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

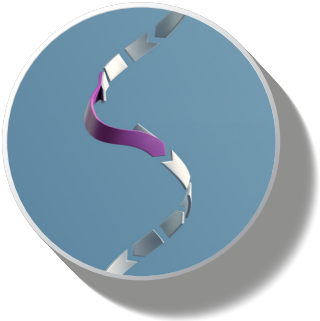
FULL-TIME EMPLOYEES	MARKETED PRODUCTS	2023 ANNUAL SALES	2023 R&D INVESTMENT	PIPELINE PROGRAMS
~1300	4	$1.1B and 7-year CAGR of **115%**	$877M representing **65%** of operating expense	40+

Data on file. Sarepta Therapeutics, Inc. 2024

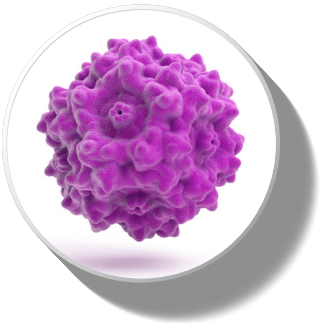
Advancing an Industry-Leading Genetic Medicine Pipeline

Our pipeline includes more than 40 programs across three scientific platforms and multiple therapeutic areas. Our most advanced programs are in Duchenne muscular dystrophy and limb-girdle muscular dystrophies (LGMDs). Sarepta's pipeline includes discovery-stage programs for other rare neuromuscular diseases, central nervous system disorders and cardiomyopathies.

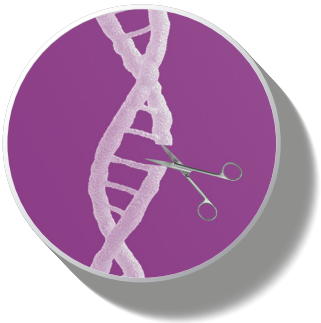
THREE SCIENTIFIC PLATFORMS



RNA PLATFORM
Proprietary technology designed to "skip" over a genetic mutation and enable the body to make a shortened version of a protein[1]



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[2]



GENE EDITING
Technology that aims to modify genetic sequences by removing sections of genetic code containing a mutation, in a precise and targeted manner[3]

[1]RNA platform. Sarepta Therapeutics, Inc. Accessed July 17, 2024. https://www.sarepta.com/science/rna-platform
[2]Gene therapy engine. Sarepta Therapeutics, Inc. Accessed July 17, 2024. https://www.sarepta.com/science/gene-therapy-engine
[3]Gene editing. Sarepta Therapeutics, Inc. Accessed July 17, 2024. https://www.sarepta.com/science/gene-editing

OUR PIPELINE

Information is current as of 6/19/2024, updates are made on a quarterly basis

Program Name	Discovery/Preclinical	Clinical
RNA Targeted Therapies PPMO[4]		
SRP-5051 (vesleteplirsen)	Duchenne	
Other Exon Targets[5]	Duchenne	
Gene Therapy		
SRP-9003 (bidridistrogene xeboparvovec)	LGMD2E/R4 β-sarcoglycan	
SRP-9004 (patidistrogene bexoparvovec)	LGMD2D/R3 α-sarcoglycan	
SRP-6004	LGMD2B/R2 Dysferlin	
Other LGMD Targets[6]	LGMD	
Other Targets	Multiple	
Gene Editing		
CRISPR/CAS9 - Duke University	Duchenne	
CRISPR/CAS9 - Harvard University	Duchenne	

[4]Peptide phosphorodiamidate morpholino oligomers
[5]Other exon targets in development: 44, 45, 50, 52, and 53
[6]Other LGMD targets in development: SRP-9005 (LGMD2C/R5 γ-sarcoglycan), SRP-9006 (LGMD2L/R12 Anoctamin 5), and SRP-9010 (LGMD2A/R1)

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. We recently explanded our Duchenne portfolio to include a total of 4 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
Burlington, MA, USA

GENETIC THERAPIES CENTER OF EXCELLENCE
Columbus, OH, USA

GENE EDITING INNOVATION CENTER
Durham, North Carolina, USA

INTERNATIONAL OFFICES
United Kingdom
Switzerland

SAREPTA THERAPEUTICS

Media Inquiries: Media@Sarepta.com | 617.274.4052

Follow us on social: