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): January 10, 2022

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 Cambridge, Massachusetts	**02142**
(Address of Principal Executive Offices)	**(Zip Code)**

Registrant's Telephone Number, Including Area Code: (617) 274-4000

(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, $0.0001 par value per share	SRPT	NASDAQ Global Select 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 2.02 Results of Operations and Financial Condition.

On January 10, 2022, Douglas S. Ingram, President and Chief Executive Officer of Sarepta Therapeutics, Inc. (the "Company") disclosed certain preliminary financial information for the year ended December 31, 2021 during the Company's presentation at the 40th Annual J.P. Morgan Healthcare Conference (the "Conference") and in discussions with third parties at the Conference. Specifically, the Company disclosed its (unaudited) cash position of approximately $2.1 billion as of December 31, 2021 and that the Company generated approximately $178.7 million in revenue (unaudited) in the fourth quarter ended December 31, 2021 and approximately $612 million in revenue (unaudited) in the year ended December 31, 2021, each from sales of EXONDYS 51® (eteplirsen) Injection, VYONDYS 53® (golodirsen) Injection and AMONDYS 45® (casimersen) Injection.

The information in this Item 2.02 is unaudited and preliminary, and does not present all information necessary for an understanding of the Company's financial condition as of December 31, 2021 and its results of operations for the three months and year ended December 31, 2021. The audit of the Company's financial statements for the year ended December 31, 2021 is ongoing and could result in changes to the information in this Item 2.02.

Item 7.01 Regulation FD Disclosure.

The disclosure in Item 2.02 above is hereby incorporated by reference into this Item 7.01.

On January 10, 2022, Mr. Ingram also presented at the Conference topline results from part two of Study 102 evaluating SRP-9001, the Company's investigational gene therapy for the treatment of Duchenne muscular dystrophy. The Company issued a press release disclosing such information. Copies of the press release and the slides presented by Mr. Ingram at the Conference on January 10, 2022 are furnished with this report as Exhibits 99.1 and 99.2, respectively.

The information in this report, including Exhibits 99.1 and 99.2 attached hereto, is furnished pursuant to Items 2.02 and 7.01 and shall not be deemed "filed" for the purposes of Section 18 of the Securities and 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 Items 2.02 and 7.01 of this report.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit Number	Description
99.1	Press release dated January 10, 2022: Sarepta Therapeutics' Gene Therapy SRP-9001 Shows Statistically Significant Functional Improvements Compared to Pre-specified Matched External Control in Part 2 of Study SRP-9001-102 for the Treatment of Duchenne Muscular Dystrophy
99.2	Sarepta Therapeutics, Inc. Presentation at the 40th Annual J.P. Morgan Healthcare Conference, dated January 10, 2022
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: January 10, 2022

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

Exhibit 99.1

Sarepta Therapeutics' Gene Therapy SRP-9001 Shows Statistically Significant Functional Improvements Compared to Pre-specified Matched External Control in Part 2 of Study SRP-9001-102 for the Treatment of Duchenne Muscular Dystrophy

- ***Participants from the placebo crossover group in Part 2 of Study SRP-9001-102, scored 2.0 points higher on the mean North Star Ambulatory Assessment (NSAA) 48 weeks after treatment with SRP-9001 compared to pre-specified matched external control cohort (p value=0.0009)***

- ***Results continue to reinforce the tolerability profile of SRP-9001 with no new safety signals identified***

CAMBRIDGE, Mass., Jan. 10, 2022 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced topline results from Part 2 of Study SRP-9001-102 (Study 102), an ongoing, randomized, double-blind, placebo-controlled clinical trial to evaluate the safety, efficacy and tolerability of a single dose of SRP-9001 (delandistrogene moxeparvovec) in 41 patients with Duchenne muscular dystrophy, 21 of whom were in the placebo crossover cohort. 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. Results were presented today at the 40th Annual J.P. Morgan Healthcare Conference.

SRP-9001-treated participants from the placebo crossover group (n=20, aged 5-8 at time of dosing SRP-9001) scored a statistically significant 2.0 points higher on the mean North Star Ambulatory Assessment* at 48 weeks compared to propensity-score weighted external controls** (p value=0.0009). Mean NSAA scores from these Part 2 participants improved 1.3 points from baseline for the SRP-9001 treated group and the NSAA scores in the external control group (n=103) declined 0.7 points from baseline. Additional results will be shared at a future medical congress.

"We are delighted to report positive results for Part 2 of our blinded, placebo-controlled Study 102 in Duchenne, where the 48-week functional benefits of SRP-9001 in patients dosed at cross-over were statistically significant when compared to pre-specified matched external controls. Furthermore, the safety profile of SRP-9001 remains consistent with the wealth of previous clinical data," said Doug Ingram, president and chief executive officer, Sarepta. "Study 102, Part 2 results add to the totality of evidence for SRP-9001 generated thus far – with promising results across multiple clinical trials and more than 80 patients dosed, encompassing a wide range of phenotypes as well as the oldest and heaviest Duchenne patients to be dosed with a full body AAV gene therapy infusion to date. The totality of results that we have seen across our multiple trials bolsters our confidence in the potential disease-modifying benefits of this therapy and reinforces our conviction in the probability of success of EMBARK, our large, phase 3 placebo-controlled global study presently underway and dosing. We are reminded daily that Duchenne is a brutal, life-ending disease and SRP-9001 is the greatest near-term hope we all have to address the need for a therapy that changes the trajectory of this disease. We will continue to move as quickly as possible to bring SRP-9001 to patients in the United States and around the world."

