# 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 18, 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 18, 2021, Sarepta Therapeutics, Inc. (the "Company") issued a press release and conducted an investor webcast presenting 12-week expression and safety results from the first 11 participants enrolled in Study SRP-9001-103, the Company's investigational gene therapy for the treatment of Duchenne muscular dystrophy. 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 18, 2021: Sarepta Therapeutics' Investigational Gene Therapy for the Treatment of Duchenne Muscular Dystrophy, SRP-9001, Demonstrates Robust Expression and Consistent Safety Profile Using Sarepta's Commercial Process Material |
| 99.2 | Presentation dated May 18, 2021: Clinical Update: Micro-dystrophin Gene Therapy Study SRP-9001-103: 12-Week Expression and Safety Data Using Commercially Representative Material |
| 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 18, 2021

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


# Sarepta Therapeutics' Investigational Gene Therapy for the Treatment of Duchenne Muscular Dystrophy, SRP-9001, Demonstrates Robust Expression and Consistent Safety Profile Using Sarepta's Commercial Process Material

– ***Results from the first 11 participants enrolled in Study 9001-103 ENDEAVOR showed robust transduction, delivering mean vector genome copies of 3.87 per nucleus***

– ***Treated patients achieved mean micro-dystrophin expression levels of 55.4% of normal as measured by western blot***

– ***Micro-dystrophin was properly localized to the muscle sarcolemma, with patients achieving mean percentage of dystrophin positive fibers of 70.5% and intensity of micro-dystrophin expression of 116.9% of normal control, as measured by immunofluorescence (IF)***

– ***Safety profile consistent with prior studies and no new safety signals identified***

CAMBRIDGE, Mass., May 18, 2021 (GLOBE NEWSWIRE) – Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced positive 12-week expression and safety results from the first 11 participants enrolled in Study SRP-9001-103, an open-label study known as ENDEAVOR being conducted in partnership with Roche. In results from the first clinical study using commercially representative material, SRP-9001 (rAAVrh74.MHCK7.micro-dystrophin) demonstrated robust expression of micro-dystrophin and no new safety signals from prior studies, supporting its potentially differentiated profile for the treatment of Duchenne muscular dystrophy. SRP-9001 is an investigational gene transfer therapy intended to deliver its micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein.

"We are delighted by these seminal results from the ENDEAVOR Study, our first trial results with SRP-9001 made by our commercial-scale manufacturing process. These data show strong transduction of the micro-dystrophin gene, resulting in robust expression of the properly localized micro-dystrophin protein, and did so with no new or unexpected safety signals," said Doug Ingram, president and chief executive officer, Sarepta. "In addition to characterizing and differentiating SRP-9001, these results confirm the extraordinary work done over the last two and a half years to build an at-scale gene therapy manufacturing process and corresponding analytics sufficient to meet the needs of the Duchenne population with what we believe will be a potentially life-changing therapy. Armed with these data, we will seek a meeting with the FDA with the goal of rapidly starting our registrational study."

In the open-label study, 20 participants between the ages of four and seven were treated with a single infusion of SRP-9001 at a dose of $1.33x10^{14}$ vg/kg. In muscle biopsies from the first 11 patients taken 12 weeks after treatment, the following results were observed:

- All patients demonstrated robust transduction, with mean micro-dystrophin expression of 55.4% of normal, as measured by western blot.

- Muscle dystrophin levels demonstrated a mean of 70.5% (baseline 12.8%) muscle fibers expressing micro-dystrophin at 12 weeks with a mean intensity at the sarcolemma of 116.9% (baseline 41.0%) compared to normal biopsies, as measured by immunofluorescence. Comparisons between baseline and post-treatment measures were statistically significant (p=0.001 for positive fibers, and p=0.002 for intensity).
- Mean vector genome copies per nucleus reached 3.87.

The safety profile of SRP-9001 observed in the first 11 participants in ENDEAVOR is consistent with the safety seen in earlier studies using clinical manufacturing process material. In line with previously reported clinical data, no clinically relevant complement activation was observed in these 11 patients. Two patients experienced serious adverse events (transaminase elevation in one patient and nausea and vomiting in a second patient) that fully resolved.

