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): October 4, 2019

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 October 4, 2019, Sarepta Therapeutics, Inc. issued a press release and conducted an investor webcast presenting positive functional results from the SRP-9003 (MYO-101) gene therapy trial to treat Limb-girdle muscular dystrophy Type 2E, or beta-sarcoglycanopathy. A copy of the press release and the presentation are furnished as Exhibits 99.1 and 99.2 and are incorporated herein by reference.

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 Number	Description
99.1	Press release dated October 4, 2019.
99.2	Presentation dated October 4, 2019, CLINICAL UPDATE: SRP-9003 BETA-SARCOGLYCANOPATHY GENE THERAPY PROGRAM LIMB-GIRDLE MUSCULAR DYSTROPHY TYPE 2E FUNCTIONAL DATA.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document).

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: October 4, 2019

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

Sarepta Therapeutics Announces Positive Functional Results from the SRP-9003 (MYO-101) Gene Therapy Trial to Treat Limb-Girdle Muscular Dystrophy Type 2E, or Beta-Sarcoglycanopathy

-- Improvements on functional measures seen in all three participants --

-- Significant reduction in creatine kinase maintained over nine months --

-- Results follow positive and robust expression and biomarker data presented earlier in 2019 --

CAMBRIDGE, Mass., Oct. 4, 2019 (GLOBE NEWSWIRE) – Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced the nine-month functional results from three Limb-girdle muscular dystrophy Type 2E (LGMD2E) clinical trial participants who received SRP-9003. SRP-9003 is an investigational gene therapy intended to transduce skeletal and cardiac muscle with a gene that codes for the full-length, native beta-sarcoglycan protein, the lack of which is the sole cause of LGMD2E.

In Cohort 1 of the SRP-9003 study, three participants ages 4-13 were treated with an infusion of SRP-9003 at a dose of $5x10^{13}$vg/kg. Improvements in functional outcomes were observed at day 270 (nine months) for all three participants.

“We have now observed consistent functional improvements, in addition to high levels of expression of the missing protein of interest and strong results in related biomarkers, in both of our first cohorts for Duchenne muscular dystrophy (SRP-9001) and LGMD2E (SRP-9003). We intend to test one higher dose of SRP-9003 in LGMD2E participants, select our clinical dose and then advance our SRP-9003 program, along with our other five LGMD programs, as rapidly as possible,” said Doug Ingram, Sarepta’s president and chief executive officer. “With the results of our first LGMD2E cohort, Sarepta continues to build its gene therapy engine, an enduring model created to design, develop and bring to the medical and patient community transformative therapies for those living with, and too often dying from, rare genetic disease.”

At Day 270, mean creatine kinase (CK) was significantly reduced compared to baseline. CK is an enzyme biomarker strongly associated with muscle damage.

At Day 270, all three participants showed improvements from baseline across all functional measures, including the North Star Assessment for Dysferlinopathy (NSAD), time to rise, four-stair climb, 100-m walk test and 10-meter walk test. These results are distinctly different from what an age-matched, natural history group would predict.

No new safety signals were observed and the safety profile seen to date supports the ability to dose escalate in the next cohort of the study. As previously disclosed, two participants in the study had elevated liver enzymes, one of which was designated a serious adverse event (SAE), as the participant had associated transient increase in bilirubin. Both events occurred when the participants were tapered off oral steroids and, in both instances, elevated liver enzymes returned to baseline and symptoms resolved following supplemental steroid treatment.

“LGMD2E is a devastating neuromuscular disease with no current treatment options so we are very pleased to observe a functional improvement in study participants who received SRP-9003,” said Jerry Mendell, M.D., principal investigator at the Center for Gene Therapy in the Abigail Wexner Research Institute at Nationwide Children’s Hospital and lead investigator for the study.

Sarepta had previously shared expression results from the study, which found that in two-month post-treatment muscle biopsies, clinical trial participants showed a mean of 51% beta-sarcoglycan positive fibers, as measured by immunohistochemistry (IHC), substantially exceeding the pre-defined 20% measure for success. Mean fiber intensity, as measured by IHC, was 47% compared to normal control.

About SRP-9003 and the Phase I/IIa Gene Transfer Clinical Trial

SRP-9003 uses the AAVrh74 vector, which is designed to be systemically and robustly delivered to skeletal, diaphragm and cardiac muscle without promiscuously crossing the blood brain barrier, making it an ideal candidate to treat peripheral neuromuscular diseases. As a rhesus monkey-derived AAV vector, AAVrh74 has lower immunogenicity rates than reported with other common human AAV vectors. The MHCK7 promoter has been chosen for its ability to robustly express in the heart, which is critically important for patients with LGMD2E, many of whom die from pulmonary or cardiac complications.

This first-in-human study is evaluating a single intravenous infusion of SRP-9003 among children with LGMD2E between the ages of four and 15 years with significant symptoms of disease.

About Limb-Girdle Muscular Dystrophy

Limb girdle muscular dystrophies are genetic diseases that cause progressive, debilitating weakness and wasting that begin in muscles around the hips and shoulders before progressing to muscles in the arms and legs.

Patients with LGMD2E begin showing neuromuscular symptoms such as difficulty running, jumping and climbing stairs before age 10. The disease, which is an autosomal recessive subtype of LGMD, progresses to loss of ambulation in the teen years and often leads to death before age 30. There is currently no treatment or cure for LGMD2E.

