

Forward-looking statements

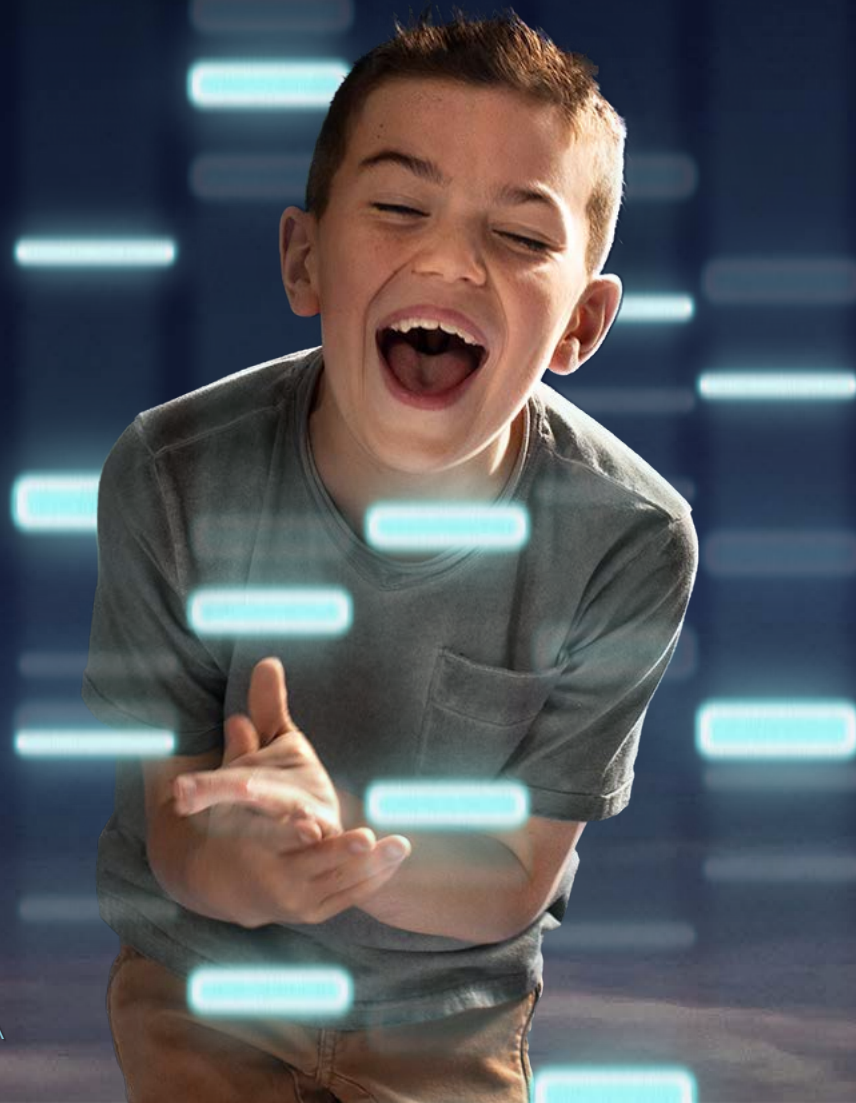
This presentation contains "forward-looking statements." Any statements that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believe," "anticipate," "plan," "expect," "will," "may," "intend," "prepare," "look," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements relating to our strategy; our program's potential to treat 1.5M patients; the potential of our collaborations and partnerships, including with Roche, the University of Massachusetts, StrideBio and Harvard University; our goal to create at least 2 novel gene therapy constructs per year for new indications; our goal of building and populating a gene editing center of excellence in Durham, NC; our gene editing approaches' potential to be available for the majority of DMD patients and to mitigate safety durability challenges facing competing approaches; the estimated number of patients suffering from DMD, LGMDs and MPS IIIA; our pipeline, technologies and programs, including with strategic partners, and their respective potential benefits, including the potential benefits of MHCK7, AAVrh74, SR2, SR3, β -SARCOGLYCAN, PMO and the potential of our PPMO to lead to more efficient dosing and to deliver PMOs to unique muscle types; our gene therapy programs' potential to treat over 70% of all known LGMDs; the potential read through of our work on LGMD2E on our other LGMD candidates; Sarepta being poised to have 3 PMOs serving the community in 2020, serving ~29% of the DMD community; our plan to expand our Gene Therapy Center of Excellence in 2020; and expected milestones, including completing the commercial process development and analytical development for SRP-9001 in H1 2020, gaining insights from the FDA on CMC and analytical assays for study 301 in Q2 2020, commencing study 301 in mid-2020, having an expression and functional read out of study 102 in early 2021, having a read out of SRP-9003 high-dose cohort study in early Q2 2020, gaining regulatory and manufacturing insight for SRP-9003 in Q4 2020, completing dosing in the LYS-SAF302 Phase 2/3 AAVance study in H1 2020, and having safety and dosing insights from study SRP-5051 in mid-2020.

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: we may not be able to comply with all FDA post-approval commitments and requirements with respect to EXONDYS 51 and VYONDY 53 in a timely manner or at all, our data for our different programs, including casimersen, SRP-9001 and SRP-9003, 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; if the actual number of patients suffering from DMD, LGMD, and MPS IIIA is smaller than estimated, our revenue and ability to achieve profitability may be adversely affected; various factors may decrease the market size of our product and product candidates, including the severity of the disease, patient demographics and the response of patients' immune systems to our product candidates; 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; 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 for the year ended December 31, 2018 or 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.

Some see slow and
steady scientific progress.

LIAM
Living with MPS IIIA



We see a revolution.

DOUG INGRAM

President and CEO

J.P. Morgan 38th Annual Healthcare Conference
January 13, 2020



Making the revolution real



DEEPE
NED
PIPELINE



ADVANCED
CLINICAL
DEVELOPMENT



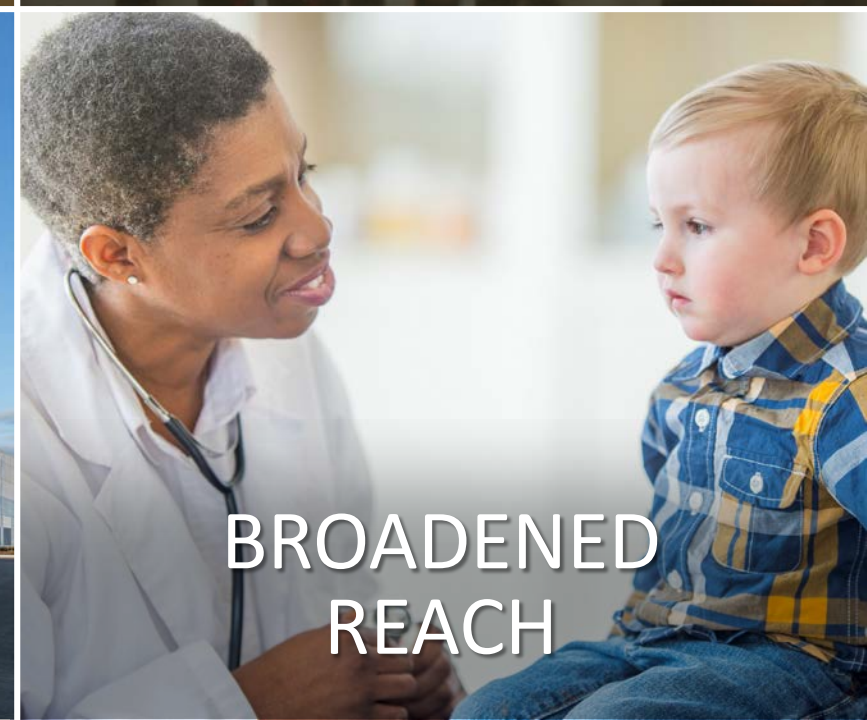
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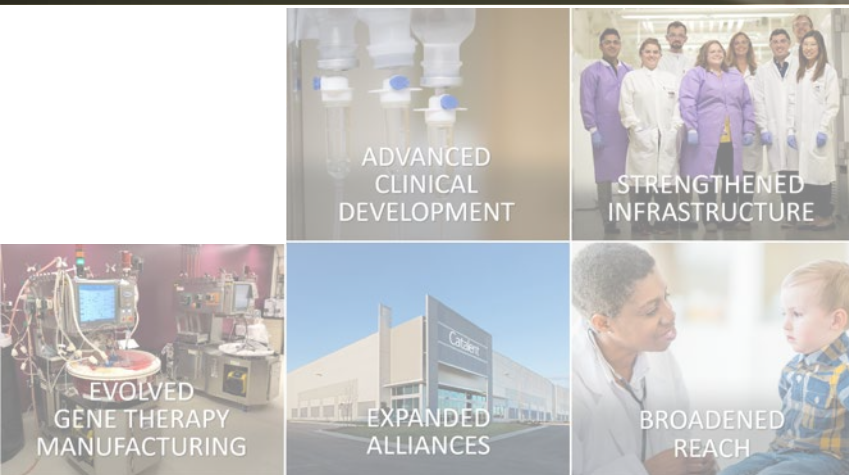
EVOLVED
GENE THERAPY
MANUFACTURING



