SAREPTA THERAPEUTICS

DOUG INGRAM
Chief Executive Officer

Sarepta Therapeutics, Inc. (NASDAQ:SRPT)
44th Annual J.P. Morgan Healthcare Conference
San Francisco, California
JANUARY 12, 2026

MAX, age 10

Dosed with
ELEVIDYS
at age 5

Forward-looking statements

In order to provide Sarepta's investors with an understanding of its current results and future prospects, forward-looking statements will be made during this presentation. 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, without limitation, statements relating to our preliminary earnings, financial projections and future operations; our pipeline, priorities and strategies; ELEVIDYS and Cohort 8 of ENDEAVOR; the potential benefits and differentiation of our siRNA programs and TRiM platform; our ongoing and planned clinical trials; and expected plans and milestones, including meeting with FDA to discuss a path to traditional approval of casimersen and golodirsen, and our expected near-term milestones in 2026 for various programs.

Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: our products or product candidates may be perceived as insufficiently effective, unsafe or may result in unforeseen adverse events; our products or product candidates may cause undesirable side effects that result in significant negative consequences following any marketing approval; we may not be able to comply with all FDA requests in a timely manner or at all; we may not be able to reach alignment with FDA regarding our market authorization and a path to traditional approval for casimersen and golodirsen; we may not be able to meet expectations with respect to sales of our products or maintain profitability; we may observe adverse reactions in our clinical trials or in patients who receive our approved products; our products may not be widely adopted by patients, payors or healthcare providers, which would adversely impact our business; the estimates and judgments the Company makes, or the assumptions on which it relies, in preparing its financial statements could prove inaccurate; we may not be able to advance all of our programs, and we may use our financial and human resources to pursue particular programs and fail to capitalize on programs that may be more profitable or for which there is a greater likelihood of success; different methodologies, assumptions and applications we use 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, these data may not be sufficient to support approval; success in 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 with advisory committee recommendations, or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; failure to retain our key personnel or an inability to attract and retain additional qualified personnel could present a challenge to our business objectives; our existing and any future indebtedness could adversely affect our ability to operate our business; our revenues and operating results could fluctuate significantly, which may adversely affect our stock price and business; the possible impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business; and those risks identified under the heading "Risk Factors" in our 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 herein. 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.

Strong Fundamentals Position Sarepta well into the Future

1 Substantial Duchenne opportunity

~80% of the addressable ambulatory population remain untreated with ELEVIDYS; PMOs continue to support revenue trajectory

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Next-generation siRNA pipeline

Preclinical data support potential differentiation of our proprietary scientific platform

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3

Profit and cash generating business; right sized cost structure to fund pipeline

Self-sustaining business for the long-term

Preliminary Q4 and Full-year 2025 Results

Cash and Investments
(as of 12/31/25)

$954M[1, 2]

Total Net Product Revenue

Q4 2025 $370M[2]

Full-year total $1.86B[2]

ELEVIDYS Net Product Revenue

Q4 2025 $110M[2]

Full-year total $899M[2]

PMO Net Product Revenue

Q4 2025 $259M[2]

Full-year total $966M[2]

1. Includes cash, cash equivalents, restricted cash and investments
2. Preliminary (unaudited)

ELEVIDYS Today

~80%

of the addressable ambulatory population remain untreated

Strategic approach

- Focus on HCP segmentation and outreach (e.g., sites of care)
- Provide deep dive opportunities around the data and science
- Expand field force
- Enhance understanding of ELEVIDYS among patient and advocacy communities
- Cohort 8: Data generated within the next 12 months will enhance our understanding of ELEVIDYS safety and efficacy

Safety evaluated in 200+ clinical trial patients and in >1,100 patients worldwide

Updated monitoring/protocol developed in conjunction with medical experts and FDA

ELEVIDYS Impact

ELEVIDYS is designed to treat the underlying cause of Duchenne muscular dystrophy, impacting the trajectory of the disease.

*Placebo crossover patients increased 1.5 points on the NSAA, while the study remained blinded. Statistically significant increases on NSAA, TTR and 10MWR when compared to a pre-specified, well-matched external controls for crossover-treated patients one year after treatment.

