SAREPTA THERAPEUTICS

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-dytsrophin protein is reasonably likely to predict clinical benefit; our belief that our internal gene therapy capabilities complimented 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 peek 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.

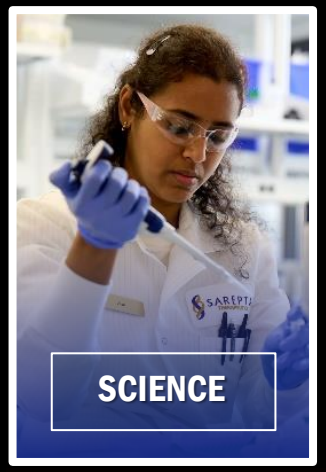
A Bellwether Moment…

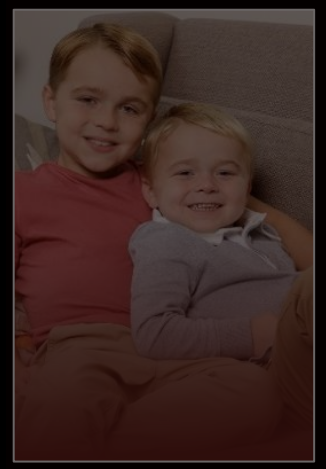
BENJAMIN
Living with Duchenne muscular dystrophy

THE OPPORTUNITY

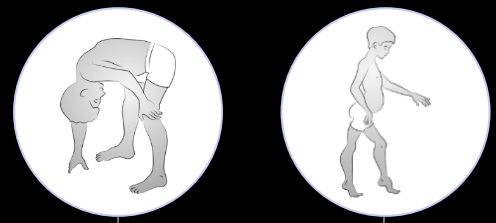
SRP-9001

Poised to transform the trajectory of
Duchenne muscular dystrophy





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

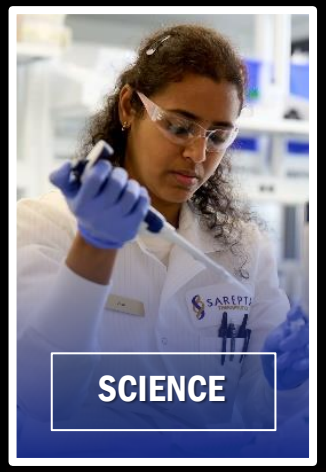


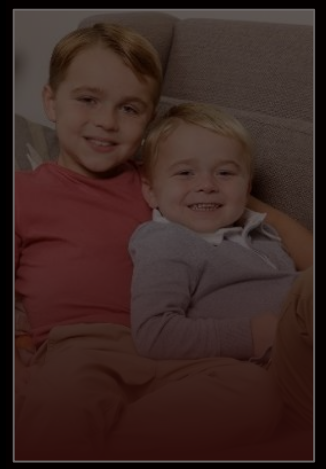
- **BIRTH** — Lack of dystrophin causes **muscle damage**[1,2,3]
- **2-5** years — **Delayed milestones**, Gower's sign and toe-walking[1,2,4]
- **5-10** years — **Difficulty walking**, balancing and climbing stairs, **change in posture**, use of **walking aids** required[1,2,4]
- **7-15** years — **Pain** and **limitations** in upper limbs[2,4,5]
- **10-14** years — **LOA** and **wheelchair** use at around **12 years**,[2,4,6,7,8] onset of **scoliosis**[2,9,10]
- **8-30** years — **Loss** of **upper limb function**[1,2,5]
- **10-30** years — **Pulmonary decline**[1]
- **15-30** years — **Cardiomyopathy** and **heart failure**[4,9]
- **20-30** years — **Night-time ventilation** progressing to **24h ventilation**[6,7,8,9,11]

