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

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SAREPTA THERAPEUTICS

Forward-looking Statements

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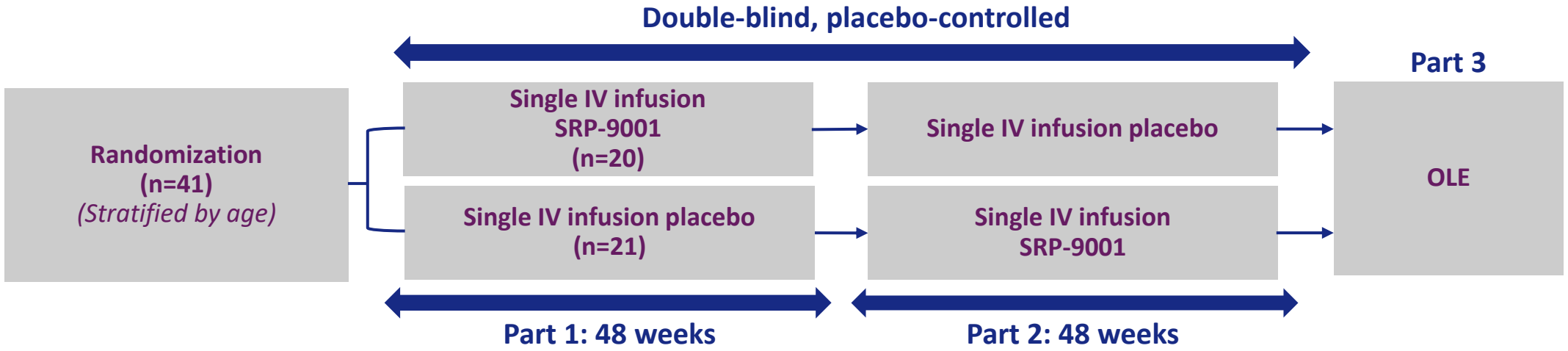
Welcome and Introduction

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%

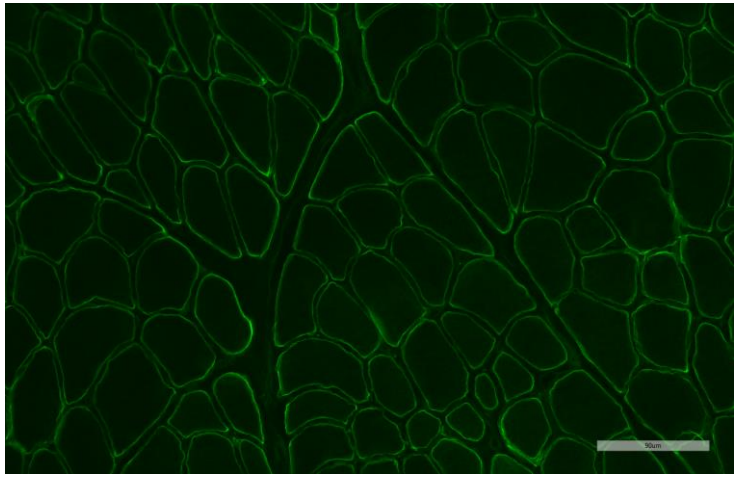
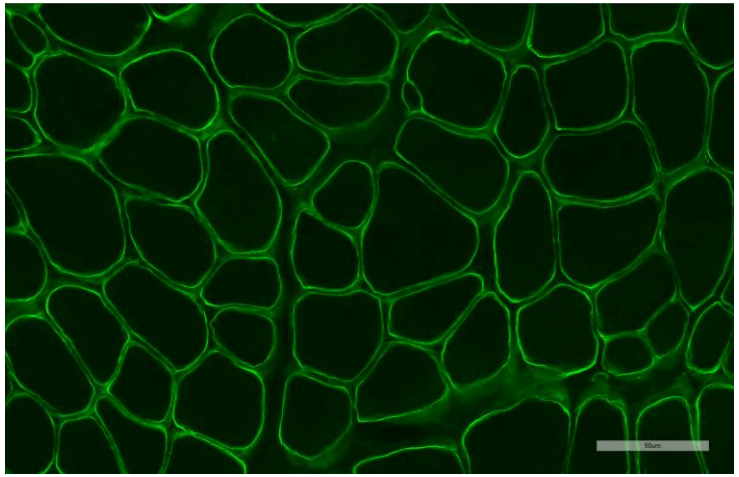
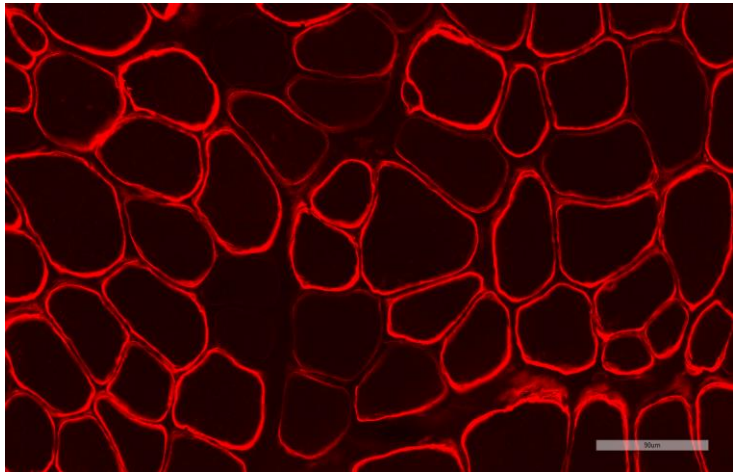
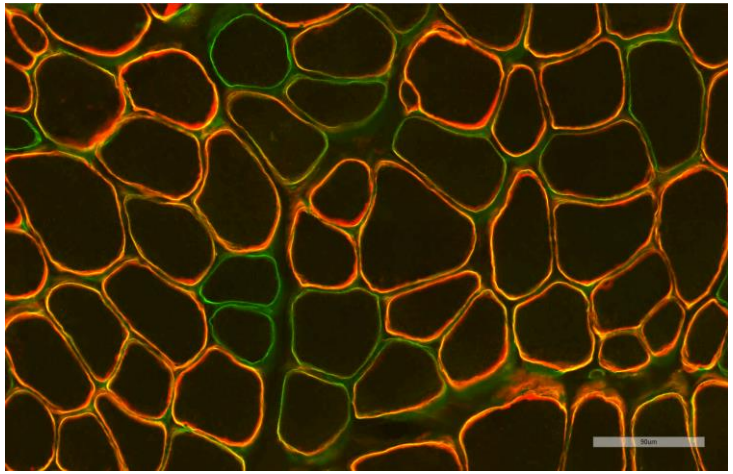
Micro-dystrophin Expression (IF)

