

**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 09, 2023**

**Sarepta Therapeutics, Inc.**

(Exact name of Registrant as Specified in Its Charter)

**Delaware**  
(State or Other Jurisdiction  
of Incorporation)

**001-14895**  
(Commission File Number)

**93-0797222**  
(IRS Employer  
Identification No.)

**215 First Street**  
**Cambridge, Massachusetts**  
(Address of Principal Executive Offices)

**02142**  
(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 9, 2023, 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, 2022 during the Company’s presentation at the 41st 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 \$2.0 billion as of December 31, 2022 and that the Company expects total net product revenue (unaudited) for the fourth quarter ended December 31, 2022 to be approximately \$235.5 million, and expects total net product revenue (unaudited) for the year ended December 31, 2022 to be approximately \$843.3 million, 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, 2022 and its results of operations for the three months and year ended December 31, 2022. The audit of the Company’s financial statements for the year ended December 31, 2022 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 9, 2023, 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 9, 2023 are furnished with this report as Exhibit 99.1 and Exhibit 99.2, respectively.

*The information in this report, including Exhibit 99.1 and Exhibit 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.*

**Forward-Looking Statements**

This Current Report contains forward looking statements. Any statements contained in this Current Report that are not statements of historical fact may be deemed to be forward-looking statements. Words such as “believes,” “anticipates,” “plans,” “expects,” “will,” “may,” “intends,” “prepares,” “looks,” “potential,” “possible” and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to the Company’s expected financial results.

These forward-looking statements involve risks and uncertainties, many of which are beyond the Company’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: the estimates and judgments the Company makes, or the assumptions on which it relies, in preparing its consolidated financial statements could prove inaccurate; the Company’s revenues and operating results could fluctuate significantly, which may adversely affect the Company’s stock price; and those risks identified under the heading “Risk Factors” in the Company’s Quarterly Report on Form 10-Q for the quarter ended September 30, 2022 as well as other SEC filings made by the Company which you are encouraged to review.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits

<b>Exhibit Number</b>	<b>Description</b>
99.1	<a href="#">Press Release dated January 9, 2023</a>
99.2	<a href="#">Sarepta Therapeutics, Inc. Presentation at the 41st Annual J.P. Morgan Healthcare Conference, dated January 9, 2023</a>
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 hereunto duly authorized.

**Sarepta Therapeutics, Inc.**

Date: January 9, 2023

By: /s/ Douglas S. Ingram

Douglas S. Ingram

President and Chief Executive Officer

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## Sarepta Therapeutics Reports Preliminary\* Fourth Quarter and Full-Year 2022 Net Product Revenues

- *The Company expects to exceed 2022 full-year guidance for net product revenues*
- *Preliminary net product revenues for the fourth quarter and full-year 2022 are expected to total \$235.5 million and \$843.3 million, respectively*
- *Preliminary year-end 2022 cash balance of approximately \$2.0 billion*

CAMBRIDGE, Mass., Jan. 9, 2023 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today reported preliminary\* fourth quarter and full-year 2022 net product revenues as part of their presentation today at the 41st Annual J.P. Morgan Healthcare Conference.

### Financial Update\* (preliminary and unaudited):

- Fourth quarter 2022 net product revenues are expected to be approximately \$235.5 million, a 32% increase over the same period in 2021. Our net product revenues do not include collaboration revenues.
- Net product revenues for the full-year 2022 are expected to be \$843.3 million, a 38% increase over the same period of 2021, which is also expected to exceed Sarepta's net product revenue guidance of \$825-840 million. Our expected net product revenues do not include collaboration revenues.
- As of December 31, 2022, the Company had preliminary cash, cash equivalents, restricted cash and investments of approximately \$2.0 billion, as compared to approximately \$2.1 billion as of December 31, 2021.

"We are pleased to have closed out 2022 on an extremely strong note with continued execution across our three RNA-based PMO therapies, delivering net product revenue that is expected to exceed our upwardly revised guidance range. These preliminary results reflect the mission-driven dedication and expertise of our teams to serve the nearly 30% of Duchenne patients who are amenable to one of our approved therapies," said Doug Ingram, president and chief executive officer, Sarepta Therapeutics.

\*These preliminary selected financial results are unaudited and subject to adjustment. Sarepta will report its final and complete fourth quarter and full-year 2022 financial results in late February 2023. The Company has not completed its financial closing procedures for the quarter or year ended December 31, 2022 and its actual results could be materially different from these preliminary financial results.

### 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

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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](http://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](http://www.sarepta.com). We encourage investors and potential investors to consult our website regularly for important information about us.*

#### **Forward-Looking Statements**

In order to provide Sarepta's investors with an understanding of its current results and future prospects, this press release contains statements that are forward-looking. 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," "may," "intends," "prepares," "looks," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to our expected financial results.

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: the estimates and judgments we make, or the assumptions on which we rely, in preparing our consolidated financial statements could prove inaccurate; our revenues and operating results could fluctuate significantly, which may adversely affect our stock price; and those risks identified under the heading "Risk Factors" in our Quarterly Report on Form 10-Q for the quarter ended September 30, 2022 as well as other SEC filings made by the Company which you are encouraged to review.