***About SRP-9001-103 (ENDEAVOR)***

Study SRP-9001-103 (Study 103) is an open-label clinical trial of SRP-9001 that has enrolled 20 participants with Duchenne muscular dystrophy between the ages of 4-7. Study 103 uses commercially representative SRP-9001 and the primary endpoint is the change from baseline in the quantity of micro-dystrophin protein expression measured by western blot at 12 weeks. Secondary outcome measures include change from baseline in micro-dystrophin expression fiber intensity as measured by immunofluorescence (IF) and micro-dystrophin expression measured by IF percent dystrophin positive fibers at 12 weeks. Exploratory endpoints include the change in vector genome copies per nucleus, North Star Ambulatory Assessment (NSAA) and certain timed functional tests. Including the initial 12-week period, patients will be followed for a total of five years.

***About SRP-9001 (rAAVrh74.MHCK7.micro-dystrophin)***

SRP-9001 is an investigational gene transfer therapy intended to deliver the micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein. Sarepta is responsible for global development and manufacturing for SRP-9001 and plans to commercialize SRP-9001 in the United States upon receiving FDA approval. In December 2019, Roche partnered with Sarepta to combine Roche's global reach, commercial presence and regulatory expertise with Sarepta's gene therapy candidate for Duchenne to accelerate access to SRP-9001 for patients outside the United States. Sarepta has exclusive rights to the micro-dystrophin gene therapy program initially developed at the Abigail Wexner Research Institute at Nationwide Children's Hospital.

***About Duchenne Muscular Dystrophy***

Duchenne muscular dystrophy (DMD) is a rare, fatal neuromuscular genetic disease that occurs in approximately one in every 3,500-5,000 males worldwide. DMD is caused by a change or mutation in the gene that encodes instructions for dystrophin. Symptoms of DMD usually appear in infants and toddlers. Affected children may experience developmental delays such as difficulty in walking, climbing stairs or standing from a sitting position. As the disease progresses, muscle weakness in the lower limbs spreads to the arms and other areas. Most patients require full-time use of a wheelchair in their early teens, and then progressively lose the ability to independently perform activities of daily living such as using the restroom, bathing and feeding. Eventually, increasing difficulty in breathing due to respiratory muscle

dysfunction requires ventilation support, and cardiac dysfunction can lead to heart failure. The condition is universally fatal, and patients usually succumb to the disease in their twenties.

### *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.

### *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.*

### *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 the potentially differentiated profile of SRP-9001 for the treatment of Duchenne muscular dystrophy; the potential for SRP-9001 to deliver micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein; the potential of our gene therapy manufacturing process and corresponding analytics to meet the needs of the Duchenne population with what we believe will be a potentially life-changing therapy and our plan to meet with the FDA with the goal of rapidly starting our registrational study.

These forward-looking statements involve risks and uncertainties that may cause actual results to differ materially from those expressed or implied in the forward-looking statements. Many of these risks and uncertainties 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 or result in an assessment that SRP-9001 provides a safe or effective treatment benefit; different methodologies or assumptions than 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 are positive, the data may not be sufficient to support approval by the FDA or foreign regulatory authorities; 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, many of which are outside of our control, including possible limitations on 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; the impact of the COVID-19 pandemic; and those risks identified under the heading “Risk Factors” in our 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 we make, 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 we face, we encourage you to review our SEC filings. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. We undertake no obligation to update forward-looking statements based on events or circumstances after the date of this press release.

Source: Sarepta Therapeutics, Inc.

***Investor Contact:***
Ian Estepan, 617-274-4052
iestepan@sarepta.com

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

EXHIBIT 99.2

# Clinical Update:

*Micro-dystrophin Gene Therapy*

*Study SRP-9001-103: 12-Week Expression and Safety Data Using Commercially Representative Material*

**DOUG INGRAM**
*President and CEO*

**LOUISE RODINO-KLAPAC, PH.D.**
*Executive Vice President and Chief Scientific Officer*

*May 18, 2021*
*8:30 a.m. ET*

SAREPTA THERAPEUTICS

# Welcome and Introduction

**Doug Ingram**
President and CEO

# 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 benefits of SRP-9001 and its vector, promoter and transgene; market opportunities; the expected future manufacturing supply of SRP-9001; and plans and expected milestones, including meeting with the FDA in mid-2021, meeting with other regulatory agencies, commencing Study 301 following the FDA meeting, and expanding Study 103 to include older ambulant and non-ambulant patients.