PROGRESSIVE & IRREVERSIBLE MUSCLE DAMAGE LEADING TO PREMATURE DEATH

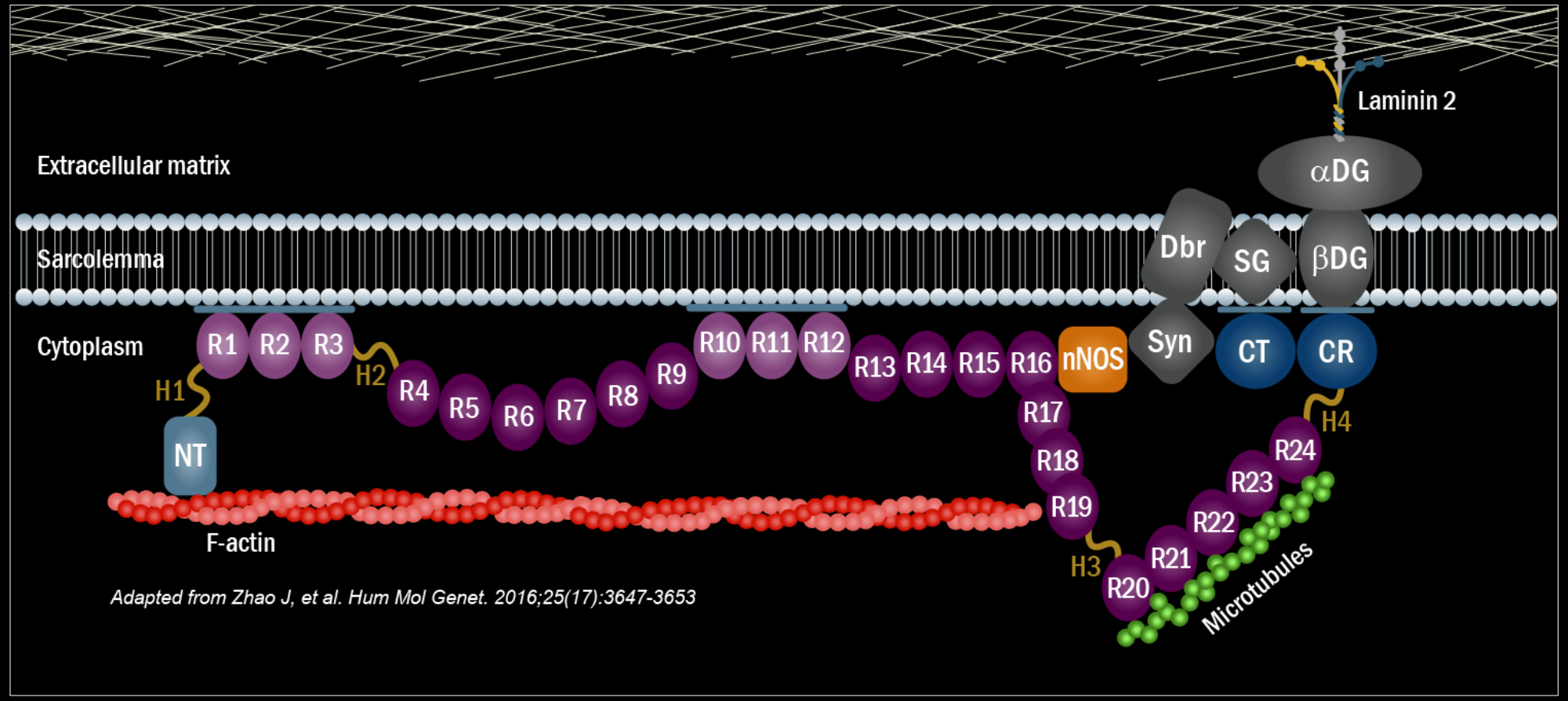
0 5 10 15 20 25 30 YEARS

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):1269-88; **6.** Rall 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 26;12(1):79; **9.** Birnkrant et al. Lancet Neurol. 2018 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.

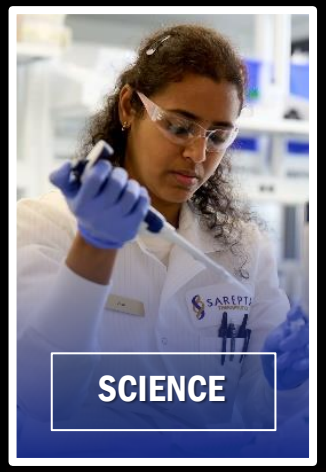


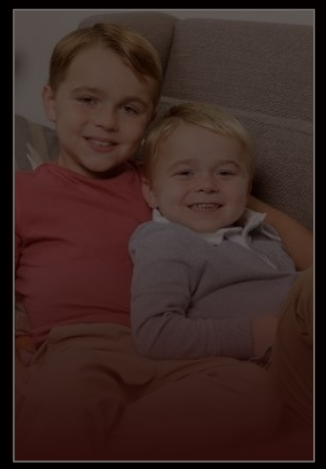


Restoring the dystrophin-associated protein complex (DAPC) restores function



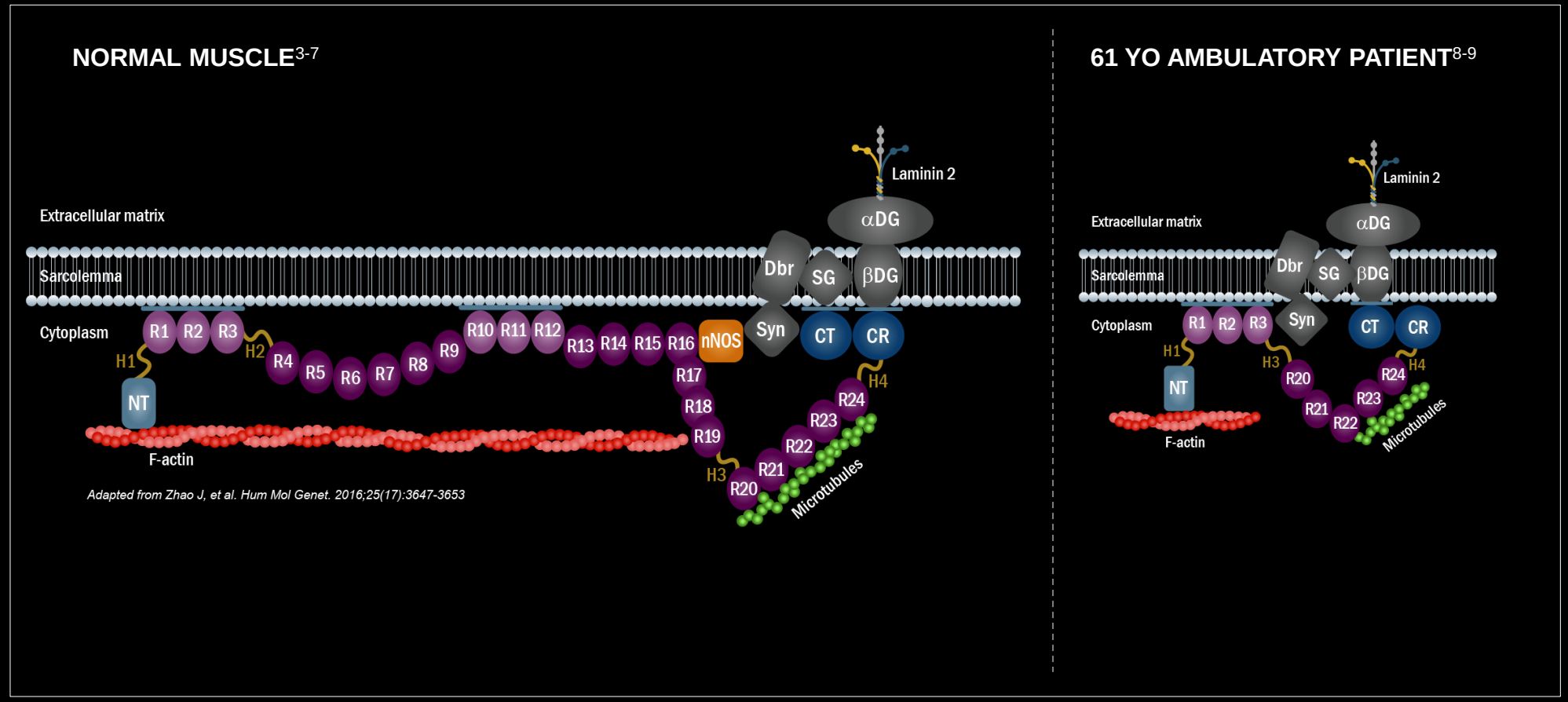
Adapted from Zhao J, et al. Hum Mol Genet. 2016;25(17):3647-3653



