



RESEARCH & CLINICAL TRIALS

Parent JOIN THE FIGHT.
Project END DUCHENNE.
Muscular
Dystrophy

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Overview

- Introduction to clinical trials
- Considerations for participating in clinical trials
- Resources for finding clinical trials
- Current drug development landscape



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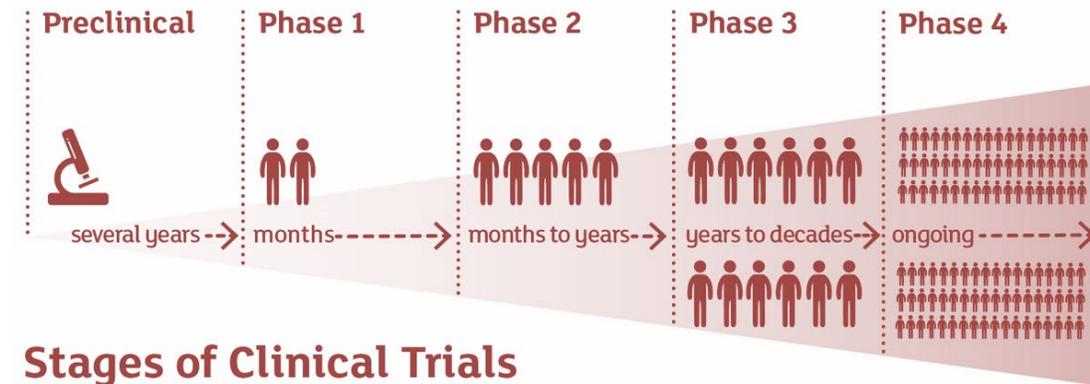
Purpose of a Clinical Trial

- Studies to understand the effect of a drug or device in a human population
- In Duchenne and Becker this has typically involved evaluating the safety and efficacy of new or repurposed drugs, referred to as ‘investigational products’
- Clinical trials are **research** not treatment – the data collected during trials is what is necessary to shift an ‘investigational product’ to an ‘approved product’ by the FDA.
 - There may be benefits from an investigational product, but there is no guarantee, the trial is how we find out if an investigational product is effective.



Phases of Clinical Trials

- Preclinical – Lab and animal studies that help us understand potential of an investigational product
- Phase 1 – First in humans, assessing safety – Small numbers, sometimes done in healthy adults before the pediatric trial
- Phase 2 – Typically dose finding studies, safety still primary endpoint, will collect functional data
- Phase 3 – Pivotal trial to show efficacy in large number of patients – Likely to include placebo
- Phase 4 – Post-marketing or post-approval studies. Continued assessment of drug safety and efficacy