Source: Sarepta Therapeutics, Inc.

#### **Investor Contact:**

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[tsorrentino@sarepta.com](mailto:tsorrentino@sarepta.com)

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**DOUG INGRAM**  
President and CEO

Sarepta Therapeutics, Inc. (NASDAQ:SRPT)  
JPMorgan Healthcare Conference  
San Francisco, California  
**JANUARY 9, 2023**

## 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 future operations, financial performance and projections; our current guidance for 2023 for our three currently approved therapies of more than \$925 million in net revenue; our opportunities in the rare disease space; potential solutions and market opportunities with our RNA technologies, gene therapy and gene editing; the potential benefits of our technologies and scientific approaches; the potential benefits of PMO and PPMO; the potentially transformative benefits of SRP-9001, including SRP-9001's potential to transform the trajectory of Duchenne, the potential restoration of DAPC, reduced CK and improved histopathology, and the potential of improved benefit received from SRP-9001 over time; our belief that the 9001-dysmorphin protein is reasonably likely to predict clinical benefit; our belief that our internal gene therapy capabilities complemented by partnerships will meet demand to launch SRP-9001, if approved, and drive competitive costs with continued improvements to drive upside; our belief that the transformative one-time therapy, SRP-9001, will cost the system less than the value it will provide to the Duchenne community; the potential of gene therapy's applicability across disease; the potential of our collaborations and partnerships; and expected milestones and plans, including our belief that we may receive an advisory Committee meeting for SRP-9001, launching SRP-9001 in the middle of 2023, if SRP-9001 is approved, having a readout of our confirmatory trial for SRP-9001 at the end of the year, expanding the available label of SRP-9001 after additional studies by 2024, publishing our perspective on the holistic approach to value innovative one-time therapies like SRP-9001, our expectation that we will have approximately 30 clinical trials ongoing by the end of 2023, continuing to build our pipeline, and our expectations related to our future financial performance, including if SRP-9001 is approved, our forecasted peak year SRP-9001 net product revenue will be nearly \$4 billion, tracking to nearly \$5 billion in total net product venue, by 2026, if we meet our strategic plan goals, including if SRP-9001 is approved, we will be cash positive and profitable by next year, and updating our guidance to include SRP-9001 net sales for 2023, if SRP-9001 is approved.

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: 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 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 impact of the COVID-19 pandemic; 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 and LGMD is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; 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; 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.

A Bellwether Moment...



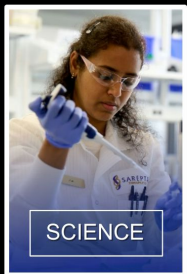
**BENJAMIN**  
Living with Duchenne  
muscular dystrophy



THE OPPORTUNITY

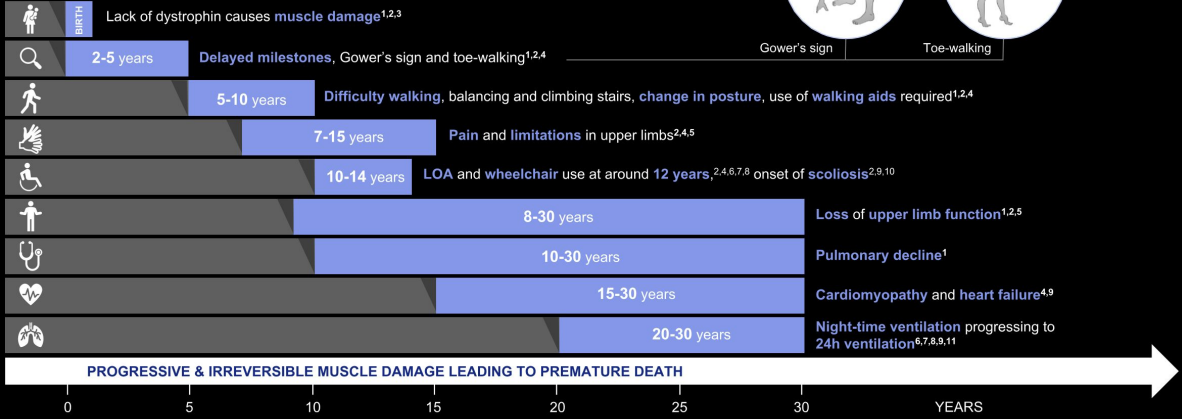
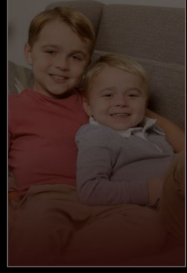
SRP-9001

