Patients can't wait for the next breakthrough in medical research.

So neither will we.

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Clinical Update: MOMENTUM (Study SRP-5051-201, Part B)

January 29, 2024

SAREPTA THERAPEUTICS

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Living with Duchenne muscular dystrophy

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 future operations, financial performance and projections, business plans, market opportunities, priorities and research and development programs and technologies; the potential benefits of our technologies and scientific approaches, including PPMO; the potential benefits of SRP-5051, including the potential for higher dystrophin and exon-skipping than eteplirsen, an accumulation of expression over time, and a favorable benefit-risk profile, including our belief that hypomagnesemia is manageable; our belief that all individuals living with Duchenne can potentially benefit from dystrophin restorative therapies; and plans and milestones, including requesting a pre-NDA meeting to discuss results with FDA, expected in Q3 2024.

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 such risks and uncertainties could materially and adversely affect our business, results of operations and trading price. Potential known risk factors include, among others, the following: the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; success in preclinical testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business; our data for our different programs, including PPMO, may not be sufficient for obtaining regulatory approval; we may not be able to comply with all FDA requests in a timely manner or at all; the expected benefits and opportunities related to our agreements with our strategic partners may not be realized or may take longer to realize than expected due to a variety of reasons, including any inability of the parties to perform their commitments and obligations under the agreements, challenges and uncertainties inherent in product research and development and manufacturing limitations; if the actual number of patients living with Duchenne is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; 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 successfully scale up manufacturing of our product candidates in sufficient quality and quantity or within sufficient timelines; 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, for various reasons including possible limitations of our 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; and those risks identified under the heading “Risk Factors” in Sarepta’s most recent Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) and in its other SEC filings.

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Welcome and Introduction

Doug Ingram

Clinical Program and Results

Louise Rodino-Klapac, PhD

SRP-5051: Next-generation RNA-based PPMO* candidate to treat Duchenne patients amenable to exon 51 skipping**

- Same precision genetic medicine backbone
- Conjugated peptide with the goal to increasing tissue penetration, exon skipping and dystrophin production
- Nonclinical data demonstrate delivery of PPMOs to all muscle, including the heart
- Study SRP-5051-201, Part A showed after 12 weeks, 30mg/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 (data presented in May 2021)

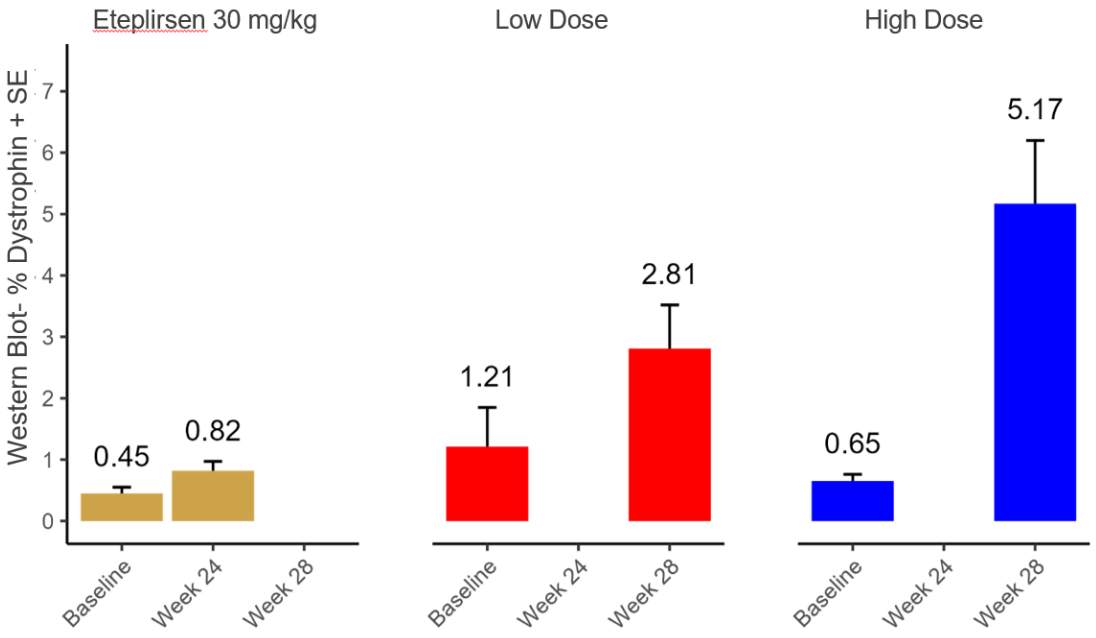
*Peptide phosphorodiamidate morpholino oligomers (PPMO)
**Approximately 13% of patients with Duchenne are amenable to exon 51 skipping