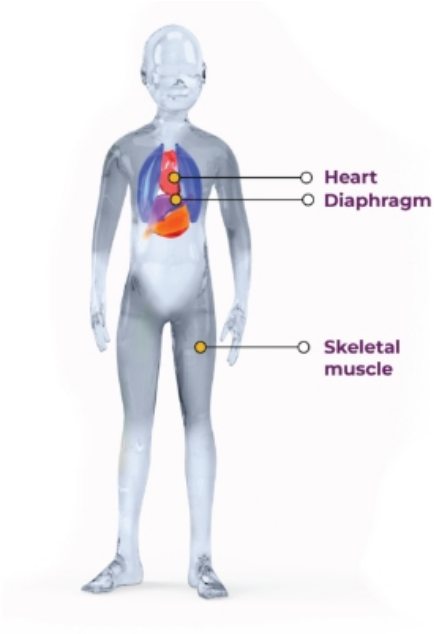
These forward-looking statements involve risks and uncertainties, many of which are beyond our control. Known risk factors include, among others: success in preclinical and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; the 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 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.

# Duchenne Muscular Dystrophy (DMD)

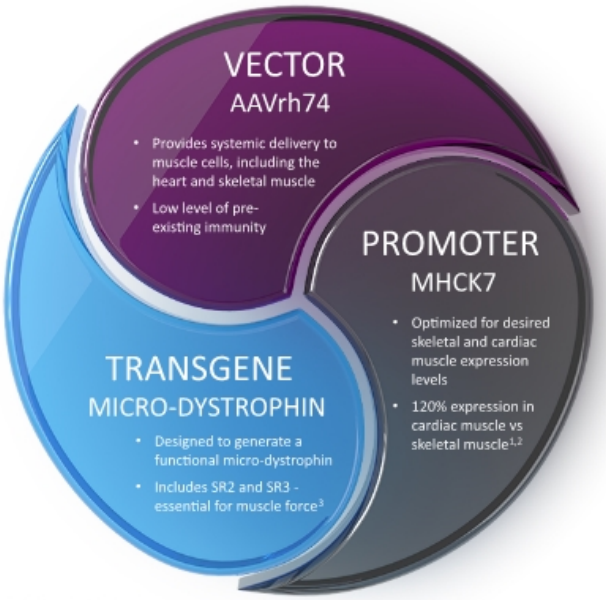
## *DMD affects approximately 1 in 3,500-5,000 males worldwide[1]*

- DMD is a rare, fatal neuromuscular genetic disease inherited in an X-linked recessive pattern[2]
- Muscle weakness becomes increasingly noticeable by 3 to 5 years of age, and most patients use a wheelchair by the time they are 11 years old[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.

# SRP-9001 Gene Therapy Construct



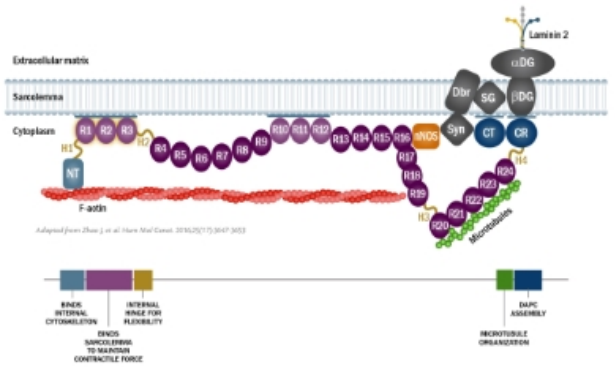


1. Potter et al. Functional and Histological Improvements Comparing 4 Micro-dystrophin Constructs in the mdx Mouse Model of DMD. ASGCT 2019.
2. Potter et al. Functional and Histological Improvements Comparing 4 Micro-dystrophin Constructs in the mdx Mouse Model of DMD. AIM 2019.
3. Nelson DM, Ervasti JM, et al. Variable rescue of microtubule and physiological phenotypes in mdx muscle expressing different miniaturized dystrophins. Human Molecular Genetics, 2018, Vol. 27, No. 12: 2090-2100 .