Poised to transform the trajectory of  
Duchenne muscular dystrophy

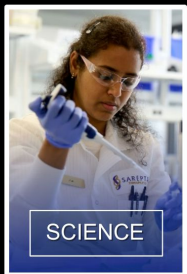


SCIENCE

# Duchenne is progressive and causes irreversible muscle damage and loss of function

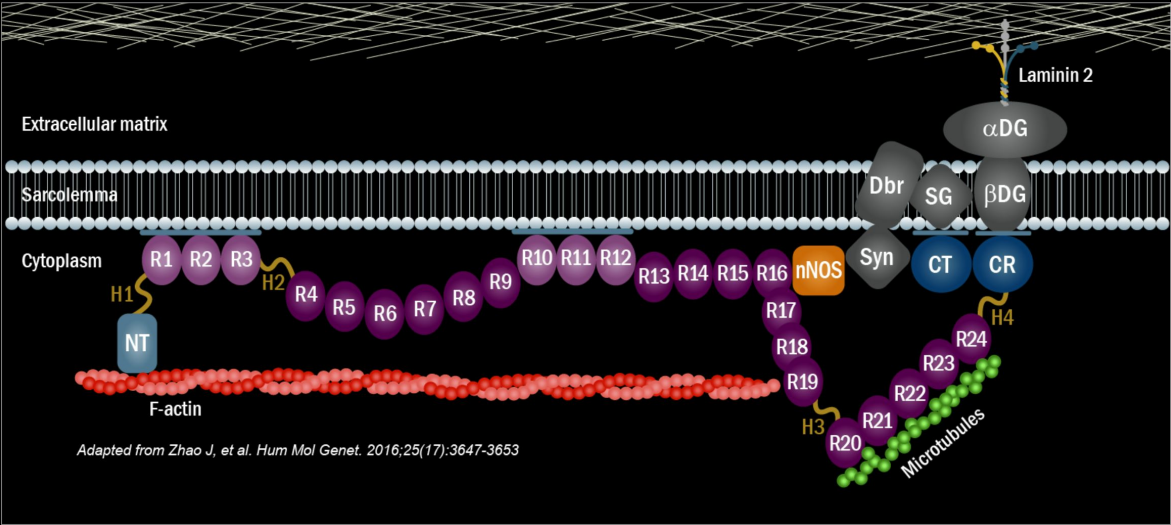
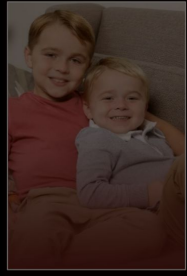


1. Birnkrant et al. Lancet Neurol. 2018 Mar;17(3):251-267; 2. Verma et al. Clin Pediatr (Phila). 2010 Nov;49(11):1011-7; 3. Aartsma-Rus et al. Hum Mutat. 2009 Mar;30(3):293-9; 4. Duchenne UK. Stages of Duchenne <https://www.duchenneuk.org/Pages/FAQs/Category/stages-of-duchenne> (last accessed August 2018); 5. Janssen et al. J Neurol. 2014 Jul;261(7):1269-86; 6. Rail and Grimm. Acta Myol. 2012 Oct;31(2):117-20; 7. Koeks et al. J Neuromuscul Dis. 2017;4(4):293-306; 8. Ryder et al. Orphanet J Rare Dis. 2017 Apr;12(1):79; 9. Birnkrant et al. Lancet Neurol. 2015 Apr;17(4):347-361; 10. Archer et al. J Spine Surg. 2016 Sep;2(3):185-19; 11. LoMauro et al. Ther Clin Risk Manag. 2015 28;11:1475-88.



SCIENCE

# Restoring the dystrophin-associated protein complex (DAPC) restores function

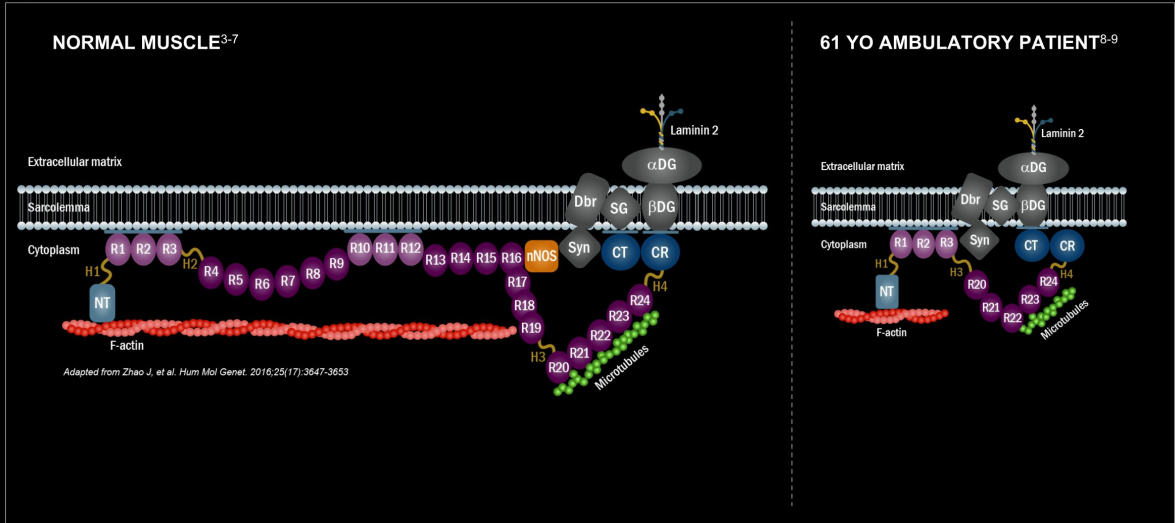
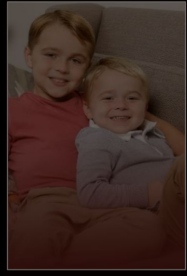