	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

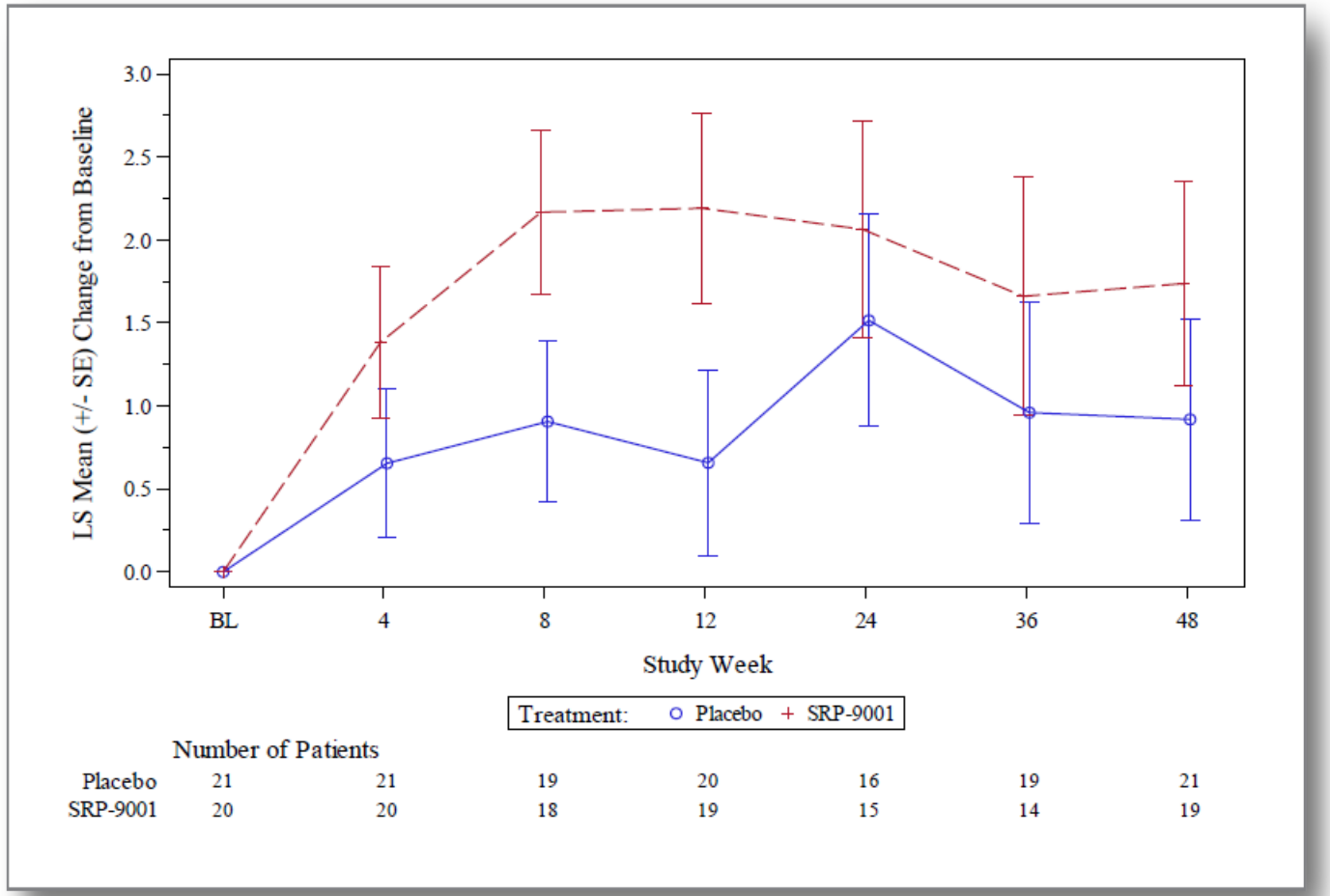
	Merosin (membrane label)	Micro-dystrophin	Merge
Baseline			NOT APPLICABLE
SRP-9001			

