SAREPTA THERAPEUTICS

Patients can't wait for the next breakthrough in medical research.

So neither will we.

Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. Every day is another 24 hours to stand up for patients, advance technology, challenge convention and **drag tomorrow into today**.

BENJAMIN
Living with Duchenne muscular dystrophy

FULL-TIME EMPLOYEES	MARKETED PRODUCTS	REVENUES FOR YEAR ENDING 12/31/2025	2025 R&D INVESTMENT
~850	4	$1.8B	$562M* representing **58%** of operating expense

Data on file. Sarepta Therapeutics, Inc. 2026

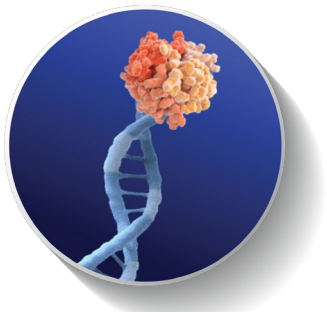
*Excludes one-time milestone payments

Advancing an Industry-Leading Genetic Medicine Pipeline

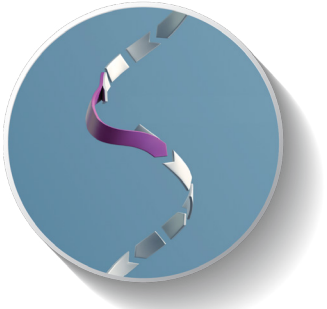
Sarepta holds leadership positions in Duchenne muscular dystrophy (Duchenne) and is building a robust portfolio of programs across muscle, central nervous system, and cardiac diseases.

Our pipeline includes numerous programs across three scientific platforms and multiple therapeutic areas.

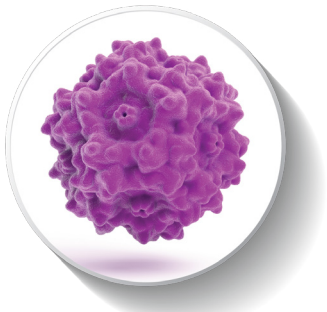
THREE SCIENTIFIC PLATFORMS



siRNA PLATFORM
Proprietary technology designed to "knockdown" or suppress overexpression of gene mutations[1]



RNA PLATFORM
Proprietary technology designed to "skip" over a genetic mutation and enable the body to make a shortened version of a protein[2]



GENE THERAPY
Delivering a new copy of a missing or malfunctioning gene with a goal of targeting the underlying biological defect that causes a certain disease[3]

[1]siRNA Platform. Sarepta Therapeutics, Inc. Accessed March 27, 2026. https://www.sarepta.com/science/sirna
[2]RNA Platform. Sarepta Therapeutics, Inc. Accessed March 27, 2026. https://www.sarepta.com/science/rna-platform
[3]Gene Therapy. Sarepta Therapeutics, Inc. Accessed March 27, 2026. https://www.sarepta.com/science/gene-therapy

OUR PIPELINE

Updated March 2026

Program Name	Discovery/Preclinical	Clinical
siRNA		
SRP-1001		Facioscapulohumeral muscular dystrophy, Type 1 (FSHD1)
SRP-1003		Myotonic dystrophy, Type 1 (DM1)
SRP-1002		Idiopathic pulmonary fibrosis (IPF)
SRP-1004		Spinocerebellar ataxia type 2 (SCA2)
SRP-1005		Huntington's disease (HD)
SRP-1007		SCA1*
SRP-1006		SCA3*
Gene Therapy		
SRP-9003 (bidridistrogene xeboparvovec)		LGMD2E/R4 β-sarcoglycan

* SCA1 = Spinocerebellar ataxia 1
SCA3 = Spinocerebellar ataxia 3

Learn more about our pipeline at sarepta.com/pipeline.

MARKETED PRODUCTS

Sarepta is a fully integrated biopharmaceutical company that is committed to delivering medicines to treat rare, genetic-based diseases, including Duchenne. Our Duchenne portfolio includes four treatments:

3 RNA exon-skipping therapies*

1 gene therapy

**Candidates received accelerated approval in the U.S., confirmatory studies are ongoing*

Learn more about our products at sarepta.com/products.

MAIN OFFICES

COMPANY HEADQUARTERS
Cambridge, MA, USA

RESEARCH AND MANUFACTURING FACILITIES
Andover, MA, USA
Bedford, MA, USA

GENETIC THERAPIES CENTER OF EXCELLENCE
Columbus, OH, USA

INTERNATIONAL OFFICES
United Kingdom
Switzerland

SAREPTA THERAPEUTICS

Media Inquiries: Media@Sarepta.com | 617.274.4052

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