# Becker muscular dystrophy (BMD) shortened dystrophin<sup>3,7</sup>

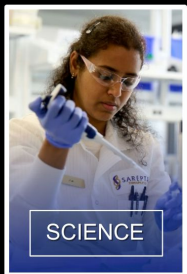
BMD patient Produces a functioning shortened version of the protein of interest<sup>1,2</sup>



SCIENCE

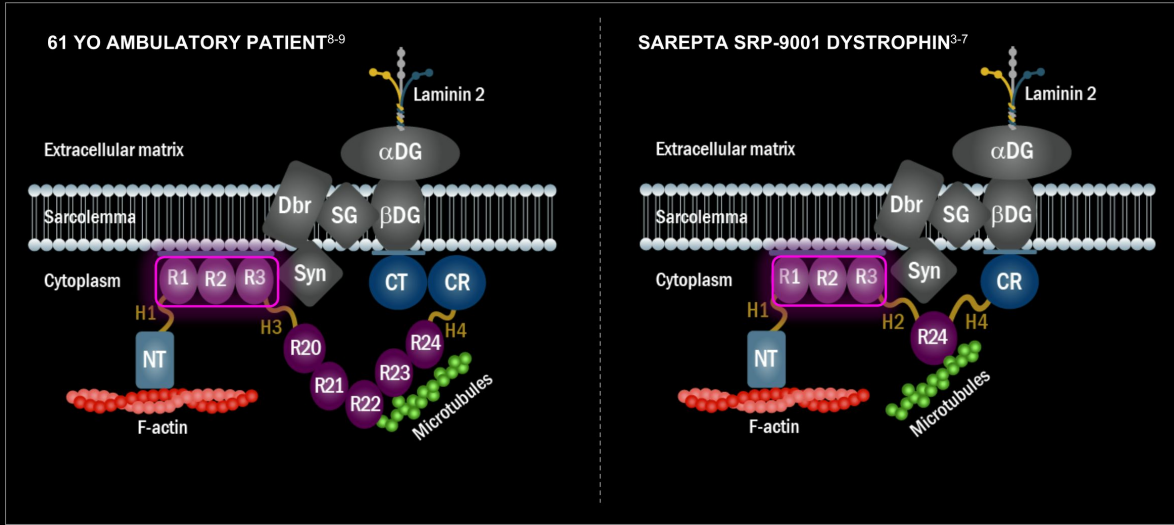
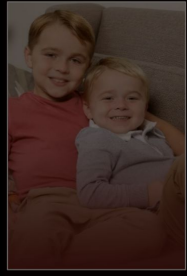


1. Niso MF, et al. *BioDrugs*. 2017;31(4):317-324. 2. Chamberlain K, et al. *Hum Gene Ther Methods*. 2016;27(1):1-12. 3. Gao Q, et al. *Compr Physiol*. 2015;5(3):1223-33. 4. Harper SQ, et al. *Nature Med*. 2002;8(3):253. 5. Nelson DM, et al. *Hum Mol Genet*. 2018;27(12):2050. 6. Farrelough T, et al. *Nat Rev Genet*. 2013;14:373-378. 7. Aartsma-Rus A, et al. *Muscle Nerve*. 2006;34(2):134-144. 8. England SB, et al. *Nature*. 1990;343(6254):180-182. 9. Wells DJ, et al. *Hum Mol Genet*. 1995;4(8):1245-1250.



# BMD shortened dystrophin protein retains critical elements of dystrophin<sup>3-7</sup>

Transgene – Produces a functioning version of the protein of interest<sup>1,2</sup>

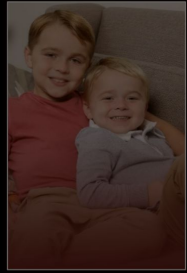


1. Niso MF, et al. *BioDrugs*. 2017;31(4):317-324. 2. Chamberlain K, et al. *Hum Gene Ther Methods*. 2016;27(1):1-12. 3. Gao Q, et al. *Comp Physiol*. 2016;5(3):1223-4. Harper SQ, et al. *Nature Med*. 2002;8(3):253. 5. Nelson DM, et al. *Hum Mol Genet*. 2018;27(12):2050. 6. Faridoughi F, et al. *Nat Rev Genet*. 2013;14:373-378. 7. Aartsma-Rus A, et al. *Muscle Nerve*. 2006;34(2):134-144. 8. England SB, et al. *Nature*. 1990;343(6254):180-182. 9. Wells DJ, et al. *Hum Mol Genet*. 1995;4(8):1245-1250.



