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): March 25, 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)

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

(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 (see General Instructions A.2. below):

☐ 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))

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 March 25, 2019, Sarepta Therapeutics, Inc. (the "Company") presented 9-month functional and creatine kinase (CK) data from baseline from the 4 patients in the Phase 1 open-label study of the Company's micro-dystrophin gene therapy candidate for Duchenne muscular dystrophy. A copy of the presentation of Louise Rodino-Klapac, Ph.D. is furnished as Exhibit 99.1 and is incorporated herein by reference.

The information in this report furnished pursuant to Item 7.01, including Exhibit 99.1 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	Clinical Update: Micro-dystrophin Study-101, Louise Rodino-Klapac, Ph.D.

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 hereunto duly authorized.

Sarepta Therapeutics, Inc.

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

Date: March 25, 2019

Exhibit 99.1

Clinical Update: Micro-dystrophin Study-101

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

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 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 early results from a clinical trial do not necessarily predict final results; 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 encouraging, 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; if the actual number of patients suffering from DMD is smaller than estimated, Sarepta’s revenue and ability to achieve profitability may be adversely affected; 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.

Micro-dystrophin Study-101 Clinical Trial Design

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Open-Label Trial Design

- **Cohort B**
  - 4 Patients
  - 4-7 years of age
- **Inclusion criteria**
  - Confirmed *DMD* mutation
  - Negative for AAVrh74 antibodies

ClinicalTrials.gov Identifier: NCT03375164.
Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Cohort B (4-7 Years of Age) Endpoints

- **Primary endpoint**
  - Safety
- **Secondary endpoints**
  - Change in micro-dystrophin expression pre- vs post-treatment
  - Decrease in CK
  - 100-meter timed test (100 m)
  - North Star Ambulatory Assessment (NSAA; 10-meter timed test included)
  - Timed up and go (TUG)
  - Ascend and descend 4 steps
  - Hand-held dynamometry (HHD)
  - Cardiac magnetic resonance imaging (at 1 year)

ClinicalTrials.gov Identifier: NCT03375164.
Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Subject Demographics at Baseline

Subject	Age (years)	CK Levels at Baseline (U/L)
1	5	20,691
2	4	23,414
3	6	34,942
4	4	29,210

ClinicalTrials.gov Identifier: NCT03375164.
Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Micro-dystrophin Study-101 Summary: All Patients (n=4)

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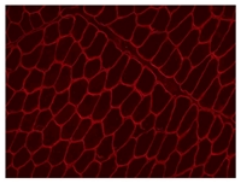
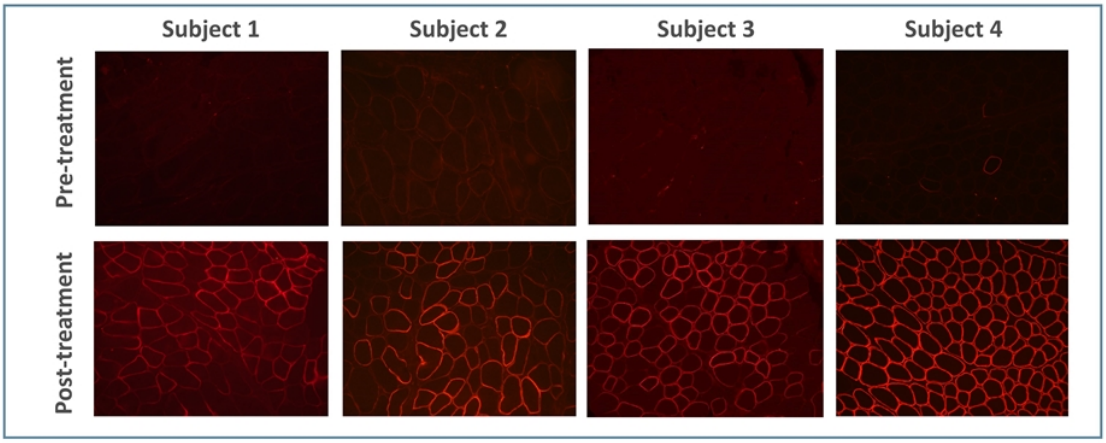
Robust Micro-dystrophin Expression in Muscle Fibers from the Gastrocnemius in All 4 Patients at Day 90