Sarepta has five LGMD gene therapy programs in development, including subtypes for LGMD2E, LGMD2D, LGMD2C, LGMD2B and LGMD2L, and holds an option for a sixth program for LGMD2A.

About Sarepta Therapeutics

Sarepta is at the forefront of precision genetic medicine, having built an impressive and competitive position in Duchenne muscular dystrophy (DMD) and more recently in gene therapies for Limb-girdle muscular dystrophy diseases (LGMD), MPS IIIA, Pompe and other CNS-related disorders, totaling over 20 therapies in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. Sarepta is fueled by an audacious but important mission: to profoundly improve and extend the lives of patients with rare genetic-based diseases. For more information, please visit www.sarepta.com.

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 our intention to test one higher dose of SRP-9003 in LGMD2E participants, select our clinical dose

and then advance our SRP-9003 program, along with our other five LGMD programs, as rapidly as possible; Sarepta continuing to build an enduring gene therapy model created to design, develop and bring to the medical and patient community transformative therapies for those living with rare genetic disease; the safety profile of SRP-9003 seen to date supporting the ability to dose escalate in the next cohort of the study; SRP-9003 being an ideal candidate to treat peripheral neuromuscular diseases; the potential benefits of the AAVrh74 vector and the MHCK7 promoter; and our mission to profoundly improve and extend the lives of patients with rare genetic-based diseases.

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 testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and initial results from a clinical trial do not necessarily predict final results; the data presented in this release 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 Sarepta utilizes to assess particular safety or efficacy parameters may yield different statistical results, and even if Sarepta believes the data collected from clinical trials of its product candidates are positive, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities; Sarepta's ongoing research and development efforts may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons, some of which may be outside of Sarepta's control, including possible limitations of company 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 even if Sarepta's programs result in new commercialized products, Sarepta may not achieve any significant revenues from the sale of such products; 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, 2018, 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.

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.

Source: Sarepta Therapeutics, Inc.

Sarepta Therapeutics, Inc.

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iestepan@sarepta.com

Media:

Tracy Sorrentino, 617-301-8566

tsorrentino@sarepta.com

Exhibit 99.2

CLINICAL UPDATE:
SRP-9003 BETA-SARCOGLYCANOPATHY GENE THERAPY PROGRAM
LIMB-GIRDLE MUSCULAR DYSTROPHY TYPE 2E FUNCTIONAL DATA

Louise Rodino-Klapac
Senior Vice President, Gene Therapy
Sarepta Therapeutics, Inc.

October 4, 2019

SAREPTA THERAPEUTICS

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 the safety profile of SRP-9003 seen to date supporting the ability to dose escalate; the potential benefits of the AAVrh74 vector and the MHCK7 promoter; our clinical programs in LGMD; and our plans regarding dose escalation, selection of final dose for registration trial and engaging with global regulatory agencies to discuss pivotal trial designs.

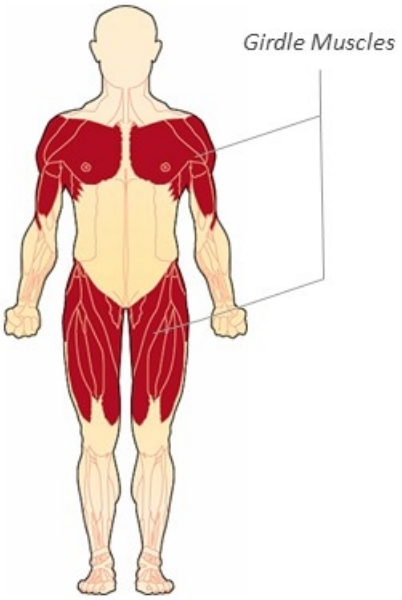
These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: 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, and initial results from a clinical trial do not necessarily predict final results; 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 Sarepta utilizes to assess particular safety or efficacy parameters may yield different statistical results, and even if Sarepta believes the data collected from clinical trials of its product candidates are positive, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities; Sarepta's ongoing research and development efforts may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons, some of which may be outside of Sarepta's control, including possible limitations of Company 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 even if Sarepta's programs result in new commercialized products, Sarepta may not achieve any significant revenues from the sale of such products; 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, 2018 or most recently filed 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. You should not place undue reliance on forward-looking statements. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except to the extent required by applicable law or SEC rules.

LGMDS ARE DEVASTATING MUSCULAR DYSTROPHIES

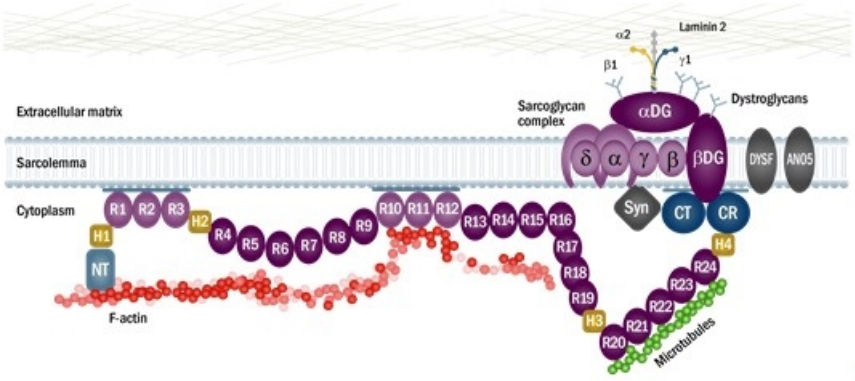
MONOGENIC, RARE NEUROMUSCULAR DISEASES

- **LGMDs are progressive, debilitating muscle-wasting diseases with no therapies[1,2]**
  - Affect males and females equally
  - Affect skeletal muscle
  - Affect cardiac muscle in some types
  - Elevated creatine kinase (CK) levels
  - Symptoms often develop before age 10
  - Loss of ambulation often in teens
  - More severe forms mimic DMD
  - Death can result before age 30
- **Consistent disease progression within each LGMD subtype**
- **Each of the ~30 LGMD subtypes is a rare disease**



