

Parent Project Muscular Dystrophy

LEADING THE FIGHT TO END DUCHENNE

Participation Matters

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MYOTONIC
DYSTROPHY
FOUNDATION

Why are we here?

A **drug development ecosystem** is a community of stakeholders (universities, companies, patient organizations, patients, government organizations) in conjunction with the nonliving components of their environment (things like regulations, economic factors, reimbursement potential), interacting as a system. These components are regarded as linked together through **clinical research** cycles and **funding** flows

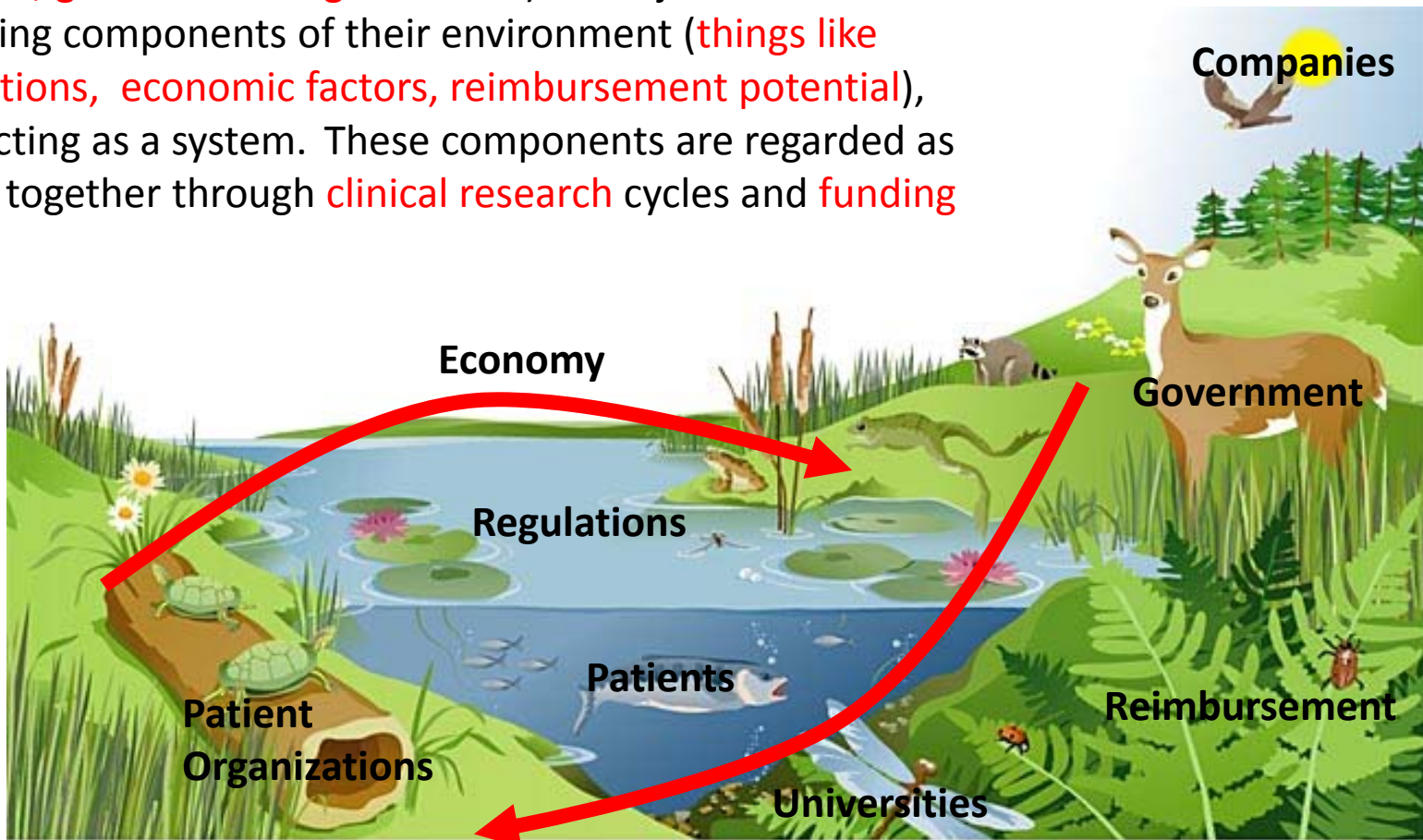


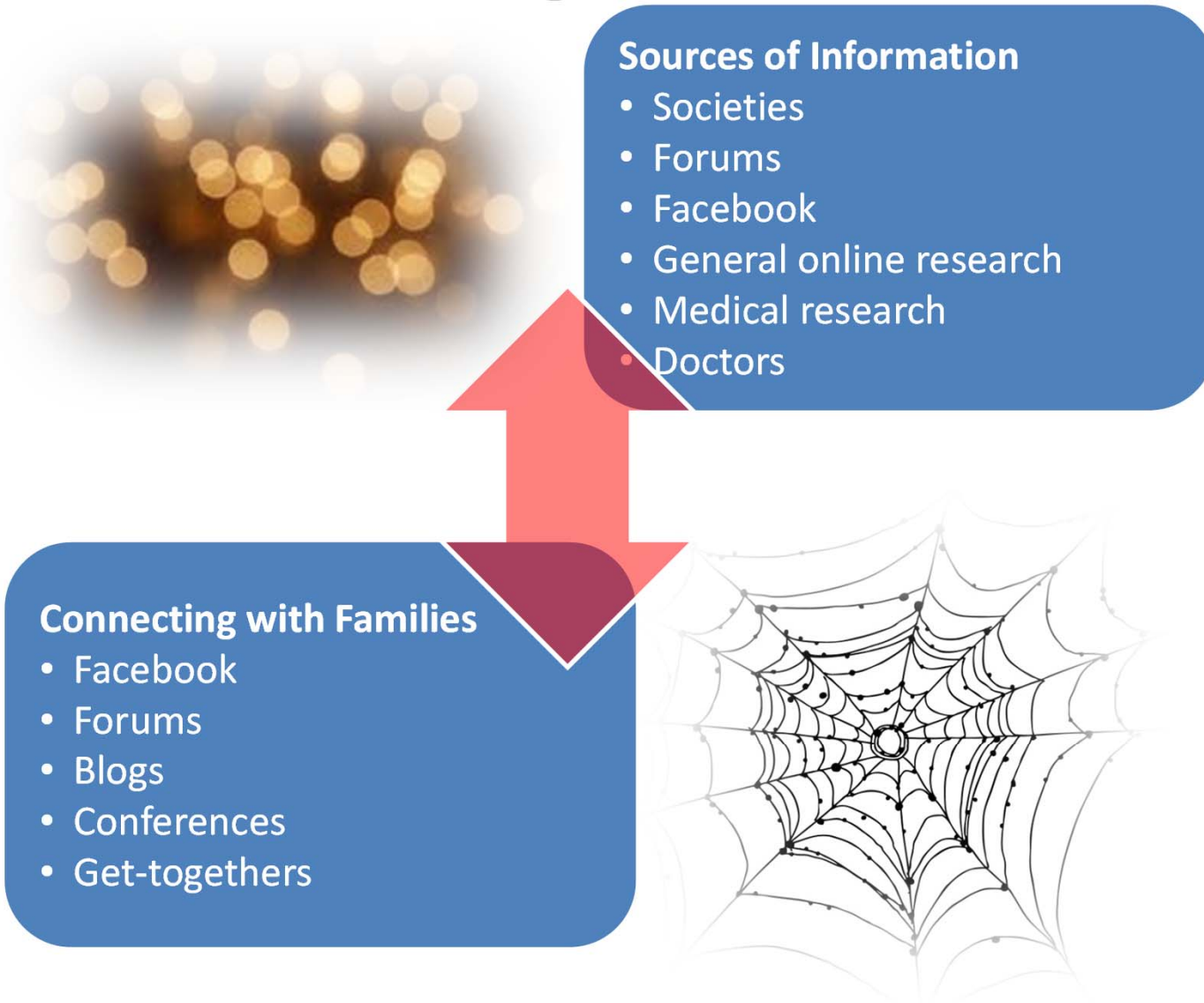
illustration by Jeff Grader / property of Delta Education