Micro-dystrophin Expression (IHC)

Normal Control



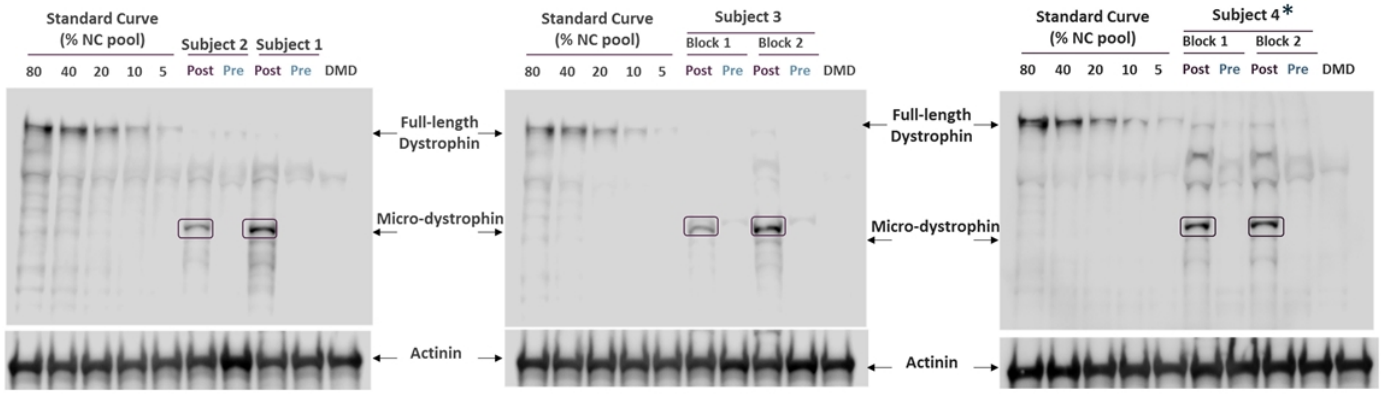


	Intensity	Percentage of Dystrophin-positive Fibers
Mean (n=4)	**96.0%**	**81.2%**

ClinicalTrials.gov Identifier: NCT03375164.

Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Detection of Micro-dystrophin Expression by Western Blot Post-treatment in All 4 Patients at Day 90



Western Quantitation Method	Mean Micro-dystrophin Expression (N=4) vs Normal
Sarepta	**74.3%** (not adjusted for fat and fibrotic tissue)
Nationwide	**95.8%** (adjusted for fat and fibrotic tissue)

p-Tyr phosphotyrosine; ULOQ, upper limit of quantification.
*Samples diluted 1:4 due to sample above ULOQ.
ClinicalTrials.gov Identifier: NCT03375164.
Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Summary of Expression Data for all 4 Patients

Micro-dystrophin Expression (IHC)

	Intensity	Percentage of Dystrophin-positive Fibers
Mean (n=4)	**96.0%**	**81.2%**

Micro-dystrophin Expression (Western Blot)

	Sarepta (not adjusted for fat/fibrosis)	Nationwide (adjusted for fat/fibrosis)
Mean (n=4)	**74.3%**	**95.8%**

Vector Genome Number

	Vector Copies/µg DNA	Copies per Nucleus
Mean (n=4)	**$>10^5$**	**3.3**

ClinicalTrials.gov Identifier: NCT03375164.
Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Clinical Data Summary for Study-101 (n=4)

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Summary of NSAA Data for All 4 Patients

NSAA Change from Baseline to Day 270

Patient	Baseline	Day 30	Day 60	Day 90	Day 180	Day 270	Change from Baseline
1	18	22	24	23	25	26	8
2	19	21	23	25	27	27	8
3	26	28	28	30	30	28	2
4	19	20	20	25	25	27	8
Mean Improvement	20.5	22.75	23.75	25.75	26.75	27	6.5

ClinicalTrials.gov Identifier: NCT03375164.
Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Summary of Clinical Data: Consistent Durable Improvement at 9 Months

Change from Baseline to Day 270

Subject	Assessment	NSAA (Δ)	Time to Rise (sec)	4 Stairs Up (sec)	100 m (sec)
1	Baseline	18	3.7	3.4	49.3
	Day 270*	26	3.0	2.3	43.2
2	Baseline	19	3.0	3.8	49.9
	Day 180*	27	3.7	2.6	48.6
	Day 270	***27***	***3.3***	***2.7***	***50.3***
3	Baseline	26	3.9	1.9	59.3
	Day 180*	30	3.4	1.8	48.4
	Day 270	***28***	***2.8***	***1.9***	***50.7***
4	Baseline	19	4.1	4.8	67.2
	Day 90*	25	2.3	2.2	50.7
	Day 270	***27***	***2.4***	***2.2***	***49.7***
Average	Change From Baseline	6.5 point Improvement	.8 second Improvement	1.2 second Improvement	7.95 second Improvement

** Last timepoint disclosed at World Muscle Society*

ClinicalTrials.gov Identifier: NCT03375164.

Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Summary of CK Data for All 4 Patients

CK Change from Baseline to Day 270

Patient	Baseline	Day 30	Day 60	Day 90	Day 180	Day 270
1	20691	-	2984	2444	18476	6317
2	23414	10427	4283	41920	6209	10494
3	34942	10430	2966	2546	9650	18855
4	29210	7215	908	1382	2580	4262

ClinicalTrials.gov Identifier: NCT03375164.
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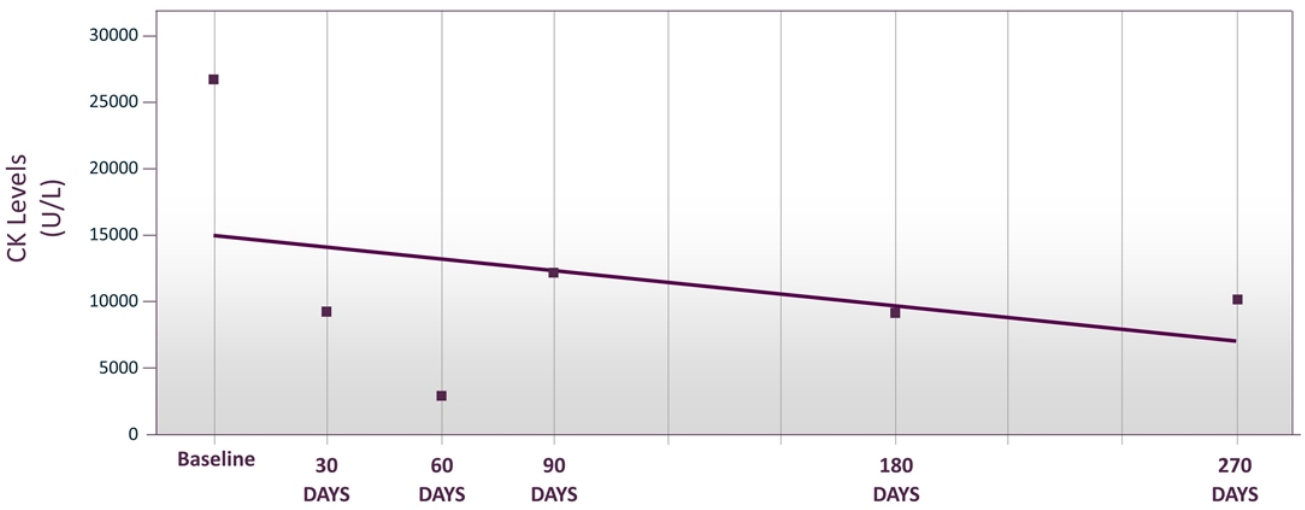
CK Significantly Decreases Over Time