EXPANDED
ALLIANCES



BROADENED
REACH



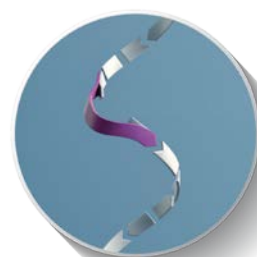
Our Pipeline in 2018

Sarepta-driven programs



GENE
THERAPY

0
PROGRAMS



RNA

11
PROGRAMS

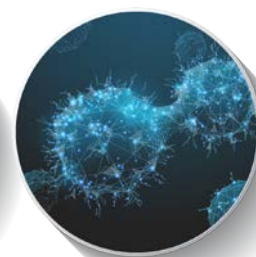
Sarepta's partnered programs



GENE
THERAPY



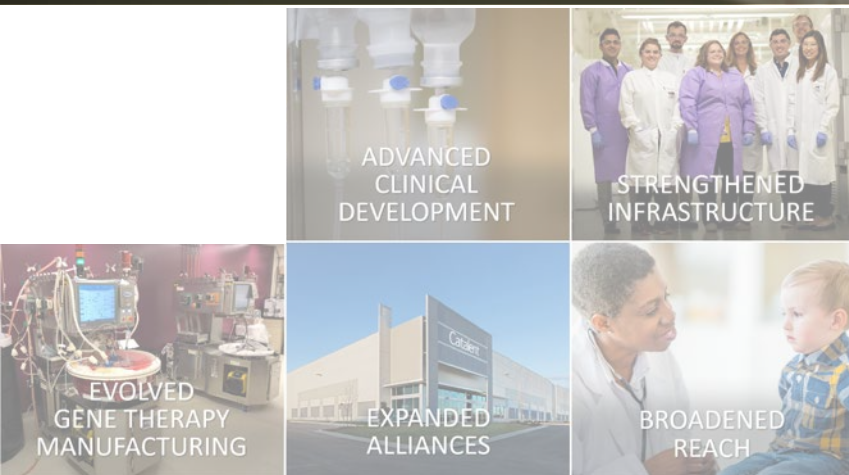
GENE
EDITING



ENABLING
TECHNOLOGIES

14
PROGRAMS

25 programs in all



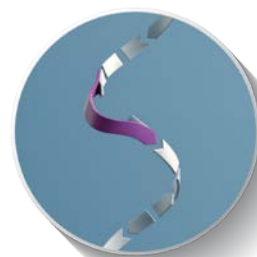
Our Pipeline in 2019

Sarepta-driven programs



GENE
THERAPY

6
PROGRAMS



RNA

14
PROGRAMS

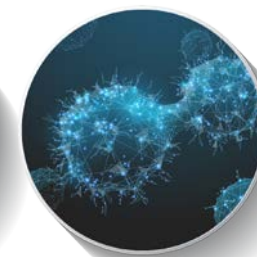
Sarepta's partnered programs



GENE
THERAPY



GENE
EDITING



ENABLING
TECHNOLOGIES

22
PROGRAMS

42 programs in all

Potential to treat 1.5M patients*

*Based on published epidemiology

J.P. MORGAN 38TH ANNUAL HEALTHCARE CONFERENCE

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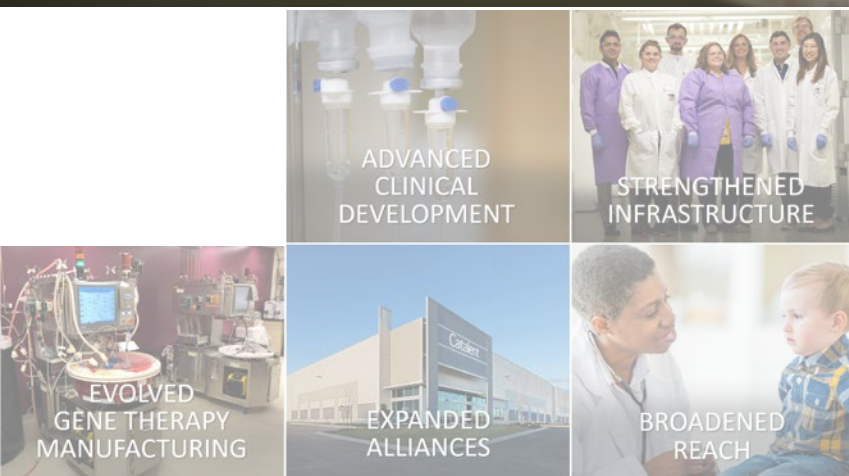
Looking to the Future: Tools and Enablers

- **AAV Technology Collaborations**

- **University of Massachusetts Medical School, Dr. Guangping Gao**
 - Focused on developing novel human-derived vectors
- **StrideBio**
 - Enhance targeting to tissues of interest
 - Expand treatable populations
 - Potential for re-dosing

- **Columbus Gene Therapy Center of Excellence - Internal Technology Development**

- Create at least 2 novel gene therapy constructs per year for new indications
- Developed assays and reagents to understand, monitor, and circumvent the immune system
- Established an efficient modular approach to create, test and select robust gene therapy constructs for new indications

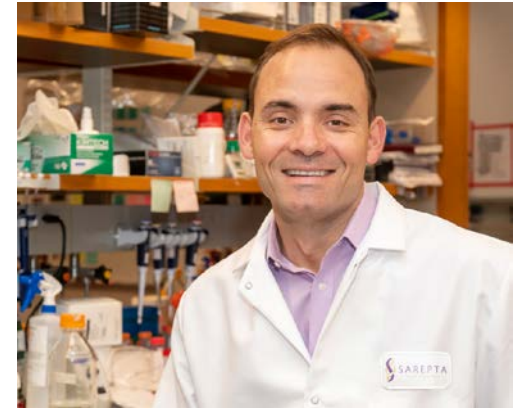




Sarepta establishes Gene Editing Innovation Center (GEIC) in Durham, NC

Charlie Gersbach, Ph.D. of Duke University to lead GEIC

Pursuing gene editing approaches potentially available for majority of DMD patients



- **Differentiated scientific approach**
 - Proprietary *dual cut strategy* for predictable and accurate editing
 - Potential to mitigate safety and durability challenges facing competing approaches
- **Led by world class scientific team**
 - **Louise Rodino-Klapac, Ph.D.,** Sr. Vice President, Gene Therapy, Sarepta
 - **Charlie Gersbach, Ph.D.,** Director, Center for Advanced Genomic Technologies, Rooney Family Associate Professor of Biomedical Engineering, Director, Center for Biomolecular and Tissue Engineering, Duke University*



*Dr. Gersbach will retain his professorship at Duke University



Sarepta establishes multi-year Research collaboration with Harvard University

Amy Wagers, Ph.D., Harvard Stem Cell Institute, to lead research project

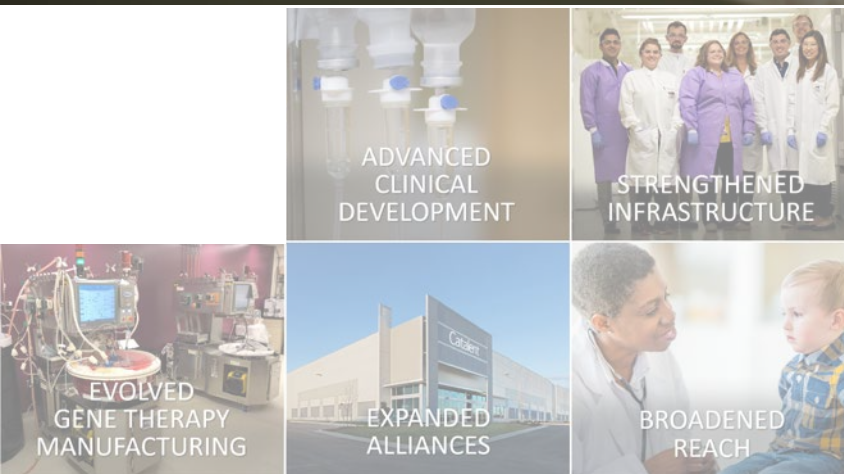
Pursuing in-vivo genome editing approach to treat DMD

- **Unique strategy**
 - Fully and precisely restore function of the dystrophin gene at its source in the DNA of stem cells (satellite cells) responsible for regenerating muscle cells
- **Led by pioneering stem cell researcher**
 - **Amy Wagers, Ph.D.**, Forst Family Professor, Stem Cell and Regenerative Biology, Co-Chair, Harvard Department of Stem Cell and Regenerative Biology, Executive Committee Member, Harvard Stem Cell Institute



Dr. Wagers's lab successfully used gene editing in mice to remove the faulty sequences in DMD to restore dystrophin function in heart, skeletal muscle and satellite cells*

*Published as "In vivo gene editing in dystrophic mouse muscle and muscle stem cells". Science. 2016 Jan 22;351(6271):407-411.

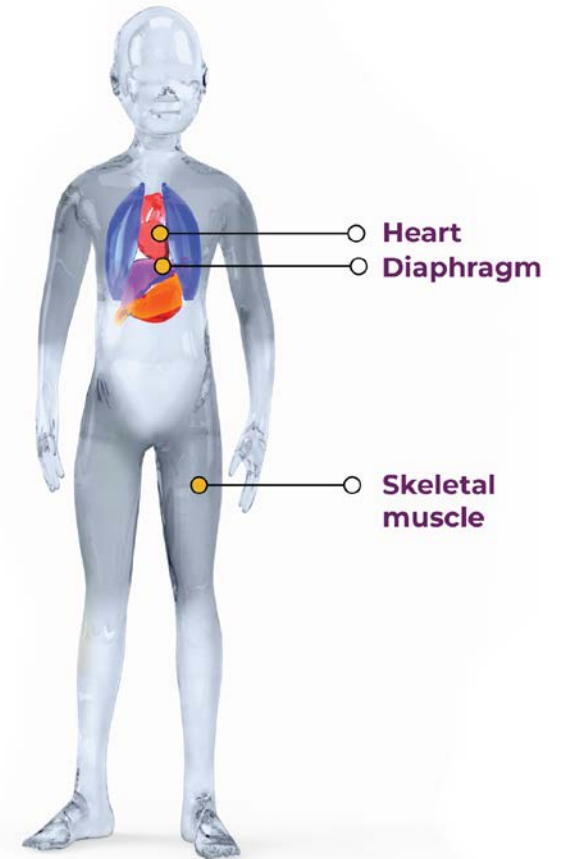


ADVANCED CLINICAL DEVELOPMENT *Gene Therapy*

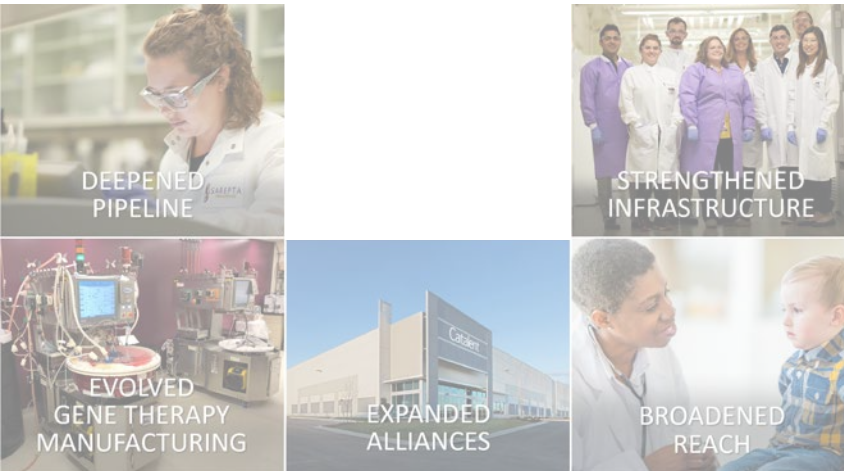
The disease Duchenne muscular dystrophy (DMD)

*DMD affects approximately
1 in 3,500-5,000 males worldwide¹*

- DMD is a rare, fatal neuromuscular genetic disease inherited in an X-linked recessive pattern²

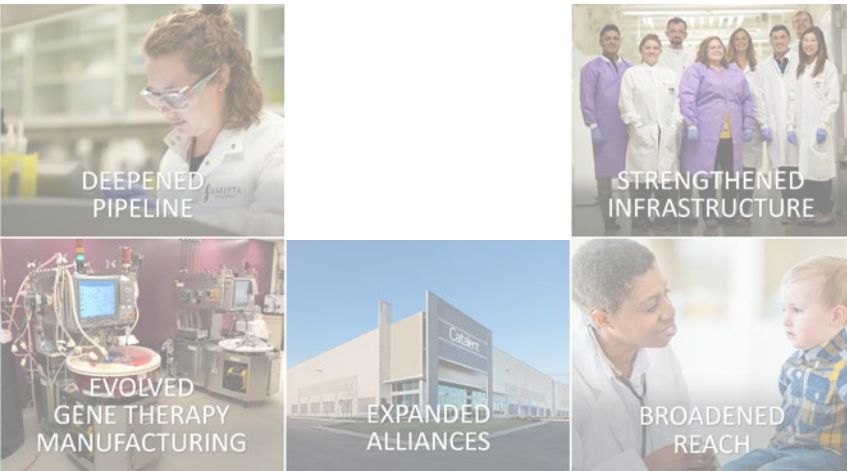


*On average, every day
DMD takes the life of
a child in the United States*



1. National Institutes of Health. Genetics Home Reference. Duchenne and Becker muscular dystrophy. <https://ghr.nlm.nih.gov/condition/duchenne-and-becker-muscular-dystrophy>. Accessed Jan 2020.
2. Hoffman EP, Brown RH, et al. Dystrophin: the protein product of the Duchenne muscular dystrophy locus. Cell. 1987;51:919-928.