The safety profile of patients treated in Part 2 of Study 102 is consistent with that seen in Part 1. There were no treatment-related serious adverse events, no deaths and no study discontinuations due to an adverse event. The most common treatment-related adverse event in patients treated in Part 2 was vomiting, similar to Part 1. For patients treated in Part 1, no new safety signals emerged after two years of follow up.

Study 102 remains ongoing and all participants continue to be monitored for safety in addition to longer-term assessments of functional outcomes.

*The NSAA is a 17-item rating scale that is used to measure functional motor abilities in ambulant individuals with Duchenne. It is used to monitor the progression of the disease and treatment effects which makes it suitable as an endpoint in clinical trials for Duchenne.

**The external control used a prospectively defined consolidated comparison group of Duchenne patients, matched for variables including age, steroid usage, baseline NSAA and timed function tests with the participants in Study 102. The prospectively defined propensity score analysis allows for a robust balancing of the multiple variables.

About Study SRP-9001-102 (Study 102)

Study SRP-9001-102 (Study 102) is a double-blind, 1:1 randomized, placebo-controlled clinical trial of SRP-9001 in 41 participants with Duchenne muscular dystrophy between the ages of 4 to 7. Study 102 uses clinical process SRP-9001 material and has two primary endpoints: micro-dystrophin expression at 12 weeks and change in NSAA total score at 48 weeks compared to placebo. Secondary endpoints include certain timed functional tests; micro-dystrophin expression measured by immuno-fluorescence fiber intensity; and micro-dystrophin expression measured by immuno-fluorescence percent dystrophin positive fibers. In Part 1, results from the treatment and placebo groups were compared through 48 weeks following treatment. In Part 2, the study remained blinded to the participants and investigators, while all participants in the placebo group crossed over to active treatment and all participants were followed for another 48 weeks while safety and efficacy were evaluated. Participants will be evaluated for five years total after treatment.

About SRP-9001 (delandistrogene moxeparvovec)

SRP-9001 (delandistrogene moxeparvovec) 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 of 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

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(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 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 the potential disease-modifying benefits of SRP-9001; our conviction in the probability of success of EMBARK; and expected plans and milestones, including sharing additional results at a future medical congress and continuing to move as quickly as possible to bring SRP-9001 to patients in the United States and around the world.

Known risk factors include, among others: success in pre-clinical trials and clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; 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 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, 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 press release. 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.

Source: Sarepta Therapeutics, Inc.

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iestepan@sarepta.com

Media Contact:

Tracy Sorrentino, 617-301-8566
tsorrentino@sarepta.com

Exhibit 99.2

Some see slow and steady scientific progress.

We see a revolution.

DOUG INGRAM
President and CEO

J.P. Morgan 40th Annual Healthcare Conference
January 10, 2022

SAREPTA THERAPEUTICS

SETH
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; the estimated number of patients suffering from Duchenne; the potential for our precision genetic medicine engine to generate a steady stream of personalized medicine therapeutics; the potential benefits of our technologies and scientific approaches; the potential benefits of SRP-9001, including the potential benefits of our SRP-9001 gene therapy construct; our belief that Study SRP-9001-102 results increase confidence in design and probability of success of Study 301 (EMBARK); the potential to seek accelerated approval for SRP-5051; our expected product revenue guidance of >$800M in 2022; and expected milestones and plans, including completing enrollment for EMBARK by mid-2022, announcing additional SRP-9001 data at a medical conference, discussing with the FDA the fastest potential path to bringing SRP-9001 to our community of patients and their families, submitting a BLA for SRP-9001 after a read out of EMBARK next year that is consistent with the totality of evidence of the benefits of SRP-9001, announcing SRP-9003-101 3-year data for low dose cohort and 2-year data for high dose cohort, finalizing our strategy for sarcoglycans (SRP-9003, SRP-9005, SRP-9004), starting our POC trial for SRP-6004 (Dysferlin) in late 2022, advancing earlier stage pipeline candidates through our partnered and internal programs, fully enrolling MOMENTUM Part B SRP-5051 by 2H22, advancing PPMO candidates for exons 45, 52 and 53, continuing the growth of our PMO-focused RNA franchise and advancing our gene editing programs and expanding our capabilities at our Gene Editing Innovation Center.

