SAREPTA THERAPEUTICS

Clinical Results and Plan Forward:

SRP-1001 for Facioscapulohumeral Muscular Dystrophy Type 1 (FSHD1)

SRP-1003 for Myotonic Dystrophy Type 1 (DM1)

March 25, 2026

Today's Speakers

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Opening Remarks

Doug Ingram
Chief Executive Officer

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 related to our priorities, technologies and research and development programs; early and limited results from our Phase 1/2 clinical trials of SRP-1001 and SRP-1003; the potential benefits of the TRiM™ platform, SRP-1001, and SRP-1003, including, but not limited to, their potential to be differentiated and best-in-class; and expected plans and milestones, including the timing of our upcoming data readouts and milestones for our siRNA programs.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's 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, and 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; differences in trial design between early-stage clinical trials and later-stage clinical trials make it difficult to extrapolate the results of earlier clinical trials to later clinical trials; interim, initial, "topline" and preliminary data from our clinical trials that we announce or publish from time to time may differ materially from final data, including data and analyses of additional cohorts; third-party data used for illustrative purposes are derived from different clinical trials conducted at different times, with differences in trial design and patient populations, and no head-to-head clinical trials have been conducted, and as a result, cross-trial comparisons may not be reliable; pre-clinical and clinical data are often susceptible to varying interpretations and analyses, and the different methodologies, assumptions and applications we utilize to assess particular safety or efficacy parameters may yield different statistical results, and even if we believe data collected from clinical trials of our product candidates are promising, these data may not be sufficient to support approval, including that FDA or foreign regulatory authorities could interpret these data in different ways from us; we rely on third parties, including in some cases our strategic partners, to conduct some aspects of our early-stage research and pre-clinical and clinical development, and accordingly, the inadequate performance by or loss of any of these third parties could affect the development and commercialization of our product candidate development, including delayed timelines; certain programs may never advance in the clinic or may be discontinued for a number of reasons, including regulators imposing a clinical hold and us suspending or terminating clinical research or trials; we face intense competition and rapid technological change, which may result in other companies discovering, developing or commercializing competitive products before our candidates; our product candidates may cause undesirable side effects, result in new safety signals or have other properties that could delay or prevent regulatory approval of product candidates, limit the commercial potential or impact the potential adoption of such candidates, if approved, or result in significant negative consequences following any potential marketing approval; we may not be able to execute on our business plans, including meeting expected or planned regulatory milestones and timelines, clinical development plans, and bringing products to markets for various reasons including possible limitations of financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, and 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 those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K 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, except as required by law.

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Strengthening Sarepta's commitment to and leadership position in rare, neuromuscular diseases

Early clinical data strengthen potential best-in-class approach for SRP-1001 and SRP-1003 to treat FSHD1 and DM1

- **Maximizing Therapeutic Delivery and Effect to Muscle**
  - Clinical experience to date matches animal-model data: The TRiM™ platform's αvβ6 integrin-targeting ligand drives potentially greater construct muscle delivery than other approaches
  - No saturation of muscle siRNA uptake observed to date, with consistent dose-dependent increases in plasma and muscle drug exposures across clinical and nonclinical studies
  - Enhanced TRiM™ siRNA chemistry improves drug stability, potentially enabling less frequent and optimized clinical dosing regimen
- **Reaching our Target to Impact Disease**
  - Successful target engagement with emerging biomarker evidence of potentially meaningful treatment efficacy
- **No Indication of Dose-related Safety Signals that would Limit Continued Dose Escalation**
  - Favorable safety and tolerability profile to date

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Differentiated Integrin-Based Delivery Technology: Targeting the Biological Root Cause of Disease

Louise Rodino-Klapac, PhD
President, R&D and Technical Operations

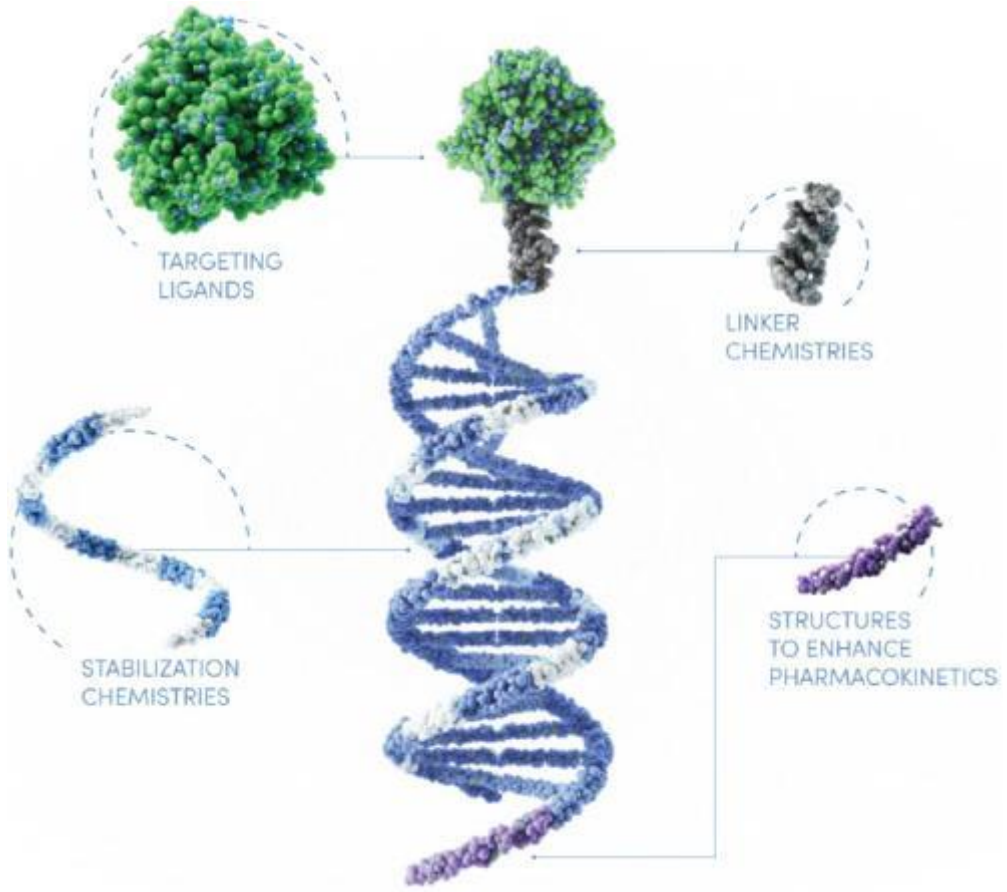
Components of TRiM™ and the science that enables our pipeline

Delivery

Tissue-specific ligands (peptide, small molecule, Ab) designed to deliver siRNA to Liver, CNS, Muscle, Heart etc.

siRNA Chemistry

Established and experimental chemical modifications designed to optimize potency, duration, and target specificity



Linker

Designed to provide stability/orientation. Linker is selected to optimize efficacy, reduce tox, and CMC complexity. Often, many known linkers are tested to select most optimal combination for each program

PK/PD Structures

Certain programs have novel features aimed at enhancing half life (e.g., lipid for SRP-1001)

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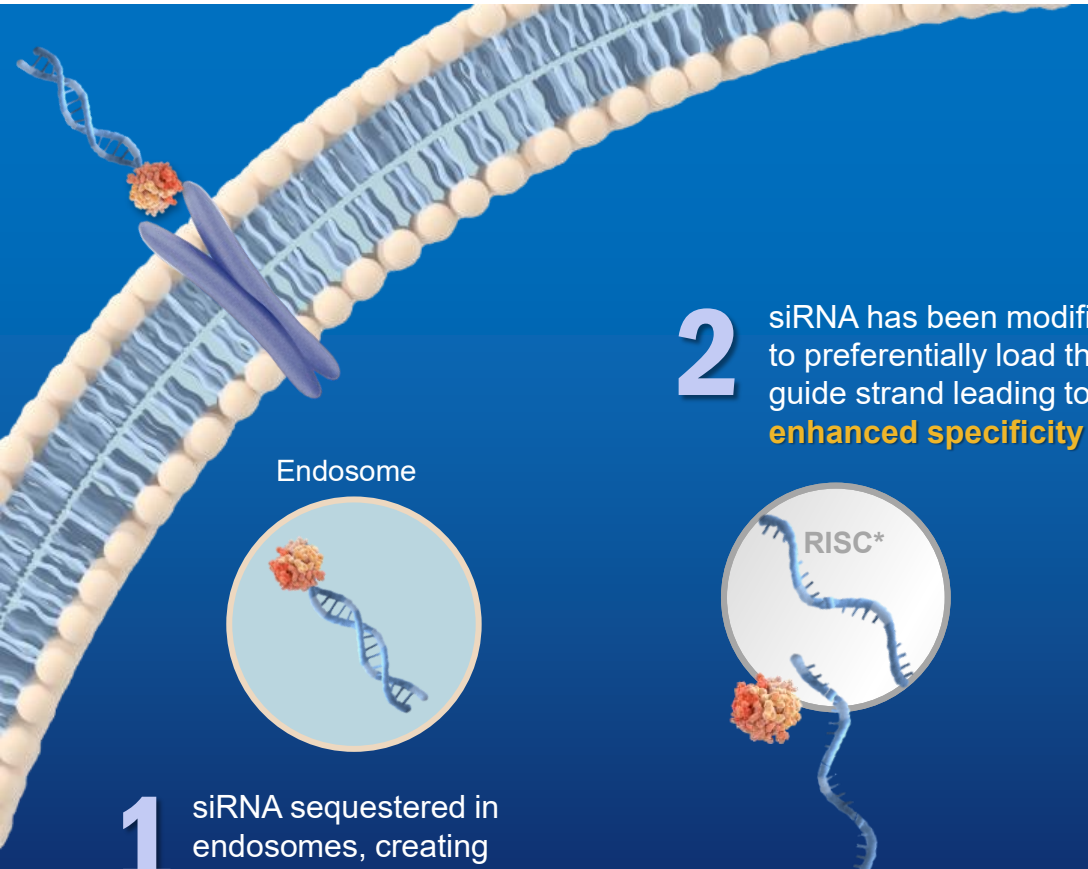
Distinct Mechanism of Action: siRNA-mediated mRNA degradation designed to enable specific target knockdown



Endosome

1 siRNA sequestered in endosomes, creating drug depot that allows for **sustained durability**

2 siRNA has been modified to preferentially load the guide strand leading to **enhanced specificity**

Passenger strand is removed

3 Complementary pairing

target mRNA

4 Catalytically active RISC cleaves multiple target mRNAs, resulting in **superior potency** over RNaseH1 dependent ASOs

Target mRNA degradation

*RNA-induced Silencing Complex (RISC)

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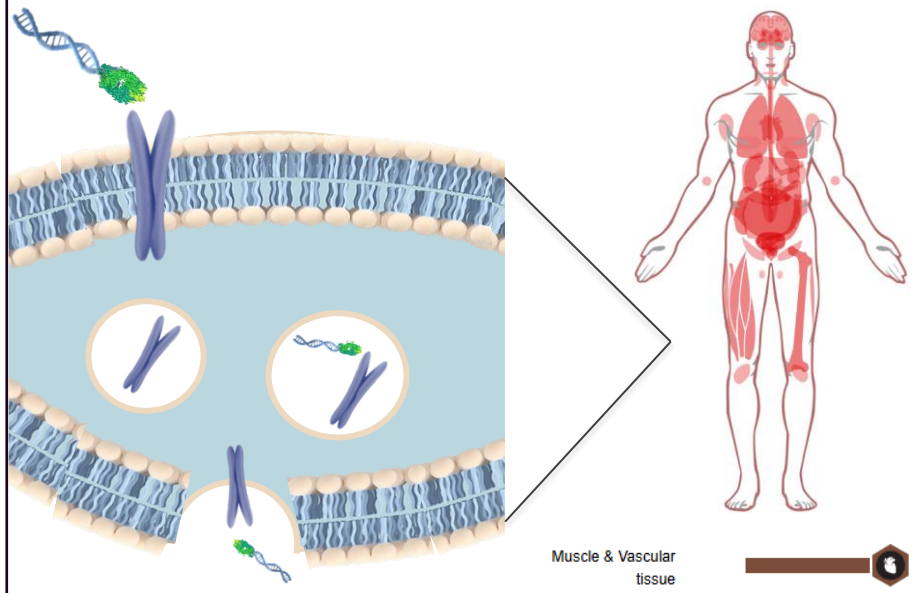
Targeting $\alpha_v\beta_6$ integrin over TfR1 for greater muscle uptake

High expression and surface availability of $\alpha_v\beta_6$ in muscle make it an ideal receptor for targeted siRNA delivery

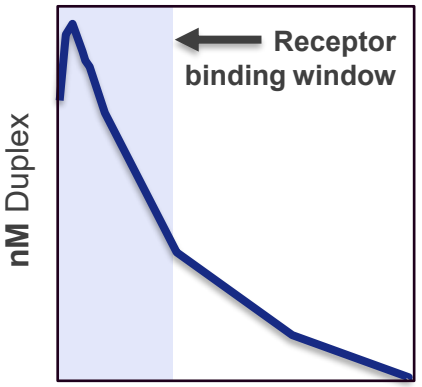
TfR1: 5%[1] of receptors available for binding on surface at any one time; majority are intracellular trafficking transferrin or being recycled to membrane



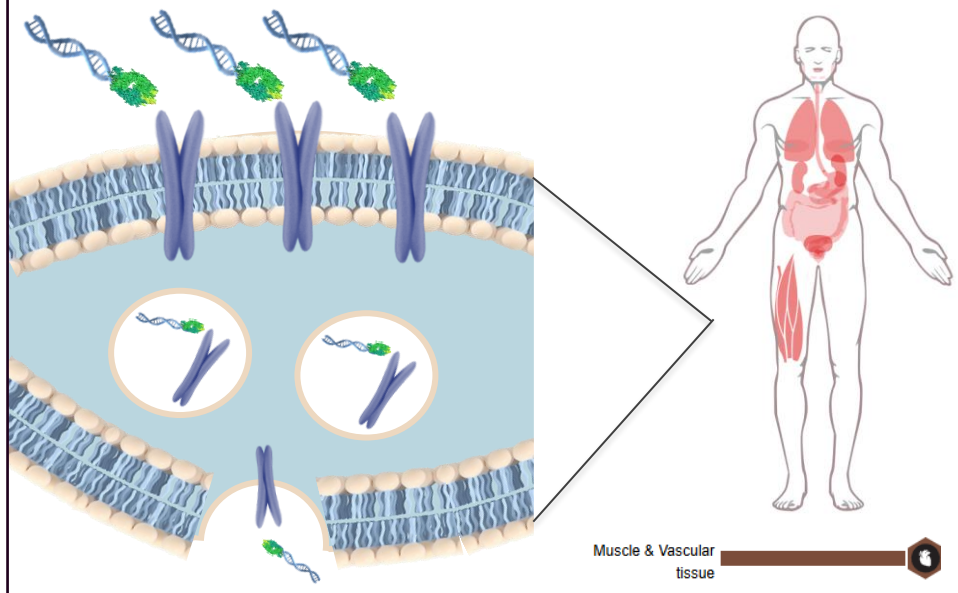
TFRC Protein Atlas

IV Delivery

Receptor recycling times range from 30 minutes to a few hours[1,2], creating short window for receptor-mediated endocytosis



$\alpha_v\beta_6$: ~40%[3] of receptors available for binding on surface at any one time; higher muscle and vascular expression than TfR1



ITGB6 Protein Atlas

[1] https://doi.org/10.3390/ph14060535
[2] https://doi.org/10.3389/fcell.2022.920303
[3] http://dx.doi.org/10.1159/000443180

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Potential advantages of siRNA integrin approach and best-in-class therapies to treat FSHD1 and DM1

Better delivery to muscle with integrin $\alpha_v\beta_6$

Optimal clinical impact	$\alpha_v\beta_6$ siRNA	TfR1mAb siRNA	TfR1fAb ASO
Potency	✓	✓	
Better muscle penetration *(e.g., delivers more Rx to muscle)*	✓		
Safety and lack of dose limiting toxicity	✓		
Longer dose interval	✓		

The tolerability of the $\alpha_v\beta_6$-targeting platform may allow for best-in-class efficacy, through higher doses and greater muscle concentration without dose-limiting AEs.

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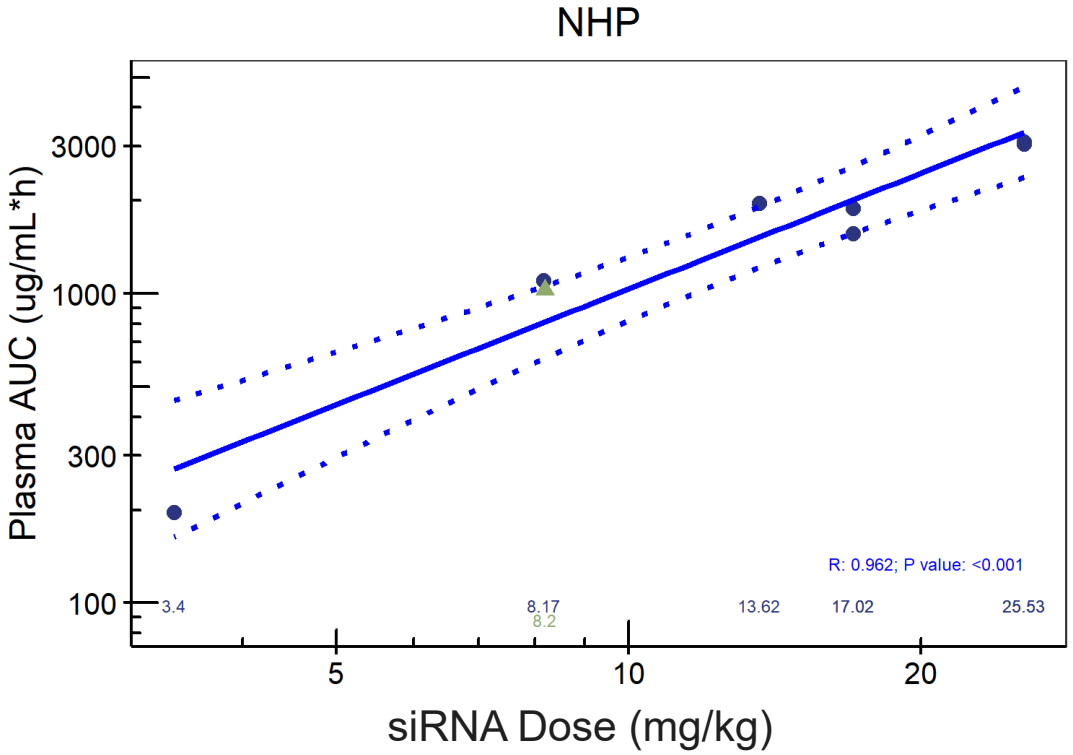
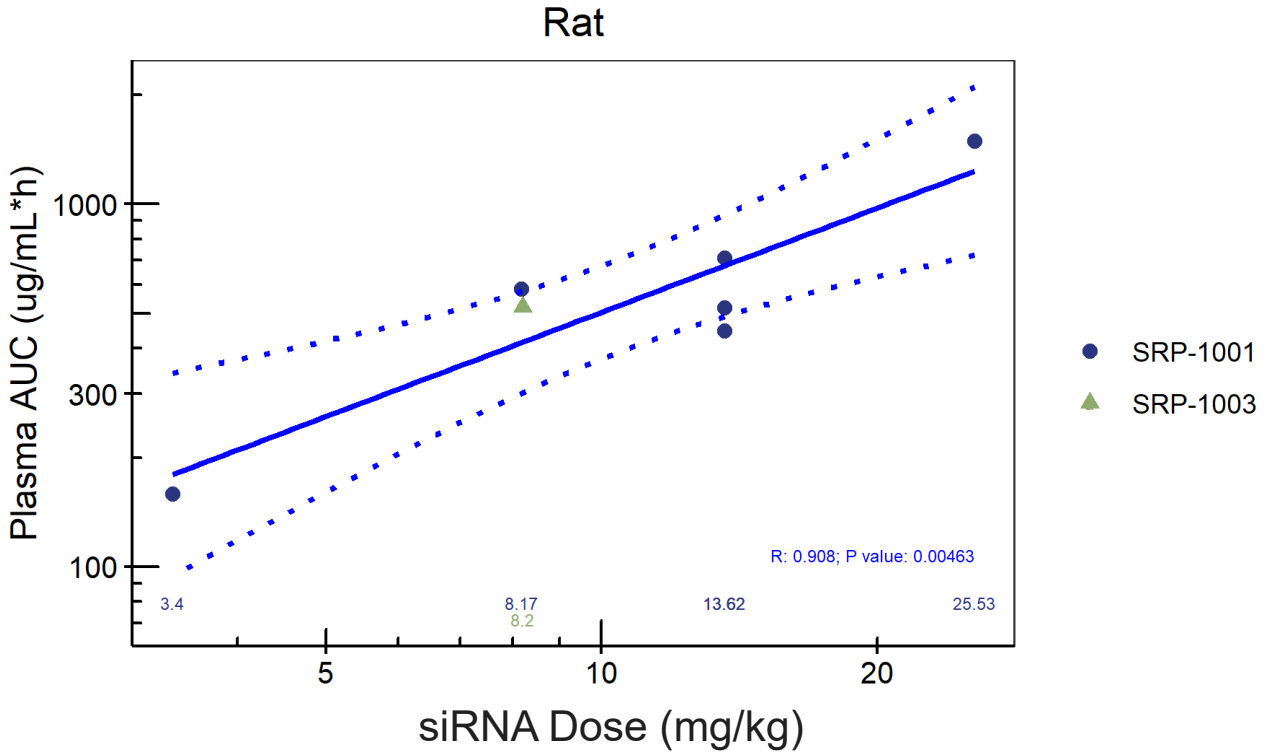
Pre-clinical in-vivo dose-dependent increase in plasma exposure via the TRiM™ platform