>1,100 patients treated

EMBARK Part 1 met all key secondary endpoints

All data confirmed by muscle MRI

EMBARK Part 2 achieved statistical significance on key endpoints*

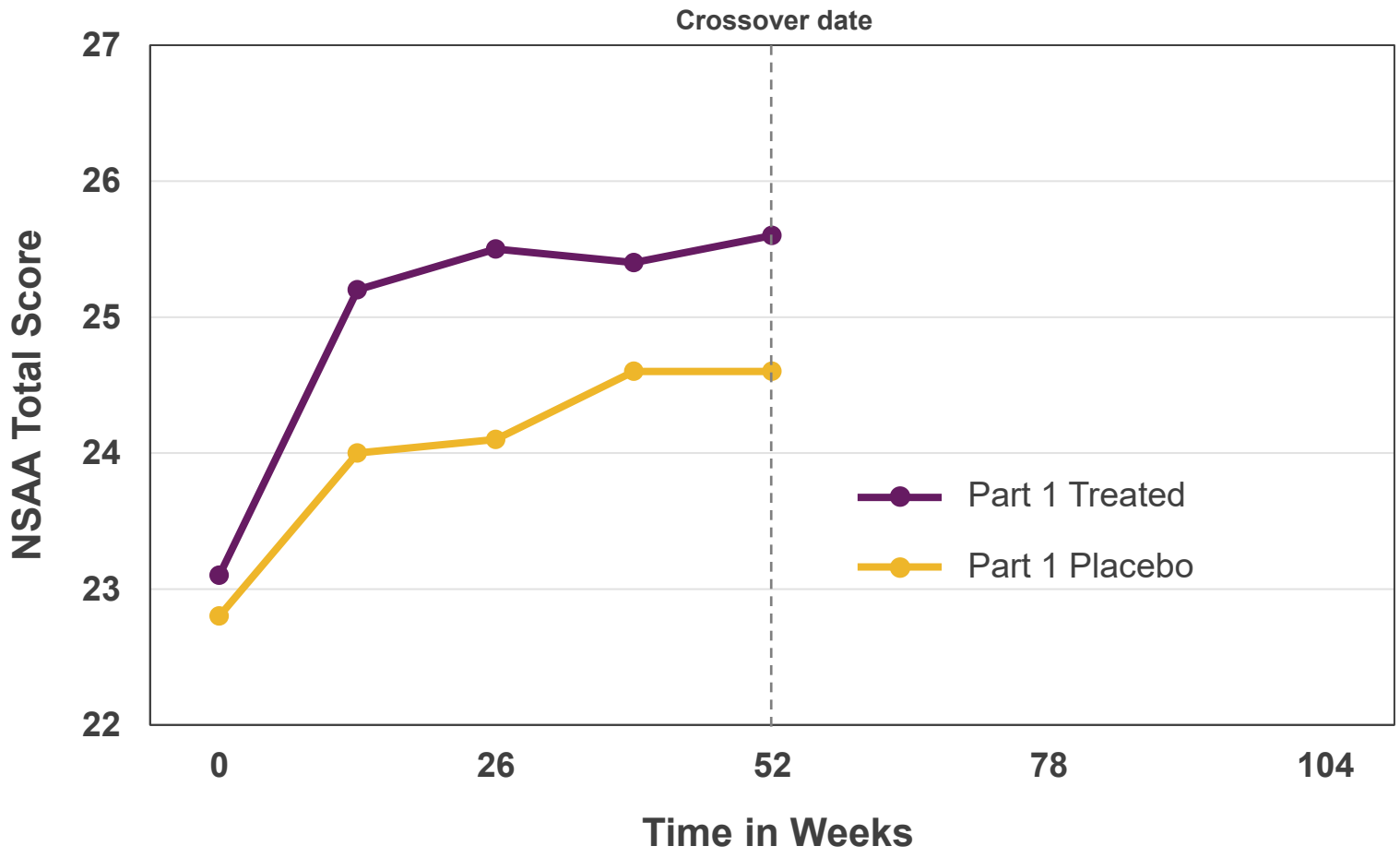
↑ **7.5**-point from BL

↑ **9.8**-point vs EC
(Study 101, NSAA 5 yrs)

