



RESEARCH & CLINICAL TRIALS

Parent JOIN THE FIGHT.
END DUCHENNE.
Project
Muscular
Dystrophy

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OVERVIEW

01.

Introduction to clinical trials

02.

Considerations for participating in clinical trials

03.

Resources for finding clinical trials

04.

Current drug development landscape



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.
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PHASES OF CLINICAL TRIALS

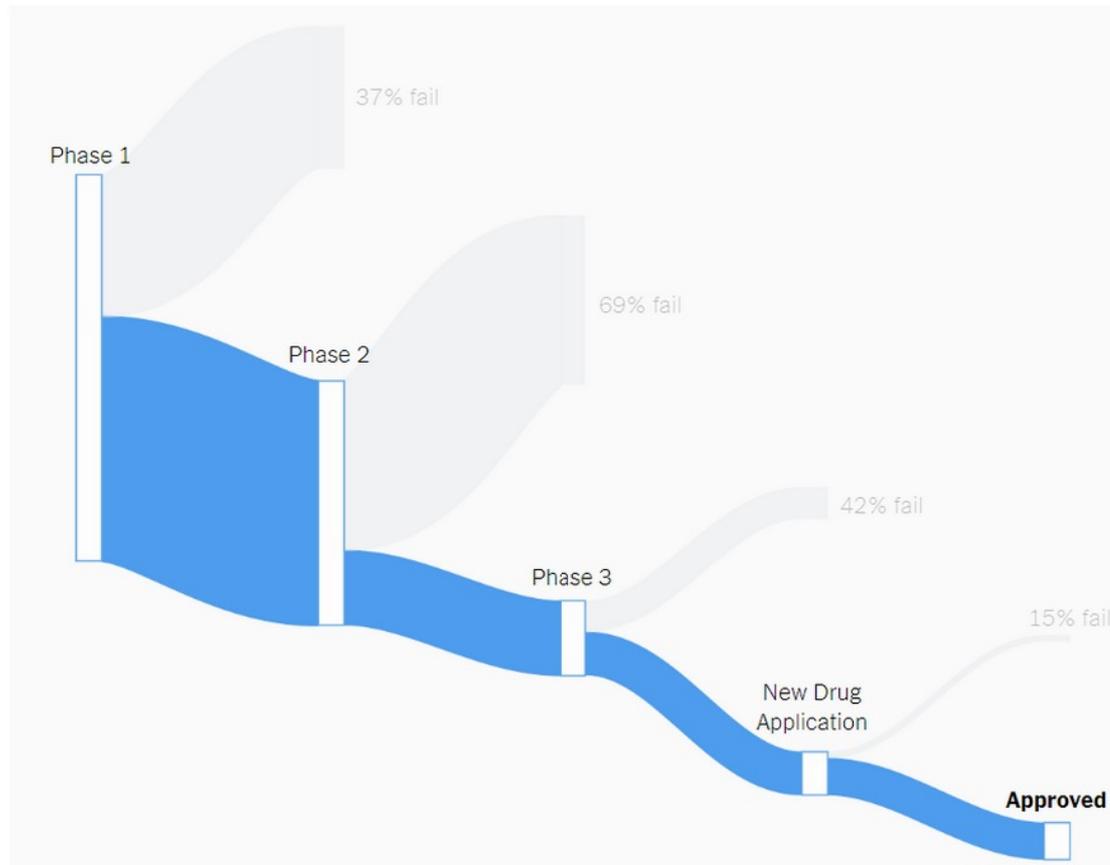
- **Preclinical** – Lab and animal studies that help us understand potential of an investigational product
- **Phase 1** – First in humans, assessing safety – Small numbers, sometimes done in healthy adults before the pediatric trial
- **Phase 2** – Typically dose finding studies, safety still primary 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