The dose-dependent increase and absence of saturation in NHP plasma exposure (up to 26 mg/kg siRNA dose) provide a clear path to increased muscle delivery and stronger pharmacodynamic activity for both SRP-1001 and SRP-1003





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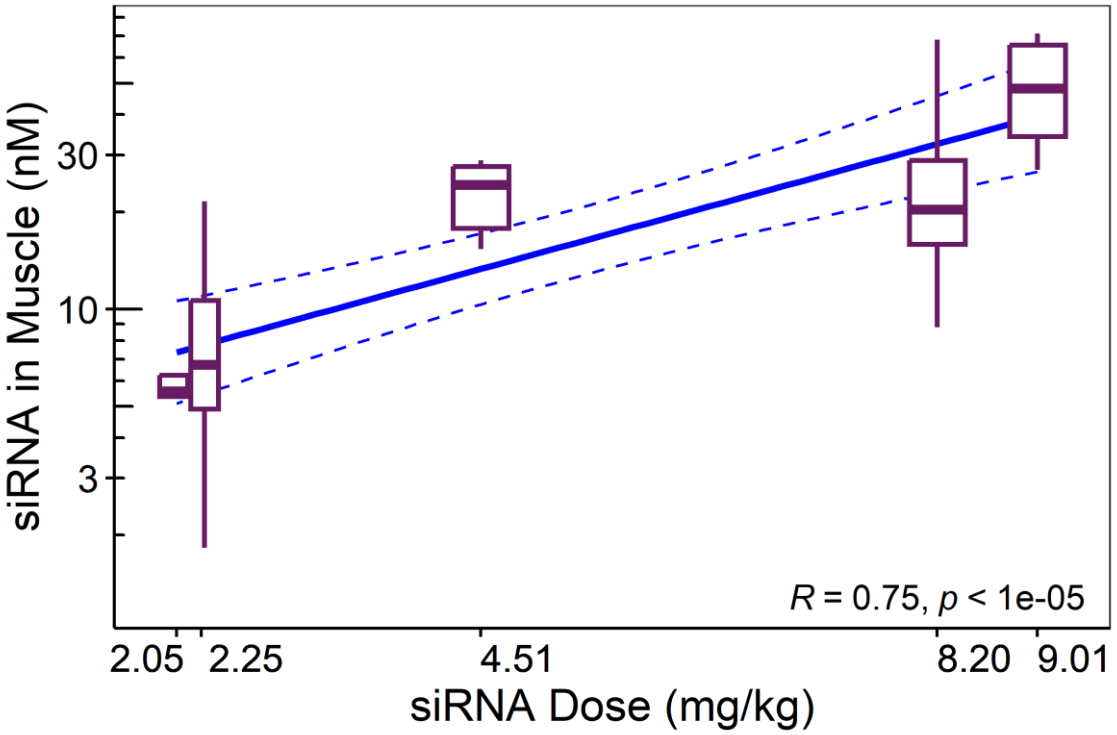
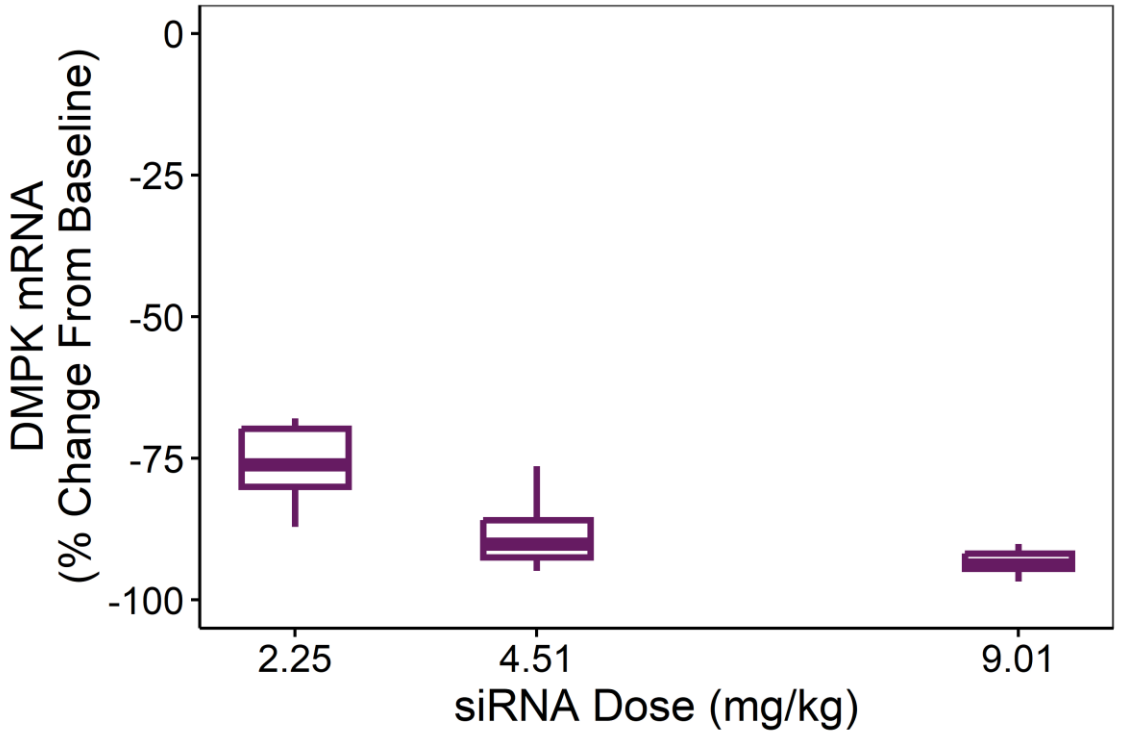
Pre-clinical in-vivo muscle siRNA delivery and mRNA knock-down via the TRiM™ platform

The linear increase in SRP-1003 plasma exposure translated into enhanced and dose-dependent muscle drug delivery, driving robust target engagement through maximal DMPK mRNA knockdown in NHPs





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Clinical Results:
SRP-1001 (FSHD1)

James Richardson, MA (Oxon), BMBCh, MBA, MRCP (Lon)
Executive Vice President, Chief Medical Officer

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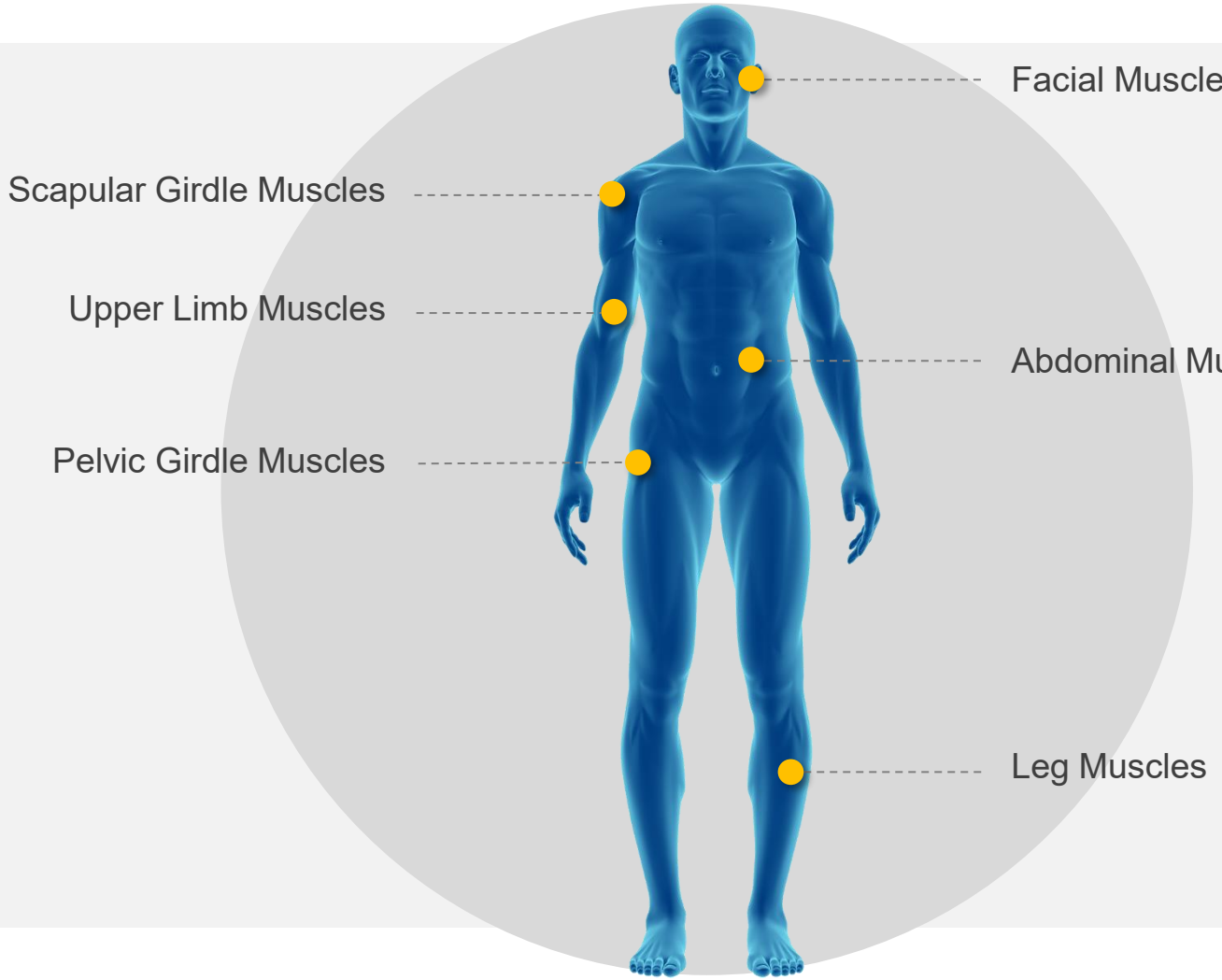
FSHD: Multiple skeletal muscles affected, beginning with the face