ENDEAVOR Cohort 8
Initiated end of 2025

ELEVIDYS Impact

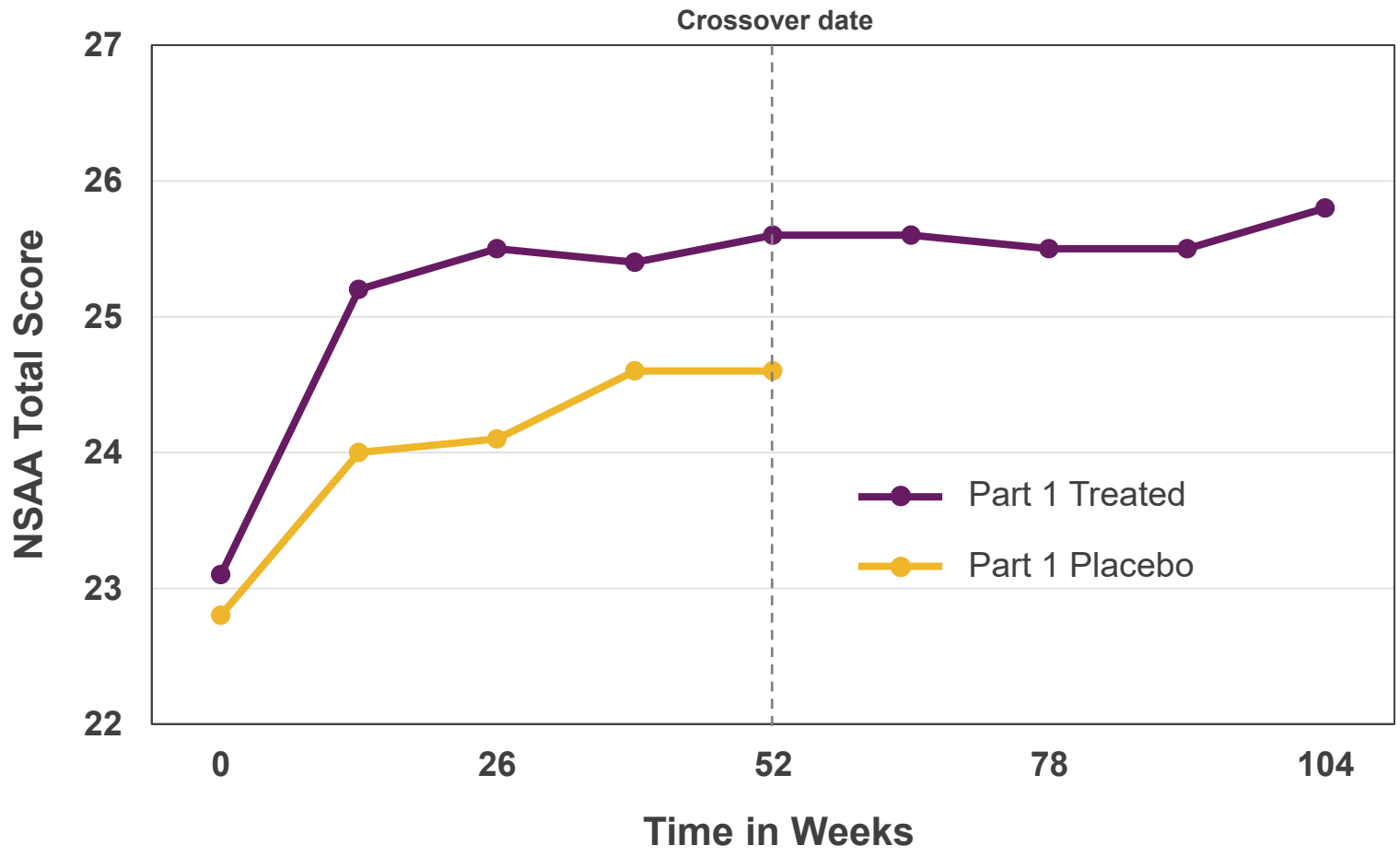
EMBARK Part 2 NSAA* Total Score: Baseline to Year 2, blinded study results



*North Star Ambulatory Assessment

ELEVIDYS Impact

EMBARK Part 2 NSAA* Total Score: Baseline to Year 2, blinded study results



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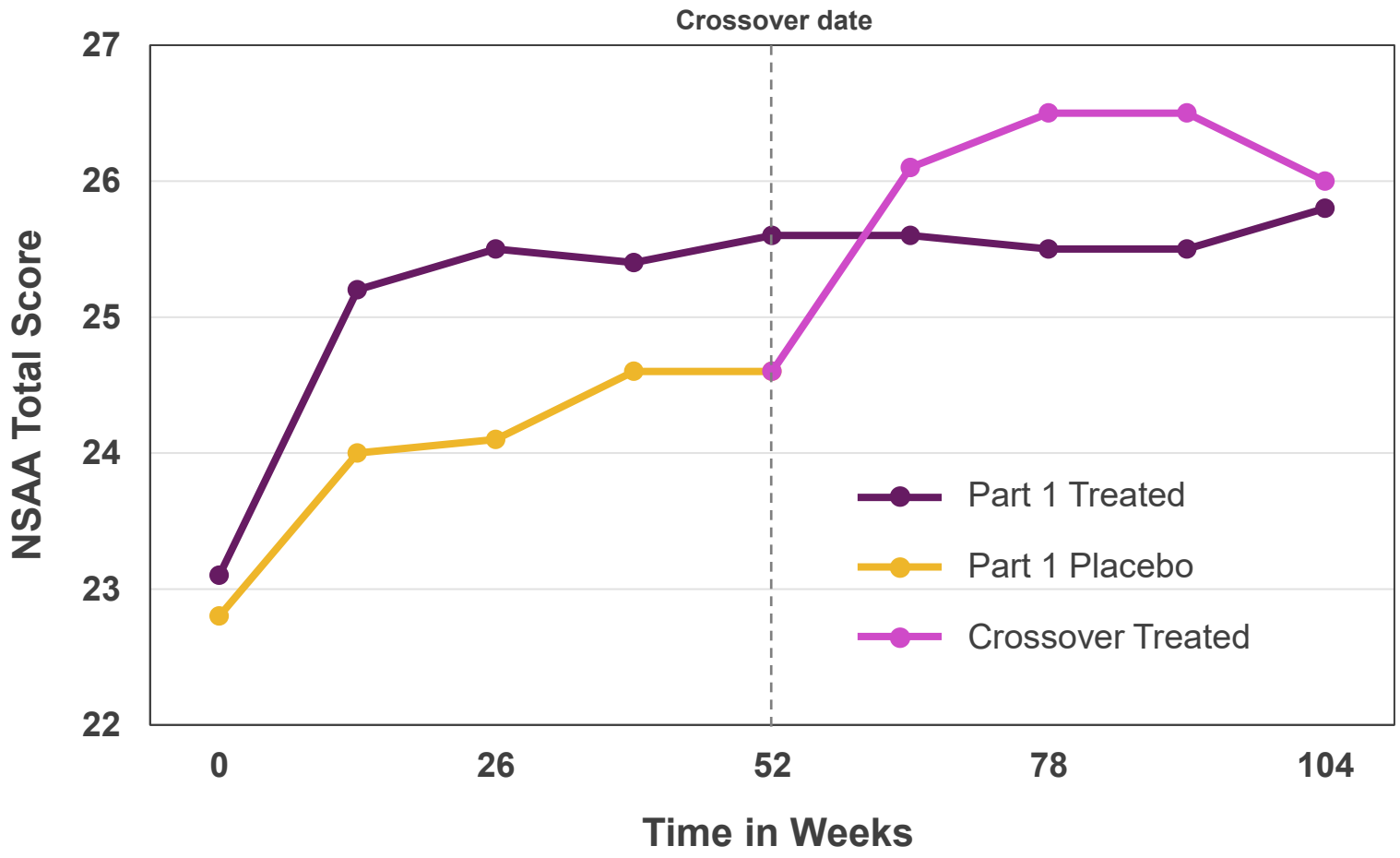
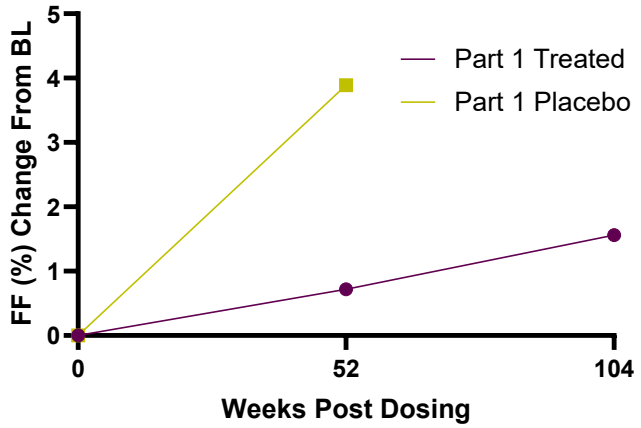