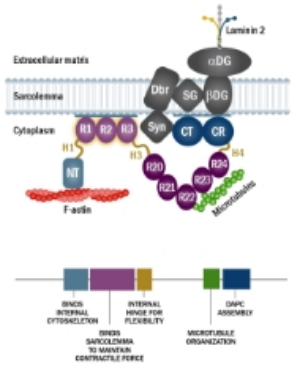
# Micro-dystrophin is a Shortened, Functional Form of Dystrophin

*Transgene – Produces a functioning version of the protein of interest*[1,2]

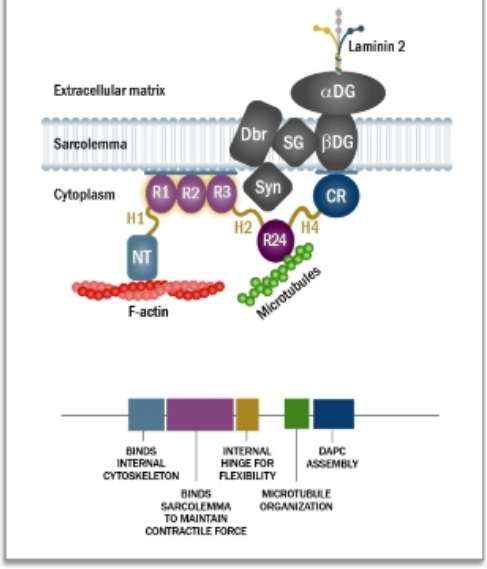
## Normal Muscle[3-8]




## 61 YO Ambulatory Patient[9-11]




## Sarepta Micro-dystrophin[4-11]




Micro-dystrophin gene therapy is investigational and has not been reviewed or approved by any regulatory authority.

1. Naso MF, et al. *BioDrugs*. 2017;31(4):317-334. 2. Chamberlain K, et al. *Hum Gene Ther Methods*. 2016;27(1):1-12. 3. Zhao J, et al. *Hum Mol Genet*. 2016;25(17):3647-3653. 4. Gao Q, et al. *Compr Physiol*. 2015;5(3):1223. 5. Harper SQ, et al. *Nature Med*. 2002;8(3):253. 6. Nelson DM, et al. *Human Mol Genet*. 2018 27(12):2090. 7. Fairclough RJ, et al. *Nat Rev Genet*. 2013;14:373-378. 8. Aartsma-Rus A, et al. *Muscle Nerve*. 2006;34(2):134-144. 9. England SB, et al. Nature. 1990;343(6254):180-182. 10. Wells DJ, et al. Hum Mol Genet. 1995;4(8):1245-1250. 11. Cooper-Olson G, Rodino-Klapac LR, Potter RA. Evaluation of the Lipid-binding Properties of Recombinant Dystrophin Spectrin-like Repeat Domains R1-3. J Neuromuscul Dis. 2021 Mar 23. doi: 10.3233/JND-200622. Online ahead of print. PMID: 33780374.

# Clinical Results:

*Micro-dystrophin Study SRP-9001-103*
*Expression and Safety Data at 12 Weeks*
*in Patients with Duchenne*

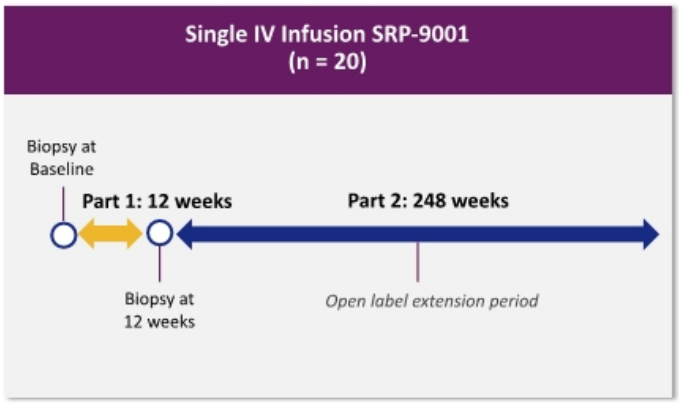
**Louise Rodino-Klapac, Ph.D.**
Executive Vice President and Chief Scientific Officer