1. NIH website. www.nih.gov. Accessed June 16, 2018.
2. MDA website. www.mda.org/disease/limb-girdle-muscular-dystrophy/causes-inheritance. Accessed June 16, 2018.

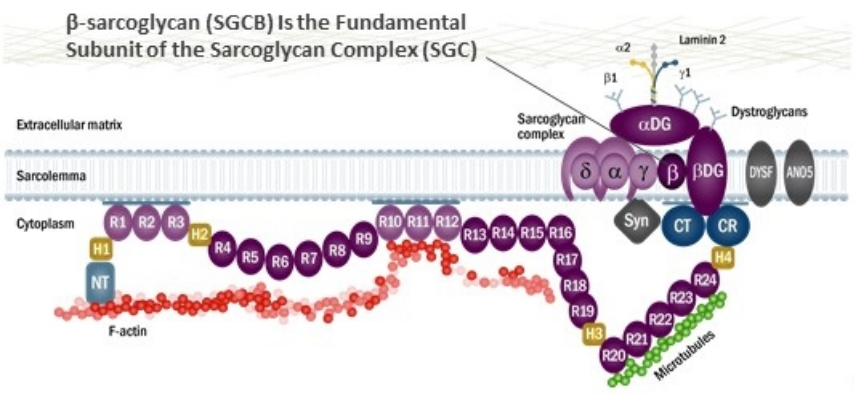
LGMD PORTFOLIO



- **Sarcoglycans** prevent muscle damage during contraction
  - All 4 functional sarcoglycans must be present to form a functional sarcoglycan complex (SCG)
    - **β-sarcoglycan (SRP-9003)**
    - **α-sarcoglycan (SRP-9004)**
    - **γ-sarcoglycan (SRP-9005)**
  - Sarcoglycan deficiency leads to dystrophin deficiency
- **Dysferlin** and **ANO5** support muscle membrane repair (MYO-201 and SRP-9006)
  - Failed muscle repair leads to chronic muscle degeneration

ANO5, anoctamin-5; FKRP, fukutin-related protein; POMT, protein-O-mannosyltransferase; TRIM, tripartite motif.

LGMD PORTFOLIO ADDRESSES MONOGENIC MUTATIONS THAT RESULT IN THE LACK OF ONE OF THE PROTEINS COMPRISING THE DYSTROPHIN-ASSOCIATED PROTEIN COMPLEX



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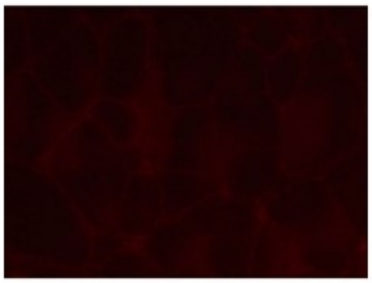
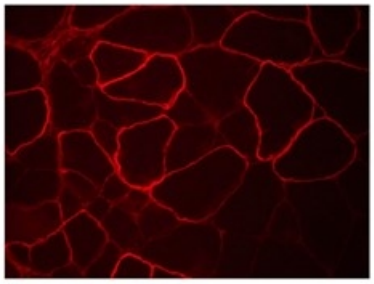
PRE-CLINICAL MODELS CORRELATED EXPRESSION AND FUNCTION

≥20 PERCENT EXPRESSION LEADS TO INCREASED FUNCTION

Pretreatment

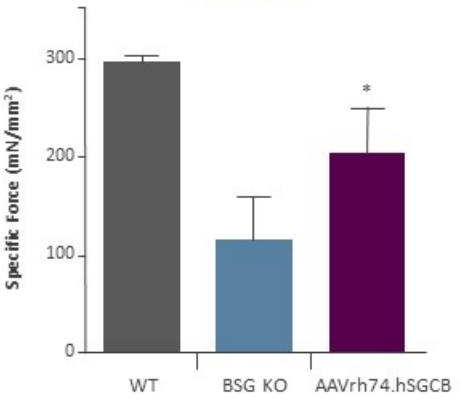


Post Treatment



5x10^{12} vg/kg

Function



LGMD2E PHASE I/II STUDY: COHORT 1 (N=3)

SAREPTA THERAPEUTICS

LGMD TYPE 2E OPEN-LABEL TRIAL DESIGN

- **Up to 6 subjects with LGMD**
  - Cohort 1: 3 subjects; 4-15 years of age, **$5x10^{13}$ vg/kg** AAVrh74.MHCK7.SGCB systemic delivery
- **Inclusion criteria**
  - A confirmed SGCB mutation in both alleles
  - Negative for AAVrh74 antibodies
  - >40% of Normal 100 meter walk test
- **60-day needle muscle biopsy**
- **Prednisone 1 day prior to gene transfer, 30 days 1 mg/kg, taper**

OUTCOME MEASURES

- **Primary endpoint**
  - **≥ 20% β–sarcoglycan expression**
  - Safety
- **Secondary endpoints, including:**
  - Decrease in CK
  - Functional endpoints
    - North Star Assessment for LGMD (NSAD)
    - 100m
    - 10m
    - 4 stairs
    - Time to rise

LGMD2E STUDY EXPRESSION RESULTS: COHORT 1 (N=3)

SAREPTA THERAPEUTICS

LGMD2E SUBJECT DEMOGRAPHICS AT BASELINE[1]

Subject	Age (years)	Mutation	Weight (kg)	CK Levels at Baseline (U/L)
1	13	Exon 3	55	10,727
2	4	Exon 4	17	12,826
3	13	Exon 3	50	10,985

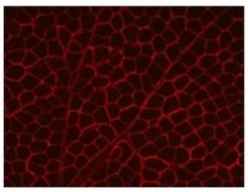
- Exons 3-6 encode for the extracellular domain of SGCB
- Mutations in these exons lead to complete absence of or severely reduced expression of SGCB, and a severe phenotype that includes cardiomyopathy[2]

β-sarcoglycan gene therapy is investigational and has not been reviewed or approved by any regulatory authority. || ClinicalTrials.gov Identifier: NCT03652259.

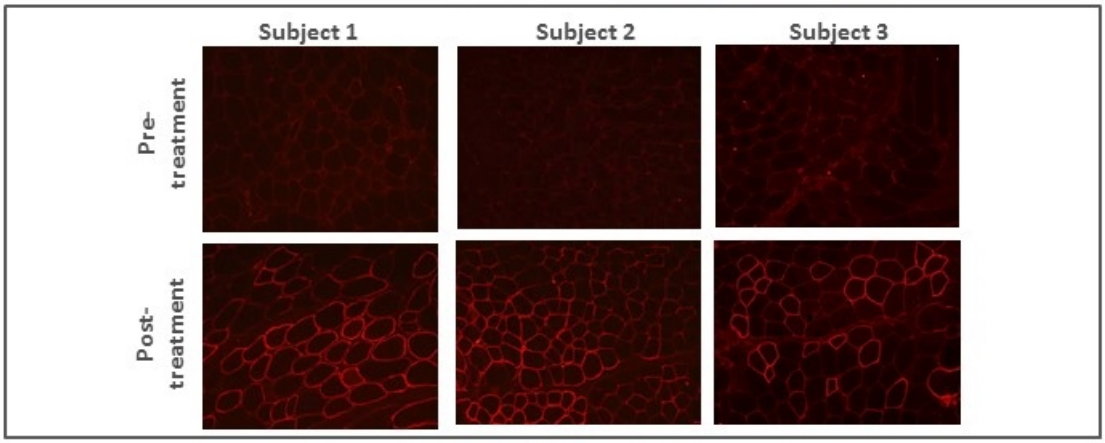
1. Sarepta Therapeutics 2019. Data on file. 2. Sempliciní C, et al. *Neurology*. 2015;84(17):1772-1781.

ROBUST β-SARCOGLYCAN EXPRESSION IN MUSCLE BIOPSIES IN ALL 3 SUBJECTS AT A DOSE OF $5X10^{13}$ VG/KG

Normal Control



Beta-Sarcoglycan Expression (IHC)



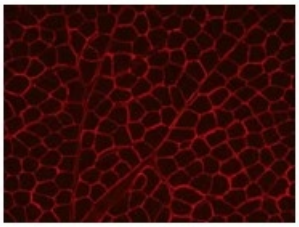
	Percentage of SGCB-positive Fibers	Intensity
Mean (n=3)	51%	47%

ROBUST β-SARCOGLYCAN EXPRESSION IN MUSCLE BIOPSIES IN ALL 3 SUBJECTS AT A DOSE OF $5X10^{13}$ VG/KG

Subject	Percentage of SGCB-Positive Fibers	Mean Intensity
1	63%	47%
2	49%	57%
3	42%	38%
Mean	**51%**	**47%**

SGCB EXPRESSION SIGNIFICANTLY UPREGULATED SGC COMPLEX AT A DOSE OF 5 X 10^{13} VG/KG

Normal Control



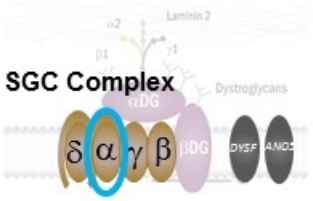
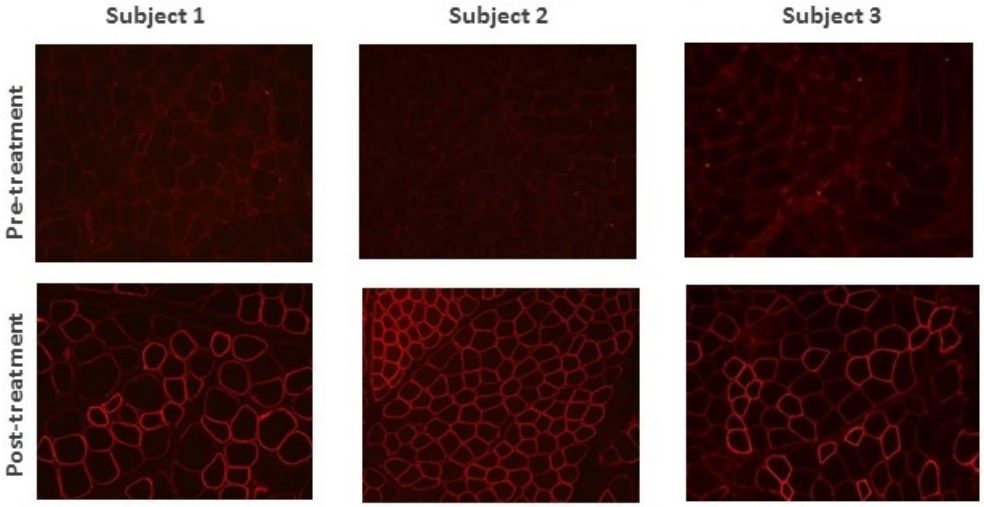