*North Star Ambulatory Assessment

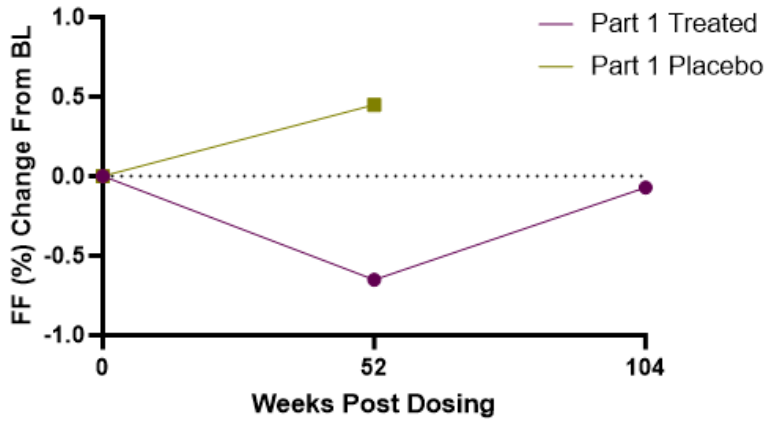
ELEVIDYS Impact

Two-Year Results: Musculoskeletal MRI - fat fraction (FF)

Vastus Lateralis



Soleus



Placebo	N=16	N=14		N=17	N=15	
Treated	N=15	N=14	N=14	N=16	N=16	N=15

Stabilization in MRI muscle FF in SRP-9001 treatment group with two years values below those seen in Part 1 placebo group

- Naarding KJ, Reyngoudt H, van Zwet EW, etal. MRI vastus lateralis fat fraction predicts loss of ambulation in Duchenne muscular dystrophy. Neurology. 2020 Mar 31;94(13):e1386-e1394.
- Barnard AM, Willcocks RJ, Triplett WT, etal. MR biomarkers predict clinical function in Duchenne muscular dystrophy. Neurology. 2020 Mar 3;94(9):e897-e909.
- Willcocks RJ, Rooney WD, Triplett WT, etal. Multicenter prospective longitudinal study of magnetic resonance biomarkers in a large duchenne muscular dystrophy cohort. Ann Neurol. 2016 Apr;79(4):535-47. doi: 10.1002/ana.24599. Epub 2016 Feb 19. PMID: 26891991; PMCID: PMC4955760.

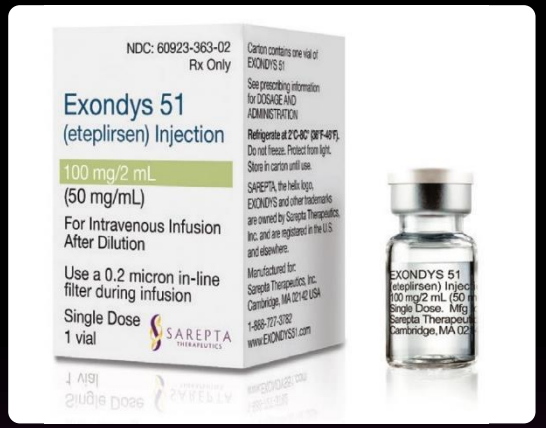
PMO Impact

EXONDYS 51, VYONDYS 53 & AMONDYS 45

Sarepta's RNA-based PMOs have an established safety profile and have successfully treated >1,800 patients worldwide across many age groups.