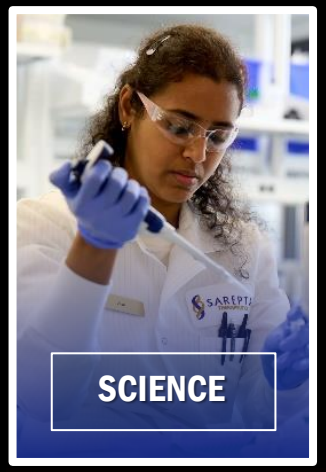


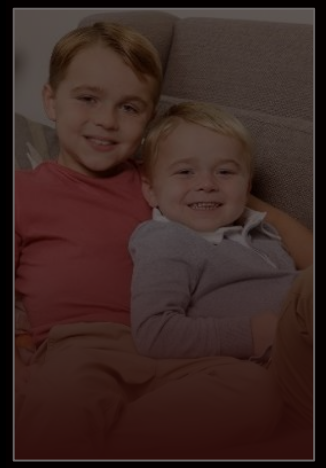
Becker muscular dystrophy (BMD) shortened dystrophin protein retains critical elements of dystrophin[3-7]

BMD patient – Produces a functioning shortened version of the protein of interest[1,2]



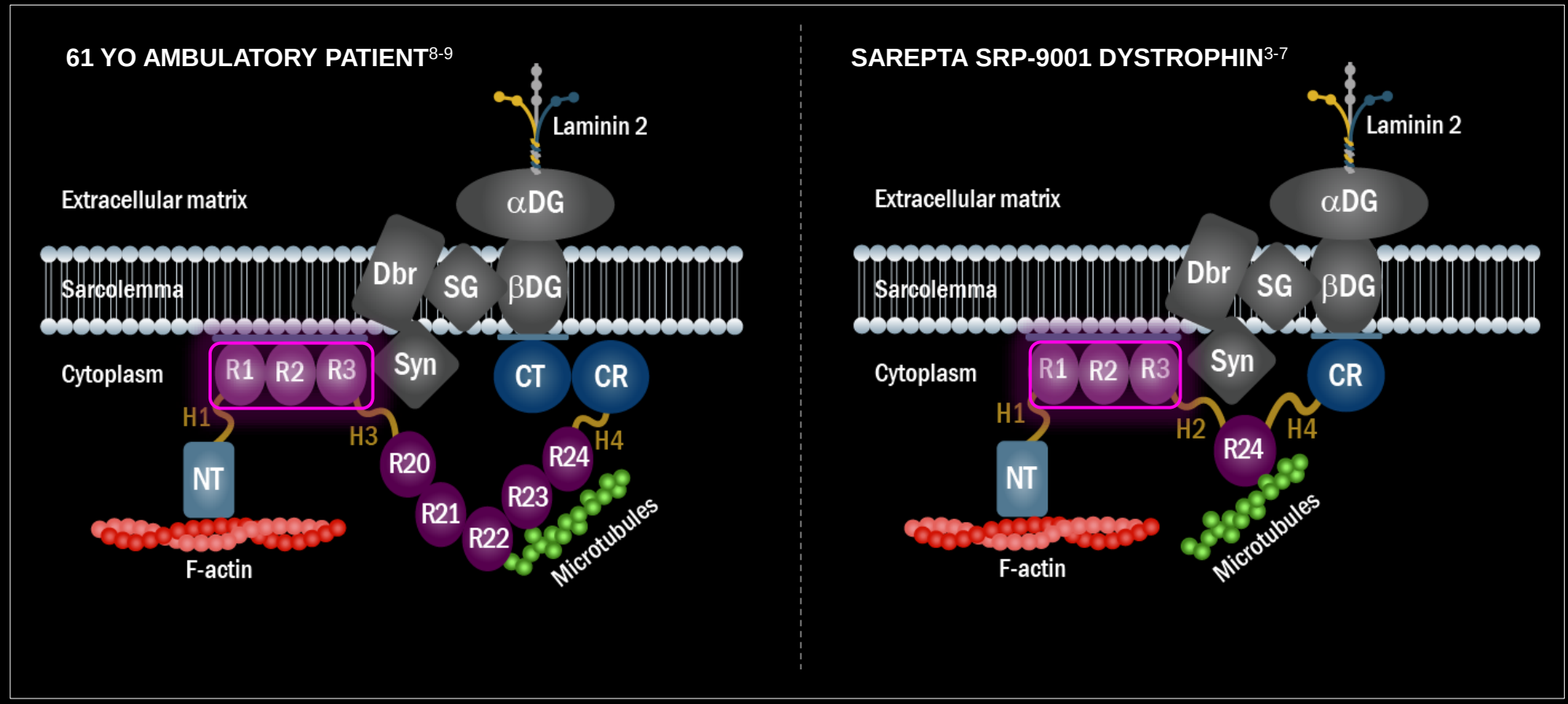
1. Naso MF, et al. *BioDrugs*. 2017;31(4):317-334. 2. Chamberlain K, et al. *Hum Gene Ther Methods*. 2016;27(1):1-12. 3. Gao Q, et al. *Compr Physiol*. 2015;5(3):1223. 4. Harper SQ, et al. *Nature Med.* 2002;8(3):253. 5. Nelson DM, et al. *Human Mol Genet.* 2018 27(12):2090. 6. Fairclough RJ, et al. *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.



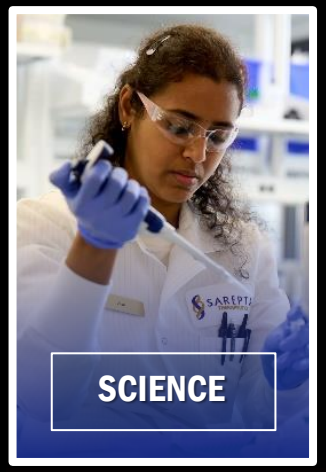


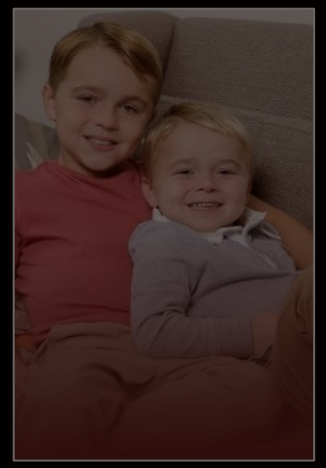
BMD shortened dystrophin protein retains critical elements of dystrophin[3-7]

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



1. Naso MF, et al. *BioDrugs*. 2017;31(4):317-334. 2. Chamberlain K, et al. *Hum Gene Ther Methods*. 2016;27(1):1-12. 3. Gao Q, et al. *Compr Physiol*. 2015;5(3):1223. 4. Harper SQ, et al. *Nature Med.* 2002;8(3):253. 5. Nelson DM, et al. *Human Mol Genet.* 2018 27(12):2090. 6. Fairclough RJ, 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.

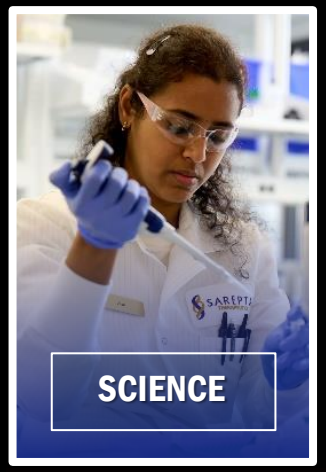


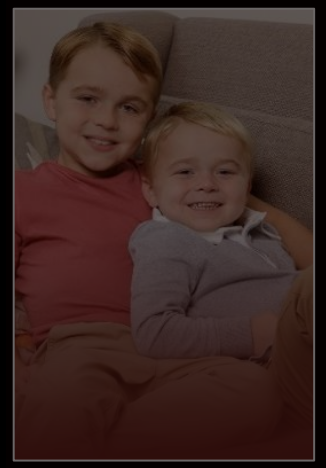


SRP-9001 has been rationally designed to maximize expression in tissues most affected by Duchenne[1-6]

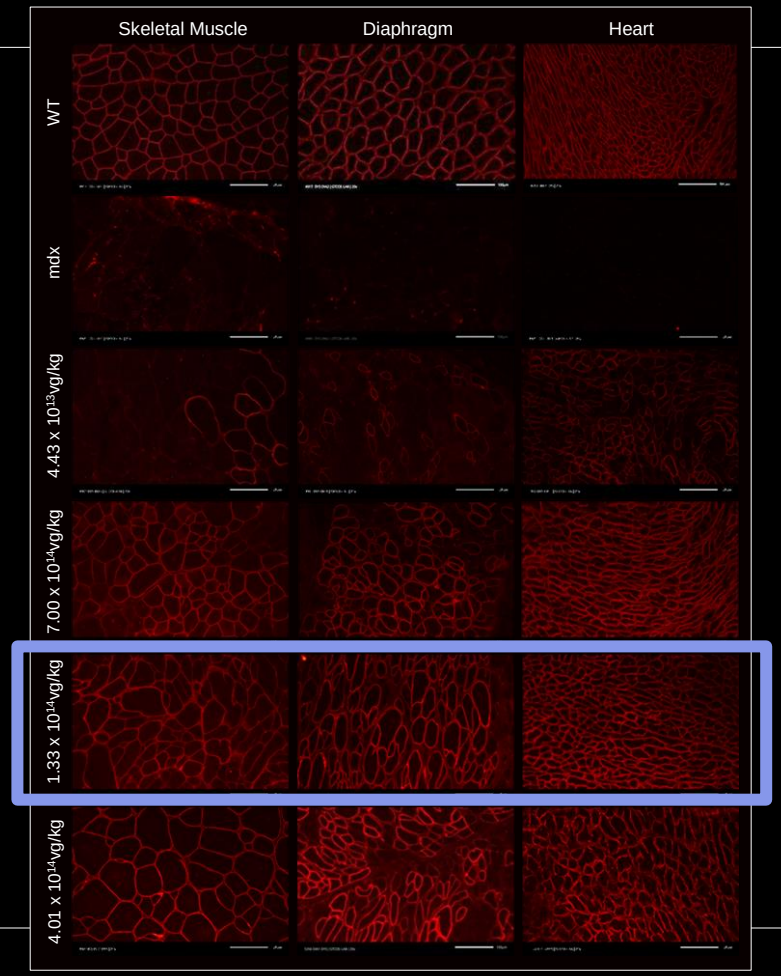
AAVrh74 Viral Vector[1-2]	MHCK7 Promoter[3]	SRP-9001 dystrophin Transgene[4-6]
Affinity for muscle	Specific to skeletal and cardiac muscle	Assembles DAPC
Relatively low level of preexisting immunity and favorable safety profile	Enhanced expression in cardiac muscle	Includes spectrin-like repeats 2 and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

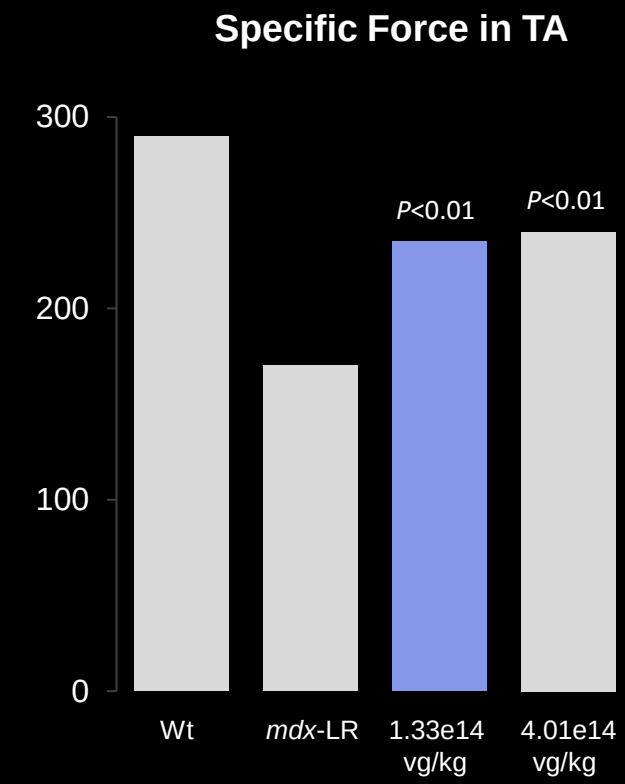
1. Mendell JR, *et al. Neurosci Lett* 2012;627:90-99. 2. Chicoine LG, *et al. Mol Ther* 2014;22:713-724. 3. Salva MZ, *et al. Mol Ther* 2007;15:320-329.
4. Rodino-Klapac LR, *et al. Hum Mol Genet* 2013;22:4929-4937
5. Harper SQ, *et al. Nat Med* 2002;8:253-261. 6. Nelson DM, *et al. Hum Mol Genet* 2018;27:2090-2100.