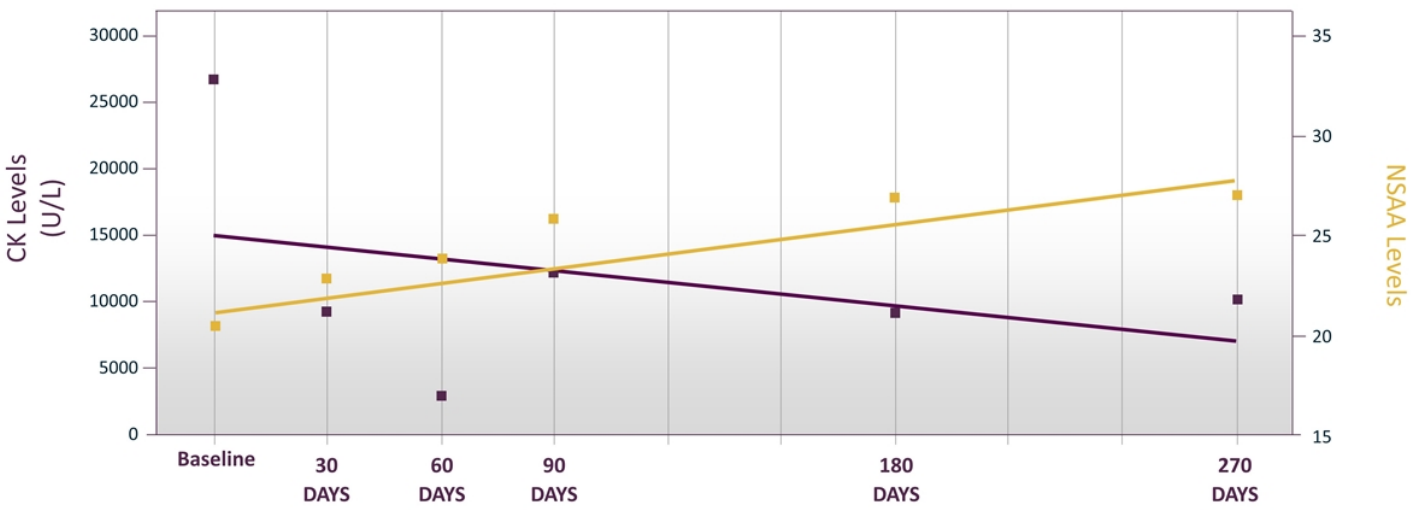
Mean CK Change from Baseline to Day 270



ClinicalTrials.gov Identifier: NCT03375164.
Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

NSAA Significantly Increases Over Time

Mean NSAA Change from Baseline to Day 270



ClinicalTrials.gov Identifier: NCT03375164.
Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Safety (n=4)

- No serious adverse events in this study
- 3 Patients had elevated γ-glutamyl transpeptidase, which resolved with steroid treatment within a week
- No other clinically significant laboratory findings
- Subjects had transient nausea generally within the first week coincident with increased steroid dosing
  - Did not correlate with liver enzyme elevations or any other abnormality

ClinicalTrials.gov Identifier: NCT03375164.
Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Summary

- All 4 treated Patients are doing well
  - Biomarkers show large magnitude of effect within 3 months (CK and dystrophin)
  - Initial functional data shows consistent and persistent improvement from Baseline to Day 270
  - Early results show treated patients performing better than DMD natural history would predict
  - Favorable safety profile to date with 9 months of follow-up
  - Currently enrolling Study-102 (24 patient placebo controlled study)

Question and Answer

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Thank You

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