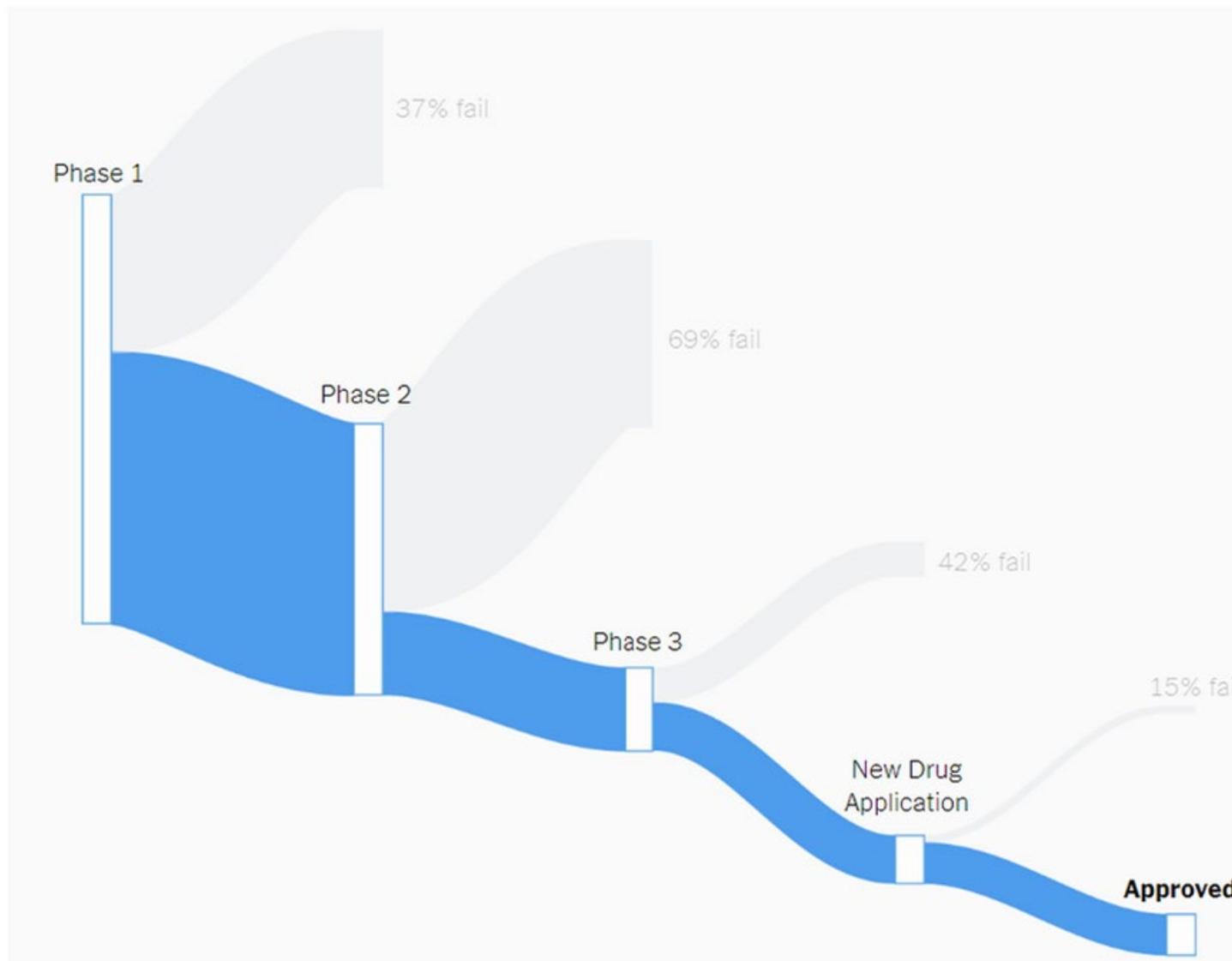


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Probability of success at each phase of research



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Frequently Used Clinical Trial Terminology

Inclusion/Exclusion Criteria

Set of requirements to be eligible for a trial.

Can include things like:

- Age
- Ambulation Status
- Steroid Use
- Genetic Variant

Placebo

- Inactive product given to patients to compare to investigational product
- Important for evaluating if the investigational product is truly providing benefit

Primary Outcome Measure

The test (functional/biomarker) to assess the effect of the investigational product

Adverse Event

- An unfavorable change in a patient's health. May or may not be related to the investigational product
- Serious Adverse Events are a type of Adverse Event that is considered life-threatening





Understanding your participation

Informed Consent

- The Informed Consent Form – is the document you will sign to agree to participate in a trial, it contains information on the trial: risks, benefits, timeline of the study.
 - You can receive a copy before signing to review with your family and physician.
 - During the consent process you will learn about the trial and it is your opportunity to ask questions of the research team.
- These can be long documents with a lot of information so come prepared:
 - Come with a list of questions you want answered, bring a notebook to write down additional questions while discussing, no question is too small!
 - Ask whatever questions you need to feel comfortable – from ‘what were the side-effects seen in the animal models?’, to ‘will you cover the cost of gas?’
- Assent – Your child may also be asked to sign a consent document (usually once 7+ years of age), investigators should be developing assent forms and explanations that help your child understand the trial.