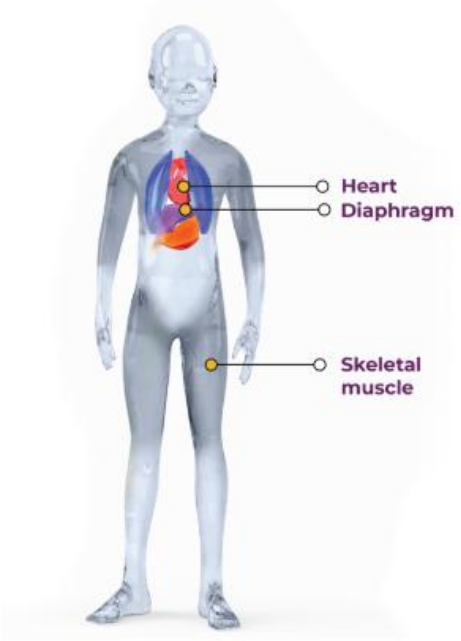
These forward-looking statements involve risks and uncertainties, many of which are beyond our control and are based on our current beliefs, expectations and assumptions regarding our business. Actual results and financial condition could materially differ from those stated or implied by these forward-looking 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: our data for our different programs, including PPMO and gene therapy-based product candidates, may not be sufficient for obtaining regulatory approval; our product candidates, including those with strategic partners, may not result in viable treatments suitable for commercialization due to a variety of reasons, including 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 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 or LGMD is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; we may not be able to comply with all FDA post-approval commitments and requirements with respect to EXONDYS 51, VYONDY 53 and AMONDYS 45 in a timely manner or at all; 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; current reimbursement models may not accommodate the unique factors of our gene therapy product candidates; 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, the ongoing COVID-19 pandemic; 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.

For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review Sarepta's filings with the SEC. We caution investors not to place considerable reliance on the forward-looking statements contained in this presentation. The forward-looking statements in this presentation are made as of the date of this presentation only and, other than as required under applicable law, Sarepta does not undertake any obligation to publicly update its forward-looking statements.

Duchenne Muscular Dystrophy (DMD)

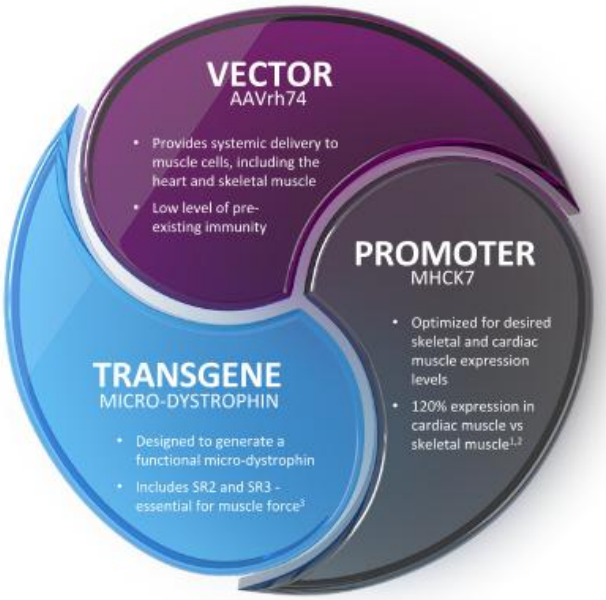
DMD affects approximately 1 in 3,500-5,000 males worldwide[1]

- 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

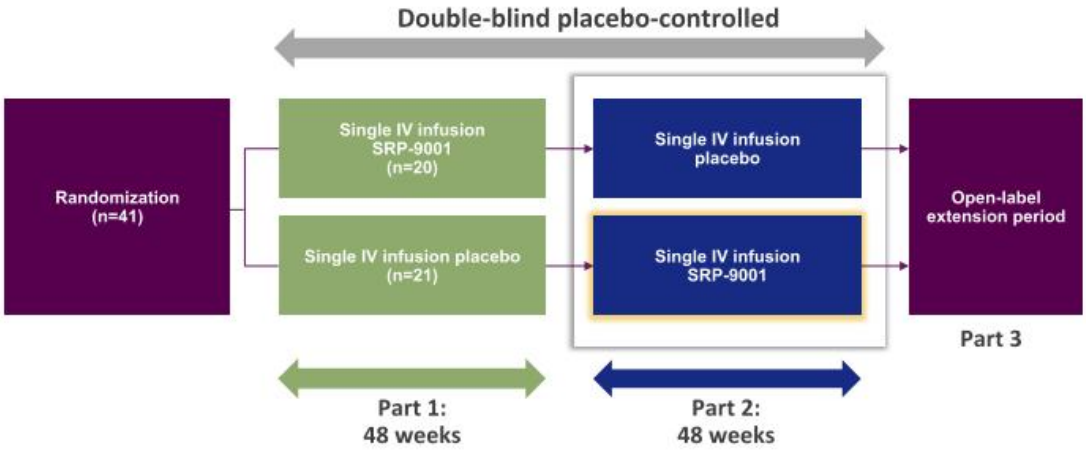


1. Potter et al. Functional and Histological Improvements Comparing 4 Micro-dystrophin Constructs in the mdx Mouse Model of DMD. ASGCT 2019.
2. Potter et al. Functional and Histological Improvements Comparing 4 Micro-dystrophin Constructs in the mdx Mouse Model of DMD. AIM 2019.
3. Nelson DM, Ervasti 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.