Muscle Weakness and Mobility

- ~50% of FSHD patients will progress to becoming dependent on a wheelchair after ~20 years
- >50% of FSHD patients have moderate to extreme level of mobility restrictions

"DUX4 Expression in FSHD Muscles: Focus on Its mRNA Regulation" *Journal of Personalized Medicine.*
"DUX4 Signaling in the Pathogenesis of Facioscapulohumeral Muscular Dystrophy", *International Journal of Molecular Sciences.*
"Genetic Cause of FSHD", *FSHD Society*; Accessed March 2022.

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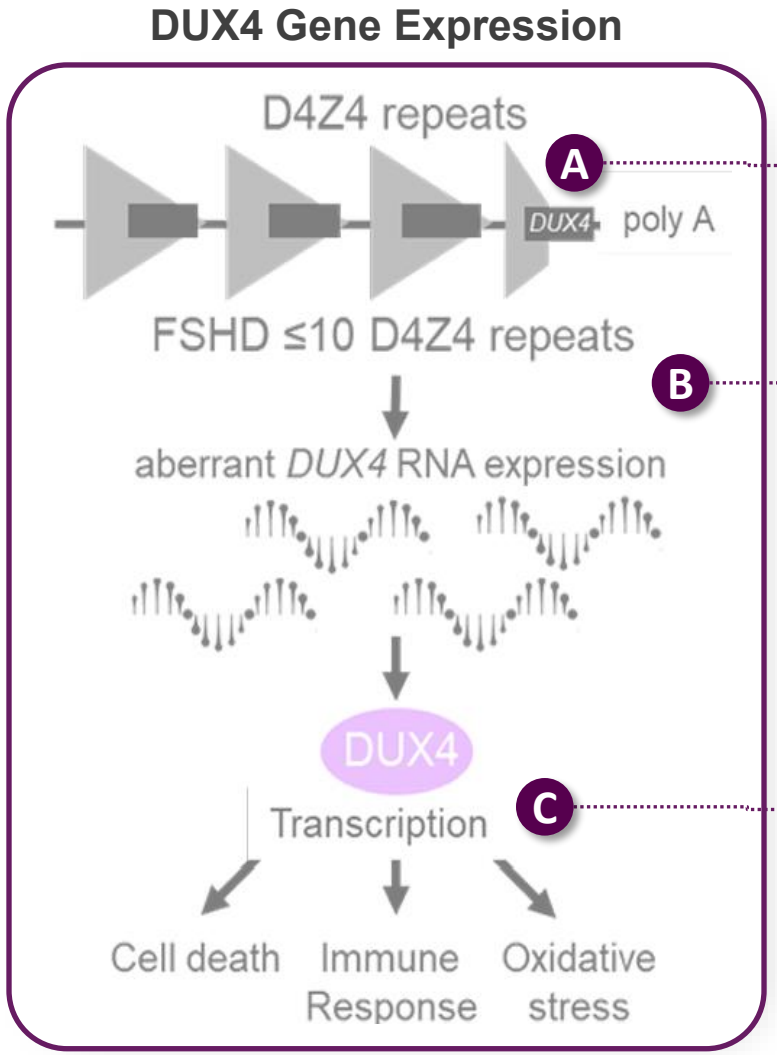
FSHD is caused by the contraction of the D4Z4 repeat, leading to the abnormal expression of DUX4, which is typically silenced early in life



DUX4 Transcription Factor

FSHD genotype leads to aberrant DUX4 expression

Downstream Cellular Consequences

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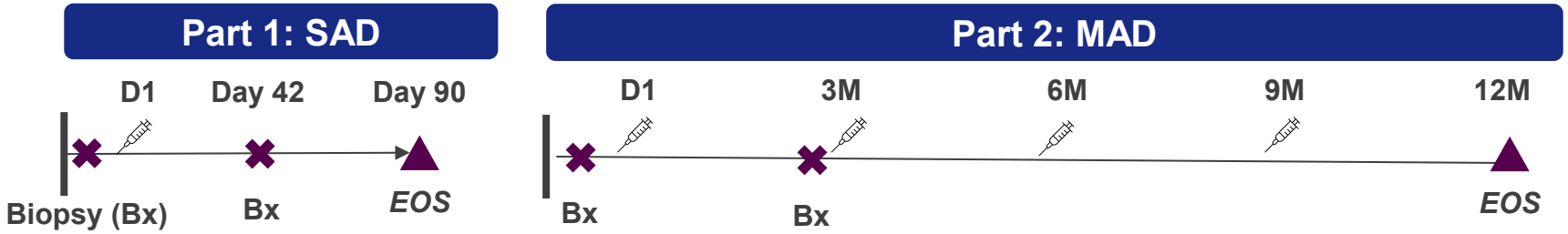
Study SRP-1001-101: A phase 1/2 randomized, placebo-controlled trial in participants aged 16-70 with FSHD1



Cohort Design

SAD

C1-4: n=6, 2:1

- Cohort 1 (1.02 mg/kg siRNA)
- Cohort 2 (2.04 mg/kg siRNA)
- Cohort 3 (4.08 mg/kg siRNA)
- Cohort 4 (8.17 mg/kg siRNA)

MAD

C5-7: n up to 12, 3:1

- Cohort 5 (4.08 mg/kg siRNA Q3M)
- Cohort 6 (8.17 mg/kg siRNA Q3M)
- Optional Cohort 7 (8.17 mg/kg siRNA Q3M), 16-70 y/o

Endpoints

Primary:
- Safety and tolerability

Secondary:
- PK in plasma

Key Exploratory Endpoints:
- siRNA muscle concentration
- Circulating DUX4 biomarker & DUX4 regulated gene expression
- Serum CK
- Functional: RWS, 6MWT, QMT, TUG
- PROs: Pain VAS, CIS, PGIC

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Baseline characteristics

	SAD				MAD		Overall		
	Cohort 1 (1.02 mg/kg) N=5	Cohort 2 (2.04 mg/kg) N=4	Cohort 3 (4.08 mg/kg) N=4	Cohort 4 (8.17 mg/kg) N=5	Cohort 5 (4.08 mg/kg) N=11	Cohort 6 (8.17 mg/kg) N=11	Pooled Placebo N=16	Treated N=40	Total N=56
Age (years)	36.2 (14.15)	49.0 (6.00)	42.8 (9.29)	34.4 (11.44)	50.2 (9.13)	51.0 (13.35)	42.8 (13.75)	45.8 (12.41)	44.9 (12.75)
Female, n (%)	0	2 (50.0)	1 (25.0)	0	2 (18.2)	2 (18.2)	5 (31.3)	7 (17.5)	12 (21.4)
Weight (kg)	84.320 (12.6428)	84.750 (25.9976)	78.063 (7.1672)	81.664 (15.0810)	83.050 (12.9954)	82.645 (15.7689)	80.627 (14.2110)	82.596 (14.3478)	82.033 (14.2076)
BMI (kg/m²)	25.66 (4.868)	27.88 (7.571)	26.20 (2.035)	24.46 (3.812)	26.10 (3.546)	26.85 (3.177)	25.07 (4.036)	26.23 (3.910)	25.90 (3.945)
CSS at Baseline	4.4 (1.95)	6.5 (1.29)	5.8 (2.06)	6.6 (1.14)	6.1 (1.14)	5.2 (1.33)	5.0 (1.67)	5.7 (1.51)	5.5 (1.57)

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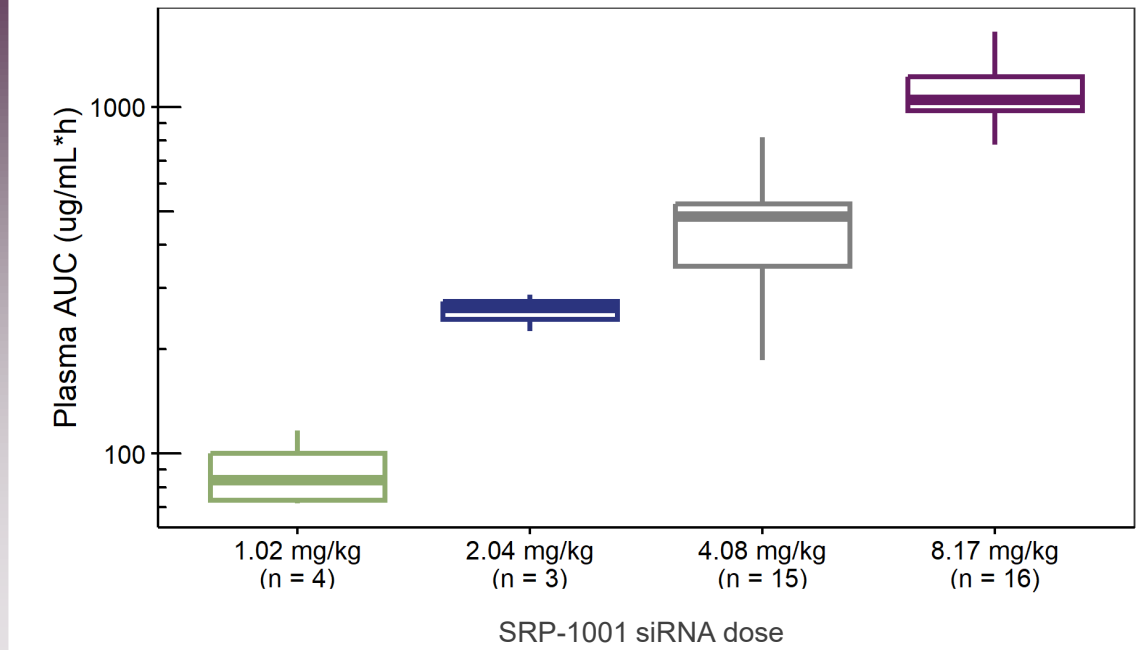
SRP-1001 shows consistent dose-dependent increase in plasma exposure up to the highest dose cohort tested so far

Plasma Exposure by Dose Level



Dose-dependent increase in plasma AUC up to 8 mg/kg, representing the highest siRNA dose evaluated to date for FSHD

AUC = area under the plasma concentration-time curve between time zero to 8 hours. Plasma PK includes data following Day 1 dose across SAD and MAD cohorts.