Risks of Participating

- The investigational product may not work or be better than routine standard of care
- You may receive placebo rather than the investigational product
 - For most studies you will eventually receive the investigational product; the access and timeline should be available in the informed consent
- Investigational products may have unwanted or harmful side-effects
- May impact your ability to participate in other clinical trials
 - Exclusion criteria often restricts the use of multiple investigational products
 - May have to wait a certain amount of time before joining another trial – referred to as a ‘washout’ to ensure the product you had been taking isn’t impacting the results of the new trial



Burdens of Participating

- Costs associated with the study
 - Gas, hotels, parking, food
 - Child care for children not in the study
 - Some costs may be reimbursed by the study Sponsor; ask about reimbursement during your informed consent process.
- Schedule of visits
 - May lose time at work or have to use vacation/personal leave
 - Missing school or other activities with their peers
 - Visits could be multiple days, weekly visits, monthly; schedule should be outlined in consent document.
 - A particular trial may not have a site that is local to your area



Benefits of Participating

- Access to the investigational product
 - Products could provide benefit and this is an opportunity to have access prior to approval
 - Many studies offer Open Label Extensions – providing you an opportunity to continue having access to the product after the initial trial is completed
- Providing benefit to the community
 - Investigational products can't be approved without appropriate testing and that requires willing participants
 - The more individuals willing to participate, the faster a trial can gather data to see if the products are providing benefit or not
- Access to medical experts and standard of care
 - Visits may occur outside of the typical 6 month visit allowing for more interaction with care providers
 - May visit with other expert care providers outside of your traditional care team

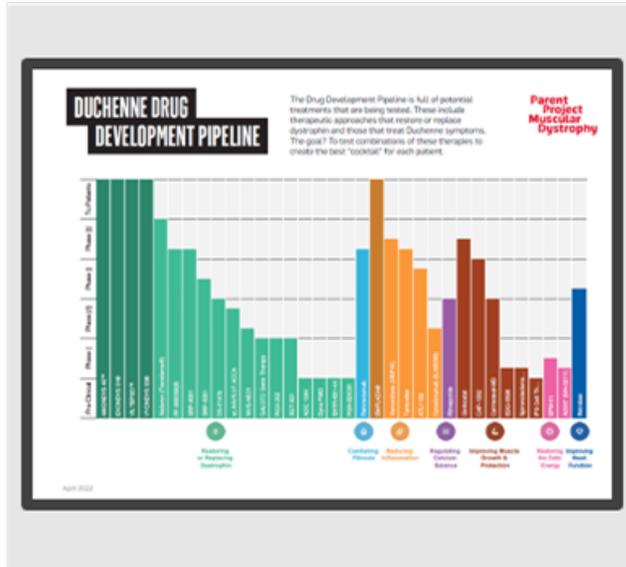


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Resources for finding trials

Resources for Finding Trials



PPMD INTERACTIVE DRUG PIPELINE

Details the different investigational and approved products in family-friendly language

parentprojectmd.org/pipeline

CLINICAL TRIALS ACTIVELY RECRUITING

Learn about actively recruiting clinical trials and studies you or your child may qualify for. parentprojectmd.org/exploretrials

Study ID	Study Name	Industry Sponsor	Age Range	Availability	Recruitment Status	Study Type	Study Phase
1	RESTORING MYOGENESIS	Progenics	18-30	Open	Recruiting	Phase I	Phase I
2	RESTORING MYOGENESIS	Progenics	18-30	Open	Recruiting	Phase I	Phase I
3	RESTORING MYOGENESIS	Progenics	18-30	Open	Recruiting	Phase I	Phase I
4	RESTORING MYOGENESIS	Progenics	18-30	Open	Recruiting	Phase I	Phase I
5	RESTORING MYOGENESIS	Progenics	18-30	Open	Recruiting	Phase I	Phase I
6	RESTORING MYOGENESIS	Progenics	18-30	Open	Recruiting	Phase I	Phase I
7	RESTORING MYOGENESIS	Progenics	18-30	Open	Recruiting	Phase I	Phase I
8	RESTORING MYOGENESIS	Progenics	18-30	Open	Recruiting	Phase I	Phase I
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10	RESTORING MYOGENESIS	Progenics	18-30	Open	Recruiting	Phase I	Phase I
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EXPLORING APPROVED & POTENTIAL THERAPIES
Duchenne research continues to progress, with multiple therapies in clinical trials. Stay up-to-date on the latest progress of drug development in Duchenne. parentprojectmd.org/pipeline

PPMD'S INVESTMENT IN INNOVATION
In order to End Duchenne, we must understand Duchenne. PPMD supports numerous scientists and clinicians pursuing important questions to break us closer to a cure. parentprojectmd.org/researchstrategy

PPMD TRIAL FINDER TOOL

Online search tool to help identify trials that you or your child qualify for

parentprojectmd.org/exploretrials



PPMD DUCHENNE REGISTRY

Once registered you will receive recruitment updates for clinical trials that you qualify for!