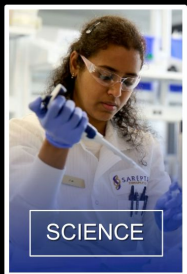
SCIENCE

# SRP-9001 has been rationally designed to maximize expression in tissues most affected by Duchenne<sup>1-6</sup>

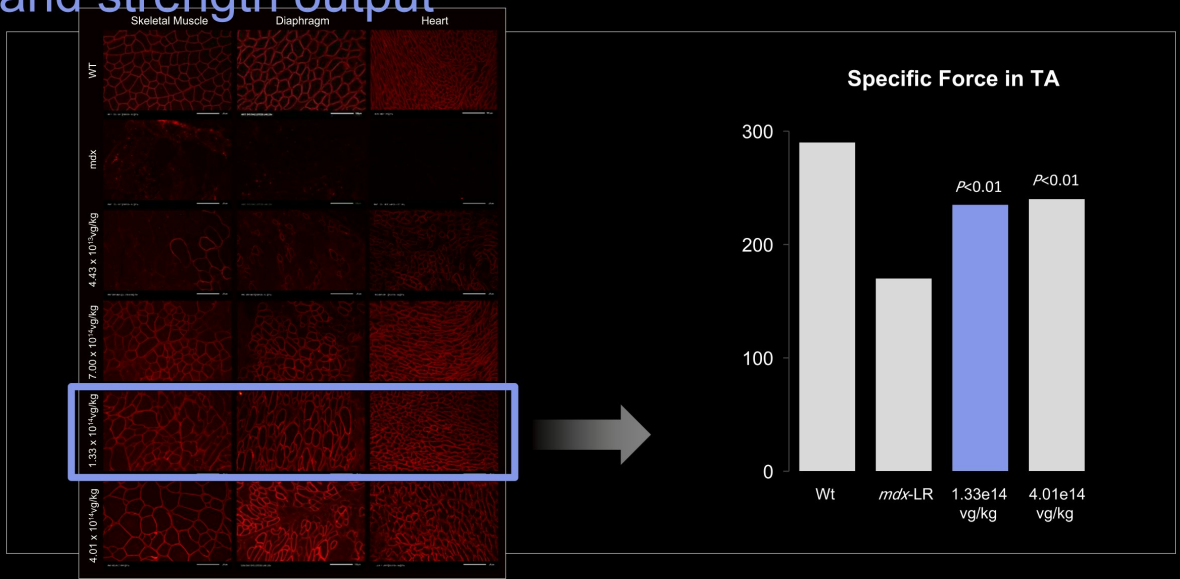
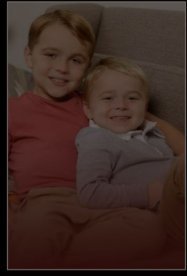


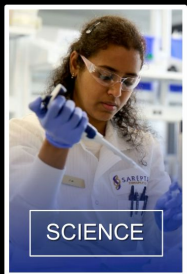
AAVrh74 Viral Vector <sup>1-2</sup>	MHCK7 Promoter <sup>3</sup>	SRP-9001 dystrophin Transgene <sup>4-6</sup>
Affinity for muscle	Specific to skeletal and cardiac muscle	Assembles DAPC
Relatively low level of preexisting immunity and favorable safety profile	Enhanced expression in cardiac muscle	Includes spectrin-like repeats 2 and 3 for maintenance of contractile force

1. Mendell JR, et al. *Neurosci Lett* 2012;627:90-99. 2. Chicoine LG, et al. *Mol Ther* 2014;22:713-724. 3. Salva MZ, et al. *Mol Ther* 2007;15:320-329.  
4. Rodino-Klapac LR, et al. *Hum Mol Genet* 2013;22:4929-4937  
5. Harper SQ, et al. *Nat Med* 2002;8:253-261. 6. Nelson DM, et al. *Hum Mol Genet* 2010;27:2090-2100.