One subject in Cohort 1 and 2 (2.08 mg/kg) with all BLOQ samples was excluded from the analysis.

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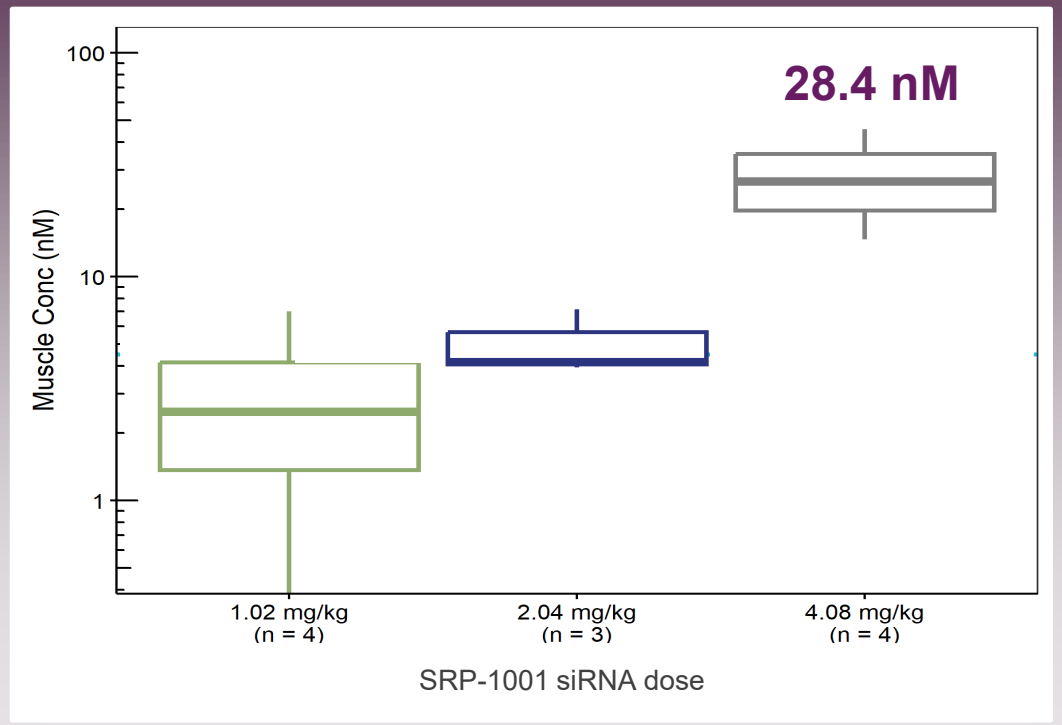
Single dose of SRP-1001 delivers 6-fold greater siRNA muscle concentration than multiple doses of transferrin-targeting ligand

SRP-1001 Muscle Concentration by Dose Level



- Dose-dependent increase in muscle concentration up to 4.08 mg/kg, representing the highest siRNA dose with muscle PK data to date
- At 42 days, SRP-1001 has achieved **28.4 nM** muscle concentration without dose-limiting toxicities that would limit further dose escalation. In comparison, the TfR1 mAb-siRNA construct has reported a median of **4.5 nM** muscle concentration at its chosen pivotal trial dose (n=7)

Muscle PK includes data from SAD cohorts.

Delivering on the RNA Revolution, Avidity corporate presentation, August 2024.
Data are derived from different clinical trials conducted at different times, with differences in trial design and patient populations.
As a result, cross-trial comparisons may not be reliable, and no head-to-head clinical trials have been conducted.
One subject in Cohort 1 (1.02 mg/kg) and 2 (4.08 mg/kg) with BLOQ samples was excluded from the analysis.

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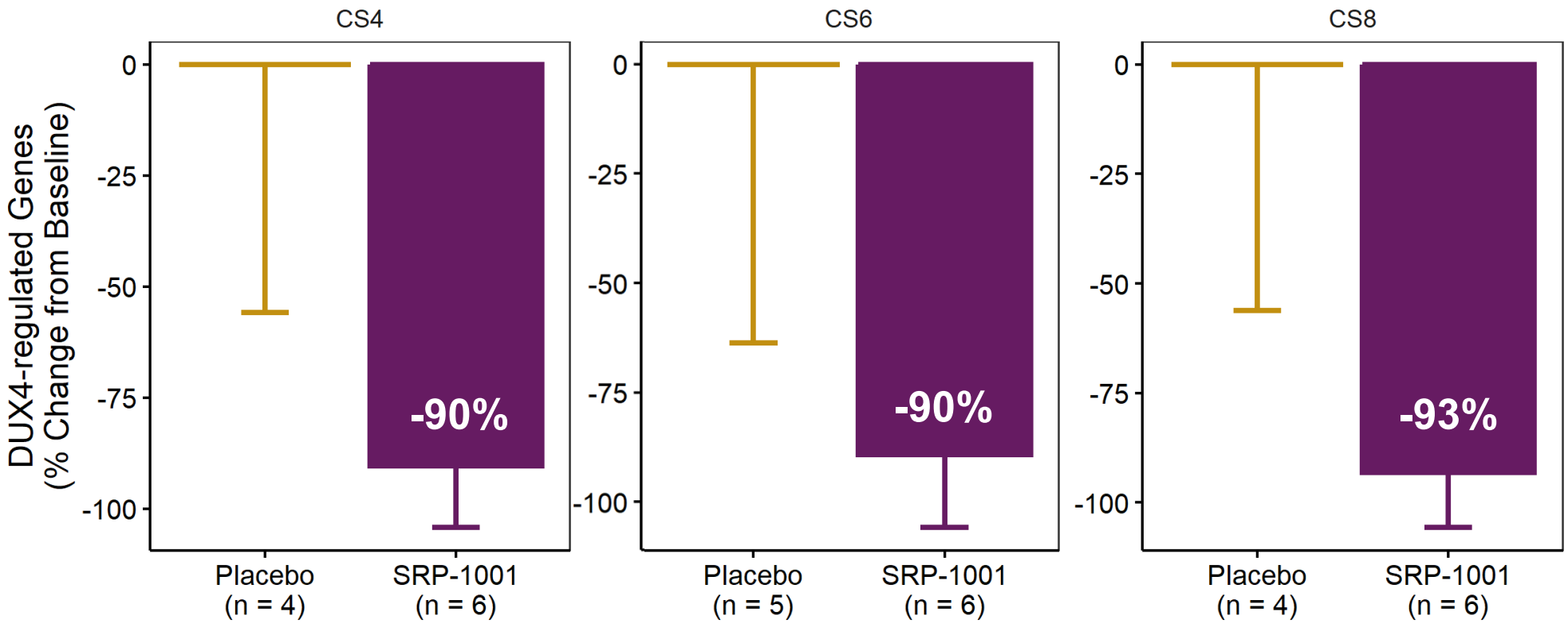
SRP-1001 suppresses DUX4-regulated genes after a single dose



SRP-1001 treatment cohorts pooled from siRNA dose cohort 1 (1.02 mg/kg), cohort 2 (2.04 mg/kg), and cohort 3 (4.08 mg/kg).

- *qPCR composite scores for CS4 (MBD3L2, TRIM43, KHDC1L, LEUTX), CS6 (MBD3L2, KHDC1L, CCNA1, PRAMEF5/6, SLC34A2, ZSCAN4), and CS8 (MBD3L2, TRIM43, KHDC1L, LEUTX, CCNA1, PRAMEF5/6, SLC34A2, ZSCAN4) were calculated as the sum across contributing target genes and normalized to baseline and placebo.
- One subject in Cohort 1 (1.02 mg/kg) excluded with all BLOQ plasma and muscle samples. One subject in Cohort 2 (2.08 mg/kg) with all BLOQ plasma and muscle samples was excluded from the analysis. Additional subjects excluded if BLOQ for dux4 genes.
- Muscle Dux4 regulated genes were characterized in MRI-guided biopsy samples collected from lower-body muscles.