MOMENTUM (Study SRP-5051-201)

- Global trial evaluating SRP-5051 in patients with Duchenne amenable to exon 51 skipping
- Dystrophin protein levels in skeletal muscle tissue following treatment with SRP-5051 were assessed, as well as safety and tolerability
- Doses (administered every 4 weeks):
  - High dose: ~30 mg/kg
  - Low dose: ~20 mg/kg
- MOMENTUM (Study SRP-5051-201, Part B) enrolled 40 patients, 50% ambulant and 50% non-ambulant, ages 8 to 21 in the United States, Canada, and Europe
  - Primary outcome: Change from baseline in dystrophin protein level at week 28
- Patients dosed in MOMENTUM Part A, who met the entrance criteria, were eligible to participate in Part B
- Throughout MOMENTUM Part B we continued to administer prophylactic magnesium supplementation and/or adjust dose to manage hypomagnesemia

SRP-5051 showed mean dystrophin expression of 5.17% at the high dose at week 28

Data also demonstrated a 12.2x increase vs. eteplirsen

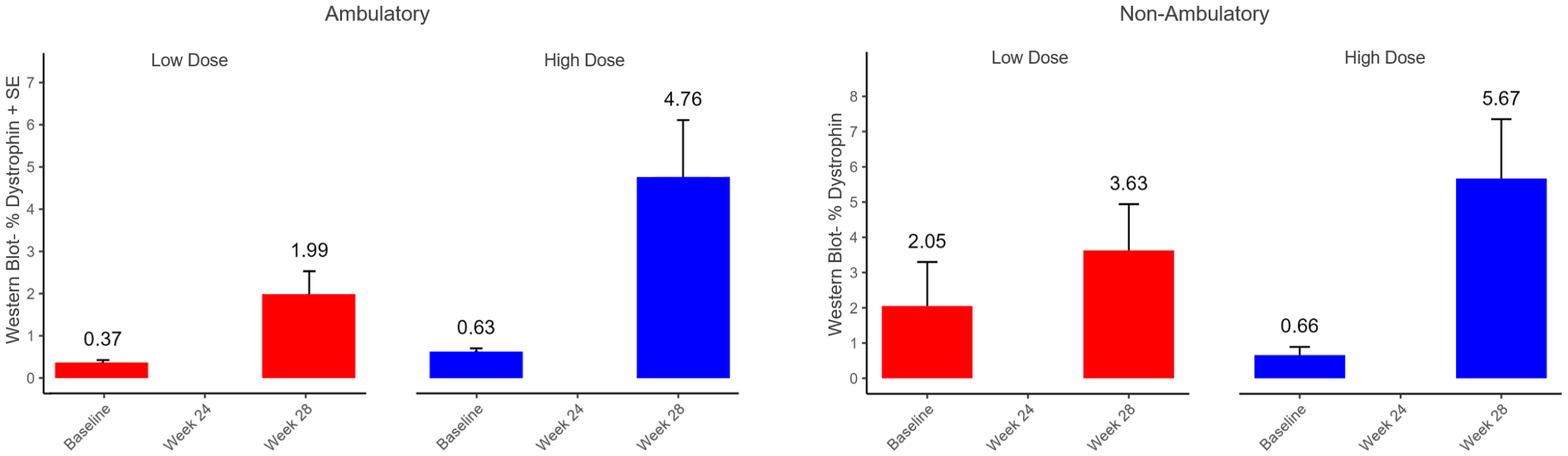


	Mean (SD) post baseline (%)	Mean (SD) change from baseline (%)	Multiplier vs. eteplirsen change from baseline
Eteplirsen (n=16)	0.82 (0.60)	0.37 (0.66)	--
SRP-5051 Low dose (n=20)	2.81 (3.19)	1.60 (2.21) P = 0.0012	**4.3x**
SRP-5051 High dose (n=20)	5.17 (4.62)	4.53 (4.78) P < 0.0001	**12.2x**