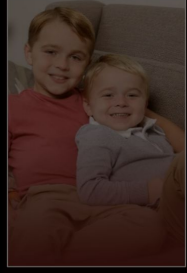


# Non-clinical proof-of-concept demonstrating a correlation between SRP-9001 dystrophin expression and strength output

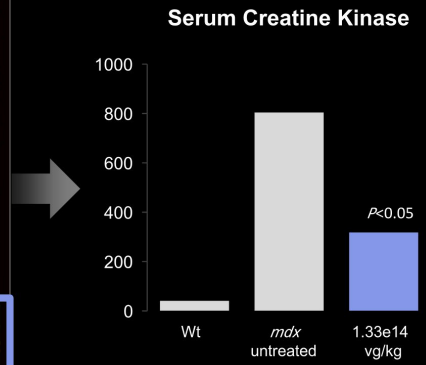
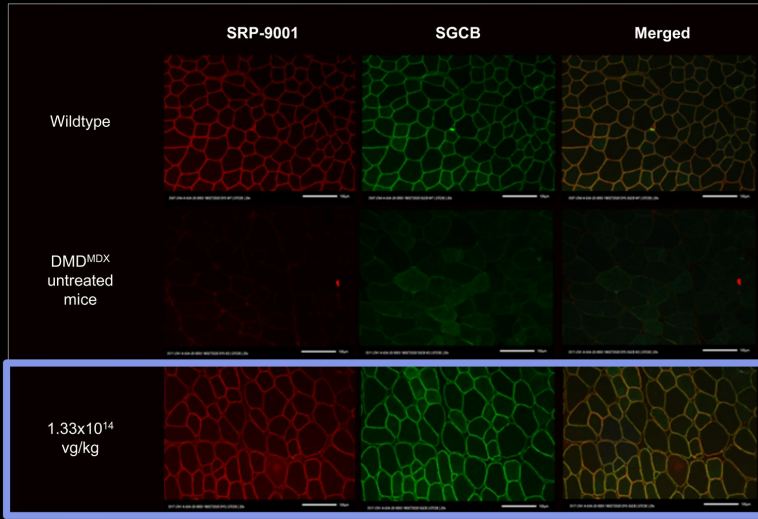




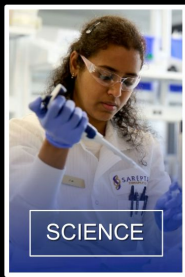
SCIENCE



# SRP-9001 treatment leads to restoration of DAPC, reduced CK, and improved histopathology

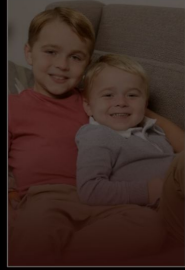






SCIENCE

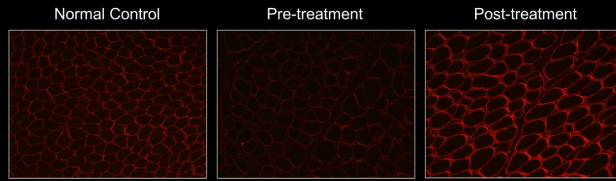
# SRP-9001 dystrophin expression, transduction, and localization at the dose of $1.33 \times 10^{14}$ vg/kg



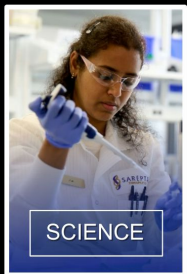
Measure	Timepoint	Study 101 (Early Development Process) (n=4)	Study 102 Part 1 & 2 Target Dose <sup>a</sup> (Early Development Process) (n=29)	Study 103 (Intended Commercial Process) (n=20)
Mean age (years) at time of biopsy	W12	5.4	7.4	6.1
Vector Genome Copy Number <sup>b</sup>	Mean change from Baseline to W12 (range)	3.3	2.9	3.4
		(1.3 - 8.1)	(0.3 - 7.3)	(0.7-9.8)
SRP-9001 Dystrophin Expression (western blot, % of normal expression)	Mean change from Baseline to W12 (range)	74.3	38.6	54.2
		(13.5 - 182.6)	(-1.1 - 114.7)	(4.8-153.9)
IF Fiber Intensity (% control)	Mean change from Baseline to W12 (range)	93.6 <sup>c</sup>	61.6	66.5
		(58.8 - 157.8)	(-7.7 - 138.1)	(-9.6 - 263.6)
PDPF, %	Mean change from Baseline to W12	81.2 <sup>c</sup>	64.1	48.3
		(73.5 - 96.2)	(-7.3 - 96.1)	(1.1 - 84.4)

<sup>a</sup> IF = immunofluorescent; PDPF = percent dystrophin positive fibers.  
 Data extraction date: 9001-101: 15 June 2021; 9001-102: 12 May 2021; 9001-103: 09 February 2022.  
<sup>b</sup> Target Dose =  $1.33 \times 10^{14}$  vg/kg by dsPCR.  
<sup>c</sup> dsPCR was used to analyze vector genome copies in Study SRP-9001-101; dsPCR was used for Studies SRP -9001-102 and -103.  
<sup>d</sup> IF and PDPF values in Study SRP-9001-101 were calculated using different methods than those used in SRP -9001-102 and -103.