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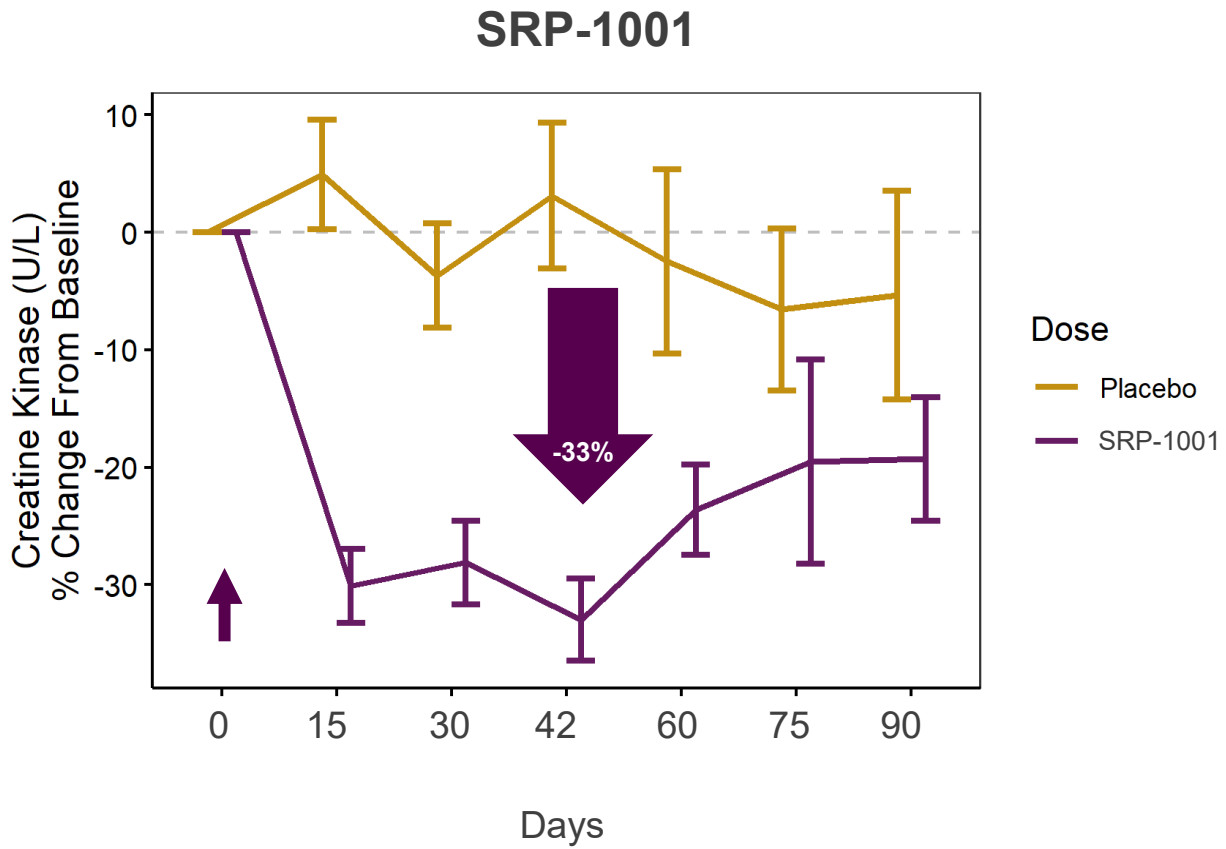
SRP-1001 drives rapid reduction in serum creatine kinase (CK) with a single dose of SRP-1001

SRP-1001 achieved a 33% reduction in CK vs placebo at Day 42



Data represent mean percent change from baseline ± SEM. Placebo (n = 16) and SRP-1001 treatment cohorts pooled from siRNA doses at 4.08 mg/kg and 8.17 mg/kg (n = 31) following a single dose administration.

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SRP-1001-101: Reassuring safety profile to date with no serious related TEAEs

TEAE Category	SAD (Active and Placebo)				MAD (Active and Placebo)		Overall
	Cohort 1 (1.02 mg/kg) N=7 n (%)	Cohort 2 (2.04 mg/kg) N=7 n (%)	Cohort 3 (4.08 mg/kg) N=6 n (%)	Cohort 4 (8.17 mg/kg) N=7 n (%)	Cohort 5 (4.08 mg/kg) N=14 n (%)	Cohort 6 (8.17 mg/kg) N=15 n (%)	Total N=56 n (%)
Any TEAE	7 (100)	4 (66.7)	5 (83.3)	5 (71.4)	10 (71.4)	12 (80.0)	43 (76.8)
Serious TEAE	0	1 (14.3)	0	0	0	0	1 (1.8)
Related TEAE [1]	2 (28.6)	0	1 (16.7)	0	6 (42.9)	1 (6.7)	10 (17.9)
Related Serious TEAE [1]	0	0	0	0	0	0	0
Related TEAE Leading to Drug Withdrawal	0	0	0	0	0	0	0
Related TEAE Leading to Study Discontinuation	0	0	0	0	0	0	0
Related TEAE Leading to Death	0	0	0	0	0	0	0

% = 100 x n/N, N = number of subjects in the population, n = number of subjects reporting event.
Treatment-emergent AEs will be defined as AEs with onset after administration of the study drug, or when a pre-existing medical condition increases in severity or frequency after study drug administration.
Adverse Events are coded using MedDRA Version 25.0.
[1] AE is considered treatment-related if the relationship to the study drug is 'Related' or missing. Subjects are counted only once at the maximum relationship.
[2] AE records with missing severity values will be imputed as 'Severe'. Subjects are counted only once at the maximum severity.

Majority of TEAEs were mild to moderate in severity

- 1 (1.8%) serious TEAE (chest discomfort) was considered not related to study drug, and is also the only severe TEAE in the study
- Most (observed in ≥ 10% of subjects) common TEAEs
  - Headache (16.1%)
  - Upper respiratory tract infection (14.3%)
- No TEAEs occurred ≥ 20% of participants
- No related TEAEs led to death, study drug discontinuation or study discontinuation

TEAEs do not appear to be dose dependent based on TEAE frequencies at the different SAD and MAD doses

SRP-1001 is safe and well tolerated to date in the ongoing study

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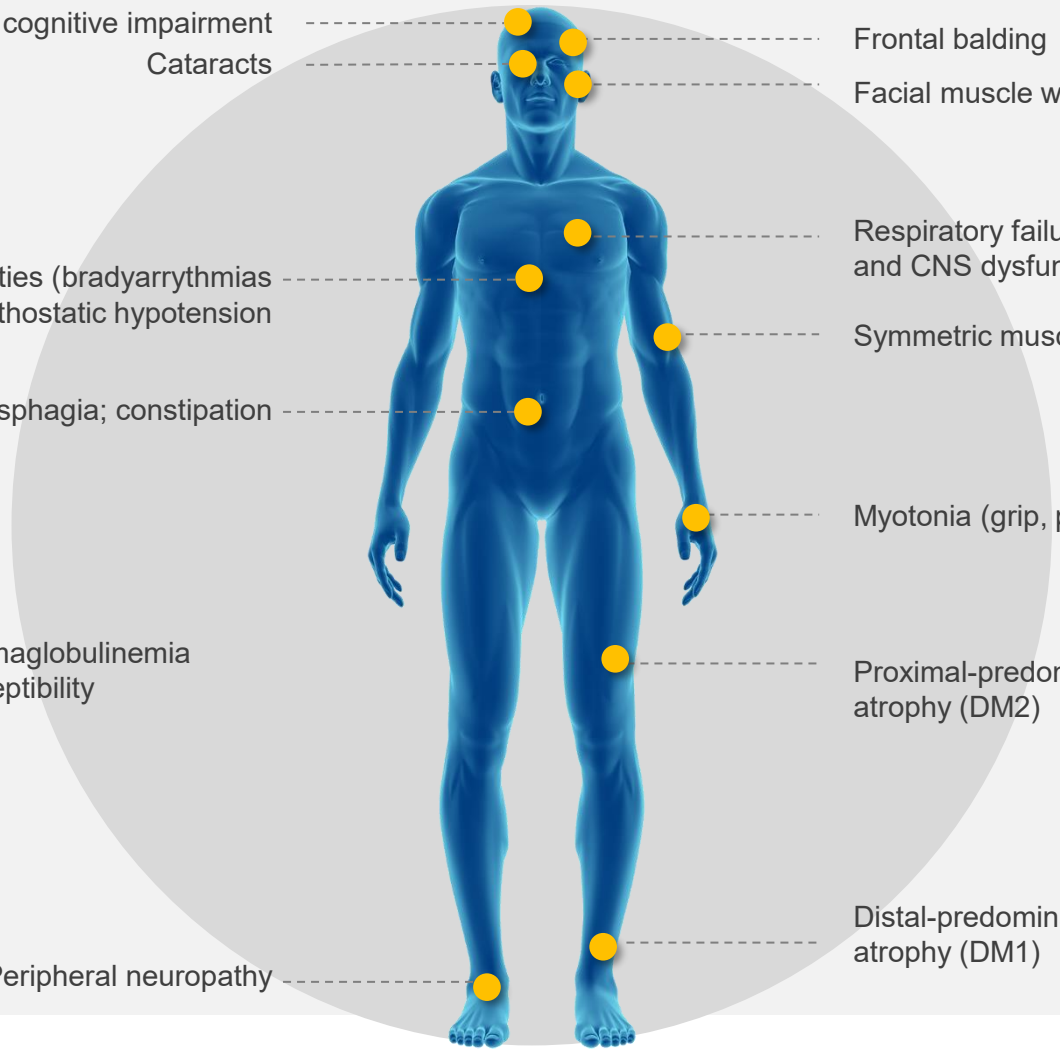
Clinical Results: SRP-1003 (DM1)

James Richardson, MA (Oxon), BMBCh, MBA, MRCP (Lon)
Executive Vice President, Chief Medical Officer

DM1: A multi-system disorder impacting skeletal and smooth muscle along with other organs



Fatigue, apathy, cognitive impairment

Cataracts

Frontal balding

Facial muscle weakness; TMJ dysfunction

Respiratory failure (skeletal muscle weakness and CNS dysfunction)

Cardiac conduction abnormalities (bradyarrythmias and ventricular tachycardia); orthostatic hypotension

Symmetric muscle pain

GI dysmotility; dysphagia; constipation

Myotonia (grip, percussion, electrical)

Systemic Symptoms

- Endocrine dysfunction
- IgG and IgM hypogammaglobulinemia
- Increased cancer susceptibility

Proximal-predominant muscle weakness and atrophy (DM2)

Distal-predominant muscle weakness and atrophy (DM1)

Peripheral neuropathy

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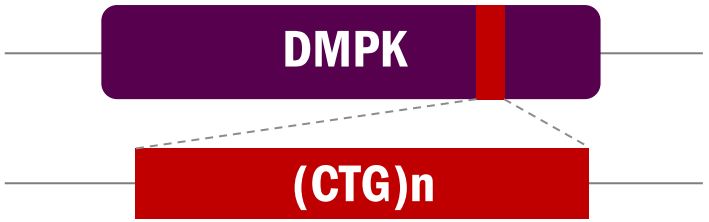
DM1: Expanded DMPK repeat, leading to multi-system impact