ADVANCED CLINICAL DEVELOPMENT *Gene Therapy*



DEEPEMED
PIPELINE

STRENGTHENED
INFRASTRUCTURE

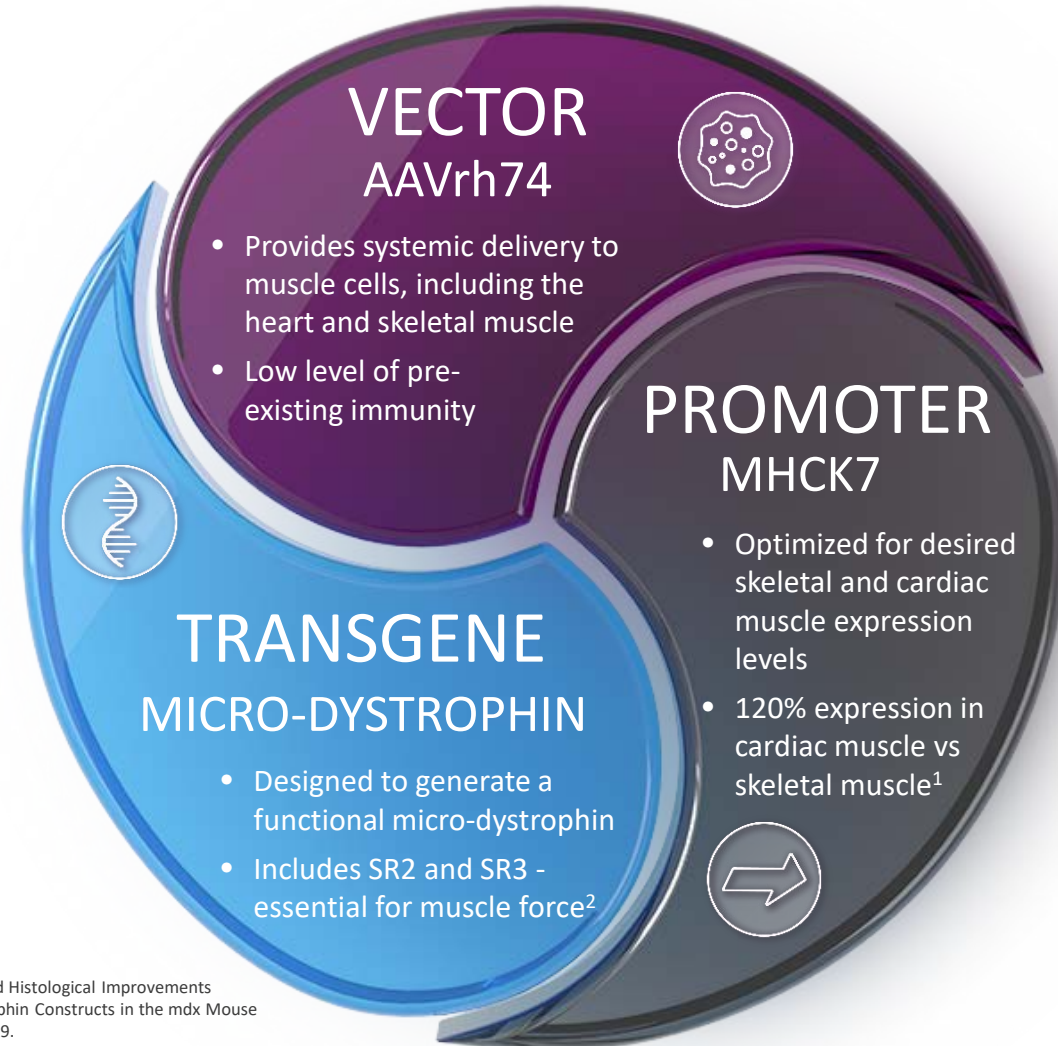
EVOLVED
GENE THERAPY
MANUFACTURING

EXPANDED
ALLIANCES

BROADENED
REACH

The science

Sarepta's differentiated gene therapy for SRP-9001



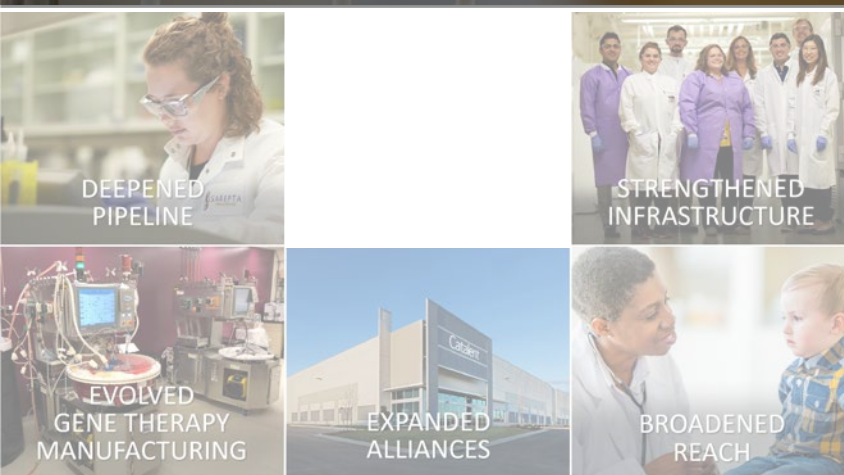
1. Potter et al. Functional and Histological Improvements Comparing 4 Micro-dystrophin Constructs in the mdx Mouse Model of DMD. ASGCT 2019.

2. Nelson DM, Ervasti JM, et al. Variable rescue of microtubule and physiological phenotypes in mdx muscle expressing different miniaturized dystrophins. Human Molecular Genetics, 2018, Vol. 27, No. 12: 2090-2100.



ADVANCED CLINICAL DEVELOPMENT

Gene Therapy



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PIPELINE

STRENGTHENED
INFRASTRUCTURE

EVOLVED
GENE THERAPY
MANUFACTURING

EXPANDED
ALLIANCES

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REACH

The clinical data

SRP-9001 demonstrated positive 9-month functional data from first 4-patient cohort

EARLY CLINICAL RESULTS MICRO-DYSTROPHIN GENE THERAPY FOR DMD*

81%

Expression of
micro-dystrophin
in muscle fibers¹

96%

Expression of
micro-dystrophin
measured by signal
intensity¹

96%

Expression of
micro-dystrophin
measured by
Western blot^{**},¹

64%

Reduction of
creatine kinase¹

NINE-MONTH CLINICAL RESULTS MICRO-DYSTROPHIN GENE THERAPY FOR DMD – AVERAGE CHANGE FROM BASELINE***

NSAA

6.5

POINT IMPROVEMENT

TIME TO RISE

.8

SECOND IMPROVEMENT

4 STAIRS UP

1.2

SECOND IMPROVEMENT

100 M

7.95

SECOND IMPROVEMENT

1. Clinical Update: Micro-dystrophin Study-101 - March 25, 2019 - Micro-dystrophin Gene Therapy Update Conference Call
- Sarepta Therapeutics Data on File
ClinicalTrials.gov Identifier: NCT03375164
Sarepta Therapeutics Data on File

*Data from the 4 patients dosed in Study NCT03375164
**NCH Western blot method
***North Star Ambulatory Assessment (NSAA),
Time to Rise, 4 Stairs Up, and 100M



ADVANCED CLINICAL DEVELOPMENT *Gene Therapy*

The progress SRP-9001 – study 102 and study 301 achievements and forward momentum

2019 Progress **STUDY 102**

- Dosed 41 patients
- Dosed placebo cross-over patients
- Study is progressing well and uninterrupted

*Data read out
scheduled
for early 2021*

2019 Progress **STUDY 301**

- Achieved commercially viable yields


*On track to
commence study
mid-2020*



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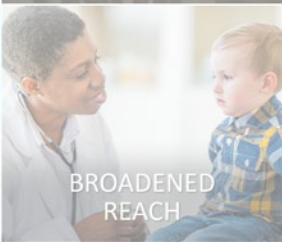
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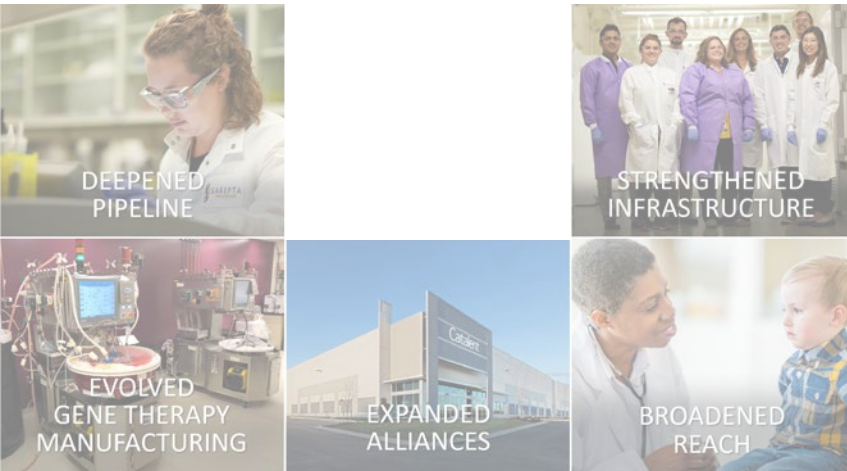


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ALLIANCES



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The disease Limb-girdle muscular dystrophies (LGMDs)

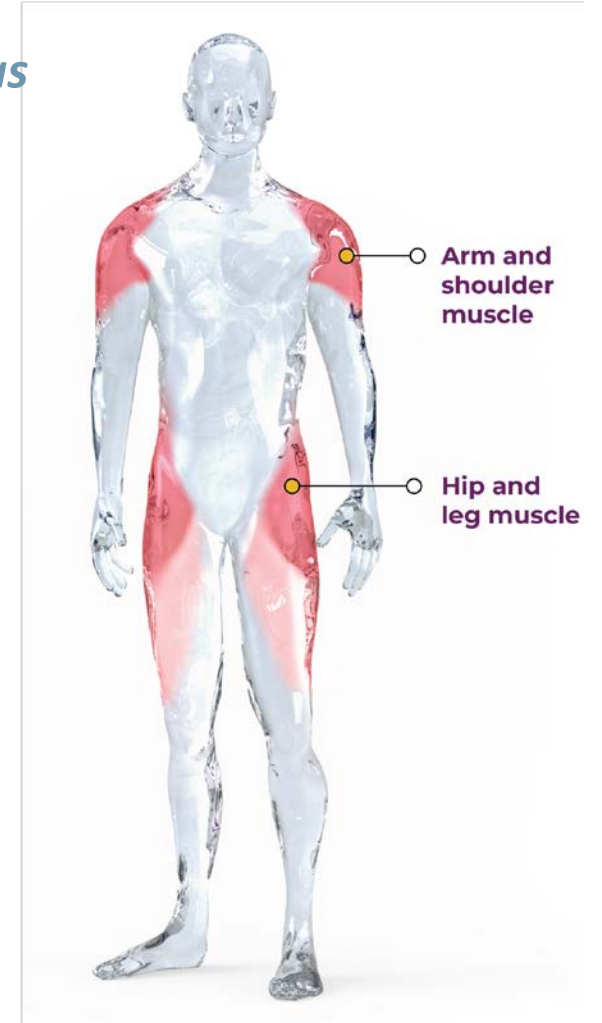
Approximate global prevalence of LGMDs as a group is 1.63 per 100,000^{1}*

Over 30 subtypes exist²

Both genders are affected equally³

- The LGMDs are a group of genetically heterogeneous, autosomal inherited (recessive more common than dominant) muscular dystrophies with a childhood to adult onset⁴

Sarepta's 6 gene therapy programs address over 70% of all known LGMDs⁵



*Prevalence estimates range from 0.56 to 5.75 per 100,000¹

1. Liewluck T, Milone M, et al. Untangling the complexity of limb-girdle muscular dystrophies. *Muscle Nerve*. 2018;58(2):167-177.