SE = Standard Error; SD = Standard Deviation

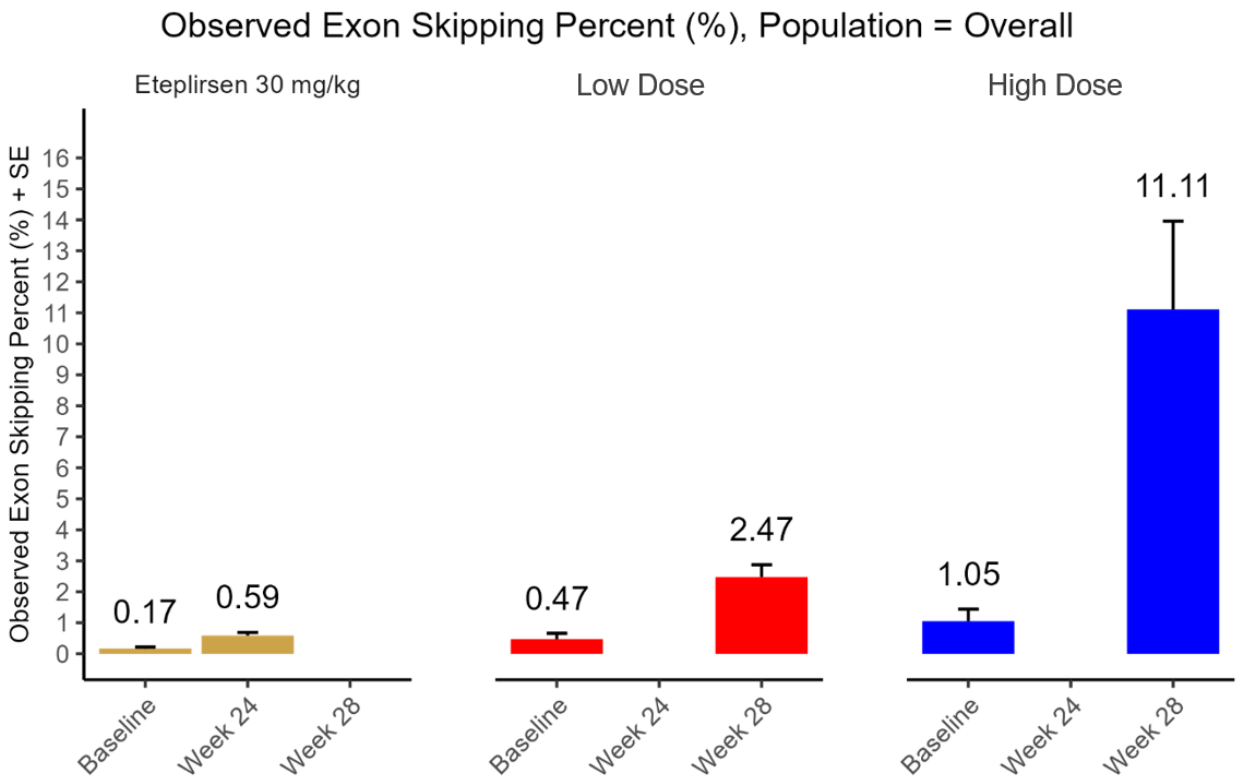
Both doses showed a change from baseline with statistically significant values

Similar expression levels observed in the ambulatory (n=21) and non-ambulatory (n=19) patient populations



SRP-5051 showed mean exon skipping of 11.11% at the high dose at week 28 (n=20)