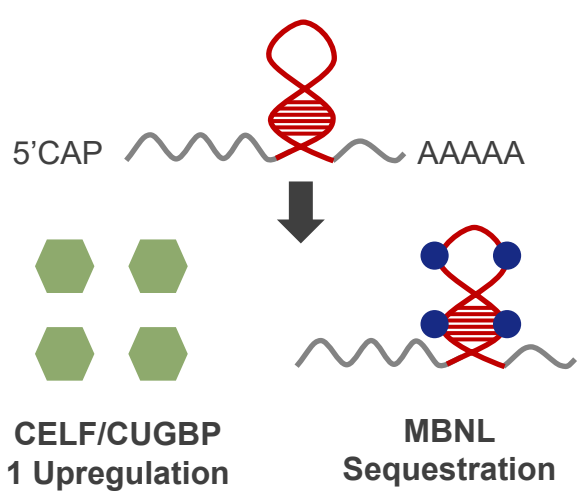
Targeting and knockdown of DMPK ***MUST*** occur in nucleus for therapeutic benefit

DM1 Genetic Cause



DNA

Expanded tri-nucleotide repeat in the *DMPK* gene (50 – 1000 repeats)



RNA

Repeats accumulate in the nucleus and create 3D structures that bind MBNL and alter CUGBP1 expression

Downstream mis-splicing of proteins implicated in cognitive, skeletal, and cardiac muscle function

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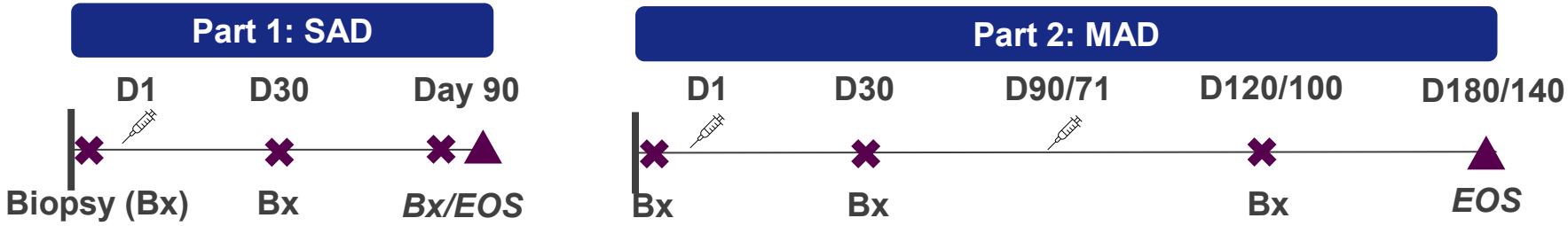
Study SRP-1003-101: A phase 1/2 randomized, placebo-controlled trial in participants aged 18-65 with DM1



Cohort Design

SAD

Cohort 1
(1.02 mg/kg siRNA) n=12

Cohort 2
(2.05 mg/kg siRNA) n=6

MAD

Cohort 3
(3.07 mg/kg siRNA Q12W) n=6

Cohort 4
(4.10 mg/kg siRNA Q12W) n=6

Cohort 5
(8.20 mg/kg siRNA Q10W) n=12

Endpoints

Primary:
- Safety and tolerability

Secondary:
- PK in plasma

Key Exploratory Endpoints:
- siRNA muscle concentration
- DMPK mRNA KD
- DMPK splicing indices
- Functional (secondary in C5): vHOT, QMT, 10MWR, TUG
- PROs: CIS, PGIC, DM1-Activ-C, MDHI (MDHI and DM1-Activ-C secondary for C5)

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Baseline characteristics

	SAD		MAD			
Mean (sd) or Freq (%)	Cohort 1 (1.02 mpkg) N=10	Cohort 2 (2.04 mpkg) N=6	Cohort 3 (<=3.07 mpkg) N=5	Cohort 4 (<=4.10 mpkg) N=4	Placebo N=11	Total N=36
Age (years)	39.2 (11.84)	36.0 (9.06)	47.0 (12.04)	41.8 (7.09)	36.3 (9.16)	39.1 (10.32)
Female, n (%)	6 (60.0)	1 (16.7)	1 (20.0)	1 (25.0)	5 (45.5)	14 (38.9)
Weight (kg)	74.97 (19.112)	63.92 (12.982)	78.34 (6.201)	72.45 (21.849)	75.31 (15.826)	73.42 (15.992)
BMI (kg/m²)	25.84 (3.862)	20.77 (3.132)	25.88 (4.272)	25.23 (5.814)	25.68 (4.656)	24.88 (4.465)

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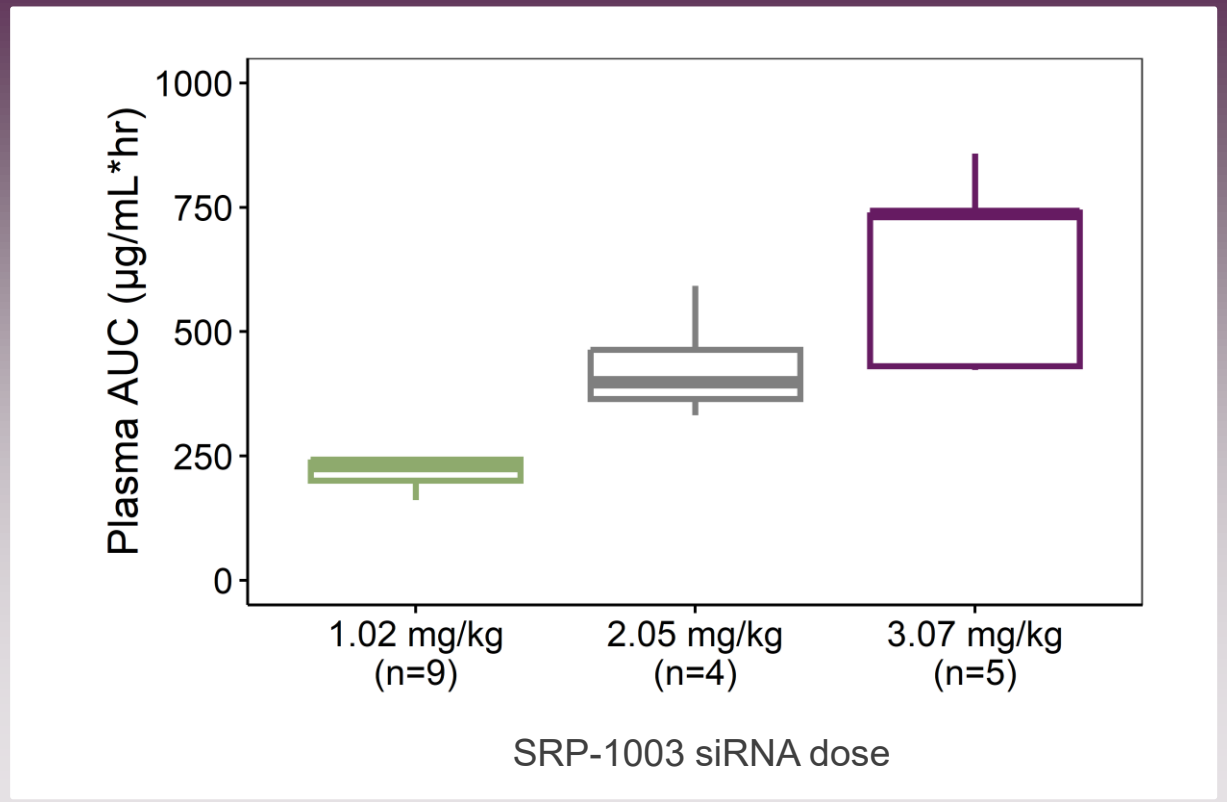
SRP-1003 shows dose-dependent increase in plasma exposure, reinforcing cross-program consistency in the TRiM™ platform

Plasma Exposure by Dose Level



Dose-dependent increase in plasma AUC up to 3.07 mg/kg, representing the highest SRP-1003 dose cohort with data to date

AUC = area under the plasma concentration-time. N=1 in cohort 1 excluded due to study conduct.