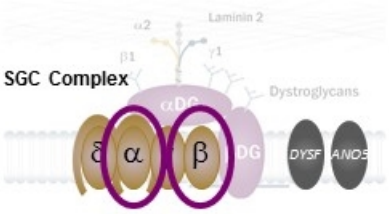
Image adapted from Fairclough RJ, et al. *Nat Rev Genet*. 2013;14(6):373-378.

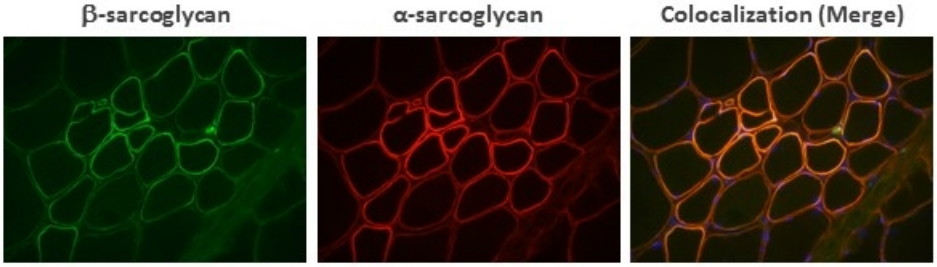
α-Sarcoglycan Expression (IHC)



β-sarcoglycan gene therapy is investigational and has not been reviewed or approved by any regulatory authority.
Sarepta Therapeutics 2019. Data on file. ClinicalTrials.gov: NCT03652259.

SGCB EXPRESSION SIGNIFICANTLY UPREGULATED SGC COMPLEX PROTEIN AT A DOSE OF $5x10^{13}$ VG/KG





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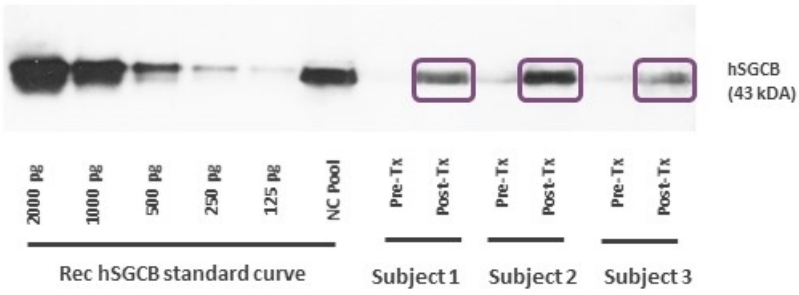
Sarepta Therapeutics 2019. Data on file. ClinicalTrials.gov: NCT03652259. Image adapted from Fairclough RJ, et al. *Nat Rev Genet*. 2012;14(6):373-378.

DETECTION OF β-SARCOGLYCAN EXPRESSION BY WESTERN BLOT POST-TREATMENT IN ALL 3 SUBJECTS AT DAY 60

Subject	Mean SGCB Expression vs Normal
1	34.7%
2	39.2%
3	34.5%
Mean	36.1%



The gene transfer delivers full-length SGCB

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Sarepta Therapeutics 2019. Data on file. ClinicalTrials.gov Identifier: NCT03652259.

β-SARCOGLYCAN EXPRESSION IS SUPPORTED BY VECTOR GENOME COUNTS

Beta-Sarcoglycan Expression (IHC)

	Percentage of Beta-Sarcoglycan-positive Fibers	Intensity
Mean (n=3)	**51%**	**47%**

Beta-Sarcoglycan (Western Blot)