Stages of Clinical Trials

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PROBABILITY OF SUCCESS AT EACH PHASE OF RESEARCH



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
- Mutation

Placebo

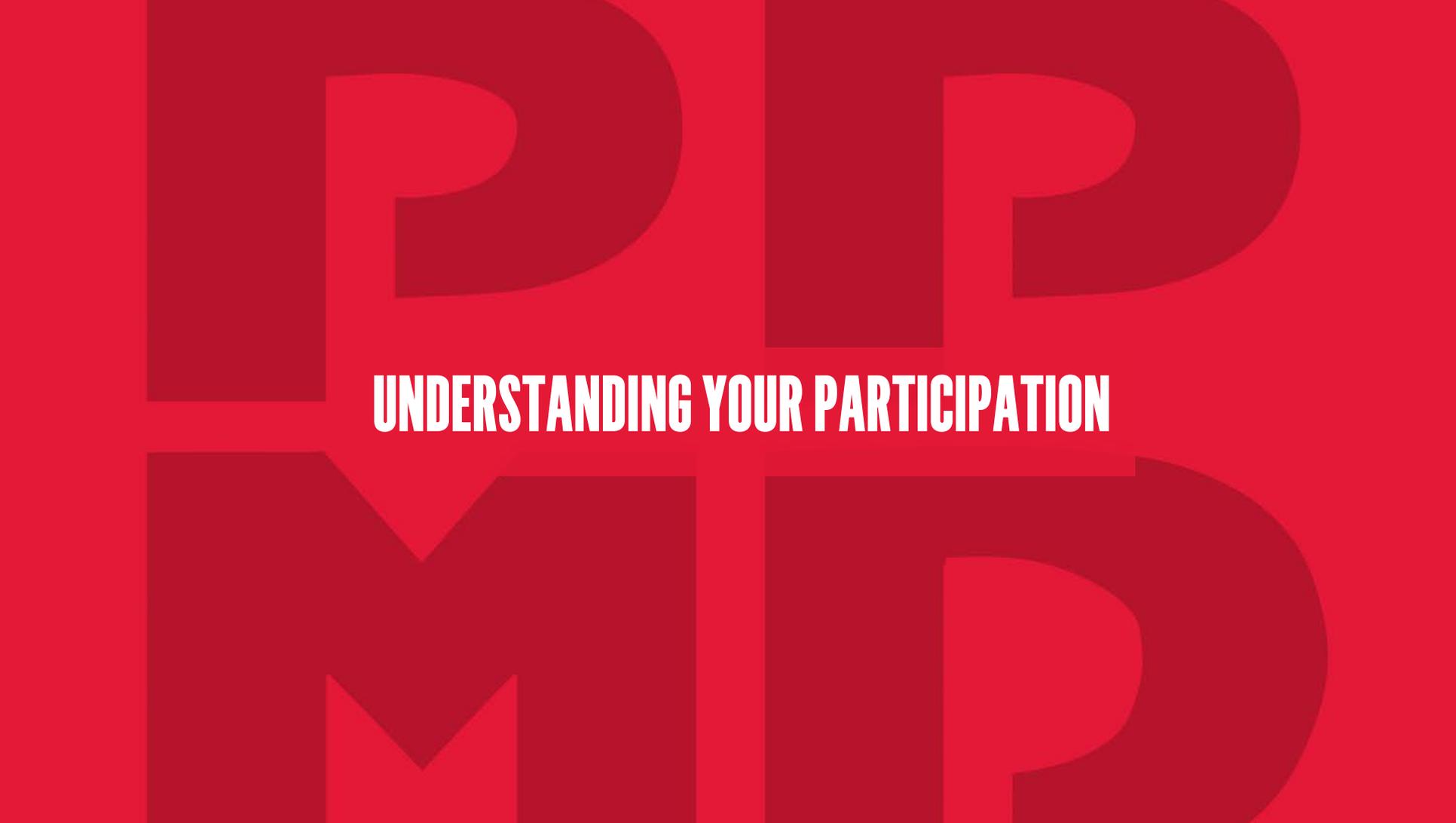
- Inactive product given to patients to compare to investigational product
- Important for evaluating if the investigational product is truly providing benefit