>1,800
patients worldwide ranging from infants to adults in their 30's and over

10+
years of clinical and commercial experience



Established safety profile

2,300+
years of life added to patients who have been on therapy

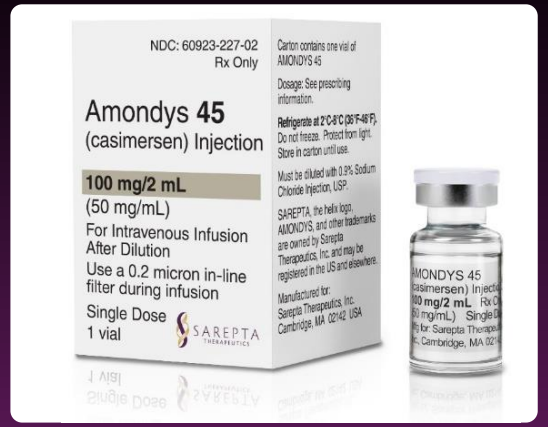
Slowing disease progression

- Delayed loss of ambulation
- Slowed pulmonary and cardiac decline
- Prolonged survival

Adherence rates exceeding

90%

underscoring clinical value



PMO Impact

EXONDYS 51, VYONDYS 53 & AMONDYS 45

The real-world evidence supports Sarepta's RNA-based PMOs which have demonstrated an over 90% adherence rate.

Real-world evidence support Sarepta's on market PMO therapies

5.4 YEAR
increase in **survival**

3-4 YEAR
delay in **loss of ambulation**

3-7 YEAR
delay in need for nighttime **ventilation** and significantly slower **pulmonary decline**

50-90%
reduction in **assisted ventilation and tracheostomy**

78%
reduction in risk of reaching **LVEF<55%** and significantly slower **cardiac decline**