2. Murphy AP, Straub V. The Classification, Natural History and Treatment of the Limb Girdle Muscular Dystrophies. *J Neuromuscular Diseases*. 2015;2(s2):S7-S19.

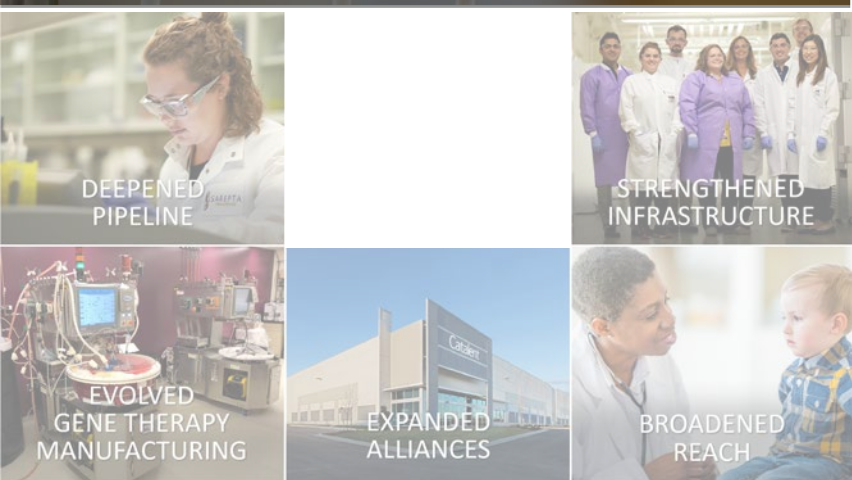
3. Muscular Dystrophy Association. Limb-girdle muscular dystrophy (LGMD). Accessed Jan 2020 4.

Liewluck T, Milone M, et al. Untangling the complexity of limb-girdle muscular dystrophies. *Muscle Nerve*. 2018;58(2):167-177.

5. Taghizadeh E, Rezaee M. *J Cell Physiol*. 2018;1-11.

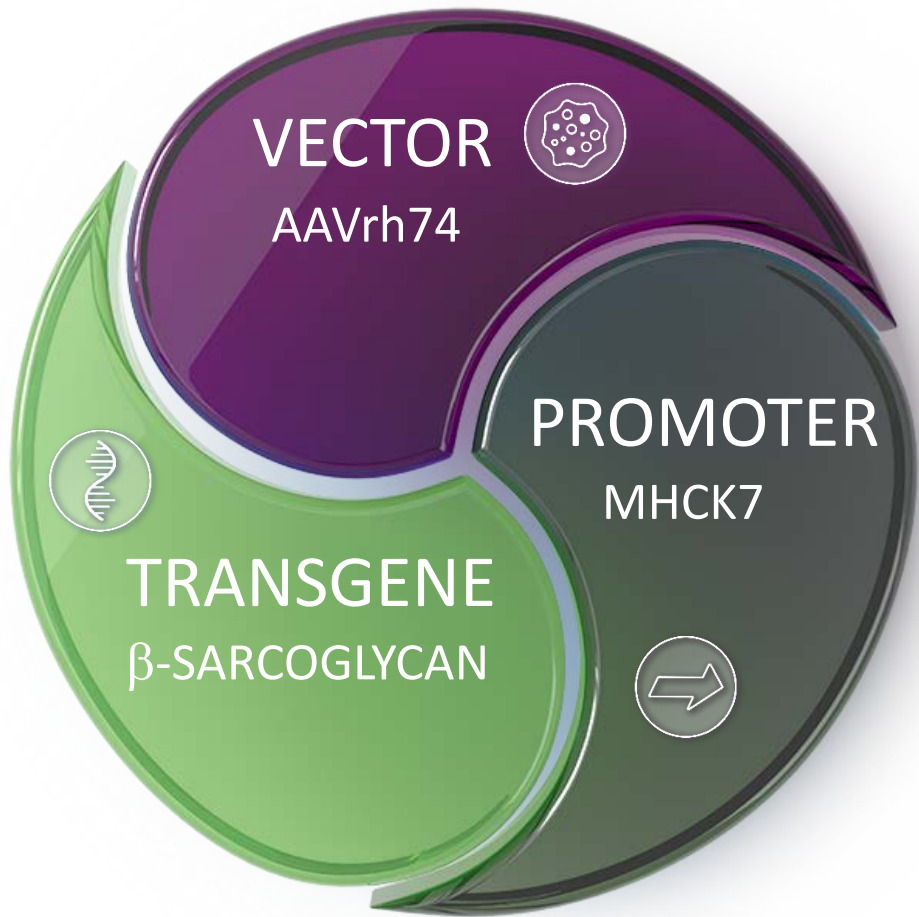


ADVANCED CLINICAL DEVELOPMENT *Gene Therapy*



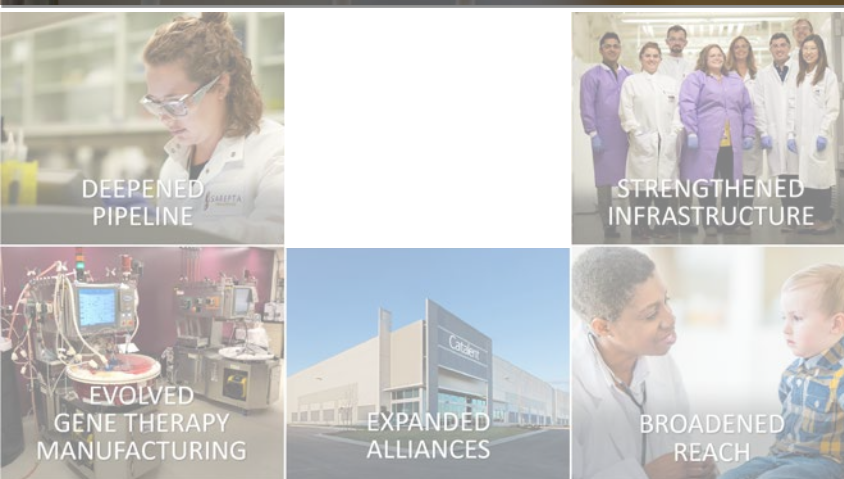
The science SRP-9003 gene therapy for LGMD2E

Designed to transduce skeletal and cardiac muscle with a gene that codes for the full-length, native β -sarcoglycan protein (the lack of which causes LGMD2E)





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DEVELOPMENT
Gene Therapy



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The clinical data

LGMD2E demonstrated consistent results

PRELIMINARY CLINICAL RESULTS β -SARCOGLYCAN GENE THERAPY FOR LIMB-GIRDLE MUSCULAR DYSTROPHY TYPE 2E^{*,1,2}

51%

Expression of β -sarcoglycan in muscle fibers

47%

Expression of β -sarcoglycan measured by signal intensity

36%

Expression of β -sarcoglycan measured by Western blot^{**}

82%

Reduction of creatine kinase

CLINICAL RESULTS β -SARCOGLYCAN GENE THERAPY FOR LIMB-GIRDLE MUSCULAR DYSTROPHY TYPE 2E^{*,2}

All patients improved in all functional endpoints at 9 months

^{*}Data from the 3 patients dosed in Study NCT03652259
^{**}NCH Western blot method

1. Sarepta Press Release – February 27, 2019
2. Sarepta Press Release – October 4, 2019



ADVANCED CLINICAL DEVELOPMENT *Gene Therapy*

The progress

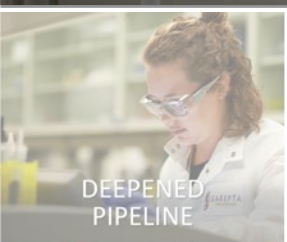
Clinical study underway for SRP-9003 in patients with LGMD2E

A Single-Center, Open-Label, Systemic Gene Delivery Study to Evaluate the Safety, Tolerability, and Efficacy of SRP-9003 Administered by Systemic Infusion in Subjects With LGMD2E (β -Sarcoglycan Deficiency)

2019 Achievement

- 2 patients dosed (30kgs and 40kgs)

*Read out from high-dose cohort,
early Q2 2020*



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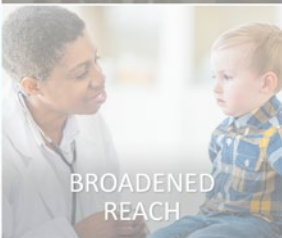
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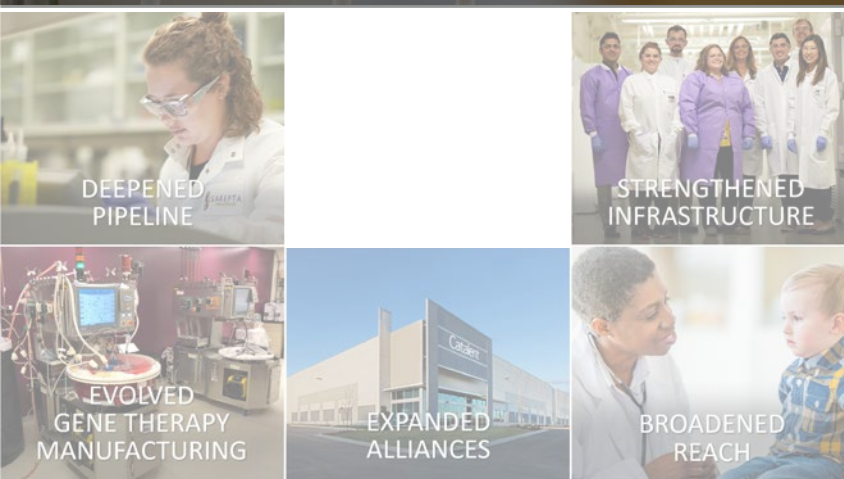
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GENE THERAPY
MANUFACTURING



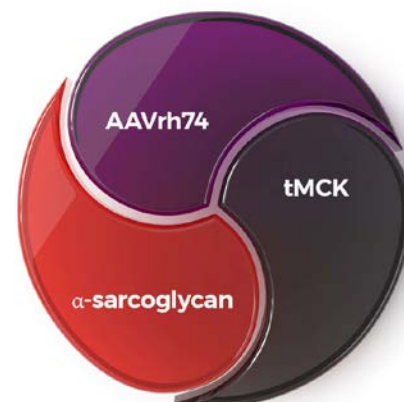
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ALLIANCES



