days in quad 2OMePS in mdx mice.
Verhaart et al Molecular Therapy-Nucleic Acids 2014 3:e418

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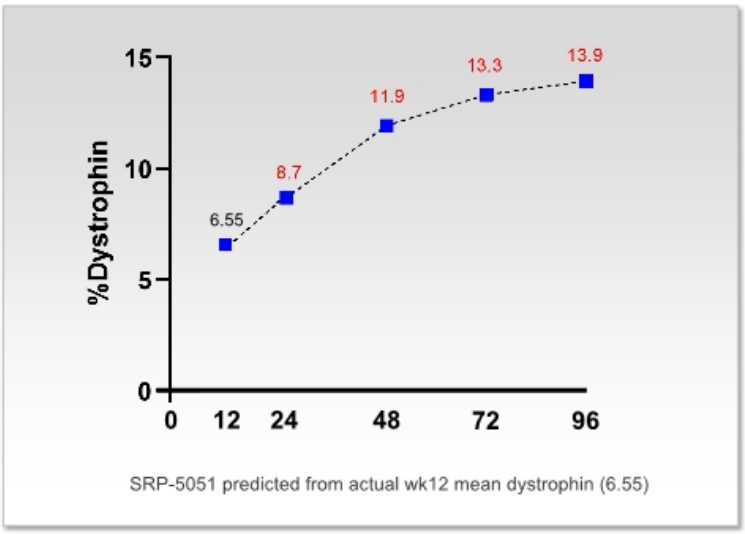

	Number of Doses at Time of Biopsy	Western Blot	Exon Skipping
Patient 1	5	12.1%	11.0%

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Safety and Clinical Summary

SRP-5051-201 MOMENTUM: Safety Experience

Adverse Event Summary

Subjects with at least one	4 mg/kg (N=3)	10 mg/kg (N=3)	20 mg/kg (N=5)	30 mg/kg (N=7)	Overall (N=18)
Treatment-emergent AE (TEAE)	2 (66.7%)	3 (100.0%)	5(100.0%)	7(100.0%)	17 (94.4%)
TEAE related to study drug	0	3 (100.0%)	2 (40.0%)	7 (100.0%)	12 (66.7%)
Grade ≥3 TEAE	0	0	0	2 (28.6%)	2 (11.1%)
Serious TEAE	1 (33.3%)	0	0	2 (28.6%)	3 (16.7%)
Serious TEAE related to study drug	0	0	0	2 (28.6%)	2 (11.1%)
TEAE leading to death	0	0	0	0	0

Severe and serious TEAEs in the 30 mg/kg cohort are summarized in the next slide

SRP-5051-201 MOMENTUM: Serious TEAEs at 30 mg/kg

Patient	Preferred Term	Severity*	Causality	Onset Day	Outcome
Patient 1	Hypomagnesemia	Grade 4	Related	3	Resolved
	Hypokalemia	Grade 4	Related	5	Resolved
Patient 3	Hypomagnesemia	Grade 4	Related	7	Resolved

* Severity is based on CTCAE laboratory value.
None of the SAEs were life-threatening, and both patients remained asymptomatic

Onset day: interval (days) from last dose

SRP-5051-201 MOMENTUM: Safety Summary

- Hypomagnesemia is a newly identified risk in the SRP-5051 program
  - Most cases were mild and asymptomatic with some serious cases occurring prior to the implementation of serum monitoring and magnesium repletion
  - Analysis of clinical and nonclinical studies (serum and urine) indicated that hypomagnesemia is both manageable and monitorable
    - Cases rapidly improved with magnesium supplementation
    - Serum monitoring of magnesium and oral supplementation with magnesium is a feasible approach to enable early detection and management going forward
  - Markers of kidney function (glomerular filtration rate) have generally been normal and have not shown any consistent relationship to the hypomagnesemia

SRP-5051-201 MOMENTUM: Clinical Summary

- 30 mg/kg arm at 12 weeks dosing drove 18x increase in exon skipping vs. eteplirsen at 24 weeks dosing
  - >4x increase in exon skipping in the 30 mg/kg arm vs. 20 mg/kg at 12 weeks
- 30mg/kg arm at 12 weeks dosing drove 8x increase in dystrophin vs. eteplirsen at 24 weeks dosing
  - ~2x increase in dystrophin in the 30 mg/kg arm vs. 20 mg/kg at 12 weeks
- Strong evidence that exon skipping and dystrophin will increase with longer term dosing
  - Predicted dystrophin trajectory of >10% expression is achievable over time with monthly dosing of SRP-5051
- Patient receiving the most doses of SRP-5051 had the highest dystrophin expression
- Benefit/risk supports continued clinical development

Next Steps for SRP-5051 Program

Next Steps

- Part A of 5051-201(MOMENTUM) is now complete
- Part B of MOMENTUM is intended to be the pivotal trial in the US
  - Protocol is being amended to mitigate the hypomagnesemia risk
    - Implementation of magnesium supplementation with appropriate monitoring
  - Will engage FDA prior to the initiation of Part B
- Have engaged international regulatory agencies on the overall clinical package and future registration strategy
- Learnings from the clinical studies of SRP-5051 will help inform the development of future PPMOs for other exons in Duchenne and other indications

Question and Answer