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



RESOURCES FOR FINDING TRIALS

IMPACT ON RESEARCH AND DRUG DEVELOPMENT



\$50 million+

invested by PPMD
into Duchenne
research & therapy
development to date



**Over 260
research grants**

awarded, supporting
nearly every major
therapeutic approach



~ 35 clinical trials

in Duchenne
at any given time



5 US drug approvals

with additional
promising therapies
in development

THERAPEUTIC STRATEGIES IN DUCHENNE



Restoring or
Replacing
Dystrophin



Combating
Fibrosis



Regulating
Calcium
Balance



Reducing
Inflammation



Improving
Muscle Growth
& Protection

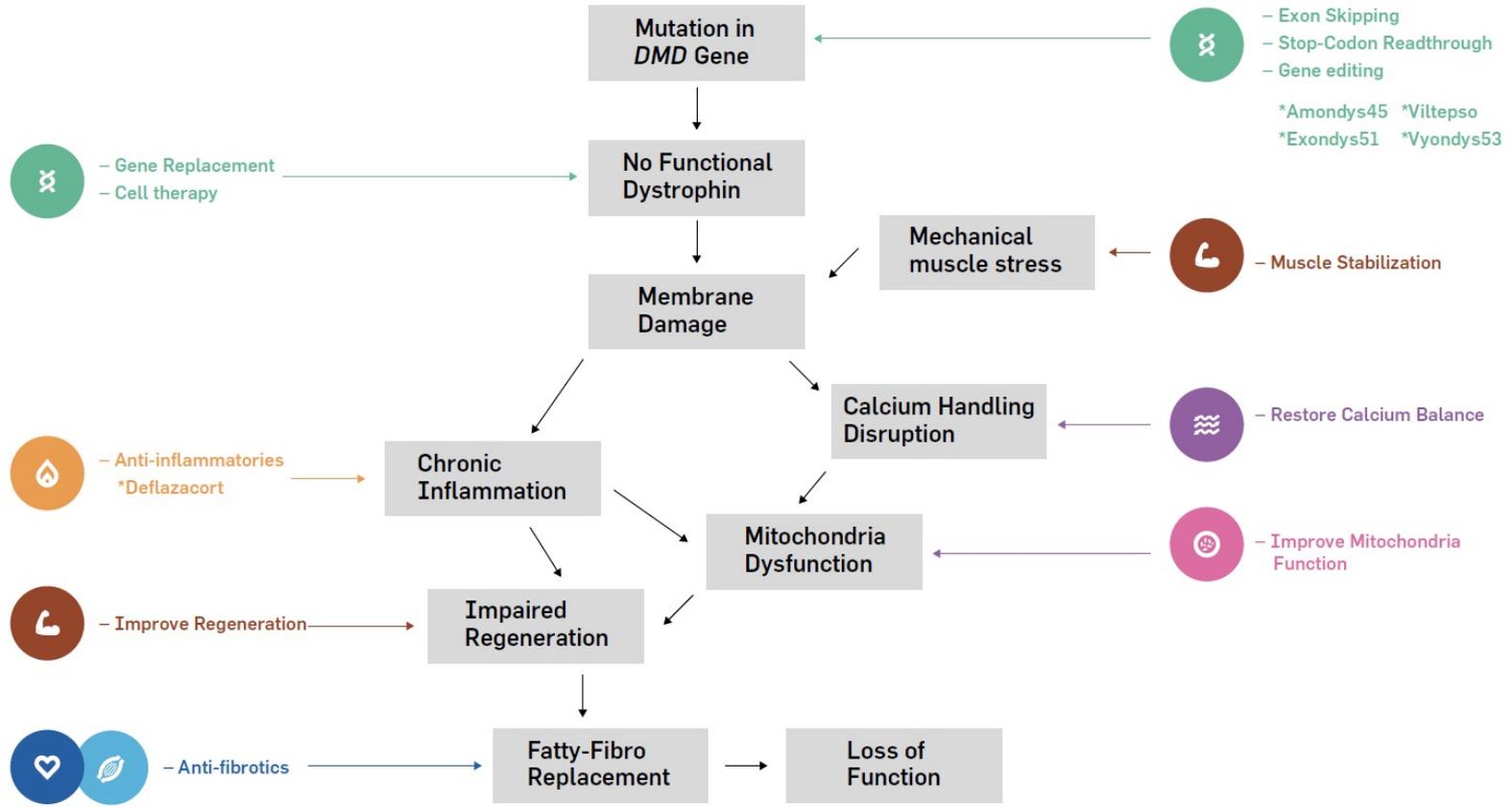


Restoring
Cellular Energy



Improving
Heart Function

THERAPEUTIC STRATEGIES IN DUCHENNE



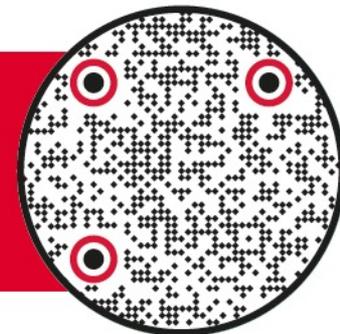
MORE INFORMATION ON TRIALS

RESEARCH | Parent Project
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Dystrophy JOIN THE FIGHT. END DUCHENNE.



CLINICAL TRIALS ACTIVELY RECRUITING

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



EXPLORE 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 answering important questions to drive us closer to a cure.

parentprojectmd.org/researchstrategy