	Percent of Normal
Mean (n=3)	**36.1%**

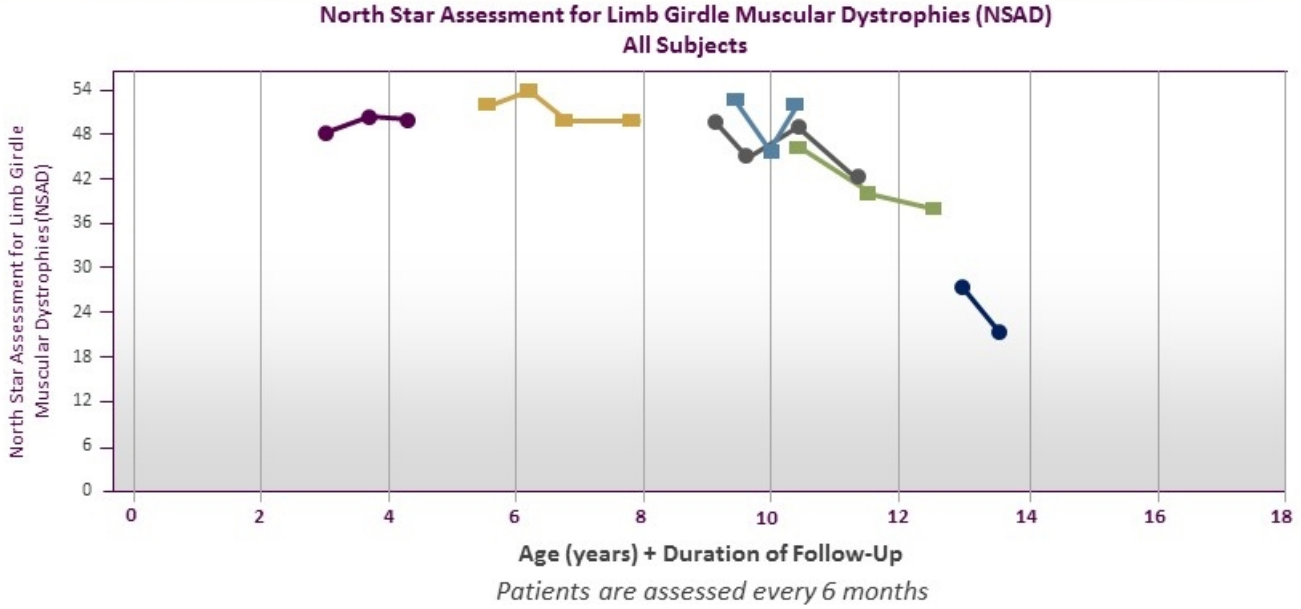
Vector Genome Number

	Vector Copies/µg DNA	Copies per Nucleus
Mean (n=3)	8.4E04	0.60

LGMD2E STUDY
FUNCTIONAL DATA SUMMARY:
COHORT 1 (n=3)

SAREPTA
THERAPEUTICS

NATURAL HISTORY DATA GENERATED BY LINDA LOWES & LINDSAY ALFANO AT NATIONWIDE CHILDREN'S HOSPITAL



Patients are assessed every 6 months

CREATINE KINASE (CK) LEVELS ARE REDUCED WITH β-SARCOGLYCAN GENE THERAPY

		CK Levels (U/L)					
Subject	Age (years)	Baseline	Day 30	Day 60	Day 90	Day 180	Day 270
1	13	10,727	619	2257	1135	1553	2300
2	4	12,826	4795	910	2159	5070	2665
3	13	10,985	687	2061	2392	10,055	1295

9 Months: 82% Reduction in CK

ClinicalTrials.gov Identifier: NCT03652259

SUMMARY OF CLINICAL DATA AT 9 MONTHS

ALL SUBJECTS SHOWED IMPROVEMENT IN ALL FUNCTIONAL MEASURES

Subject	Assessment	NSAD	Time to Rise (sec)	4 Stairs Up (sec)	100 m (sec)	10 m (sec)
1	Baseline	40	5.0	2.4	49.3	5
	Day 270	41	4.1	2.3	43.2	4.5
2	Baseline	48	1.5	1.6	59.3	3.4
	Day 270	54	1.2	1.3	48.4	3.2
3	Baseline	41	3.5	2.8	49.9	5.2
	Day 270	47	3.0	1.9	48.6	4.3