30%
reduction in **ER and hospital visits**

>90% compliance on long-term therapy

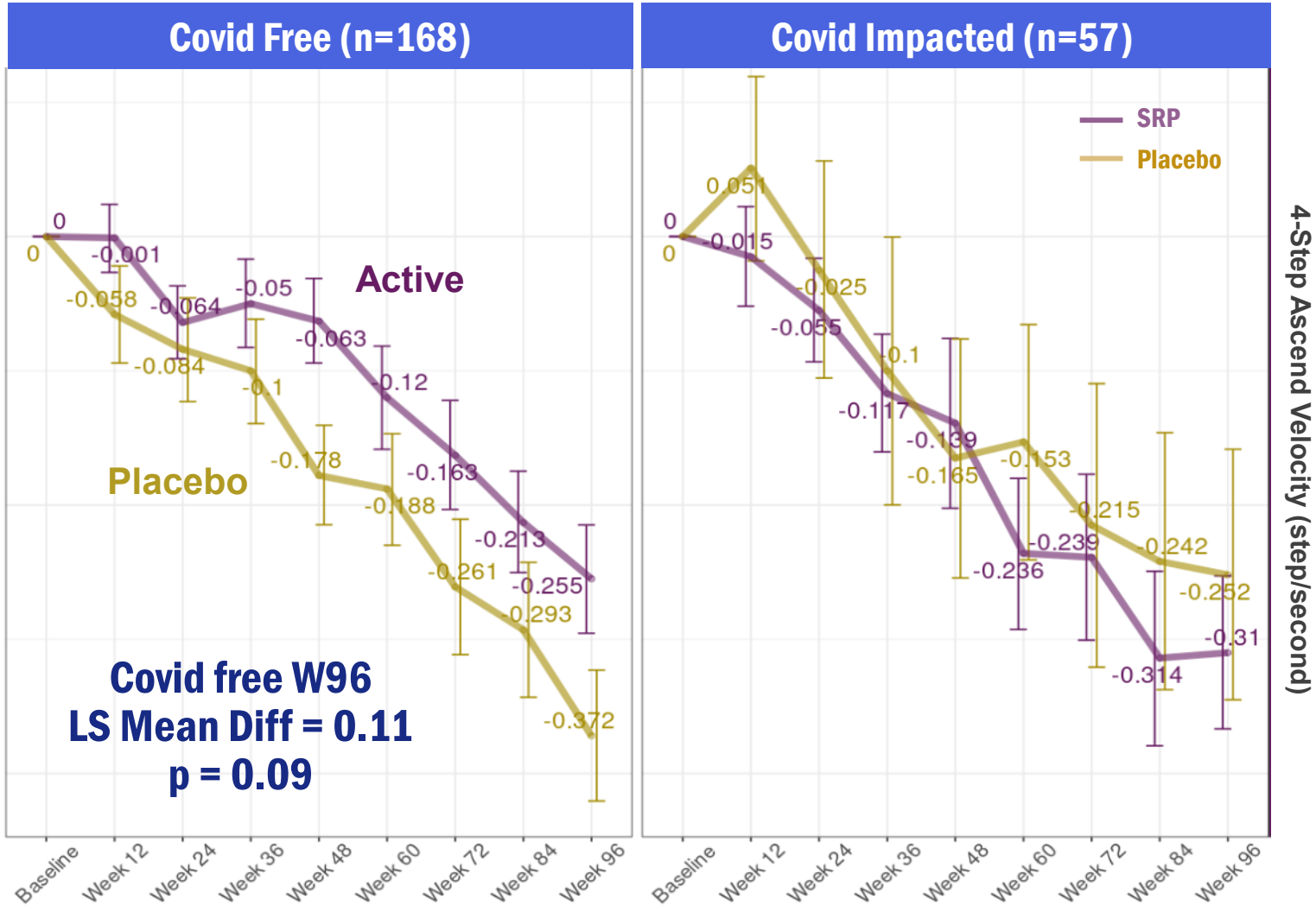
PMO Impact

EXONDYS 51, VYONDYS 53 & AMONDYS 45

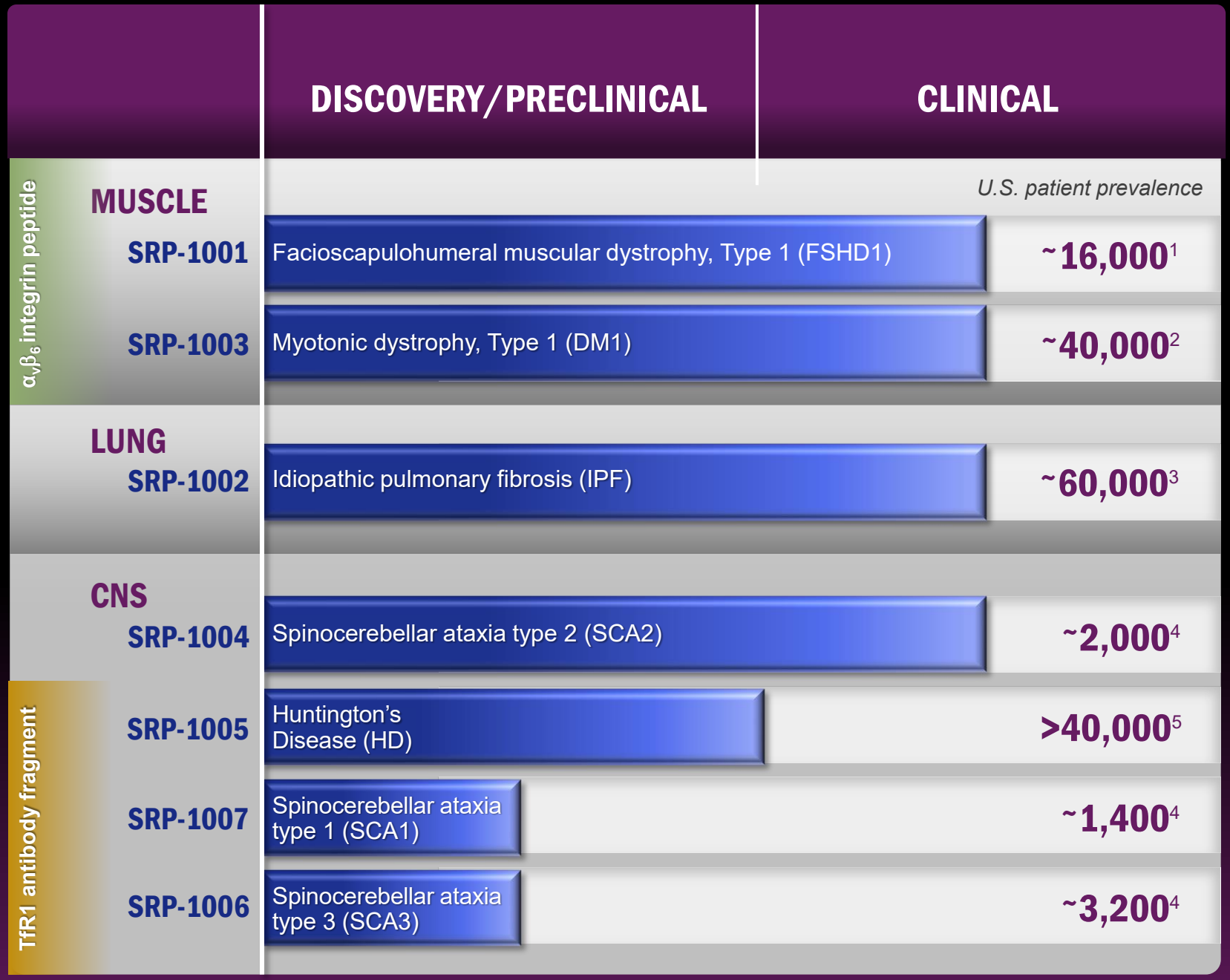
~30%

reduction in disease progression over 2 years on the 4SA

Mean trend in participants not impacted by COVID demonstrates meaningful change



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)

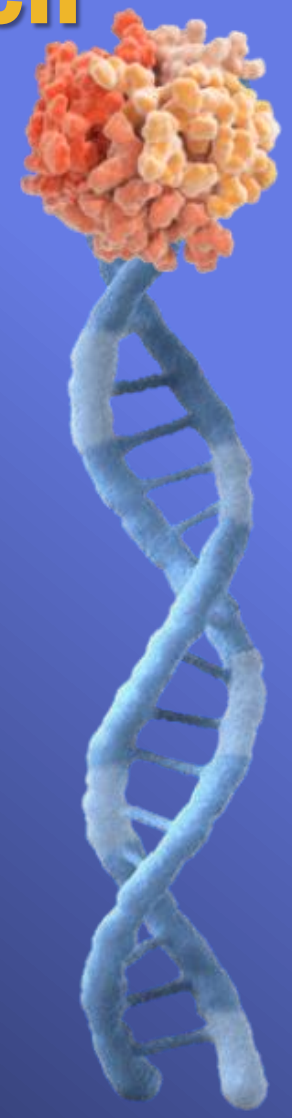
TRiM Platform

A next-generation approach

Delivery
Superior tissue targeting

siRNA Design
Effective and efficient target knock-down, improved durability, specificity, and reduction of off-target effects

Safety/Dosing
- Favorable safety profile
- Potential to dose higher vs other therapies



Optimal components support potential best-in-class therapies in FSHD and DM1

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

Optimal clinical impact	$\alpha_v\beta_6$	TfR1 mAb	ASO
Higher dosing	✓		
Better muscle penetration *(e.g., delivers more Rx to muscle)*	✓		
Safety	✓		
Potency	✓		

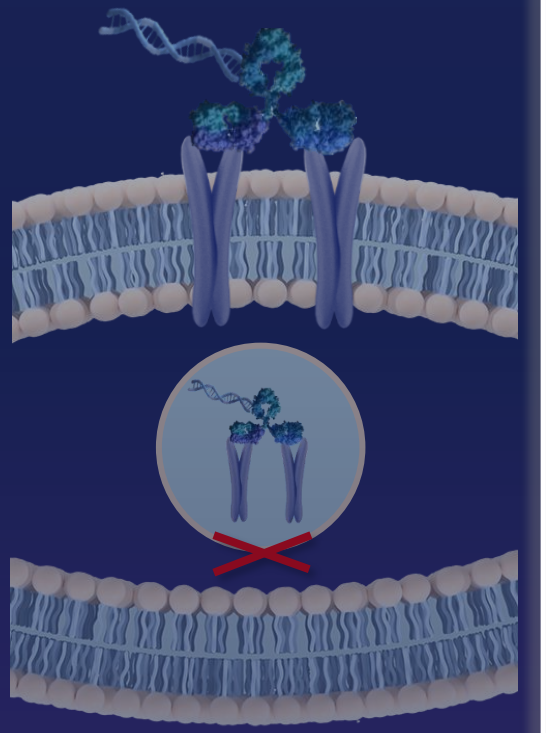
The tolerability of the $\alpha_v\beta_6$-targeting platform may allow for best-in-class efficacy, through higher doses and greater muscle concentration without dose-limiting AEs.

Our approach to crossing the blood-brain barrier

EXISTING APPROACH

Divalent Binding

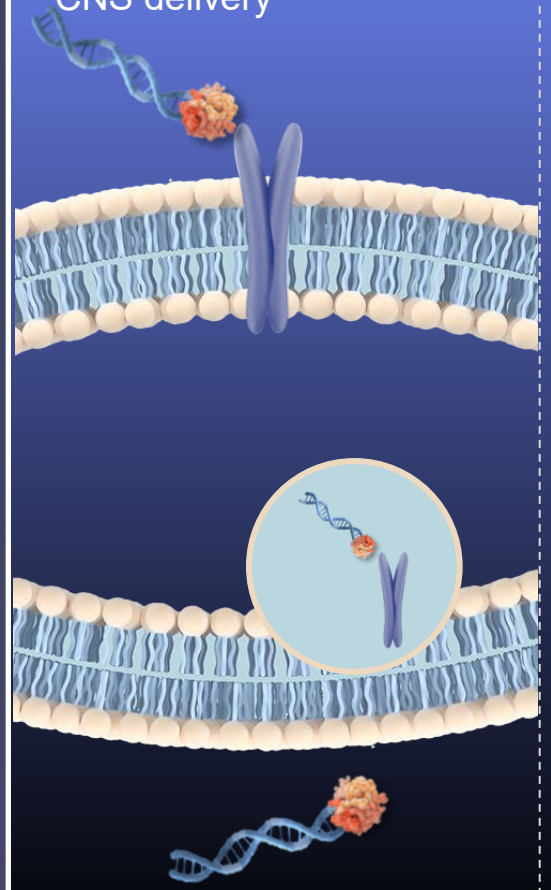
to TfR1 with mAb promotes receptor degradation and/or recycling