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)

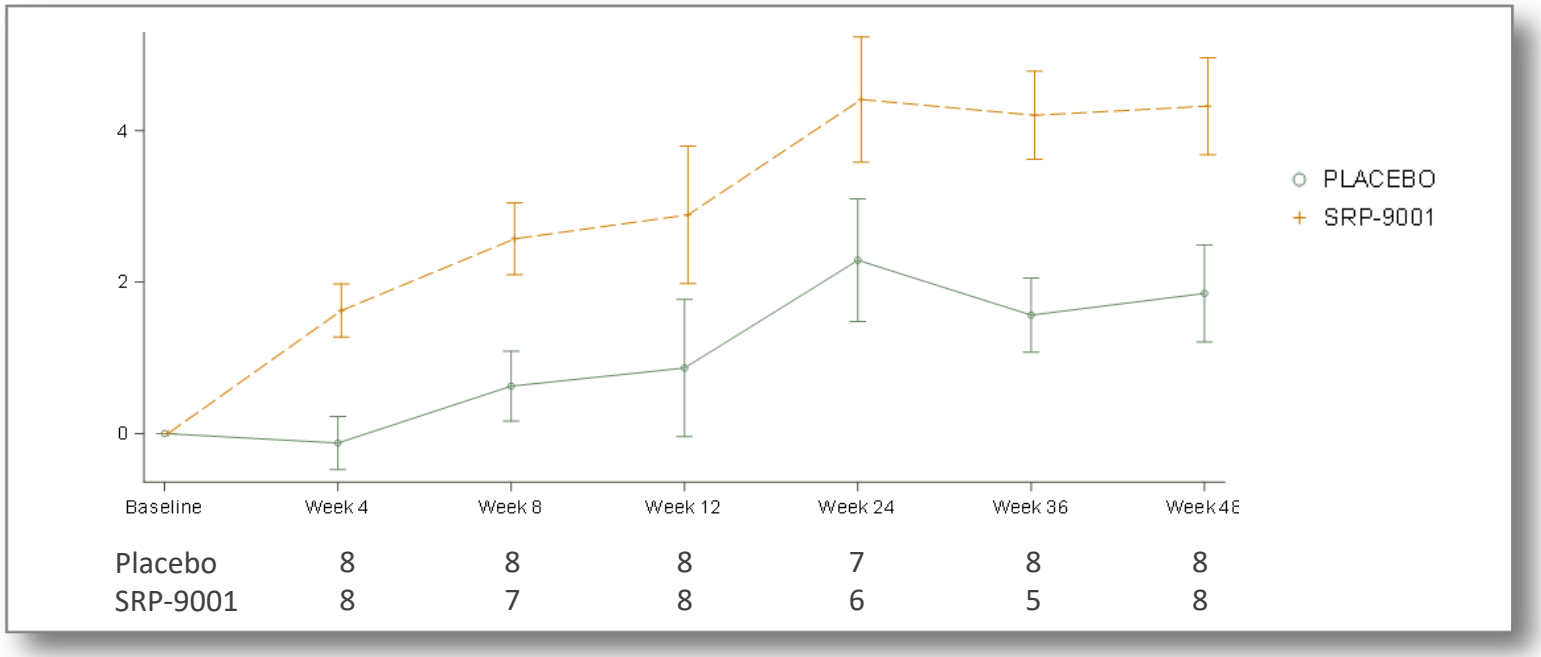


- Separation shown at every timepoint between SRP-9001 and placebo groups
- Baseline analysis at 48 weeks:
  - Treatment group showed 1.7-point increase compared to baseline (P=0.0090)
  - Placebo group showed 0.9-point increase compared to baseline (P=0.1411)

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

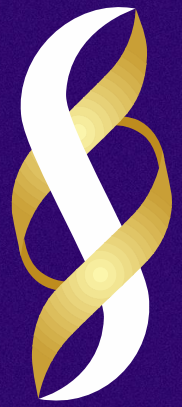
Next Steps

- 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



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