SUMMARY OF CLINICAL DATA AT 9 MONTHS

ALL SUBJECTS SHOWED IMPROVEMENT IN ALL FUNCTIONAL MEASURES

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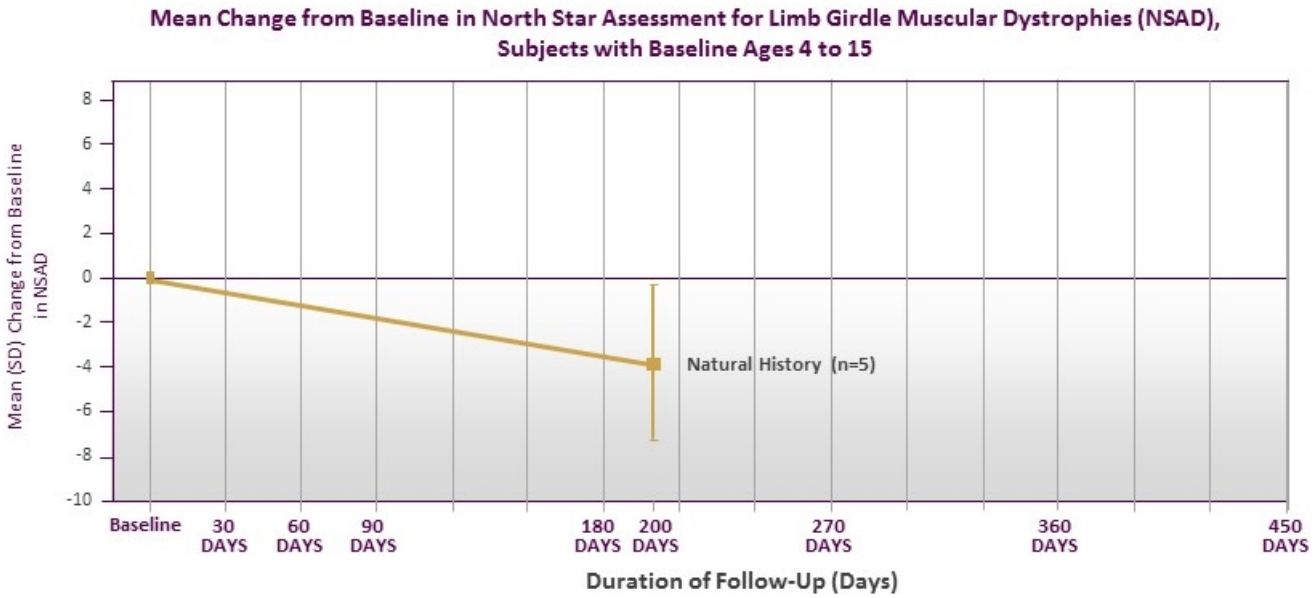
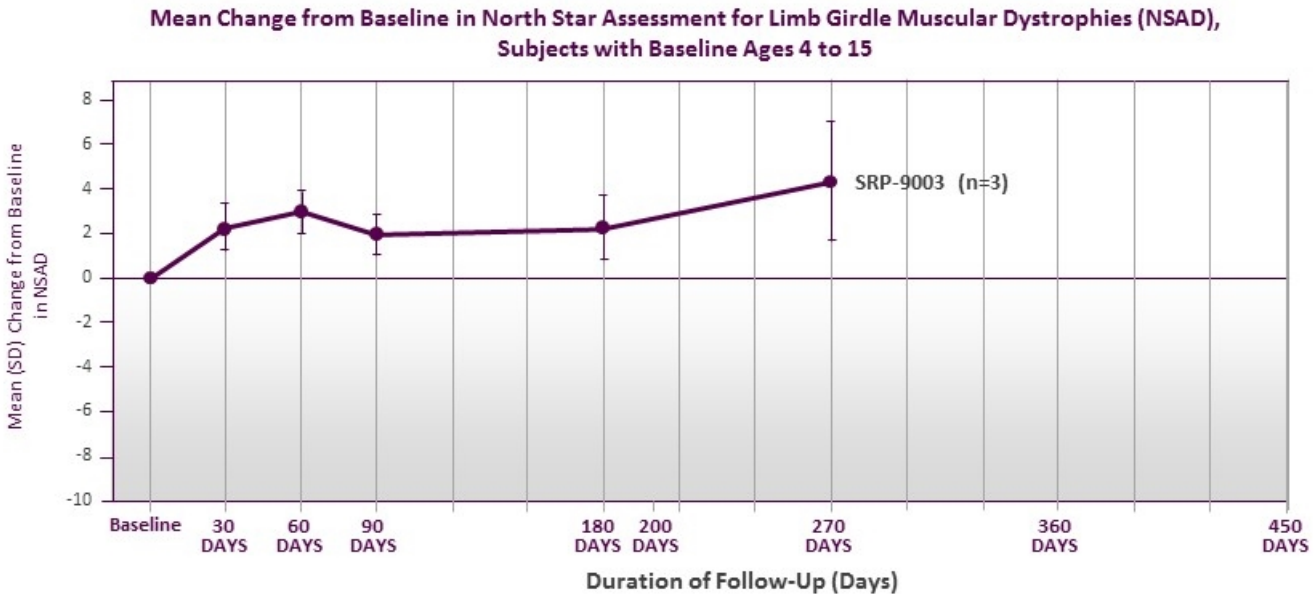
BASELINE DEMOGRAPHICS OF AGE-MATCHED NATURAL HISTORY CONTROL GROUP (4-15 YEARS)

Subject	Age (years)
1	5
2	12
3	10
4	9
5	9

AGE-MATCHED LGMD2E NATURAL HISTORY COHORT

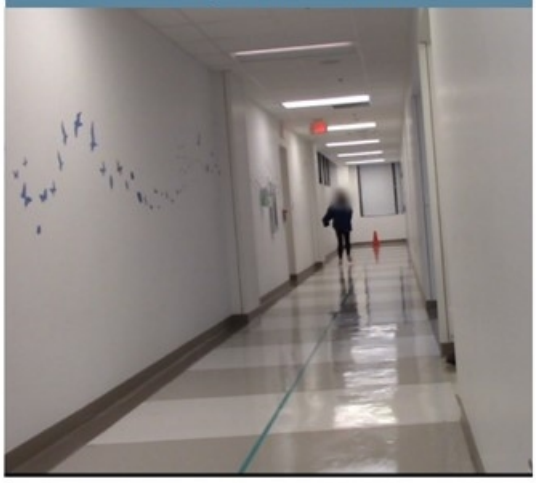


SRP-9003 TREATED LGMD2E PATIENTS (LOW DOSE COHORT 1)