SRP-9001-102: Study Design

Randomized, double-blind, placebo-controlled study

An ongoing Phase 2 study is evaluating the safety, efficacy and tolerability of a single IV dose of SRP-9001 ($1.33x10^{14}$ vg/kg), compared to placebo, in boys with DMD aged 4–7 years old



External Control (EC) Comparison

- **Purpose of Propensity Score Weighting**
  - Used with the external comparison group which allowed balancing of relevant covariates such as age, baseline function (NSAA, RFF and 10MWR)
  - Use of a multi-variate approach is more robust than just using age
- **Study SRP-9001-102 Part 2 Statistical Analysis Plan** (for including the external comparison plans)
  - Prospectively defined, finalized and submitted to the FDA prior to database lock and review of Part 2 data
- **External Control Data Sources**
  - CINRG Duchenne Natural History Study (DNHS)
  - Pre-Publication Steroid Study
  - Lilly Study (H6D-MC-LVJJ)
- **Inclusion/Exclusion Criteria for External Controls**
  - Age matched at baseline
  - On a stable dose or dose equivalent of oral corticosteroids for at least 12 weeks before baseline; patients on 10 day on/10 day off regimen will be excluded
  - NSAA score ≥ 13 and ≤ 30 at baseline
  - Rise from the floor ≤ 10.4 seconds at baseline
  - 10MWR ≤ 9.1 seconds at baseline
- **Analysis Method**
  - Propensity Score Weighting Based on Age, NSAA, RFF and 10MWR
- **Primary Analysis**
  - Part 2 cross-over cohort (N=21)

SRP-9001-102: Propensity Score Weighting Balanced the Covariates Well

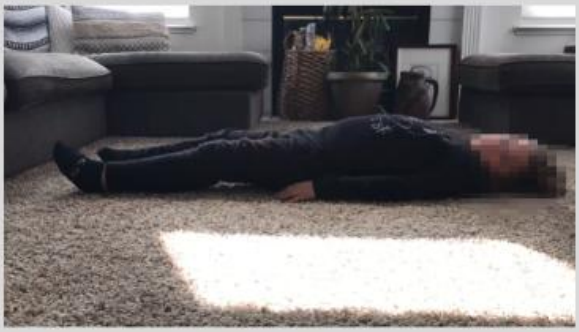
Study 102 Placebo Crossover Analysis Set

Parameter	Before Propensity Weighting					After Propensity Weighting	
	SRP-9001 (N=21)	CINRG (N=14)	Pre-Pub Steroid Study (N=83)	Lilly (N=29)	Total (N=126)	SRP-9001 (N=20)	External Control (N=103)
Age							
Mean (SD)	7.23 (1.10)	6.42 (0.93)	6.50 (0.89)	7.95 (0.47)	6.83 (1.02)	**7.24 (1.12)**	**7.03 (0.42)**
Median	7.03	6.28	6.37	7.92	6.57	7.07	6.97
Q1, Q3	6.28, 8.48	5.67, 7.32	5.86, 6.97	7.58, 8.17	6.01, 7.73	6.28, 8.49	6.17, 8.00
Min, Max	5.27, 8.89	5.40, 8.36	5.11, 8.71	7.08, 8.92	5.11, 8.92	5.27, 8.89	5.13, 8.92
NSAA Total Score							
Mean (SD)	23.6 (3.7)	25.1 (4.1)	23.8 (4.3)	23.5 (4.9)	23.9 (4.4)	**23.8 (3.7)**	**23.5 (1.9)**
Median	24	25.5	24	26	24.5	24.5	24
Q1, Q3	22.0, 26.0	24.0, 28.0	20.0, 28.0	19.0, 28.0	20.0, 28.0	22.0, 26.5	20.0, 27.0
Min, Max	13, 30	16, 30	13, 30	13, 30	13, 30	13, 30	13, 30
Time to Rise from the Floor							
Mean (SD)	4.02 (1.30)	4.62 (2.40)	4.65 (1.74)	6.07 (2.22)	4.97 (2.01)	**4.02 (1.34)**	**3.92 (0.59)**
Median	3.9	3.87	4.3	5.5	4.55	3.8	3.7
Q1, Q3	3.00, 4.50	3.10, 6.90	3.30, 5.30	4.50, 7.40	3.50, 6.00	2.95, 4.70	3.00, 4.60
Min, Max	2.40, 7.20	2.02, 10.10	1.90, 10.20	2.90, 10.40	1.90, 10.40	2.40, 7.20	1.90, 10.20
Time of 10MWR							
Mean (SD)	4.84 (1.12)	4.86 (1.28)	5.26 (0.93)	5.84 (1.17)	5.35 (1.07)	**4.84 (1.15)**	**4.83 (0.40)**
Median	4.7	4.78	5.4	5.9	5.3	4.65	4.9
Q1, Q3	4.20, 4.90	4.10, 5.10	4.50, 6.00	5.10, 6.70	4.60, 6.00	4.20, 5.00	4.10, 5.50
Min, Max	3.80, 9.10	3.03, 8.60	3.40, 7.50	3.80, 8.30	3.03, 8.60	3.80, 9.10	3.03, 8.00