Expression of DAPC Proteins in Muscle Fibers from the Gastrocnemius of Subject 4

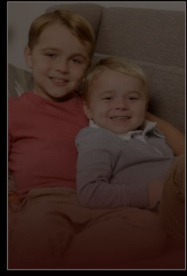


$\beta$ -Sarcoglycan



SCIENCE

# Vast body of pre-clinical and clinical data support SRP-9001 as disease-modifying



Strong Expression	Reduction in CK	Positive Biomarkers
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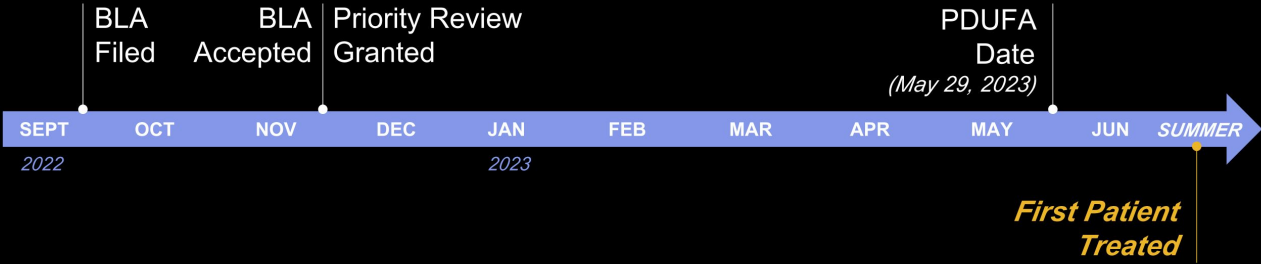
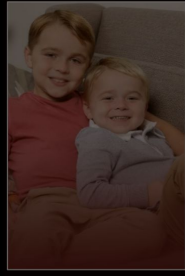
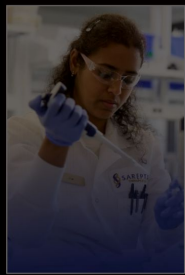


Improved benefit over time<sup>1</sup>

2 point <sup>2</sup>	2.4 point <sup>3,4</sup>	3.2 point <sup>3</sup>	5 point <sup>4</sup>	9.4 point <sup>5</sup>
1 year	1 year	1 year	2 year	4 year
STUDY 102 Part 2	point <sup>3,4</sup> Efficacy Analysis at Target Dose	STUDY 103	STUDY 102 Part 1	STUDY 101
20 patients	52 patients	20 patients	20 patients	4 patients

1. All NSAA scores are compared to propensity-matched external control group.  
 2. Calculated using least square means. Mendell, J. et al, WMS Conference 2022 and data on file.  
 3. Calculated using least square means. Zaidman, C. et al, ICNMD Conference 2022 and data on file.  
 4. Calculated using median. Data on file.  
 5. Calculated using least square means. Mendell, J. et al, ICNMD Conference 2022 and data on file.

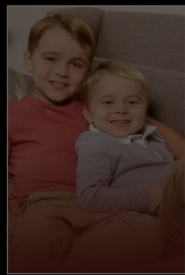
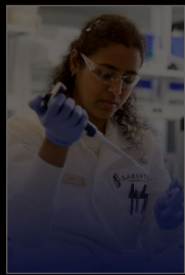
# SRP-9001 pathway



# Internal gene therapy capabilities complimented by partnerships: Meeting demand to launch SRP-9001

Dedicated Sarepta Facilities and Capabilities

External Partnership Overview



## Analytical, Process Development & Quality Control

- Vector & drug product development
- Non-clinical tox manufacturing
- Fully equipped AD/QC labs
- Validated methods for titrating/release



## Investments in FTEs and Infrastructure




- >30k ft<sup>2</sup> facilities in Andover and Burlington, MA
- >300 dedicated staff for technical operations and manufacturing support
- Expanding gene therapy capacity in Bedford, MA facility



## Continued Innovation and Improvement

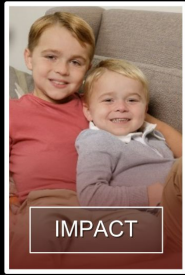
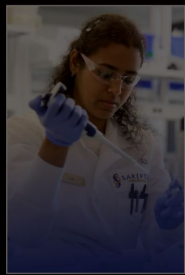
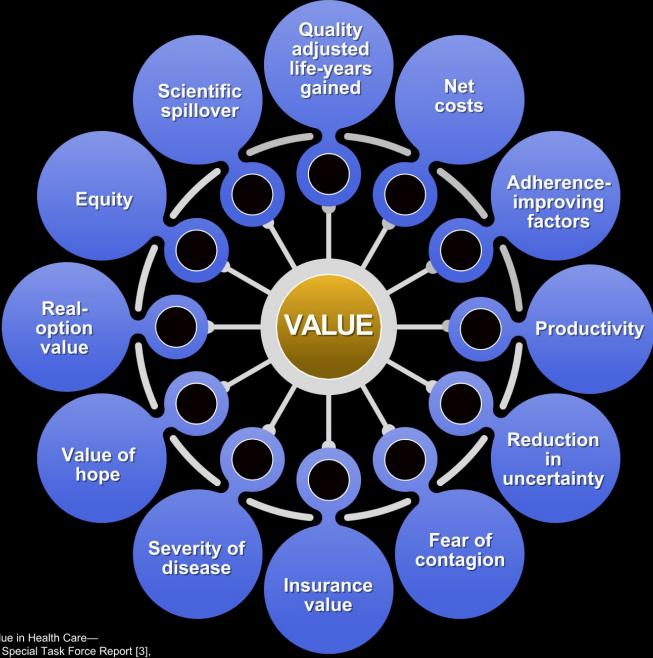
- Approximately 140,000 sq. ft. for early research and development, as well as process development (Columbus, OH)
- Developing next-gen technologies to improve efficiencies and reduce COGS (e.g., suspension manufacturing process)



External Partner	Description	Status
	Plasmid Production	Dedicated capacity for Sarepta portfolio
	Vector Production (Drug Substance & Drug Product)	Dedicated space for Sarepta
	Analytical Testing	Dedicated FTEs to support Sarepta programs