# SRP-9001-103*

ENDEAVOR

- **Study 103**
  - Ongoing phase 1b open-label study using commercially representative material of SRP-9001
  - Four U.S. sites
  - Boys with Duchenne, ages 4 to <8
  - 20 patients, today's data from first 11 patients

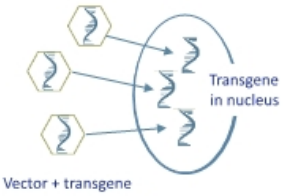
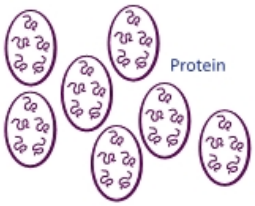
| NUMBER OF PATIENTS | AGE |
|---|---|
| 2 | 4-5 |
| 9 | 6-7 |




- **Dose**
  - Weight based dosing: $1.33 \times 10^{14}$ vg/kg

*ClinicalTrials.gov Identifier: NCT04626674

# Questions to Consider When Evaluating Gene Transfer Therapies

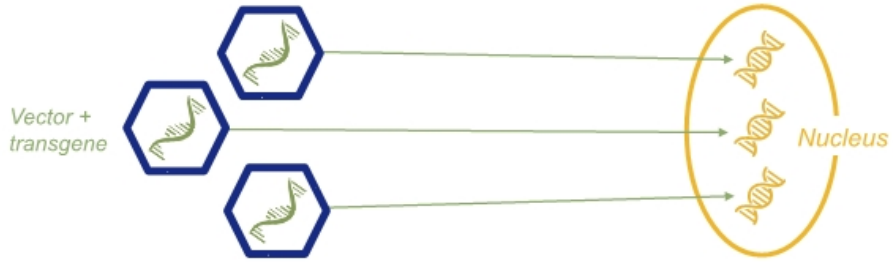
| | 1 | 2 | 3 | 4 |
|---|---|---|---|---|
| **QUESTION** | Is the transgene DNA inside muscle cells? | Is the desired protein made? | Is the protein at the cell membrane? How much is there? | What was the safety and tolerability experience? |
| **EXPERIMENT** | Vector Genome Copies/Nucleus | Western Blot | Immunofluorescence | Safety |
| |   |   | **% Positive Fibers (PDPF):** % cells with protein<br>**Intensity of Fluorescent Signal (IF):** How strong is expression in cells with protein? |  |

# Micro-dystrophin Transduction by Vector Genome Count in First 11 Patients

QUESTION

1. Is the transgene DNA inside muscle cells?
2. Is the desired protein made?
3. Is the protein at the cell membrane? How much is there?
4. What was the safety and tolerability experience?

BASED ON MUSCLE BIOPSIES FROM THE GASTROCNEMIUS MUSCLE AT WEEK 12




| | MEAN VECTOR GENOME COPIES PER NUCLEUS (SD, standard deviation) |
|---|---|
| N=11 | **3.87** (2.44) |

# Micro-dystrophin Expression by Western Blot at Week 12 Post-treatment in the First 11 Patients


BASED ON MUSCLE BIOPSIES FROM THE GASTROCNEMIUS MUSCLE AT WEEK 12

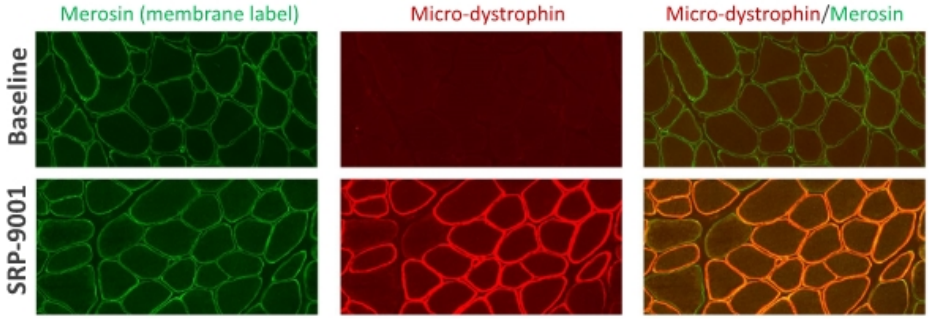
| | MEAN PERCENT NORMAL MICRO-DYSTROPHIN EXPRESSION (SD) |
|---|---|
| **N=11** | **55.4%** (43.4%) |