A One-point Change on the NSAA Shows a Distinct and Observable Difference

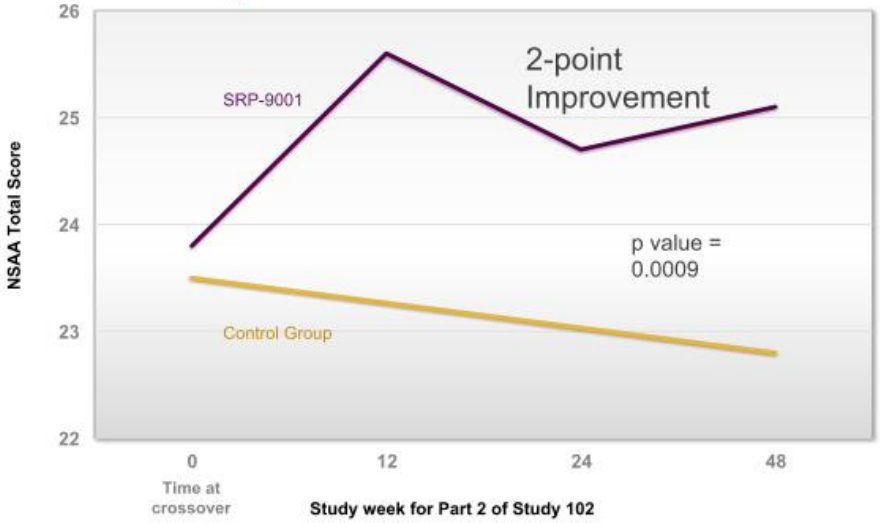
Stand from supine
NSAA: 2:1 change

Stand from supine
NSAA: 0



Video provided by presenter. Consent given for use.
NSAA, NorthStar Ambulatory Assessment.

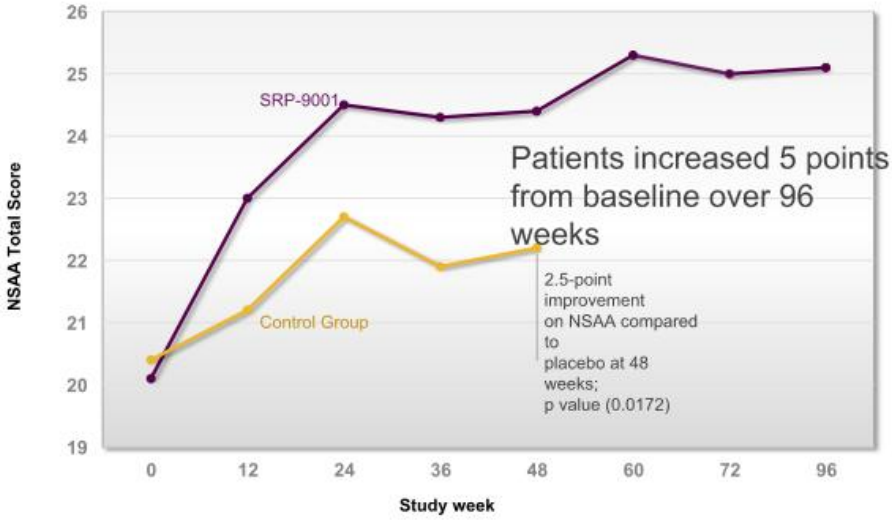
SRP-9001-102: Significant 2-point Improvement on NSAA in Patients Receiving SRP-9001 in Part 2 Compared to External Control Group at Week 48



- Mean age of patients at time of baseline NSAA, **7.24** years of age
- Change from baseline in NSAA at week 48 is +1.3 points in SRP-9001 group (n=20) vs. -0.7 in matched natural history controls (n=103) with a p value of 0.0009

SRP-9001-102: 96 Week Data in 4 to 5-year-old Cohort

Positive 5-point NSAA improvement from baseline sustained in SRP-9001 treated 4 to 5-year-olds at week 96



- Mean age of patients at week 48, 5.95 years of age (n=8)

Mean NSAA score across all ages (4 to 7-year-olds) treated in Part 1 remained stable out to week 96

SRP-9001-102: Safety Summary

- The safety profile of patients treated in Part 2 is consistent with that seen in Part 1
  - There were no treatment-related serious adverse events, no deaths and no patient study discontinuations due to an adverse event
  - The most common related adverse event in patients treated in Part 2, similar to Part 1, was vomiting
    - 16/21 treated patients in Part 2
- The total number of treatment-related treatment-emergent adverse events in year 2 for patients dosed in Part 1 was 6% of all treatment-emergent adverse events, similar to the placebo rate in Part 1 (5%) as assessed by the investigators who were blinded to assignment in both Part 1 and 2

Treatment-related Treatment-emergent Adverse Events* in Part 2

Safety Population – Part 2 Data

Preferred Term	SRP-9001 Placebo-crossover patients	SRP-9001 treated in Part 1	Total
	(N=21), n(%)	(N=20), n(%)	(N=41), n(%)
Subjects with any Treatment-Related TEAE	20 (95.2)	4 (20.0)	24 (58.5)
Vomiting	16 (76.2)	0	16 (39.0)
Decreased appetite	15 (71.4)	0	15 (36.6)
Nausea	10 (47.6)	1 (5.0)	11 (26.8)
Abdominal pain upper	8 (38.1)	1 (5.0)	9 (22.0)
Gamma-glutamyltransferase increased	6 (28.6)	0	6 (14.6)
Thrombocytopenia	5 (23.8)	0	5 (12.2)
Pyrexia	4 (19.0)	0	4 (9.8)
Glutamate dehydrogenase increased	3 (14.3)	0	3 (7.3)
Blood bilirubin increased	2 (9.5)	0	2 (4.9)
Fatigue	2 (9.5)	0	2 (4.9)
Headache	2 (9.5)	0	2 (4.9)
Ketonuria	1 (4.8)	1 (5.0)	2 (4.9)
Lethargy	2 (9.5)	0	2 (4.9)
Myalgia	2 (9.5)	0	2 (4.9)
White blood cell count decreased	2 (9.5)	0	2 (4.9)