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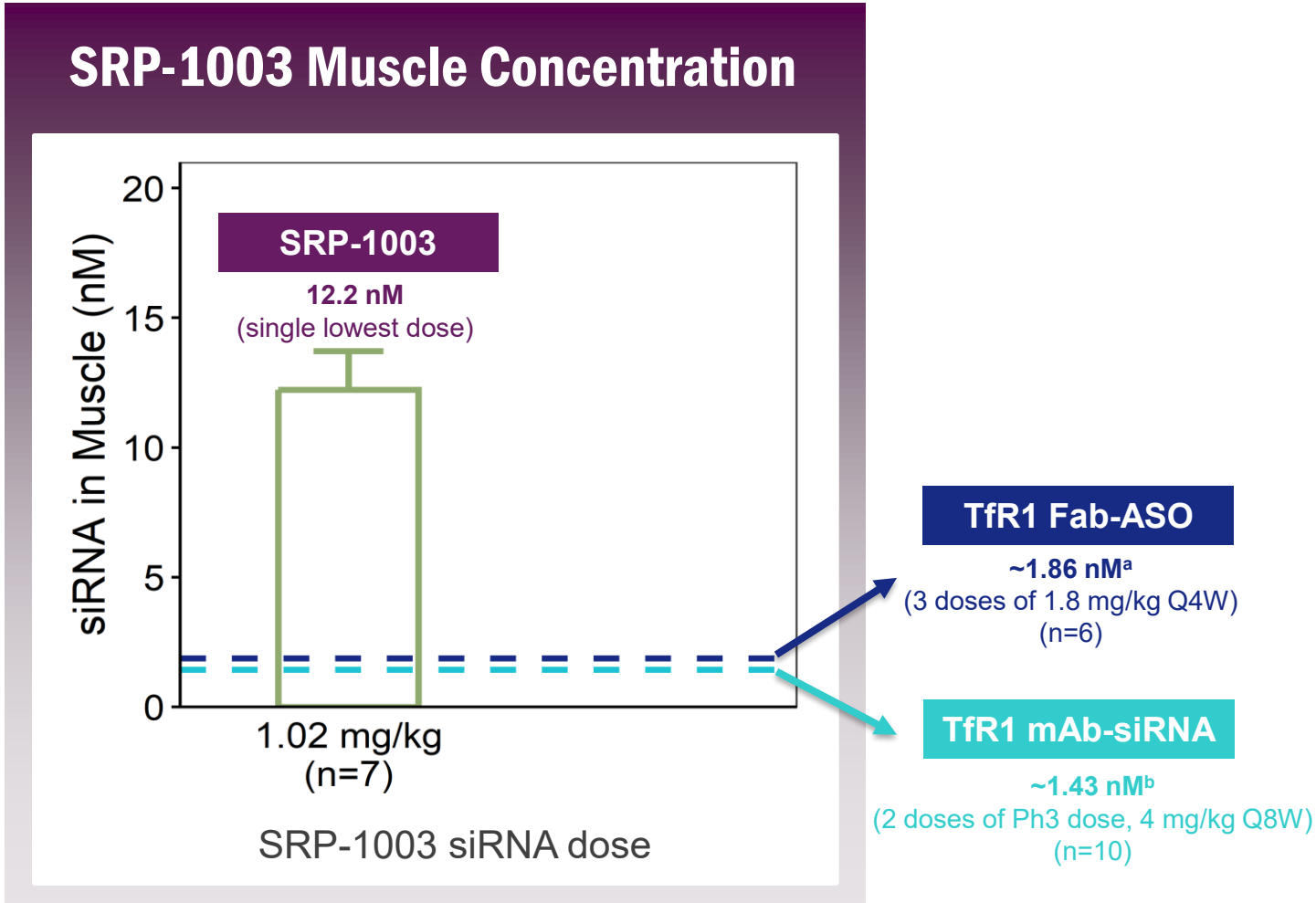
Superior muscle delivery with a single low dose of SRP-1003



- SRP-1003 in the lowest dose cohort (1.02 siRNA mg/kg) mediates robust siRNA delivery into the muscle
- A single low dose of SRP-1003 significantly outperforms repeated dosing of TfR1 mAb-siRNA and TfR1 Fab-ASO in muscle drug delivery

a Initial data from the ACHIEVE Trial of DYNE-101 in Adults With Myotonic Dystrophy Type 1 (DM1), World Muscle Society Annual Congress, Prague, Czechia, October 8–12, 2024; (original values reported in ng).
b An Antibody–Oligonucleotide Conjugate for Myotonic Dystrophy Type 1, *New England Journal of Medicine*, Downloaded from nejm.org, February 23, 2026.
Data are derived from different clinical trials conducted at different times, with differences in trial design and patient populations. As a result, cross-trial comparisons may not be reliable, and no head-to-head clinical trials have been conducted. One subject excluded due to study conduct.

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SRP-1003-101: Reassuring safety profile with no serious related TEAEs to date

TEAE Category	SAD (Active and Placebo)		MAD (Active and Placebo)		Overall
	Cohort 1 (1.02 mg/kg) N=14 n (%)	Cohort 2 (2.04 mg/kg) N=9 n (%)	Cohort 3 (≤3.07 mg/kg) N=7 n (%)	Cohort 6 (≤4.10 mg/kg) N=6 n (%)	Total N=36 n (%)
Any TEAE	12 (85.7)	6 (66.7)	7 (100)	3 (50.0)	28 (77.8)
Serious TEAE	2 (14.3)	0	0	0	2 (5.6)
Related TEAE [1]	2 (14.3)	0	3 (42.9)	0	5 (13.9)
Related Serious TEAE [1]	0	0	0	0	0
Related TEAE Leading to Drug Withdrawal	0	0	0	0	0
Related TEAE Leading to Study Discontinuation	0	0	0	0	0
Related TEAE Leading to Death	0	0	0	0	0

% = 100 x n/N, N = number of subjects in the population, n = number of subjects reporting event.
Treatment-emergent AEs will be defined as AEs with onset after administration of the study drug, or when a pre-existing medical condition increases in severity or frequency after study drug administration.
Adverse Events are coded using MedDRA Version 25.0.
[1] AE is considered treatment-related if the relationship to the study drug is 'Related' or missing. Subjects are counted only once at the maximum relationship.

Majority of TEAEs were mild to moderate in severity

- Three serious TEAEs in 2 subjects (5.6%) **unrelated to study drug**
  - Fatal Arrhythmia
  - Limb abscess
  - Emotional distress
- Most common TEAEs (observed in ≥ 10% of subjects)
  - Headache and URTI (13.9% each)
- No TEAEs occurred ≥ 20% of participants

TEAEs do not appear to be dose dependent based on TEAE frequencies at the different SAD/MAD doses

SRP-1003 appears safe and well tolerated to date in the ongoing study

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Conclusions from clinical data to date

- Well-tolerated with no dose-limiting toxicity allowing us to continue dose escalating and the potential to drive differentiated efficacy

- Superior muscle concentration

- Successful target engagement with emerging biomarker evidence of potentially meaningful treatment efficacy

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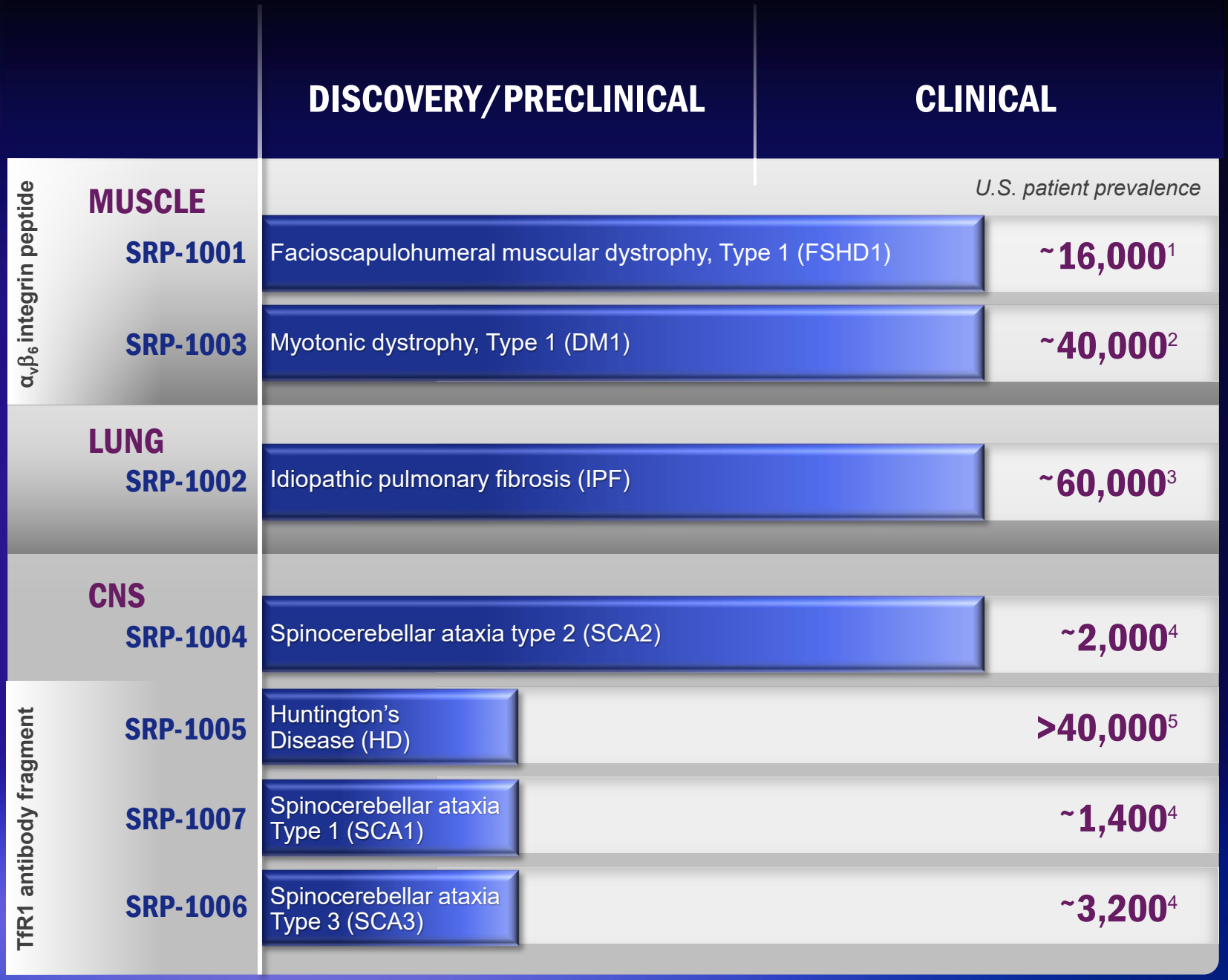
Closing Remarks

Doug Ingram

Chief Executive Officer

siRNA Pipeline



1. Kabelac Z, et al. Neurology. 2020;94(15_Supplement)1561. (Diagnosed patients in the U.S.)
2. Pascual-Gilabert M, López-Castel A, Artero R. Myotonic dystrophy type 1 drug development: a pipeline toward the market. Drug Discovery Today. 2021;26(7):1765-72. doi: 10.1016/j.drudis.2021.03.024. (Diagnosed patients in the U.S.)
3. Data on file. (U.S.)
4. Ruano et al, Neuroepidemiology 2014. (Diagnosed patients in the U.S.)
5. https://hdsa.org/what-is-hd/. (People in the U.S. affected)

siRNA Programs: Upcoming milestones over the next 12 – 18 Months

SRP-1001

FSHD1

MAD study data
– 2H 2026

SRP-1003

DM1

MAD study data
– 2H 2026

Casi22 data
– 2H 2026

SRP-1005

Huntington's

Commence dosing
– 1H 2026

Proof-of-biology data
– 1H 2027

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Clinical Results and Plan Forward:

SRP-1001 for Facioscapulohumeral Muscular Dystrophy Type 1 (FSHD1)

SRP-1003 for Myotonic Dystrophy Type 1 (DM1)

March 25, 2026