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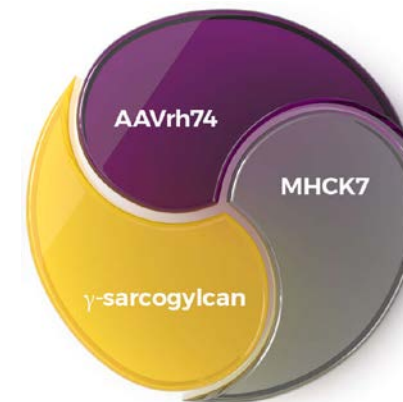
Gene therapy engine at work across LGMD programs



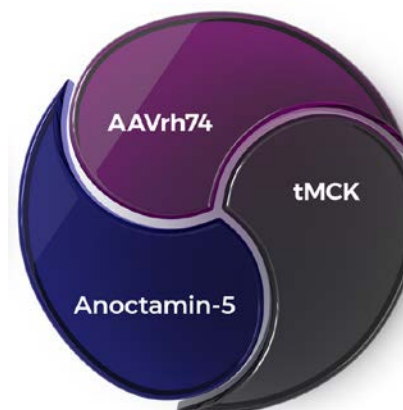
LGMD2D



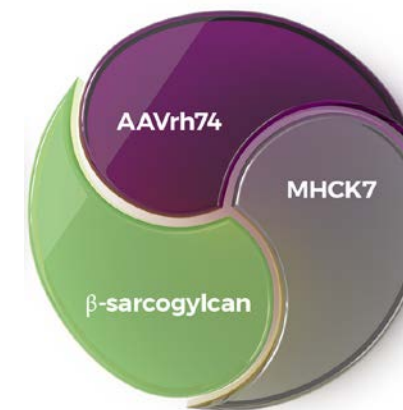
LGMD2B



LGMD2C



LGMD2L



LGMD2E

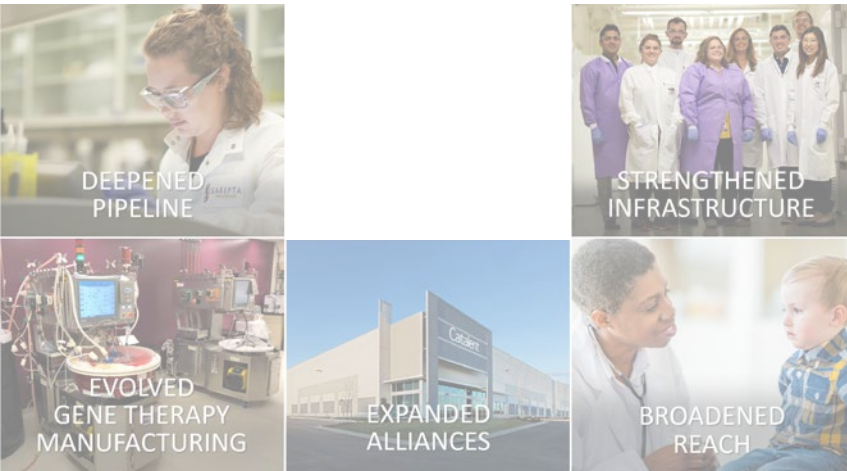
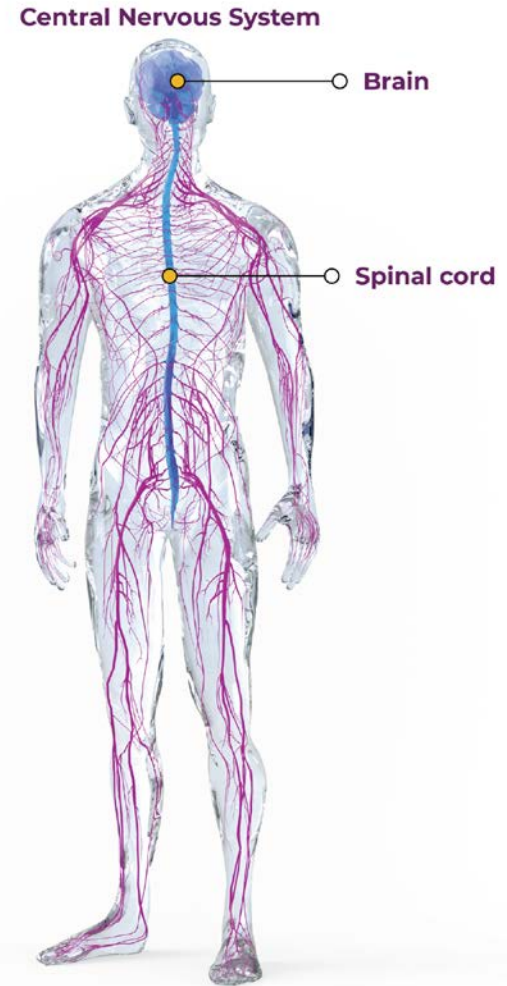
ADVANCED CLINICAL DEVELOPMENT *Gene Therapy*

The disease Mucopolysaccharidosis Type IIIA (MPS IIIA)

MPS IIIA has a worldwide incidence of up to 1.62 per 100,000 live births¹

- Signs and symptoms usually become apparent in early childhood and include, developmental delays (e.g., speech problems), challenging behaviors, extreme hyperactivity and poor sleep²

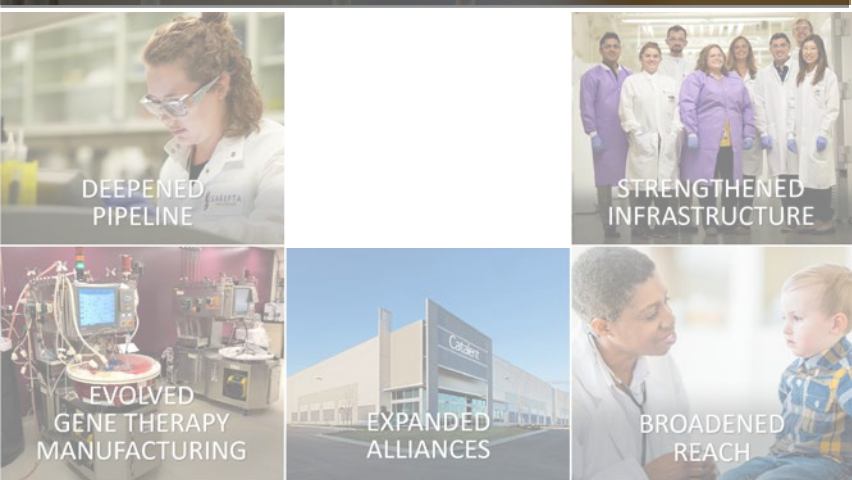
No approved treatment indicated for MPS IIIA²



1. Zelei T, Csetneki K, et al. Epidemiology of Sanfilippo syndrome: results of a systematic literature review. Orphanet J of Rare Dis. 2018;13(1):53.
2. National Institutes of Health. Genetic and Rare Diseases Information Center. Mucopolysaccharidosis type IIIA. Accessed Jan 2020.



ADVANCED CLINICAL
DEVELOPMENT
Gene Therapy



The science
LYS-SAF302, partnered with Lysogene,
a gene therapy for MPS IIIA





ADVANCED CLINICAL DEVELOPMENT *Gene Therapy*

The progress

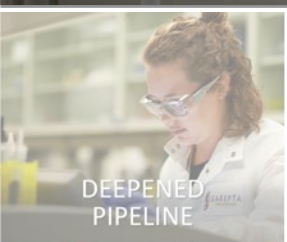
LYS-SAF302, a gene therapy in clinical development for patients with MPS IIIA

2019 Progress

Phase 2/3 AAVance study

- Initiated Phase 2/3 study in MPS IIIA patients
- Completed 2-year natural history study in MPS IIIA (serve as control arm for Phase 2/3)
- Enrolled 15 patients in Phase 2/3 study


Complete dosing (N=20) – H1 2020



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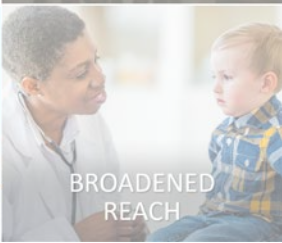
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ADVANCED CLINICAL DEVELOPMENT RNA

The science: Proprietary PMO Technology

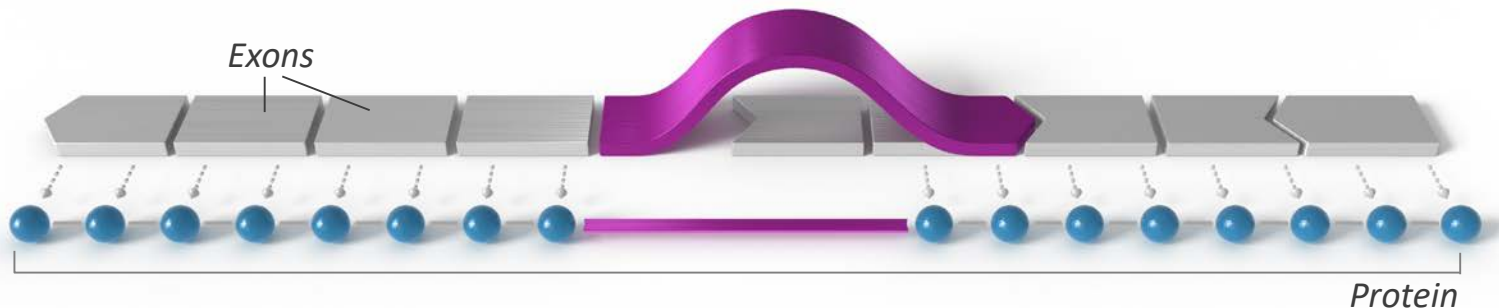
Phosphorodiamidate morpholino oligomer (PMO) Technology

Specificity: Enhanced affinity for targeting pre-mRNA for precise binding to the selected RNA target

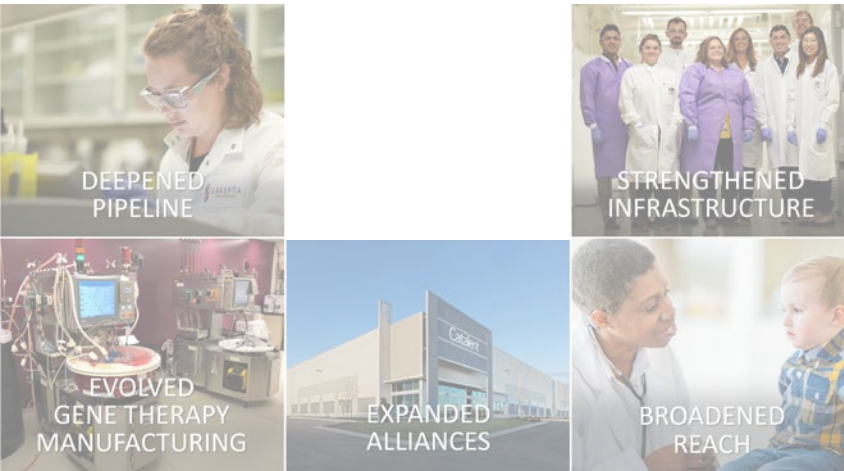
Stability: Highly resistant to degradation by enzymes

Versatility: Ability to rapidly design and construct drug candidates that are specific for human or pathogen RNA; and target specific tissues

Safety: Built upon a charge-neutral backbone, which may be reflected in tolerability



The PMO directs the splicing machinery to skip an exon when processing the pre-mRNA. As a result, the alternate mRNA allows for the production of a shortened, functional dystrophin protein.