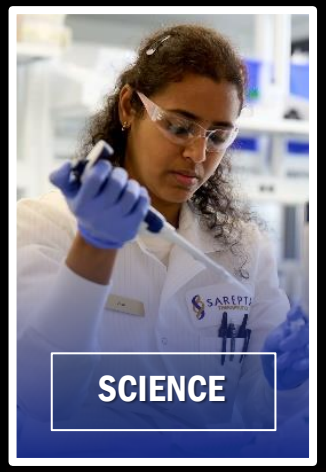


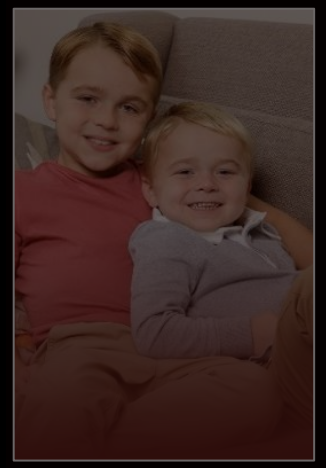


Non-clinical proof-of-concept demonstrating a correlation between SRP-9001 dystrophin expression and strength output

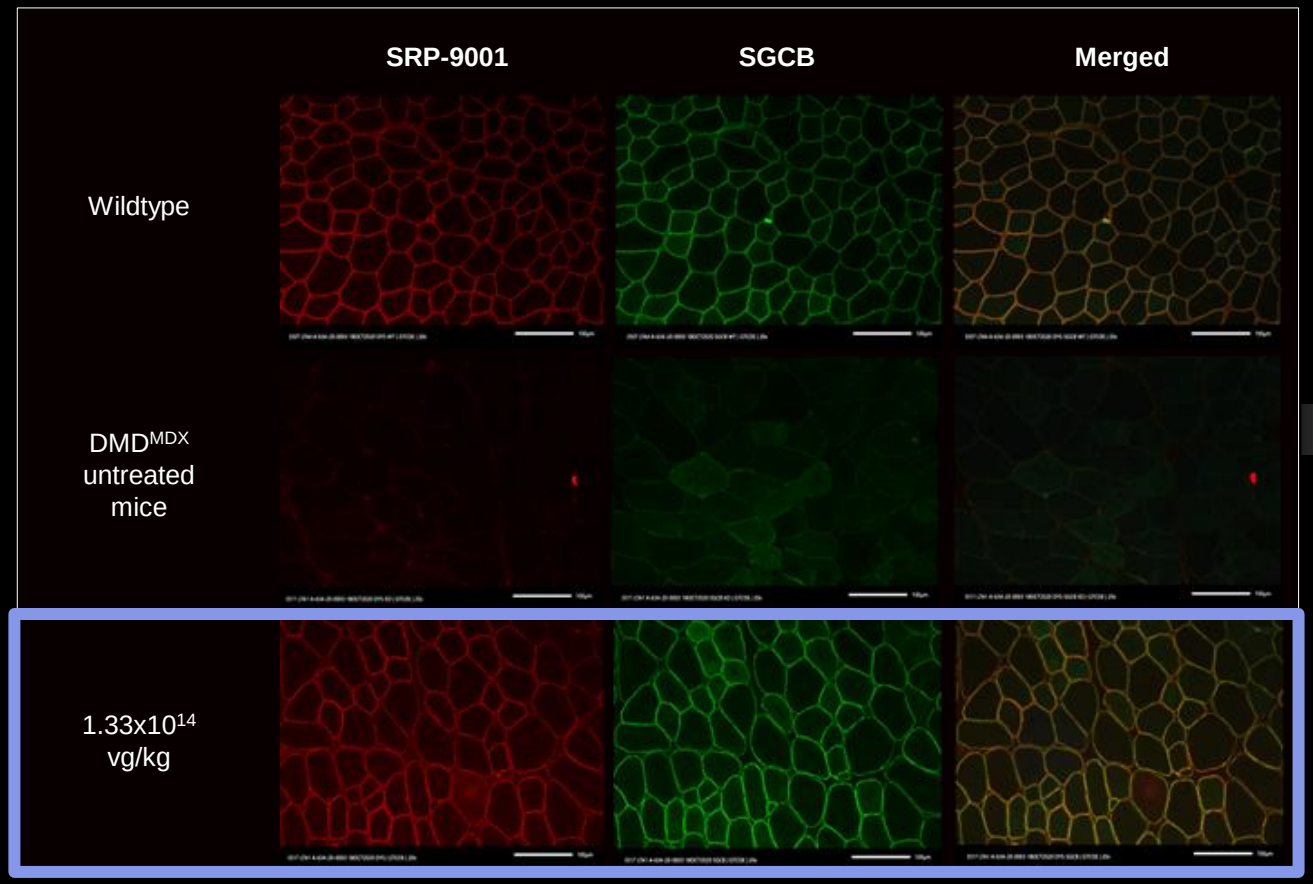


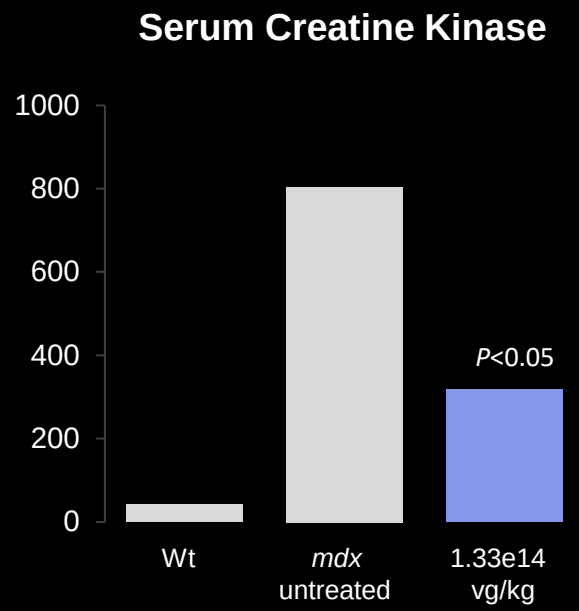


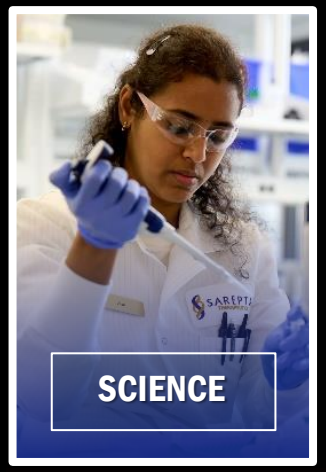


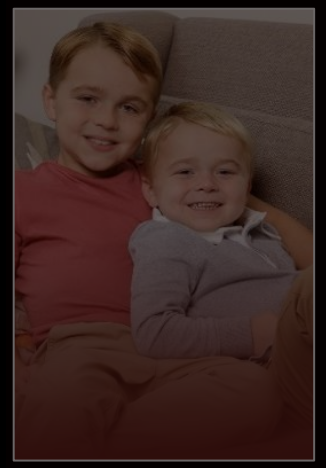


SRP-9001 treatment leads to restoration of DAPC, reduced CK, and improved histopathology







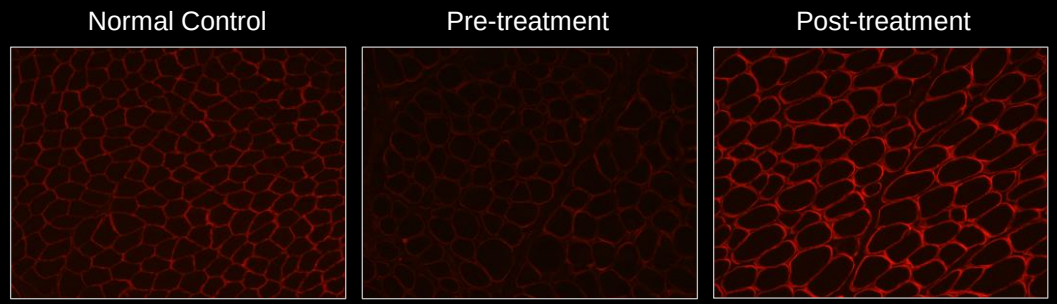


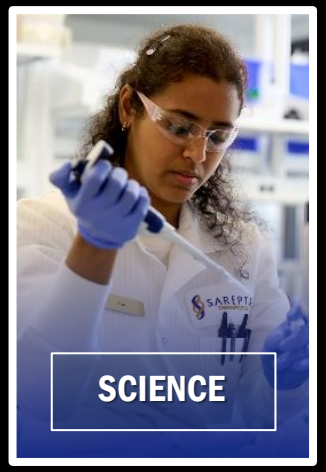
SRP-9001 dystrophin expression, transduction, and localization at the dose of 1.33 x 10^{14} vg/kg

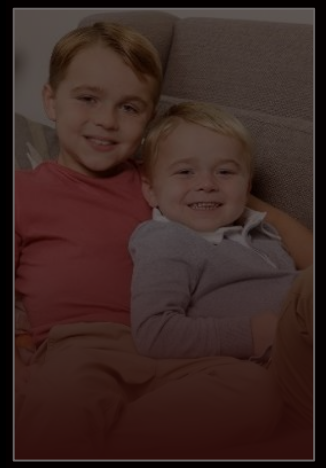
Measure	Timepoint	Study 101 (Early Development Process) (n=4)	Study 102 Part 1 & 2 Target Dose [a] (Early Development Process) (n=29)	Study 103 (Intended Commercial Process) (n=20)
Mean age (years) at time of biopsy	W12	5.4	7.4	6.1