DuchenneRegistry.org



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PPMD's Impact on Research and Drug Development



\$50 million+

invested by PPMD into
Duchenne research &
therapy to date



*Over 260
research grants*

awarded, supporting
nearly every major
therapeutic approach



~35 clinical trials

in Duchenne at any
given time



*5 U.S.
drug approvals*

with additional
promising
therapies in
development



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Therapeutic Strategies in Duchenne



Restoring or
Replacing
Dystrophin



Combating
Fibrosis



Regulating
Calcium
Balance



Reducing
Inflammation



Improving
Muscle Growth
& Protection



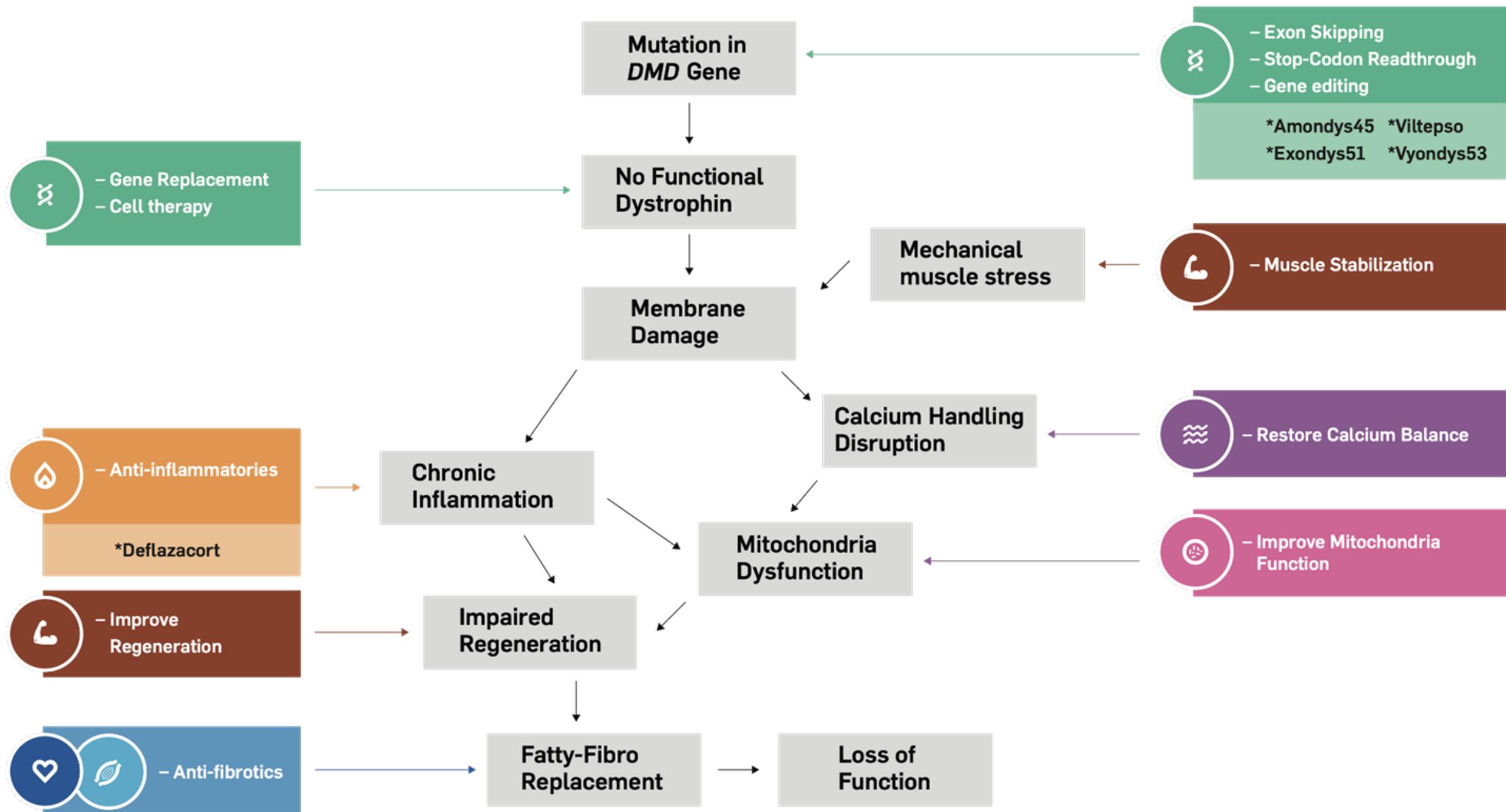
Restoring
Cellular Energy



Improving
Heart Function



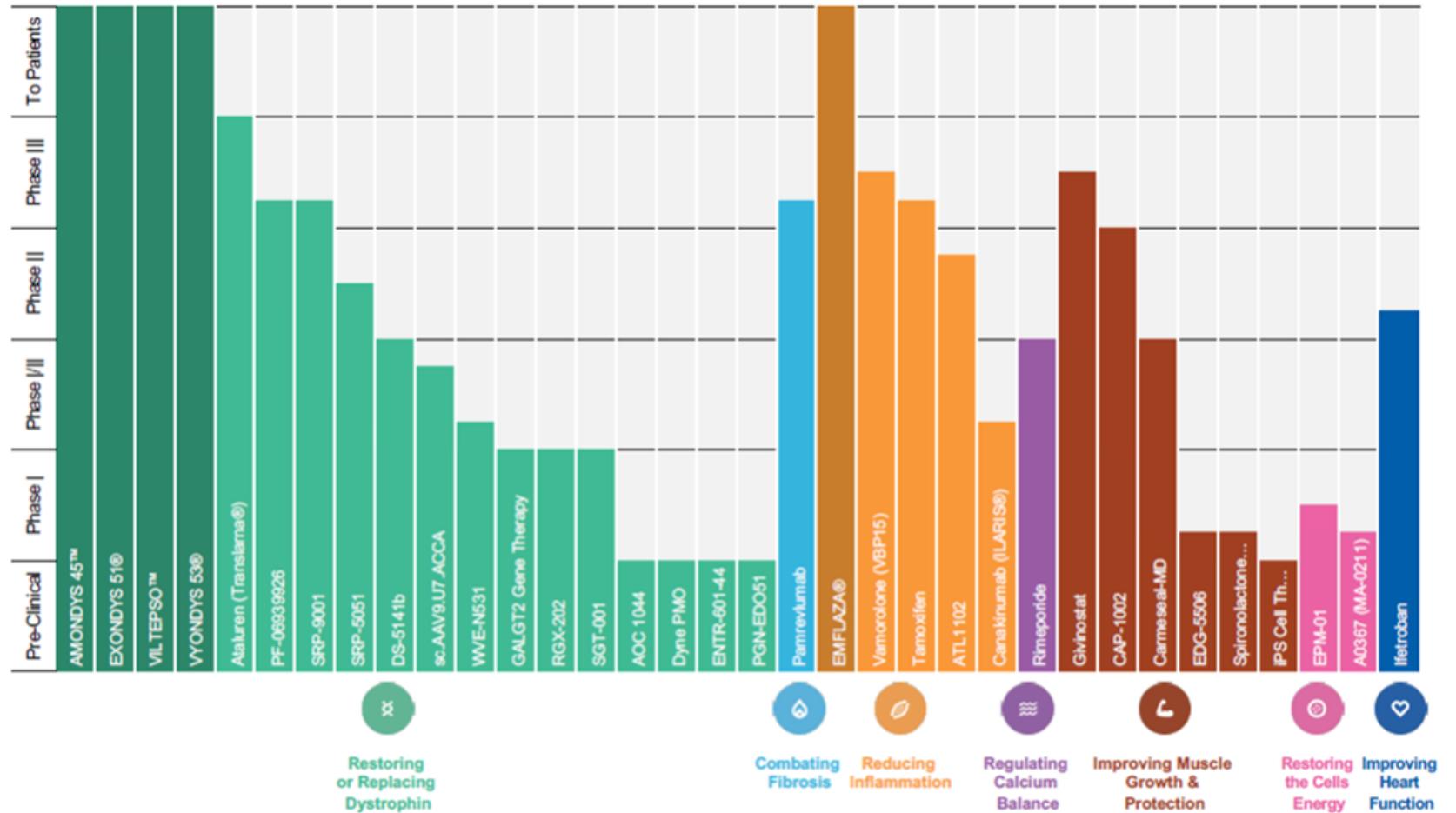
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DUCHENNE DRUG DEVELOPMENT PIPELINE