**TEAEs seen in at least 2 subjects*

SRP-9001-102: Conclusions

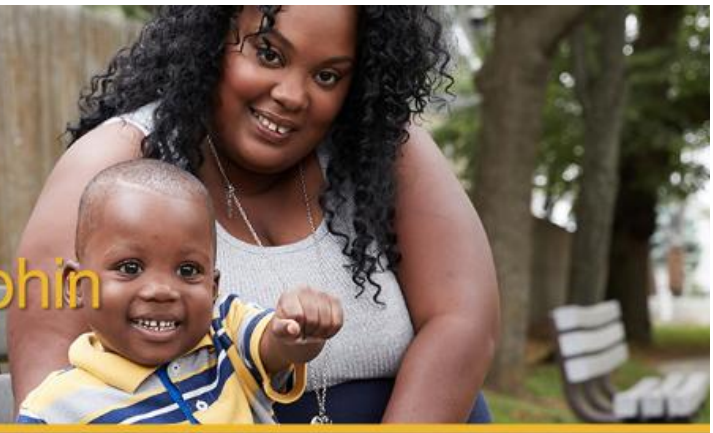
Results increase confidence in design and probability of success of Study 301 (EMBARK)

- Patients treated in Part 2 Study 102 improved 2 points vs. predefined external control group
  - Results were highly statistically significant
  - Results are consistent with observed treatment effect and increase our growing body of clinical evidence
- Observed durable response out to 2 years of treatment
- Total NSAA score of treated patients vs. placebo demonstrated a positive increase at all post-treatment time points
- No new safety signals observed and profile remains differentiated

2021 – 2022

GENE THERAPY

SRP-9001 micro-dystrophin Program Results for All Studies



STUDY 101

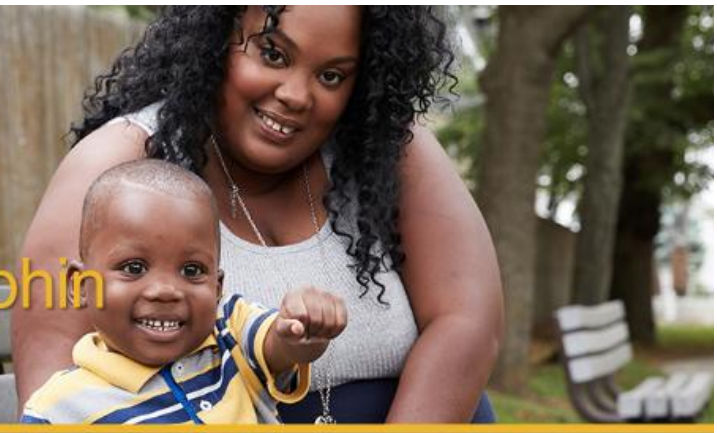
4 patients
4 to 7-year-olds

8.6-point improvement on NSAA compared to matched natural history group at 3 years; p value (<0.0001)

2021 – 2022

GENE THERAPY

SRP-9001 micro-dystrophin Program Results for All Studies



STUDY 101

4 patients

4 to 7-year-olds

8.6-point improvement on NSAA compared to matched natural history group at 3 years; p value (<0.0001)

STUDY 102 – Part 1

16 patients

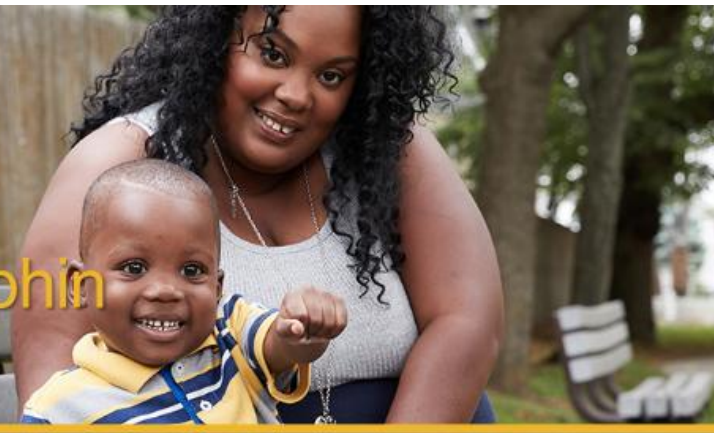
4 to 5-year-olds

2.5-point improvement on NSAA compared to placebo at 1 year; p value (0.0172)

2021 – 2022

GENE THERAPY

SRP-9001 micro-dystrophin Program Results for All Studies



STUDY 101
4 patients
4 to 7-year-olds

8.6-point improvement on NSAA compared to matched natural history group at 3 years; p value (<0.0001)