Vector Genome Copy Number [b]	Mean change from Baseline to W12 (range)	**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 [c]**	**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 [c]**	**64.1**	**48.3**
		(73.5 - 96.2)	(-7.3 - 96.1)	(1.1 - 84.4)

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
[a] Target Dose = 1.33 x 10^{14} vg/kg by ddPCR
[b] qPCR was used to analyze vector genome copies in Study SRP-9001-101; ddPCR was used for Studies SRP-9001-102 and -103.
[c] 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







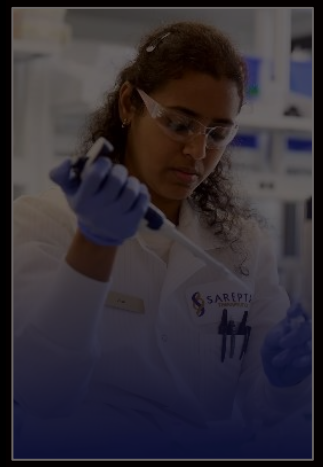
Vast body of pre-clinical and clinical data support SRP-9001 as disease-modifying

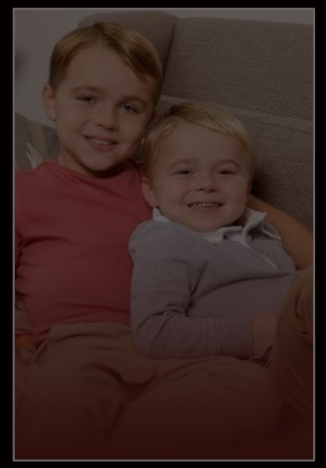
Strong Expression	Reduction in CK	Positive Biomarkers

Improved benefit over time[1]

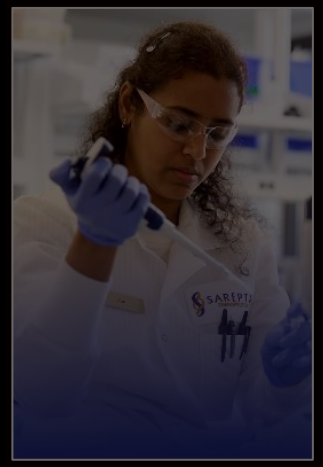
2 point[2]	2.4 point[3]	3.2 point[3]	5 point[4]	9.4 point[5]
1 year	1 year	1 year	2 year	4 year
STUDY 102 *Part 2*	Integrated Efficacy Analysis at Target Dose	STUDY 103	STUDY 102 *Part 1*	STUDY 101
20 patients	52 patients	20 patients	20 patients	4 patients