The Drug Development Pipeline is full of potential treatments that are being tested.

These include therapeutic approaches that restore or replace dystrophin and those that treat Duchenne symptoms (such as those that protect muscles by reducing fibrosis and inflammation). The goal? To test combinations of these therapies to create the best "cocktail" for each patient.





CLINICAL TRIALS ACTIVELY RECRUITING

RESTORING OR REPLACING DYSTROPHIN

Study Drug	Study Name	Industry/ Institution	Age (years)	Ambulation	Mutation Specific	Steroid Use	ClinicalTrials.gov ID
Ataluren	Ataluren in Participants From >6 Months to <2 Years of Age	PTC Therapeutics	0.5-2	Ether	Yes - Non-specific mutation	No specific requirement	NCT03336826
Eteplirsen	MISSION	Sarepta Therapeutics	4-13	Ambulatory	Yes - Amenable to exon 51 skipping	Currently using	NCT03442430
SGT-001	IGNITE DMD	Solid Biosciences	4-17	Ether	No	Currently using	NCT03368742
SRP-5051	MOMENTUM	Sarepta Therapeutics	4-9	Ether	Yes - Amenable to exon 51 skipping	No specific requirement	NCT03407066
SRP-9001	EMBARK	Sarepta Therapeutics	4-7	Ambulatory	Yes	Currently using	NCT03066221
Viltolarsen	Galactic53	NS Pharma	8+	Ether	Yes - Amenable to exon 53 skipping	Currently using	NCT04655280
Viltolarsen	RACER53	NS Pharma	4-7	Ambulatory	Yes - Amenable to exon 53 skipping	Currently using	NCT03080188
Pamrevlumab	LENTALOS TWO	FibroGen, Inc.	6-11	Ambulatory	No	Currently using	NCT03632410
Canakinumab (Ilaris)	Pilot Trial of Canakinumab (Ilaris)	Children's Research Institute	2-5	Ambulatory	No	Currently using	NCT03368804
Tadalafil	Tadalafil as Adjuvant Therapy for DMD	University of Florida	7-18	Ambulatory	No	No specific requirement	NCT05125775
ASP0367	ASP0367 in Participants with Duchenne	Astellas Pharma	6- 6	Ether	No	Currently using	NCT04184882
Iletroben	FIGHTDMD	Cumberland Pharmaceuticals	7+	Ether	No	No specific requirement	NCT03340375

COMBATING FIBROSIS

REDUCING INFLAMMATION

IMPROVING MUSCLE GROWTH & PROTECTION

RESTORING THE CELLS ENERGY

IMPROVING HEART FUNCTION

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Visit parentprojectmd.org/exploretrials or scan the QR code.

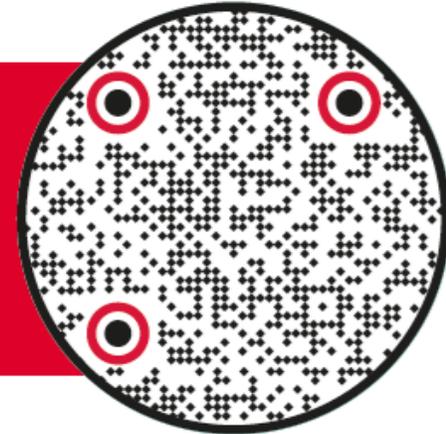


More information on trials can be found on the Clinical Trials Handout



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