*Hybrid approach will drive competitive costs with continual improvements to drive upside*

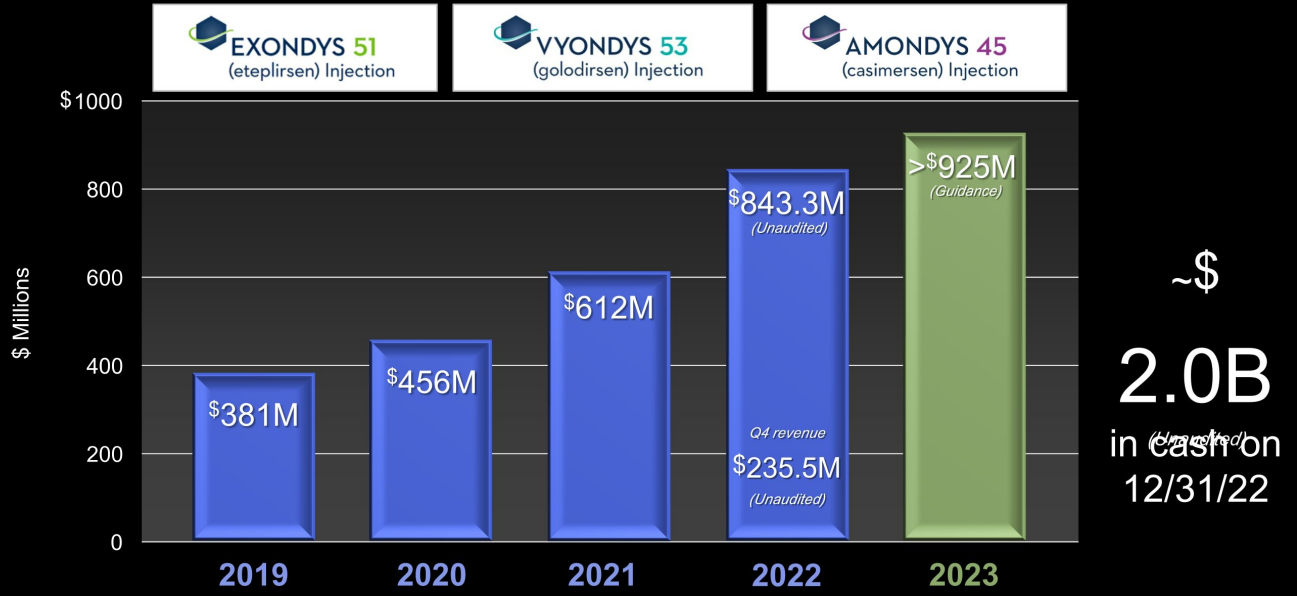
# A holistic approach to assessing innovative treatments



IMPACT

Lakdawalla, et al. Defining Elements of Value in Health Care—  
A Health Economics Approach: An ISPOR Special Task Force Report [3].  
Value in Health, Volume 21, Issue 2, 2018, Pages 131-139.  
ISSN 1098-3015, <https://doi.org/10.1016/j.vhal.2017.12.007>

# Robust total product revenue for RNA-based PMO franchise in Duchenne



Revenues represent net product revenues from PMO franchise.

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# Growth strategy: Leveraging current and future opportunities as we secure our leadership position in genetic medicine

**4<sup>\$</sup>B**  
peak-year sales

**SRP-9001- lead gene therapy and possibility of 4<sup>th</sup> FDA-approved medicine**

**Substantial revenue-generating base business**

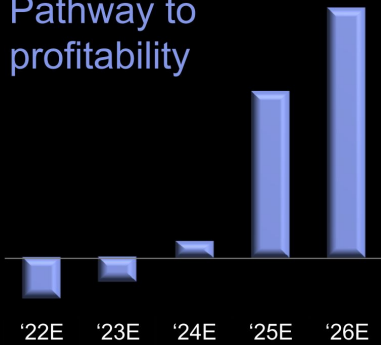


Driven by **3** on-market therapies

With revenues approaching

**\$1B**

Pathway to profitability



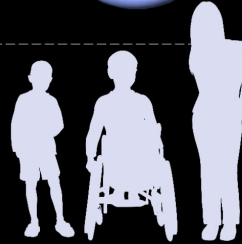
**3** Proprietary technology platforms

- RNA
- Gene Therapy
- Gene Editing


**40+** programs

**Deep, advancing pipeline in neuromuscular, cardiac, and neuro to drive future growth**

**3 late-stage programs in Duchenne and limb-girdle muscular dystrophy type 2E**



**~30** clinical trials ongoing by year-end



BUT MOST  
IMPORTANTLY,  
WE ARE ENABLING  
A FUTURE.





SAREPTA  
THERAPEUTICS

**DOUG INGRAM**  
President and CEO

Sarepta Therapeutics, Inc. (NASDAQ:SRPT)  
JPMorgan Healthcare Conference  
San Francisco, California  
**JANUARY 9, 2023**