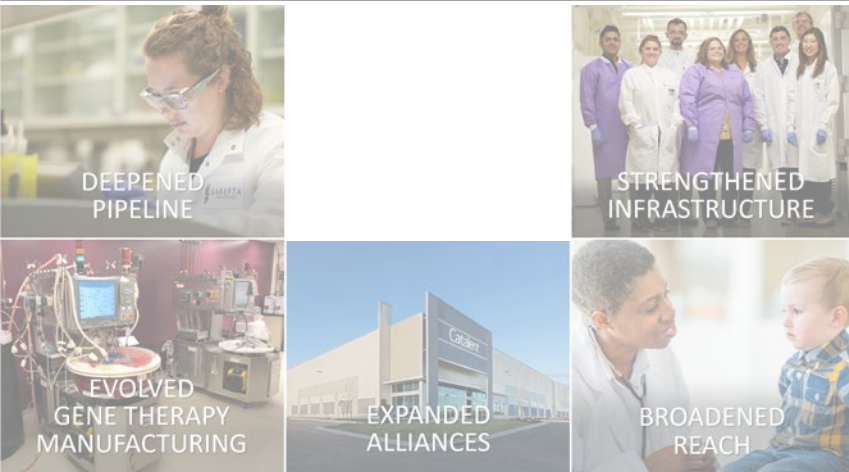




ADVANCED CLINICAL DEVELOPMENT *RNA*

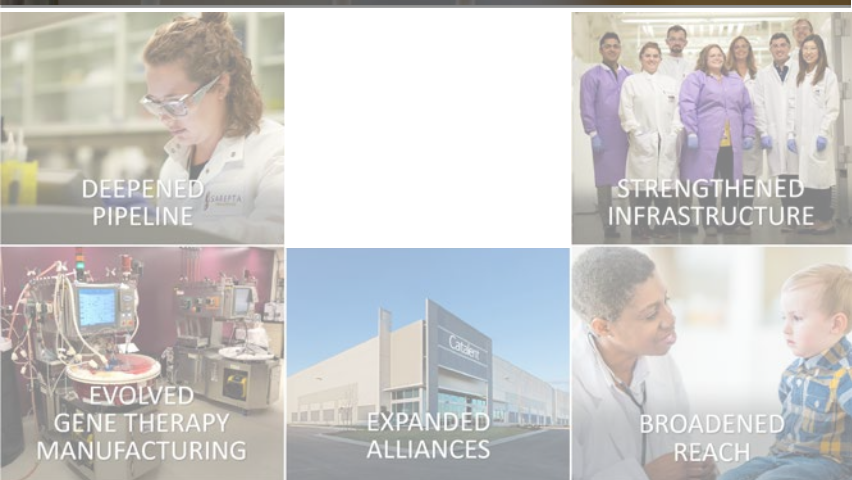
2019 accomplishments: Proprietary PMO Technology

- Completed U.S. submission for VYONDYS 53 (Q1 2019)
- Announced positive dystrophin expression results for casimersen to treat exon 45 amenable patients (Q1 2019)
- Received a Complete Response Letter (CRL) for VYONDYS 53 (August 2019)
- Received approval for VYONDYS 53 (December 2019)
 - The fastest CRL reversal in FDA history

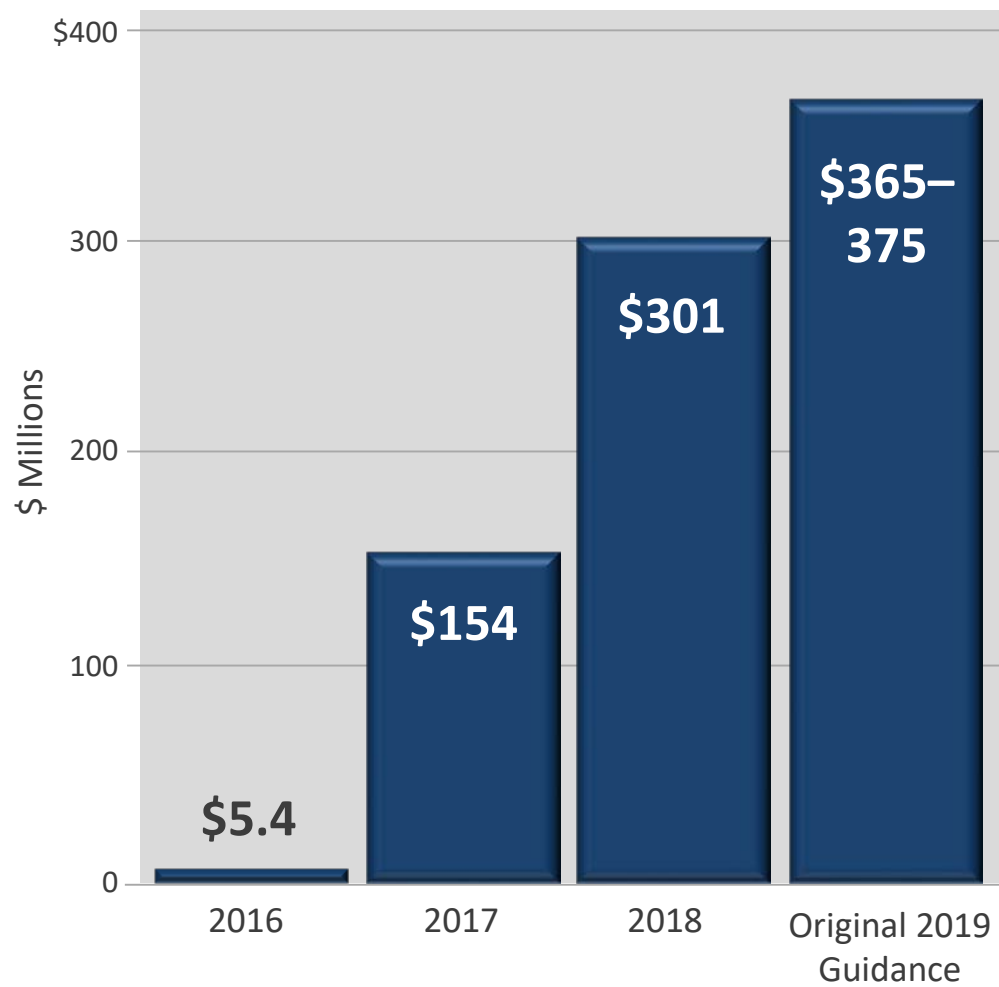




ADVANCED CLINICAL DEVELOPMENT *RNA*



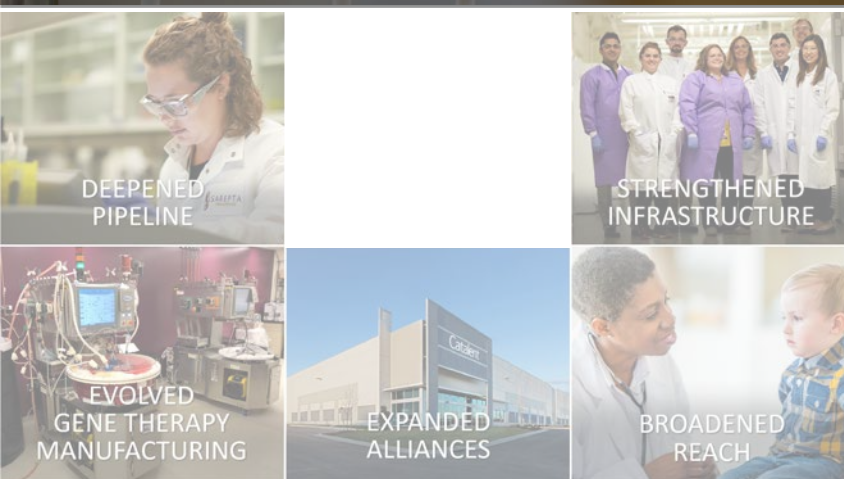
EXONDYS 51 Revenue Since 2016 Launch*



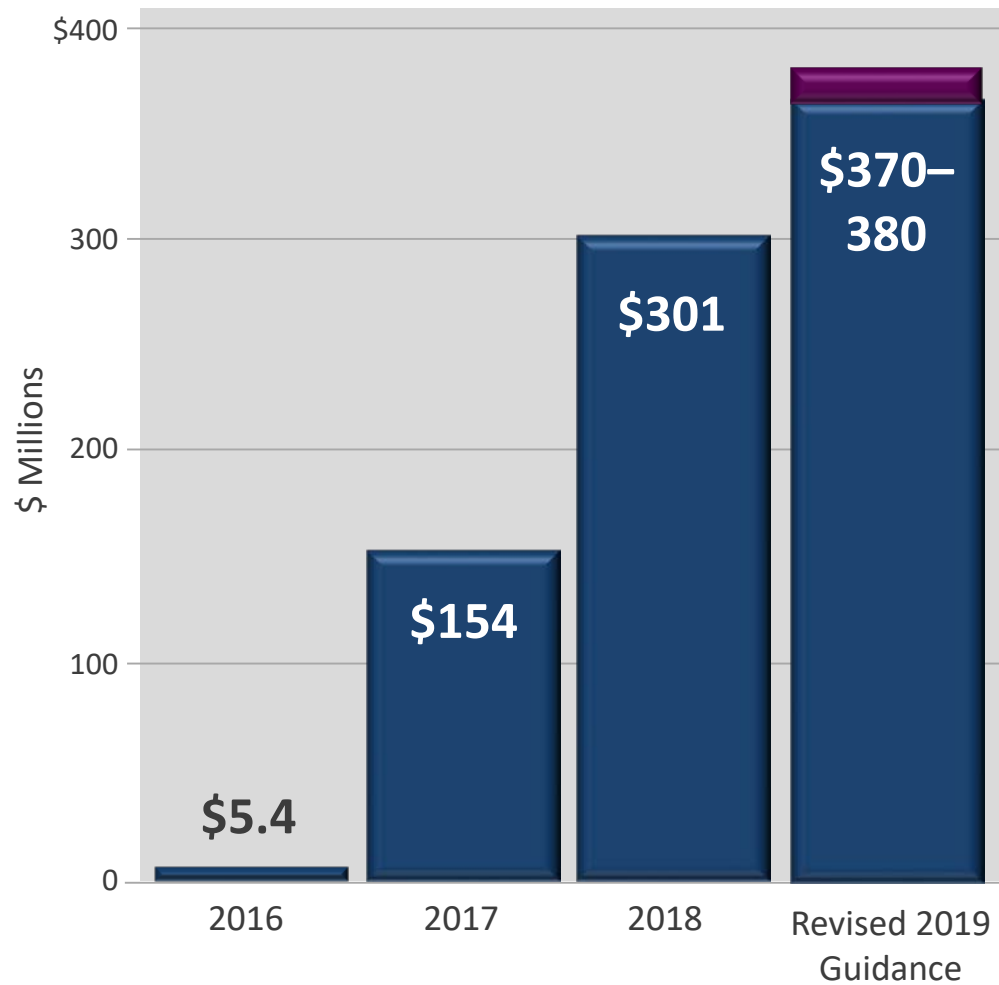
*Candidate received accelerated approval in the U.S., confirmatory studies required