SYNERGISTIC NEXT-GENERATION APPROACH

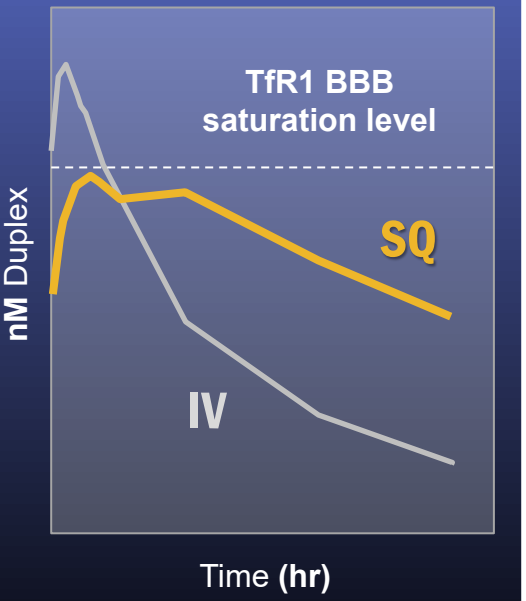
Monovalent Binding

to TfR1 with fAb promotes receptor transcytosis and CNS delivery



Delivery

Pharmacokinetics of subcutaneous delivery increases the time window of TfR1-ligand interactions without saturating the amount of available receptors to bind



TfR1-binding designed to optimize CNS delivery in Huntington's disease (HD)

SRP-1005 HD program employs a subcutaneous route of administration delivering siRNA across the BBB to the source of disease in the deep brain.

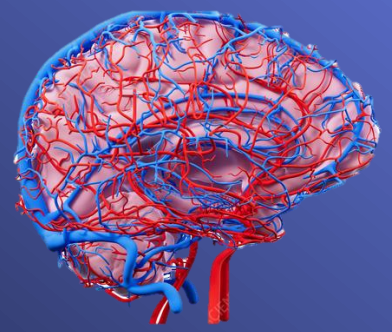
TRiM CNS-SC platform demonstrates improved delivery to deep brain in NHP compared to direct CSF injection

CSF delivery via IT injection

Accessing the brain via intrathecal injection (IT) is limited by CSF flow

J. Pers. Med. 2022, 12(12), 1979; https://doi.org/10.3390/jpm12121979

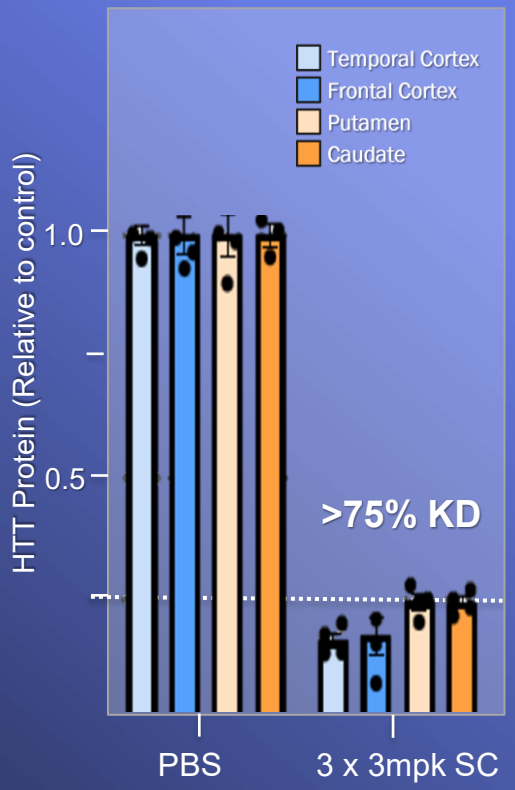
Systemic delivery via SC injection



Accessing the brain via the blood-brain barrier through TfR1-mediated crossing leads to greater deep brain distribution

Thom Leach / Science Photo Library

SRP-1005 in NHP

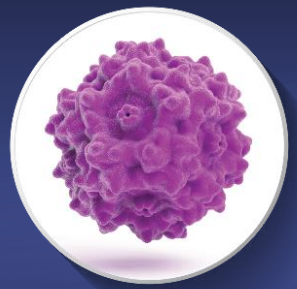


Strong Financial Foundation

- **Efficient cost structure fueling future profitability**
  - Strategic expense management and financial discipline enables sustainable profit and cash flow generation
- **Robust cash position**
  - Base business cash flow positive (excluding Arrowhead transaction and milestone payments) in 2025
  - All near- and mid-term inflection points for pipeline advancement are fully funded
- **Optimized balance sheet enabling growth and flexibility**
  - Refinanced convertible debt removes any liquidity concerns
  - Optionality from undrawn $600M revolving credit facility

Upcoming Milestones

Numerous value-building milestones expected throughout 2026, across our three differentiated scientific technology platforms.



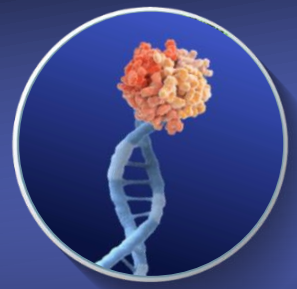
GENE THERAPY

ELEVIDYS
Duchenne
ENDEAVOR Cohort 8 complete primary endpoint data collection – 2H 2026

RNA

EXONDYS 51
Duchenne
MIS51ON data readout – end of 2026

VYONDYS 53 and AMONDYS 45
Duchenne
- FDA meeting
- Full data ESSENCE study (medical meeting or manuscript) – by end of 2026



siRNA

SRP-1001
FSHD
Phase 1/2 preliminary data – 1Q 2026

SRP-1003
DM1
Phase 1/2 preliminary data – 1Q 2026

SRP-1005
Huntington's Disease
Commence dosing – 1H 2026

We know who we're fighting for

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