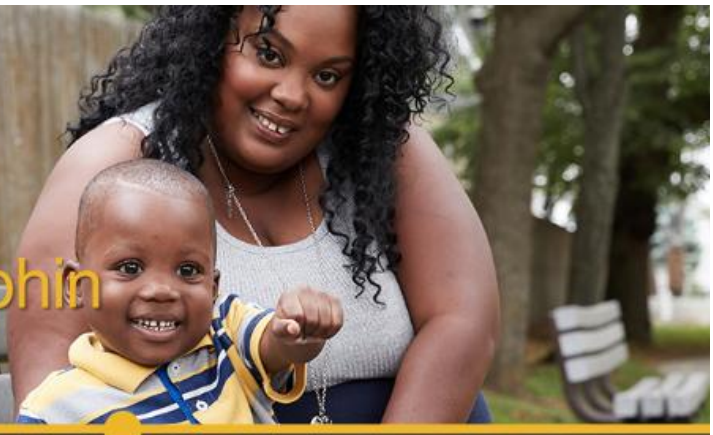
STUDY 102 – Part 1
16 patients
4 to 5-year-olds

2.5-point improvement on NSAA compared to placebo at 1 year; p value (0.0172)

STUDY 102 – Part 1
12 patients
6 to 7-year-olds

2.9-point improvement on NSAA compared to matched natural history group at 1 year; p value (0.0129)

[1]SRP-9001 treated 6-7 YO (N=12) were matched to natural history patients (N=21) from LEUVEN, Telethon and [illegible] for [illegible] NSAA at Week 48 is -0.3 in SRP-9001 group vs. -3.2 in matched natural history controls with a p value of 0.0129



2021 – 2022

GENE THERAPY

SRP-9001 micro-dystrophin Program Results for All Studies

STUDY 101
4 patients
4 to 7-year-olds

8.6-point improvement on NSAA compared to matched natural history group at 3 years; p value (<0.0001)

STUDY 102 – Part 1
16 patients
4 to 5-year-olds

2.5-point improvement on NSAA compared to placebo at 1 year; p value (0.0172)

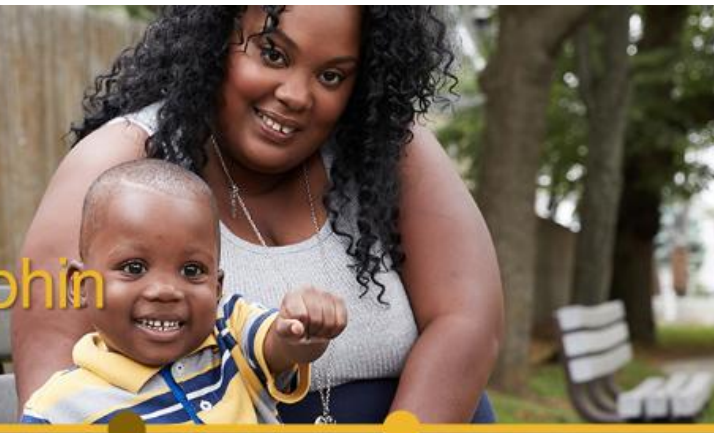
STUDY 102 – Part 1
12 patients
6 to 7-year-olds

2.9-point improvement on NSAA compared to matched natural history group at 1 year; p value (0.0129)

STUDY 102 – Part 2
21 patients
5 to 8-year-olds

2-point improvement on NSAA compared to matched natural history group at 1 year; p value (0.0009)

2021 – 2022



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SRP-9001 micro-dystrophin Program Results for All Studies

STUDY 101
4 patients
4 to 7-year-olds

8.6-point improvement on NSAA compared to matched natural history group at 3 years; p value (<0.0001)

STUDY 102 – Part 1
16 patients
4 to 5-year-olds

2.5-point improvement on NSAA compared to placebo at 1 year; p value (0.0172)

STUDY 102 – Part 1
12 patients
6 to 7-year-olds

2.9-point improvement on NSAA compared to matched natural history group at 1 year; p value (0.0129)

STUDY 102 – Part 2
21 patients
5 to 8-year-olds

2-point improvement on NSAA compared to matched natural history group at 1 year; p value (0.0009)

STUDY 103 – Cohort 1
First 11 patients
4 to 7-year-olds

3-point improvement on NSAA from baseline at 6 months

Study 103 is open label
[2]6 months, data cut 10/07/21

2021 – 2022

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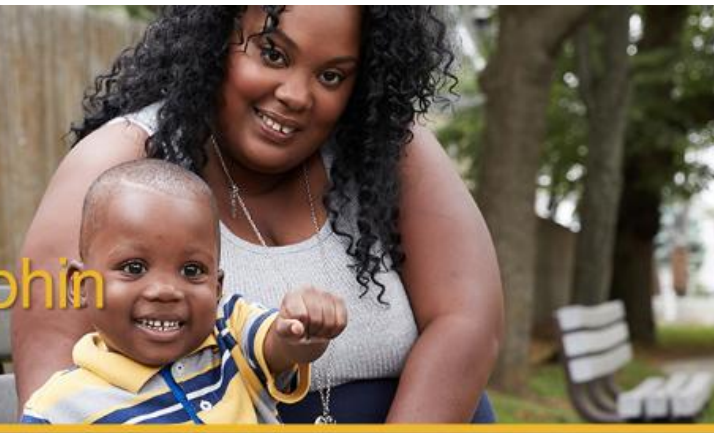
SRP-9001 micro-dystrophin



