

Some see slow and steady scientific progress.

We see a revolution.

May 1, 2022



KAI
Living with Duchenne
muscular dystrophy

MED-US-SRP-45-0148

Forward Looking Statements

This presentation contains "forward-looking statements." Any statements that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believe," "anticipate," "plan," "expect," "will," "may," "intend," "prepare," "look," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to our opportunities in the rare disease space; market opportunities with respect to our RNA technologies, gene therapy and gene editing; the potential benefits of our technologies and scientific approaches; the potential of our collaborations and partnerships; and expected timelines and milestones.

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 could materially and adversely affect our business, results of operations and trading price. Potential known risk factors include, among others, the following: our data for our different programs may not be sufficient for obtaining regulatory approval; our product candidates, including those with strategic partners, may not result in viable treatments suitable for commercialization due to a variety of reasons, including the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; success in preclinical testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful; the expected benefits and opportunities related to our agreements with our strategic partners may not be realized or may take longer to realize than expected due to a variety of reasons, including any inability of the parties to perform their commitments and obligations under the agreements, challenges and uncertainties inherent in product research and development and manufacturing limitations; 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, and 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.

Meet the Patient Affairs Team



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SVP, Global Health Policy



Siobhan Fitzgerald
Executive Director



Allison Kreuzer
Director



Tamara Wyzanski
Associate Director



Hannah Rosenberg
Senior Manager

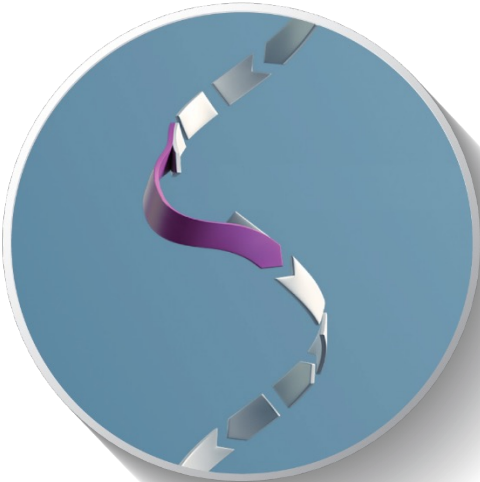


Kate Pecora
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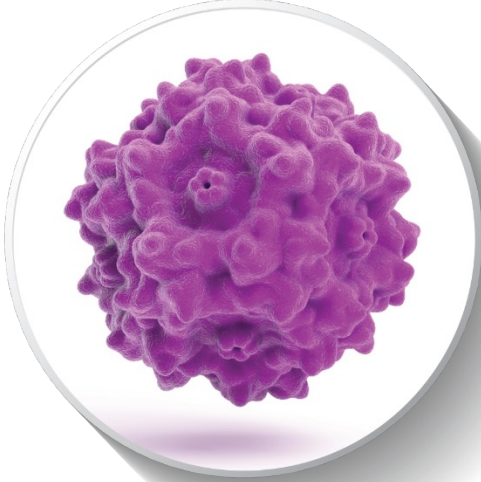
Sarepta's Experience in Duchenne



RNA THERAPY
(PMO and PPMO)

14

PROGRAMS



GENE THERAPY

4

PROGRAMS



GENE EDITING

2

PROGRAMS

20 *programs in all*

10⁺ *years dedicated to Duchenne R&D*

Three PMO programs to address up to ~30% of Duchenne population

ETEPLIRSEN:
PMO for skipping
of Exon 51

Granted US
Accelerated Approval
in **2016**

GOLODIRSEN:
PMO for skipping
of Exon 53

Granted US
Accelerated Approval
in **2019**

CASIMERSEN:
PMO for skipping
of Exon 45

Granted US
Accelerated Approval
in **2021**

Currently Recruiting U.S. Clinical Trials-RNA Platform

MiSSION

Momentum

(5051 201)

Trial status	Recruiting	Recruiting
Product name	eteplirsen	SRP-5051 (PPMO)
Trial phase	Phase 3	Phase 2
NCT identifier	NCT03992430	NCT04004065
Exon skip amenability	51	51
Trial description	144-week study to compare the safety and efficacy of a high dose of eteplirsen compared to the 30mg/kg dose eteplirsen.	Two-part multiple ascending dose study (MAD).
Participant age*	4– 13 years	7 – 21 years
Participant ambulation status*	Ambulatory individuals	Ambulatory and non-ambulatory individuals
Primary functional assessment	North Star Ambulatory Assessment (NSAA)	Safety and Dystrophin Expression



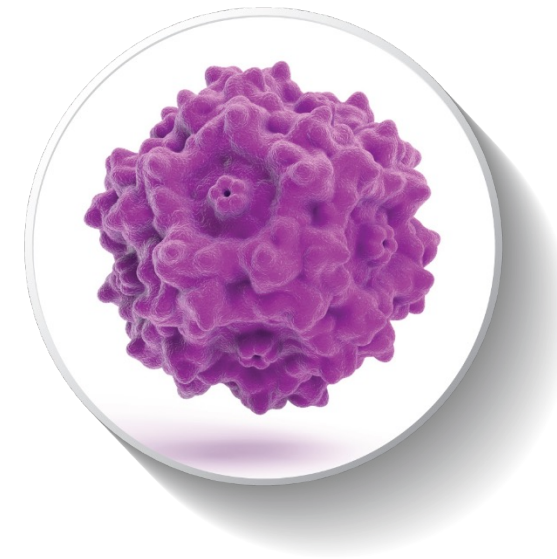
**Other inclusion/exclusion criteria apply. Please contact study sites for more details.*

SRP-5051 is investigational and has not been reviewed or approved by any regulatory authority.

Currently Recruiting U.S. Clinical Trials- Gene Therapy Platform



Trial status	Recruiting
Product name	SRP-9001 (Gene Therapy)
Trial phase	Phase 3
NCT identifier	NCT05096221
Exon skip amenability	Multiple, exclusions apply**
Trial description	Randomized, double-blind, placebo-controlled study to evaluate the safety and efficacy of gene transfer therapy.
Participant age*	≥ 4 to < 8 years
Participant ambulation status*	Ambulatory
Primary functional assessment	North Star Ambulatory Assessment (NSAA)



*Other inclusion/exclusion criteria apply. Please contact study sites for more details.

**Please contact study site for more details.

SRP-9001 micro-dystrophin gene therapy is investigational and has not been reviewed or approved by any regulatory authority.

Thank You

To connect with us further, please contact the Patient Affairs team at Advocacy@Sarepta.com

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