ADVANCED CLINICAL DEVELOPMENT *RNA*



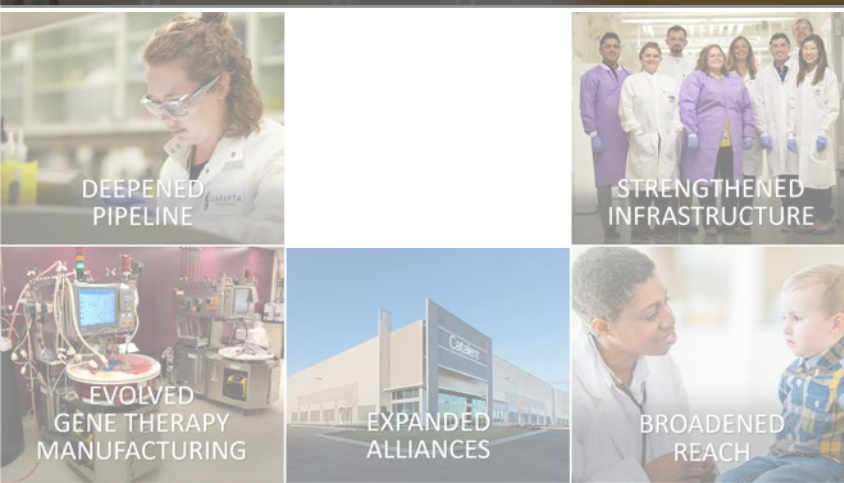
EXONDYS 51 Revenue Since 2016 Launch*



*Candidate received accelerated approval in the U.S., confirmatory studies required



ADVANCED CLINICAL DEVELOPMENT RNA



DEEPE
PIPELINE

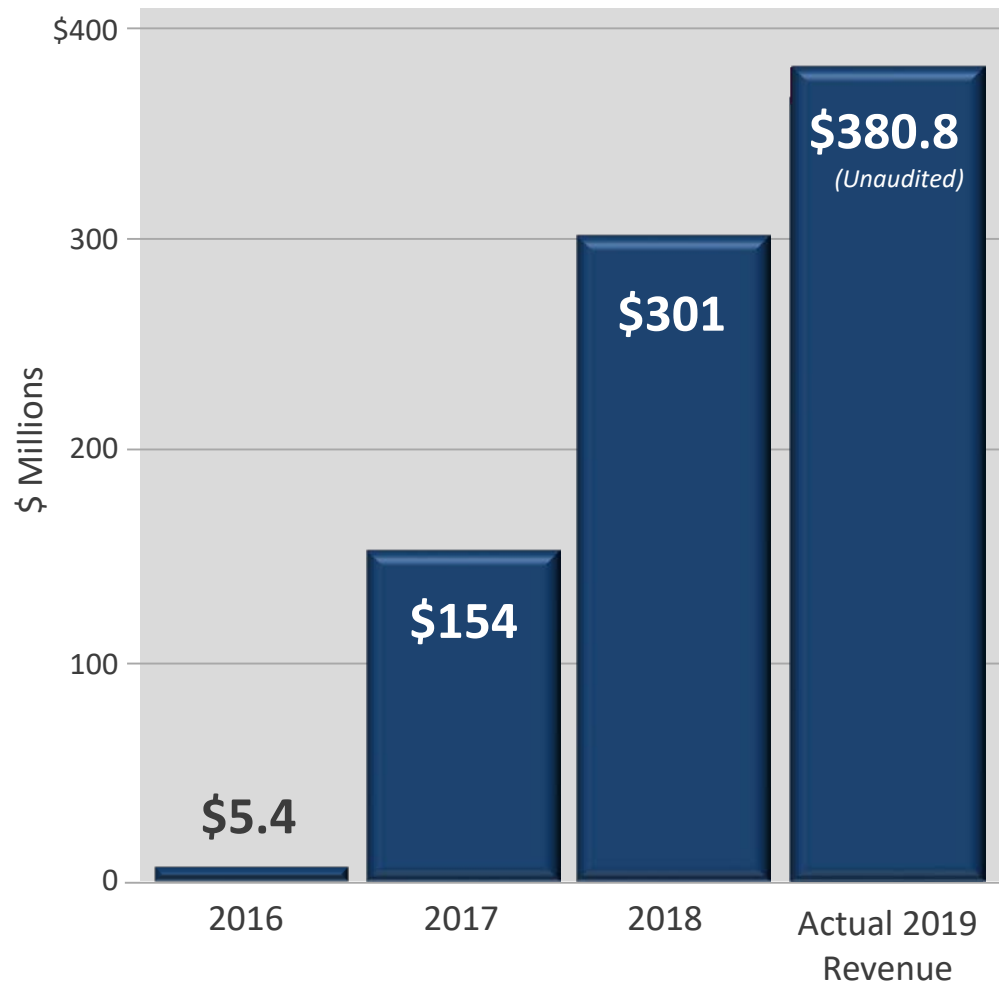
STRENGTHENED
INFRASTRUCTURE

EVOLVED
GENE THERAPY
MANUFACTURING

EXPANDED
ALLIANCES

BROADENED
REACH

EXONDYS 51 Revenue Since 2016 Launch*



- Q4 revenue
\$100.1M
(Unaudited)

*Candidate received accelerated approval in the U.S., confirmatory studies required

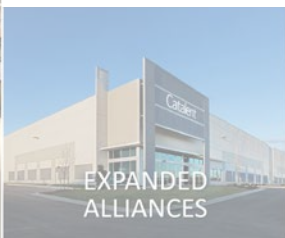
ADVANCED CLINICAL DEVELOPMENT RNA



DEEPE
PIPELINE



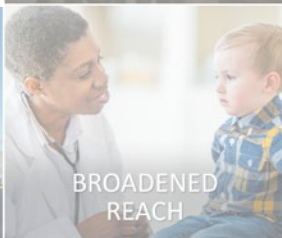
EVOLVED
GENE THERAPY
MANUFACTURING



EXPANDED
ALLIANCES

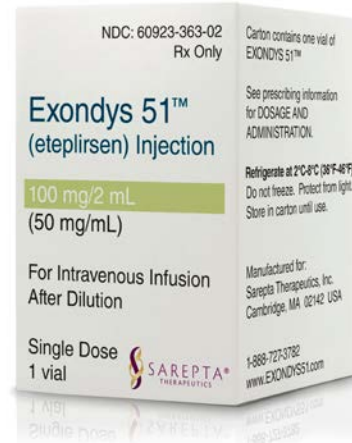


STRENGTHENED
INFRASTRUCTURE

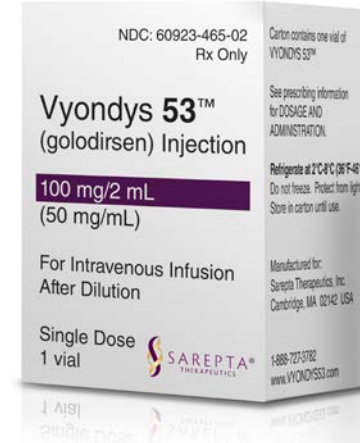


BROADENED
REACH

Poised to have 3 PMOs serving the community in 2020



September 2016:
Approved to treat patients
with a confirmed genetic
mutation that is amenable
to exon 51 skipping
(13% of DMD population)*



December 2019:
Approved to treat patients
with a confirmed genetic
mutation that is amenable
to exon 53 skipping
(8% of DMD population)*

**CASIMERSEN
(SRP-4045)**

January 2020:
Initiated rolling
NDA submission
(8% of DMD population)

*Candidate received accelerated approval in the U.S., confirmatory studies required



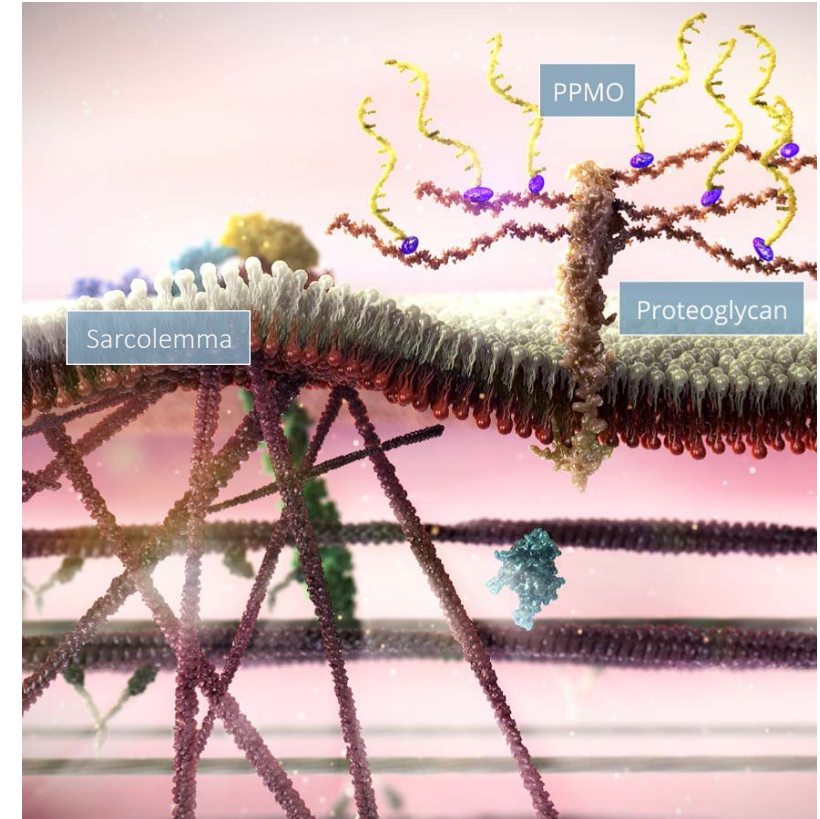
ADVANCED CLINICAL DEVELOPMENT RNA

The science: PPMO Next-Generation Technology


*Peptide phosphorodiamidate
morpholino oligomer (PPMO)
Technology*

Enhances PMO

- Conjugated peptide greatly increases cell penetration
- Could potentially lead to more efficient dosing for patients
- Able to deliver PMOs to unique muscle types (e.g., heart)



DEEPEMED
PIPELINE



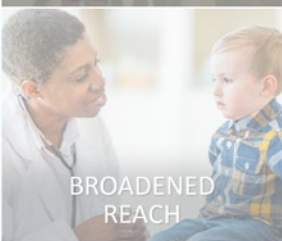
STRENGTHENED
INFRASTRUCTURE



EVOLVED
GENE THERAPY
MANUFACTURING



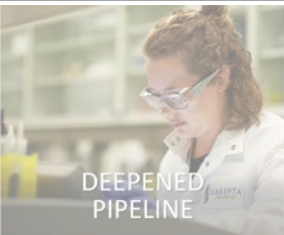
EXPANDED
ALLIANCES



BROADENED
REACH



ADVANCED CLINICAL DEVELOPMENT *RNA*



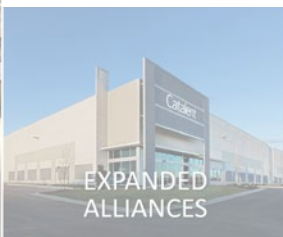
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PIPELINE