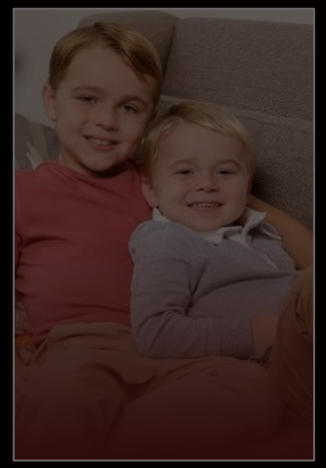
1. All NSAA scores are compared to propensity-matched external control group.
2. Calculated using least square means. Mendell, J. et al, WMS Conference 2022 and data on file.
3. Calculated using least square means. Zaidman, C. et al, ICNMD Conference 2022 and data on file.
4. Calculated using median.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

Analytical, Process Development & Quality Control

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

Investments in FTEs and Infrastructure

- >30k ft^2 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

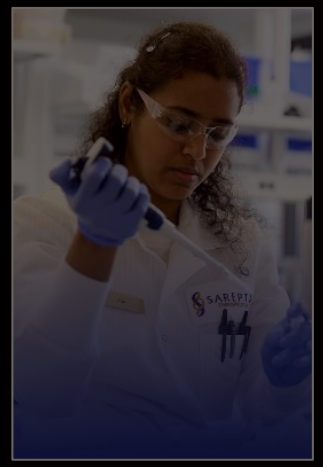
- Approximately 140,000 sq. ft. for early research and development, as well as process development (Columbus, OH)
- Developing next-gen technologies to improve efficiencies and reduce COGS (e.g., suspension manufacturing process)

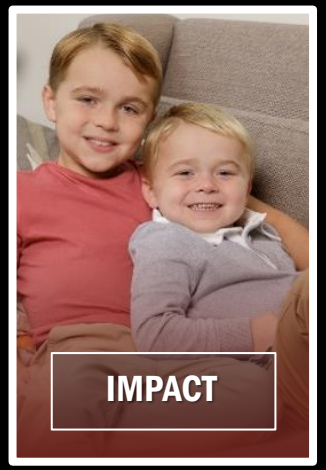
+

External Partnership Overview

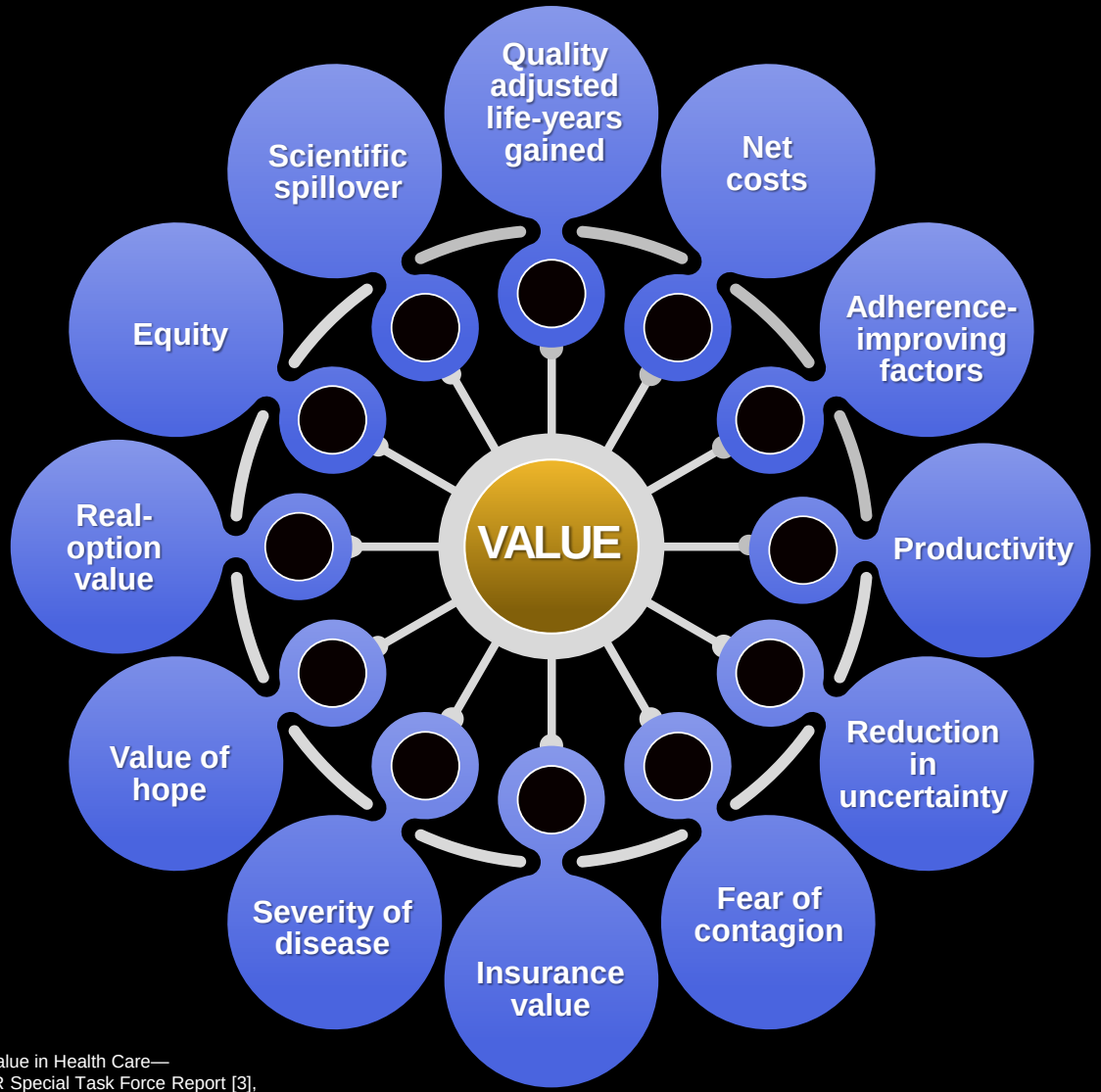
External Partner	Description	Status
aldevron	Plasmid Production	Dedicated capacity for Sarepta portfolio
Catalent	Vector Production (Drug Substance & Drug Product)	Dedicated space for Sarepta
PPD	Analytical Testing	Dedicated FTEs to support Sarepta programs