Data also demonstrated 24.6x increase vs. eteplirsen

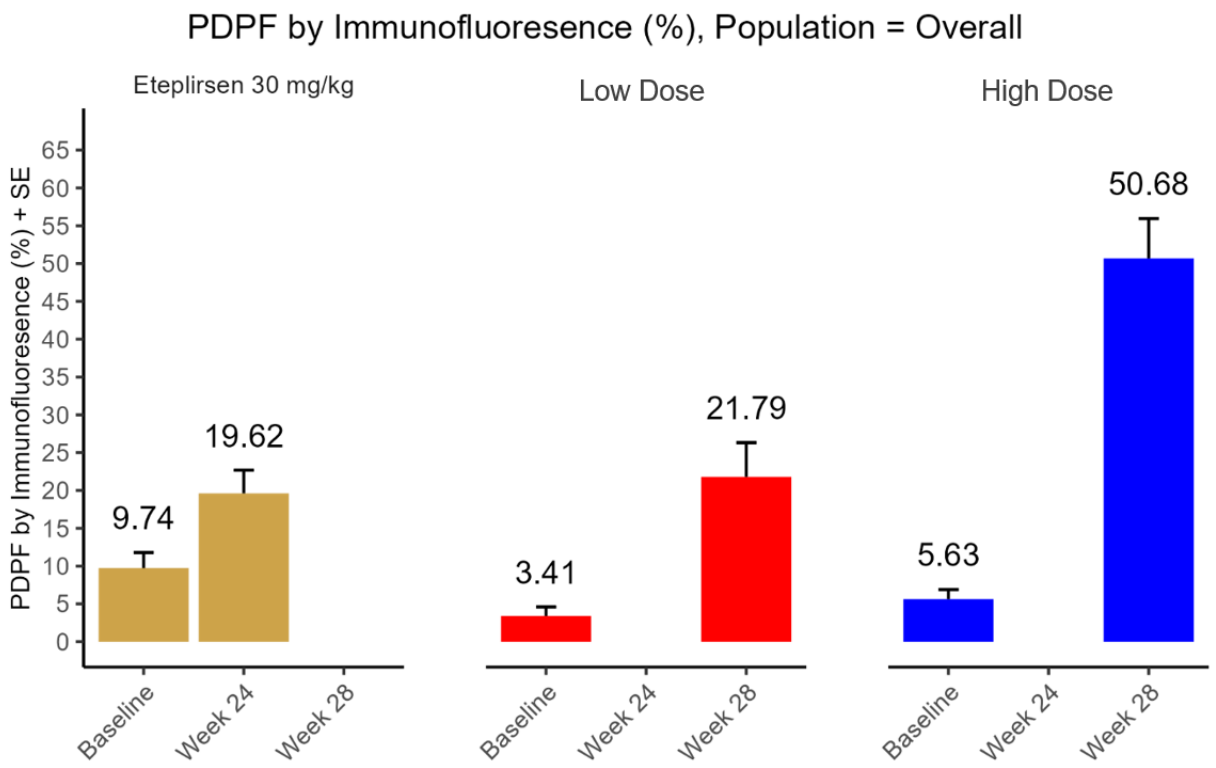


Exon skipping	Mean post baseline (%)	Mean change from baseline (%)	Multiplier vs. eteplirsen change from baseline
Eteplirsen (n=16)	0.59	0.41	--
SRP-5051 Low dose (n=19*)	2.47	2.00	**4.9x**
SRP-5051 High dose (n=20)	11.11	10.07	**24.6x**

*1 patient had degraded sample

SRP-5051 showed mean PDPF of 50.68% at the high dose at week 28

Data also demonstrated 4.6x increase vs. eteplirsen



	Mean post baseline (%)	Mean change from baseline (%)	Multiplier vs. eteplirsen change from baseline
Eteplirsen (n=16)	19.62	9.88	--
SRP-5051 Low dose (n=20)	21.79	18.38	**1.9x**
SRP-5051 High dose (n=20)	50.68	45.05	**4.6x**

Safety Results

MOMENTUM (SRP-5051-201, Part B): Safety experience overview

Adverse Event Summary (mean 12 months of dosing)

	Low Dose (N=32) n (%)	High Dose (N=29) n (%)	Overall (N=62) n (%)
Subjects with Treatment-Related TEAEs	31 (96.9%)	28 (96.6%)	60 (96.8%)
Treatment-Related AEs in > 5% of subjects			
Hypomagnesemia	30 (93.8%)	28 (96.6%)	59 (95.2%)
Hypokalemia	12 (37.5%)	14 (48.3%)	26 (41.9%)
Nausea	2 (6.3%)	3 (10.3%)	5 (8.1%)
Vomiting	2 (6.3%)	2 (6.9%)	4 (6.5%)
Glomerular filtration rate decreased	1 (3.1%)	3 (10.3%)	4 (6.5%)
Treatment-Related SAEs			
Hypomagnesemia	1 (3.1%)	3 (10.3%)	4 (6.5%)
Hypokalemia	1 (3.1%)	2 (6.9%)	3 (4.8%)
Treatment-Related Discontinuations	0	0	0

Source: Table 14.3.1.1.1 Overall Summary of Treatment-Emergent Adverse Events Safety Set – Part B
Data Cutoff Date: 02-NOV-2023

Summary and next steps

Efficacy

- Achieved study's primary endpoint
- Demonstrated a statistically significant increase in dystrophin expression at both doses
- At the high dose at week 28, SRP-5051 showed dystrophin production 12.2x higher than eteplirsen
- SRP-5051 demonstrated mean dystrophin expression of 5.17% at the high dose at 28 weeks

Safety

- The data support a positive benefit-risk profile for SRP-5051
- Throughout MOMENTUM Part B, we continued to administer prophylactic magnesium supplementation and/or adjust dose to manage hypomagnesemia
- No treatment-related discontinuations occurred in the study

Next Steps

- Request pre-NDA meeting to discuss results with FDA, expected timing Q3 2024
- Study remains ongoing

Q&A

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