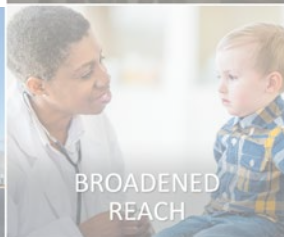
STRENGTHENED
INFRASTRUCTURE



EVOLVED
GENE THERAPY
MANUFACTURING



EXPANDED
ALLIANCES



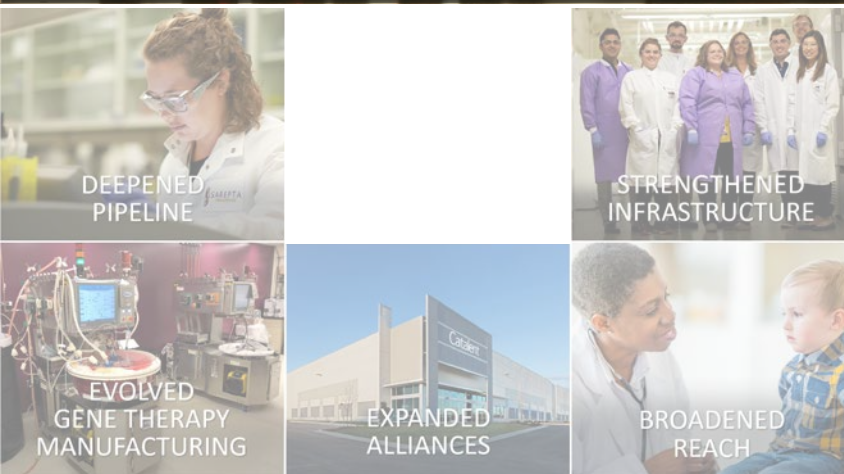
BROADENED
REACH

The science: PPMO Next-Generation Technology

2019 Progress **STUDY for SRP-5051**

- Initiated MAD study in patients with DMD

*Safety and dosing insights
in mid-2020*



Outstanding talent

- We have grown to nearly 800 employees across 4 sites in the U.S.

New 2019 Hires

<i>Clinical Development</i>	65
<i>Regulatory Affairs & Quality</i>	47
<i>Technical Operations</i>	46
<i>Research</i>	26

The number of employees shown reflects headcount added in 2019 and not the total number in the function indicated



Expanded locations and capabilities

- **Columbus, Ohio Gene Therapy Center of Excellence**

- **Overview**

- Planned expansion of facility in 2020
- All employees have deep AAV expertise

- **Strategic Imperatives**

- Execute on current internal and partnered programs
- Continue to build an enduring gene therapy engine
- Advance business development/alliance management strategy

- **Burlington, Massachusetts**

- Established intellectual hub for advanced scale-up and tech transfer capabilities

- **Andover, Massachusetts**

- Expanded lab and office facility (36 acres in total)
- Enhanced Pharmaceutical and Analytical Development capabilities



Significant progress on gene therapy manufacturing

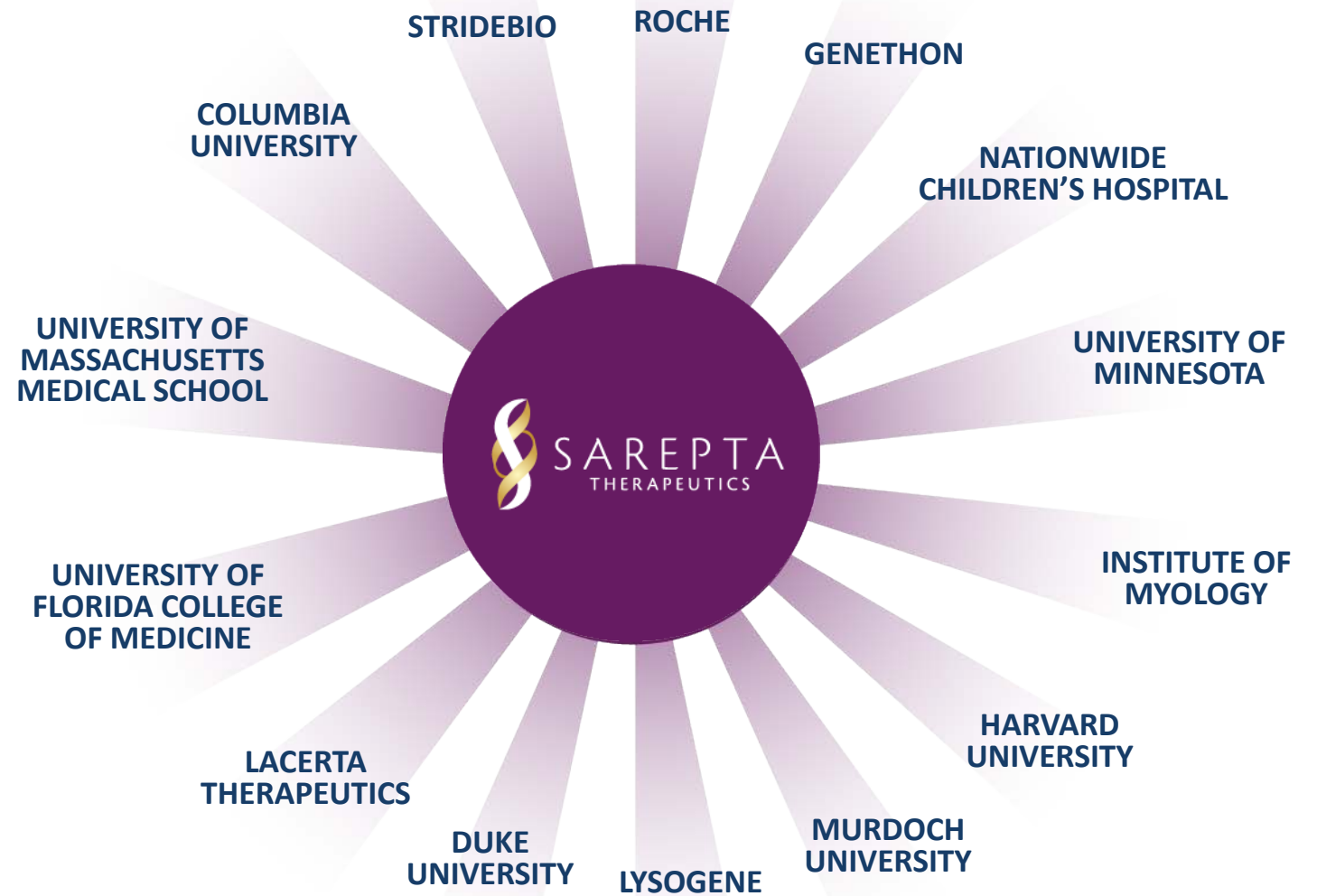
- Expanded Relationships with Thermo Fisher (Brammer) and Catalent (Paragon) to increase capacity
 - Broad access to capacity in two locations
 - Includes dedicated capacity and additional expansion options
 - Completed single-use SRP-9001 facility at Lexington, Massachusetts
- Commenced LGMD process development and analytical development work

Advanced Process Development and Analytical Development for SRP-9001

Achieving yields sufficient to commence study 301 and support commercial launch of SRP-9001



Establishing a **distinct partnership model** to drive future success





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REACH



Sarepta and Roche: Ex-US Strategic Alliance, SRP-9001 micro-dystrophin gene therapy for DMD

The most advanced and scientifically viable gene therapy to treat patients with DMD

*Together, we will reach
more DMD patients
faster than we could
on our own*

- Roche's reputation and expertise in neuromuscular and DMD is significant and its global reach and capabilities are unmatched

*Possibility of success
greatly increased*

- Greater than \$2B in cash to support our goals
- Focused on strategic priorities

Ranked as the largest gene therapy licensing deal and the largest single candidate ex-US licensing deal in history

A New Addition to Sarepta's Board



JOHN C. MARTIN, Ph.D.

Industry veteran; former Chairman, CEO and President, Gilead Sciences

- Grew portfolio to 24 approved therapies (total annual revenues, \$32B+)
- Transformed Gilead's HIV and Hepatitis C portfolios, benefitting patients worldwide

Upcoming milestones (Next 12-18 Months)

Duchenne

SRP-9001 (micro-dystrophin) *Gene Therapy*

- Complete commercial process development and analytical development (H1)
- Gain insights from U.S. Food and Drug Administration on CMC and analytical assays for study 301 (Q2)
- Commence study 3 (mid-year)
- Expression and functional read out of study 102 (early 2021)

SRP-5051 *PPMO*

- Obtain safety and dosing insight (mid-year)

LGMD2E

SRP-9003 *Gene Therapy*

- Read out of high-dose cohort and select dose (Q2)
- Gain regulatory and manufacturing insight (Q4)

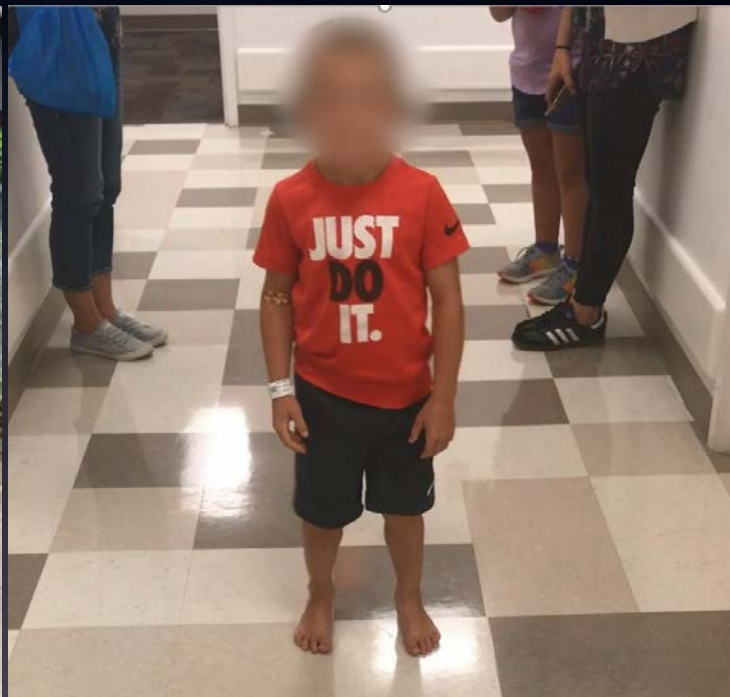
MPS IIIA

LYS-SAF302 *Gene Therapy*

- Complete dosing (H1)



Connor
LIVING WITH
DUCHENNE



Jamison
LIVING WITH
LGMD2E



Lulu
LIVING WITH
MPS IIIA



Billy
LIVING WITH
DUCHENNE



#DragTomorrowIntoToday

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ZYLER
Living with Duchenne