STUDY 301

EMBARK

- Only global gene therapy clinical trial underway
- Poised for success based on the clinical evidence generated to date
- On track to complete enrollment by mid-2022

Commercialized Therapeutics and Pipeline Programs

Robust and Expanding RNA Franchise in Duchenne

21 consecutive quarters of revenue growth; CAGR of 40% FY2017-FY2021; ~30% revenue growth anticipated (2022 vs 2021)

$

2.1B

in cash on 12/31/21 (Unaudited)

Generating a Steady Stream of Personalized Medicine Therapeutics

3 Platforms, 40+ Programs, 2 Pivotal Clinical Trials

RNA

- Duchenne Muscular Dystrophy (PMO)
- Duchenne Muscular Dystrophy (PPMO)

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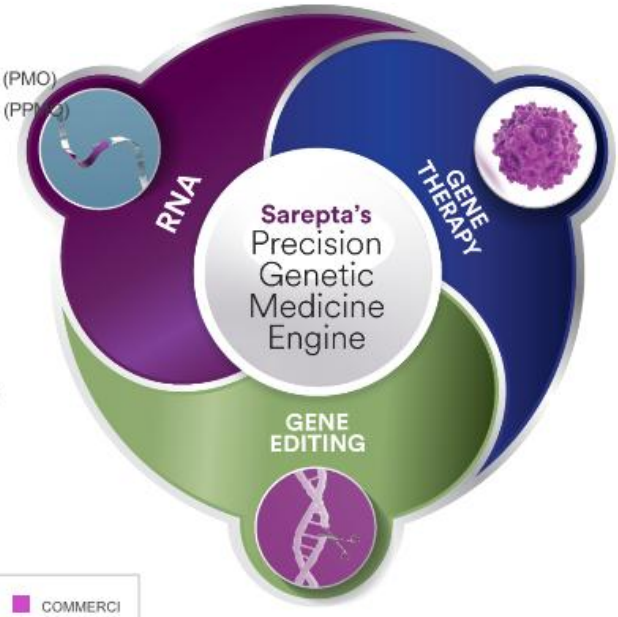
- Duchenne Muscular Dystrophy



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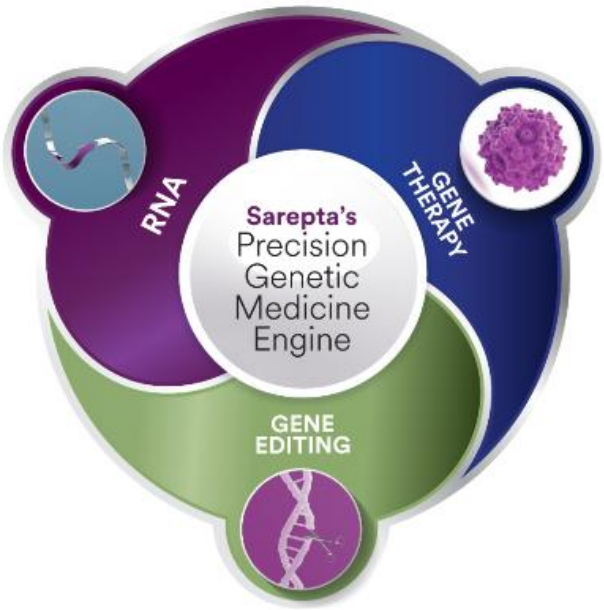
- Duchenne Muscular Dystrophy
- Limb-girdle Muscular Dystrophy
- Charcot-Marie-Tooth
- Mucopolysaccharidosis IIIA
- Cardiomyopathy
- Central Nervous System Disorders
- Pompe Disease
- Niemann-Pick Disease
- Rett Syndrome
- Dravet Syndrome
- Angelman Syndrome
- Muscle/Central Nervous System
- Emery-Dreifuss Muscular Dystrophy
- Multiple Sclerosis

DISCOVERY | PRECLINICAL | CLINICAL | COMMERCIAL

2022 Milestones



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- **Duchenne:**
  - SRP-9001-301 EMBARK fully enrolled by mid-2022
  - SRP-9001 additional data at medical conferences
- **Limb-girdle muscular dystrophy:**
  - SRP-9003-101 (3-year data for low dose cohort and 2-year data for high dose cohort)
  - Finalize strategy for sarcoglycans (SRP-9003, SRP-9005, SRP-9004)
  - SRP-6004 (Dysferlin) anticipate starting POC trial in late 2022
- **Advance earlier stage pipeline candidates (partnered and internal programs)**

RNA

- MOMENTUM Part B SRP-5051 fully enrolled by 2H22
- Advance PPMO candidates for exons 45, 52, 53
- Continued growth of PMO-focused RNA franchise, expected 2022 product revenue of >$800M

GENE EDITING

- Continue to advance gene editing programs for Duchenne
- Expand capabilities at our Gene Editing Innovation Center (Durham, NC)

Dragging tomorrow into today

#DraggingTomorrowIntoToday