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



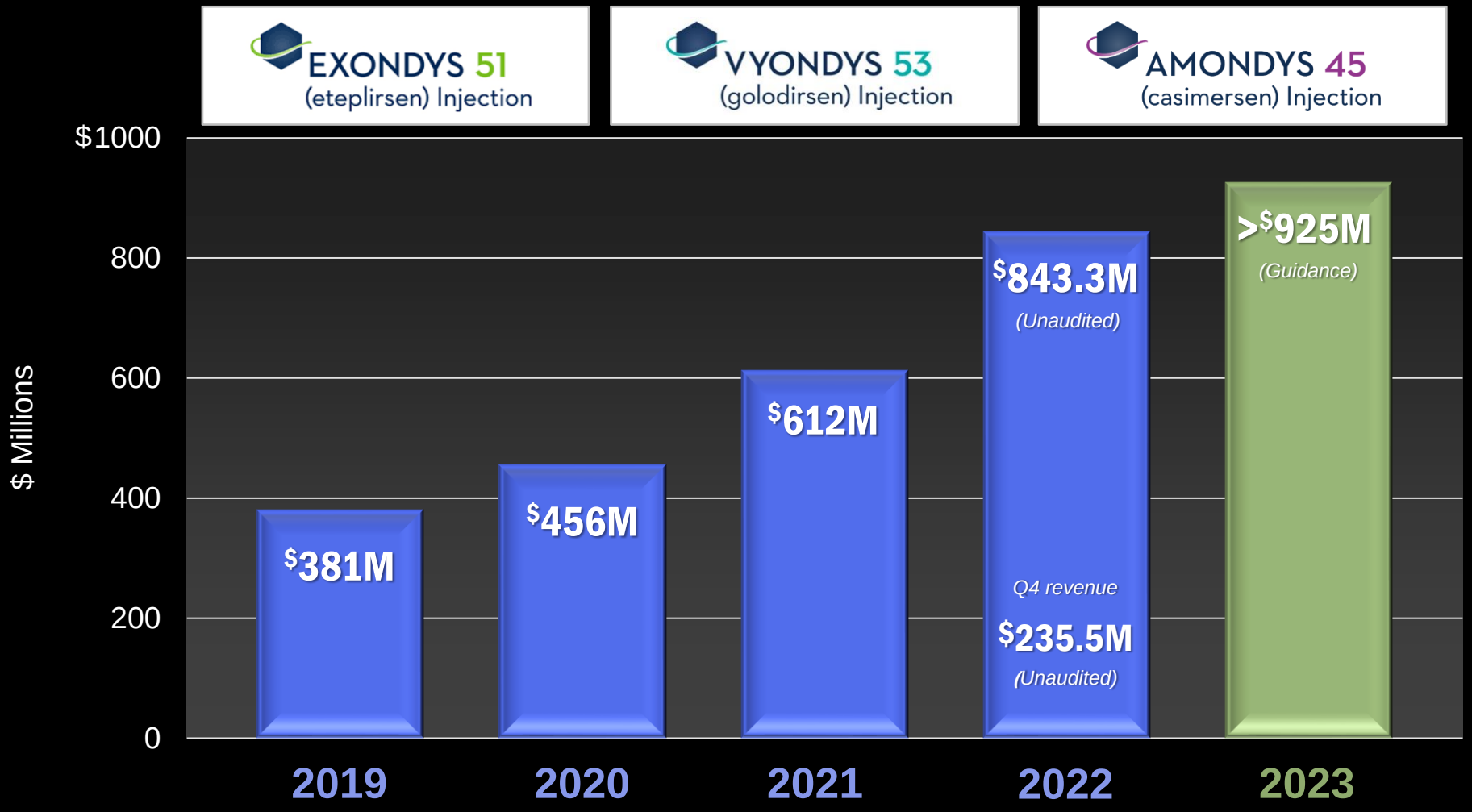


A holistic approach to assessing innovative treatments



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.jval.2017.12.007.

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



~$2.0B in cash on 12/31/22 *(Unaudited)*

Revenues represent net product revenues from PMO franchise.

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

$4B
peak-year sales

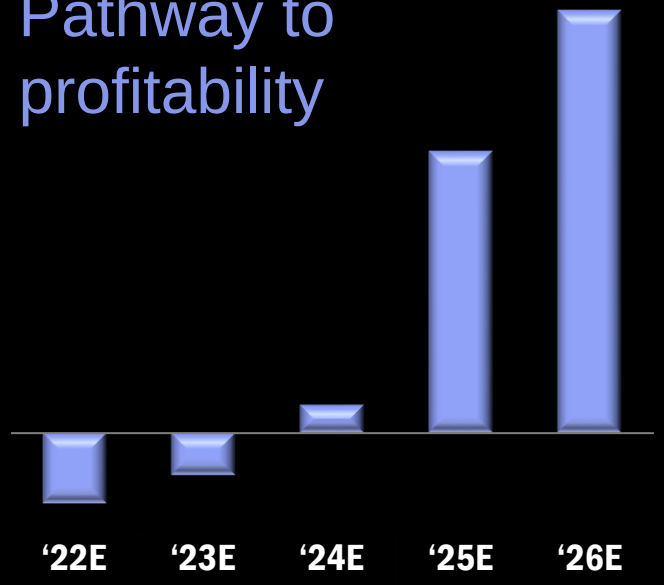
SRP-9001- lead gene therapy and possibility of 4th FDA-approved medicine

Substantial revenue-generating base business

Driven by **3** on-market therapies

With revenues approaching **$1B**

Pathway to profitability



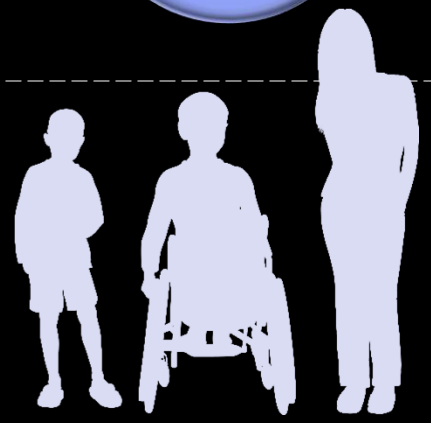
3 **Proprietary technology platforms**

- 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