# Micro-dystrophin Percentage of Dystrophin Positive Fibers and Intensity at Week 12 Post-treatment in the First 11 Patients

QUESTION

1. Is the transgene DNA inside muscle cells?
2. Is the desired protein made?
3. Is the protein at the cell membrane? How much is there?
4. What was the safety and tolerability experience?

BASED ON MUSCLE BIOPSIES FROM THE GASTROCNEMIUS MUSCLE AT WEEK 12




| | MEAN PERCENTAGE OF DYSTROPHIN-POSITIVE FIBERS (SD) | MEAN INTENSITY (SD) |
|---|---|---|
| N=11 | **70.5 %** (23.4%) | **116.9 %** (44.6%) |

*Mean baseline values were 12.8% and 41% for dystrophin positive fibers and intensity, respectively*

# Study 102 Part 2 Placebo Crossover Patients (Clinical Process Material) and Study 103 Patients (Commercially Representative Process Material) Show Consistent Results

## Micro-dystrophin Clinical Process Material

*Study 102 Part 2 Placebo Crossover Patients, Mean (n=11)*

| Vector Genome Copies per Nucleus | % of Normal Expression | % Dystrophin Positive Fibers | % Intensity |
|---|---|---|---|
| 2.62 | 51.7% | 79.2% | 100.6% |

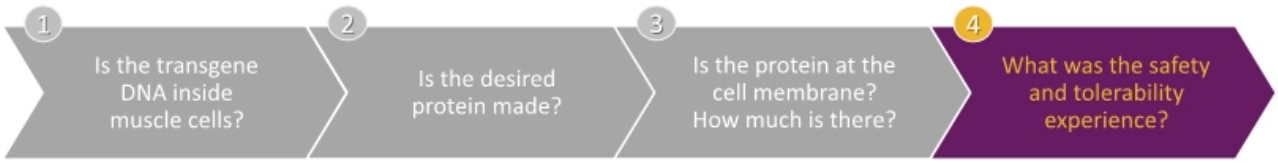
## Micro-dystrophin Commercially Representative Process Material

*Study 103 Patients, Mean (n=11)*

| Vector Genome Copies per Nucleus | % of Normal Expression | % Dystrophin Positive Fibers | % Intensity |
|---|---|---|---|
| 3.87 | 55.4% | 70.5% | 116.9% |

# SRP-9001-103 Safety Experience

QUESTION




- Safety was consistent with previous experience with SRP-9001
- 79 treatment-emergent adverse events in 11 patients
  - Most common adverse event was vomiting
    - Typical onset within first week, mild, and treated with standard antiemetics
  - Increase in liver enzymes were transient and responsive to steroids
    - No signs of impaired liver function in any patient
- SAEs in 2 patients that fully resolved
  - 1 patient with increased transaminases who was treated with intravenous steroids
  - 1 patient with nausea and vomiting
- No clinically relevant complement activation observed

# Key Takeaways

## *SRP-9001 Provides a Differentiated Profile for Duchenne*

- Confirmed characteristics of commercially representative SRP-9001
  - Robust transduction – 3.87 mean vector genome copies per nucleus
  - Mean robust expression with proper localization to the sarcolemma membrane
    - Western blot – 55.4%
    - Positive Fibers – 70.5%
    - Intensity – 116.9%
  - Consistent safety profile
    - Safe, well tolerated and consistent safety profile with clinical manufacturing process material
      - No clinical complement manifestations
- Study 103 results provide confirmation of manufacturing process and analytics; sufficient capacity to supply the Duchenne population

# Next Steps

1. *Meet with the FDA, mid-year 2021; and other regulatory agencies*
2. *Commence Study 301 following FDA meeting*
3. *Expand Study 103 to include older ambulant and non-ambulant patients*

# Question and Answer