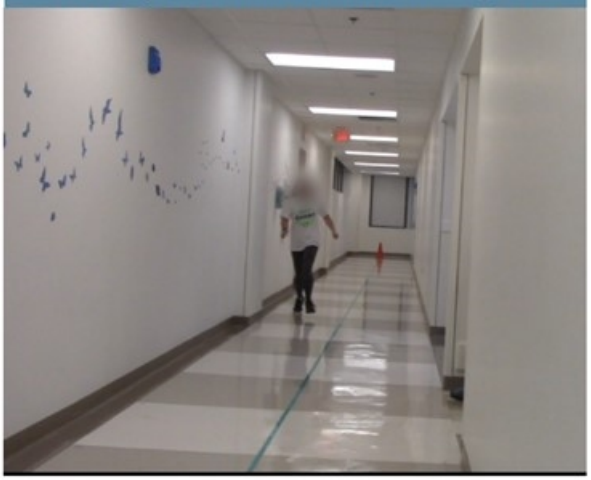


PATIENT 1: 100M RUNNING

100 m baseline running
Limited hip extension/flexion



100 m 9 months post gene therapy
Good hip extension/flexion; faster speed



PATIENT 2: TRUNK CONTROL

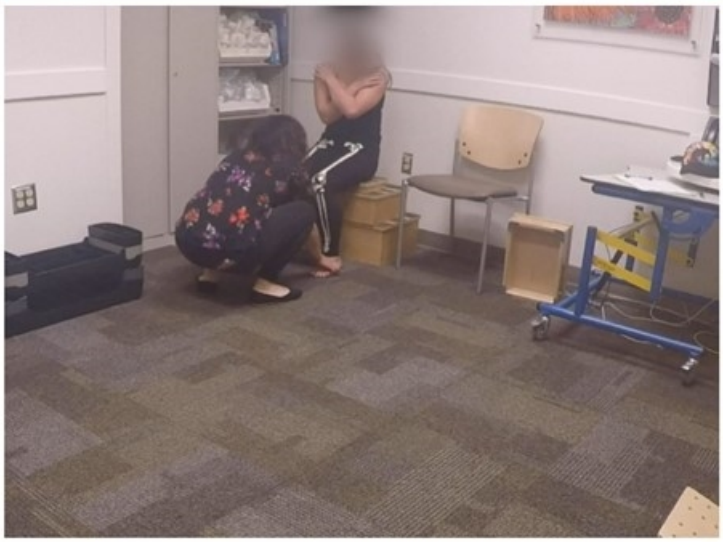
Baseline
Poor Trunk Control

9 months Post Gene Therapy
Clinic Visit

PATIENT 3: GETTING UP FROM SITTING

Baseline Getting up from Sitting

9 months post gene therapy



SAFETY TO DAY 270 (N=3)

- 2 subjects had elevated liver enzymes, 1 of which was designated an SAE, as the subject had associated transient increase in bilirubin
  - Both events occurred when the subjects were tapered off oral steroids
  - Elevated liver enzymes returned to baseline and symptoms resolved within days following supplemental steroid treatment
- 2 patients had transient mild nausea generally within the first week coincident with increased steroid dosing
  - Did not correlate with liver enzyme elevations or any other abnormality
- No other clinically significant laboratory findings

SUMMARY

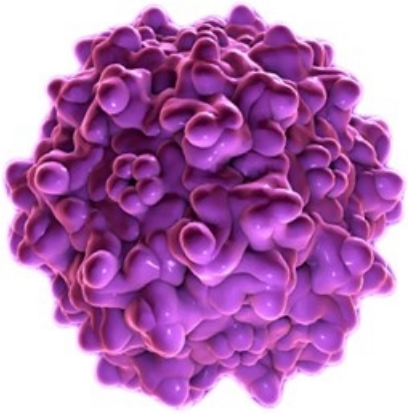
Construct optimized for use in LGMD

- AAVrh74 efficiently transduces all muscle types
- Low pre-existing immunity for AAVrh74
- MHCK7 promoter allows for cardiac and skeletal transgene muscle expression

Preliminary clinical results

- Widespread beta-sarcoglycan expression across all patients at a systemic dose of **$5x10^{13}$ vg/kg**
- Substantial reduction in CK
- Consistent improvement in all functional measures in all patients
- Safety profile supports dose escalation

FUTURE CLINICAL DEVELOPMENT:
DOSE ESCALATION TO IDENTIFY REGISTRATIONAL TRIAL DOSE



AAVrh74.MHCK7.SGCB
(SRP-9003)

Next Steps:

- Dose Escalation: 4-fold increase
- Final dose for registration trial will be selected from 2 doses studied
- Engagement with global regulatory agencies to discuss pivotal trial designs

SAREPTA'S CURRENT CLINICAL PROGRAMS IN LGMD

Partnered Program: Calpain (LGMD2A)
NCH: Dr Zarife Sahenk

	LGMD2E	LGMD2D	LGMD2B	LGMD2C	LGMD2L
Program	SRP-9003	SRP-9004	MYO-201	SRP-9005	SRP-9006
Target Function	Stabilizes DAPC, prevents muscle damage during contraction	Stabilizes DAPC, prevents muscle damage during contraction	Muscle membrane repair	Stabilizes DAPC, prevents muscle damage during contraction	Muscle membrane repair

Programs shown are investigational at Sarepta Therapeutics, Inc. and have not been reviewed or approved by any regulatory authority.
Sarepta Therapeutics 2019. Data on file

QUESTIONS & ANSWERS

SAREPTA THERAPEUTICS

CLINICAL UPDATE:

SRP-9003 BETA-SARCOGLYCANOPATHY GENE THERAPY PROGRAM
LIMB-GIRDLE MUSCULAR DYSTROPHY TYPE 2E FUNCTIONAL DATA

Louise Rodino-Klapac
Senior Vice President, Gene Therapy
Sarepta Therapeutics, Inc.

October 4, 2019