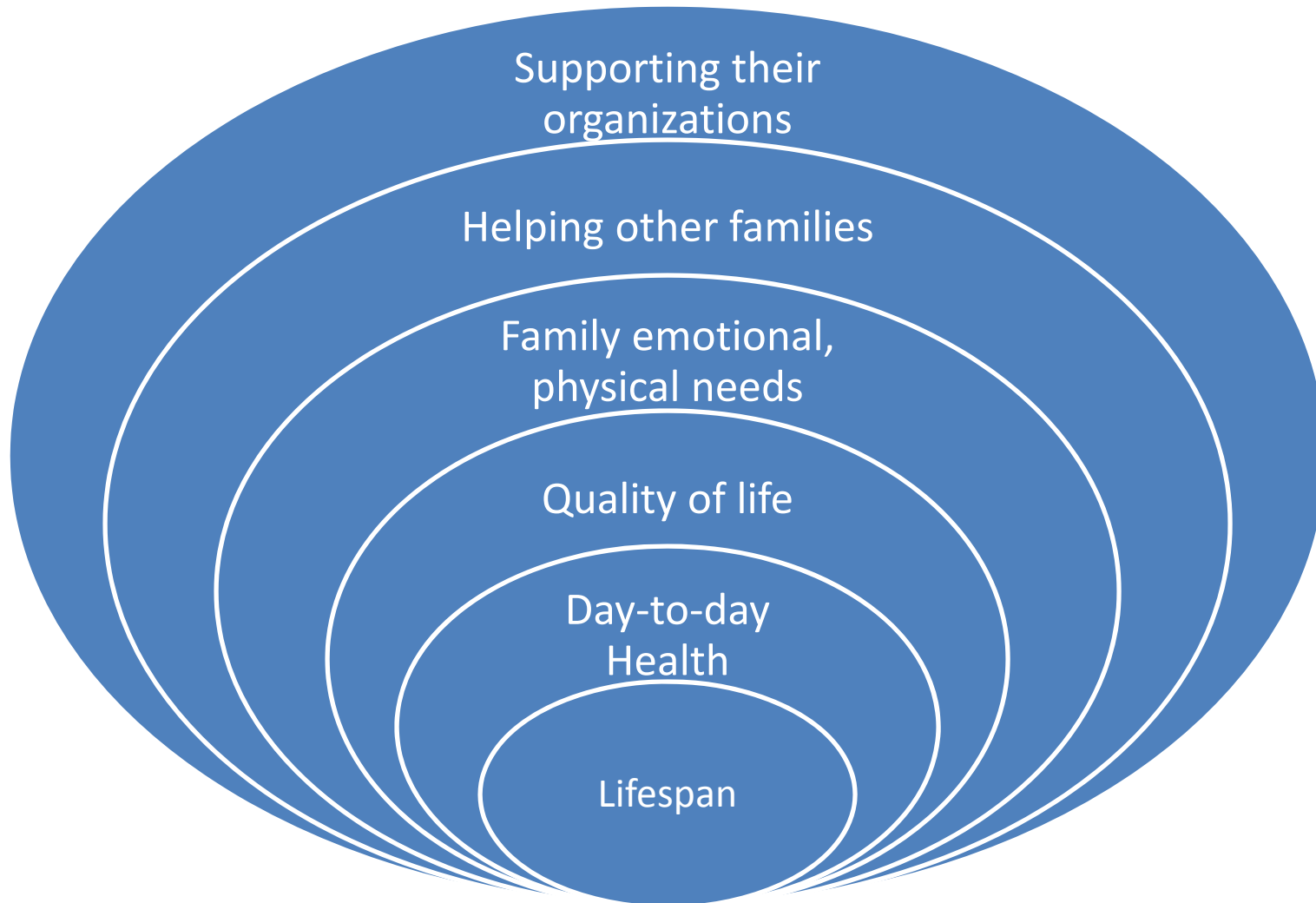
Everything important begins here



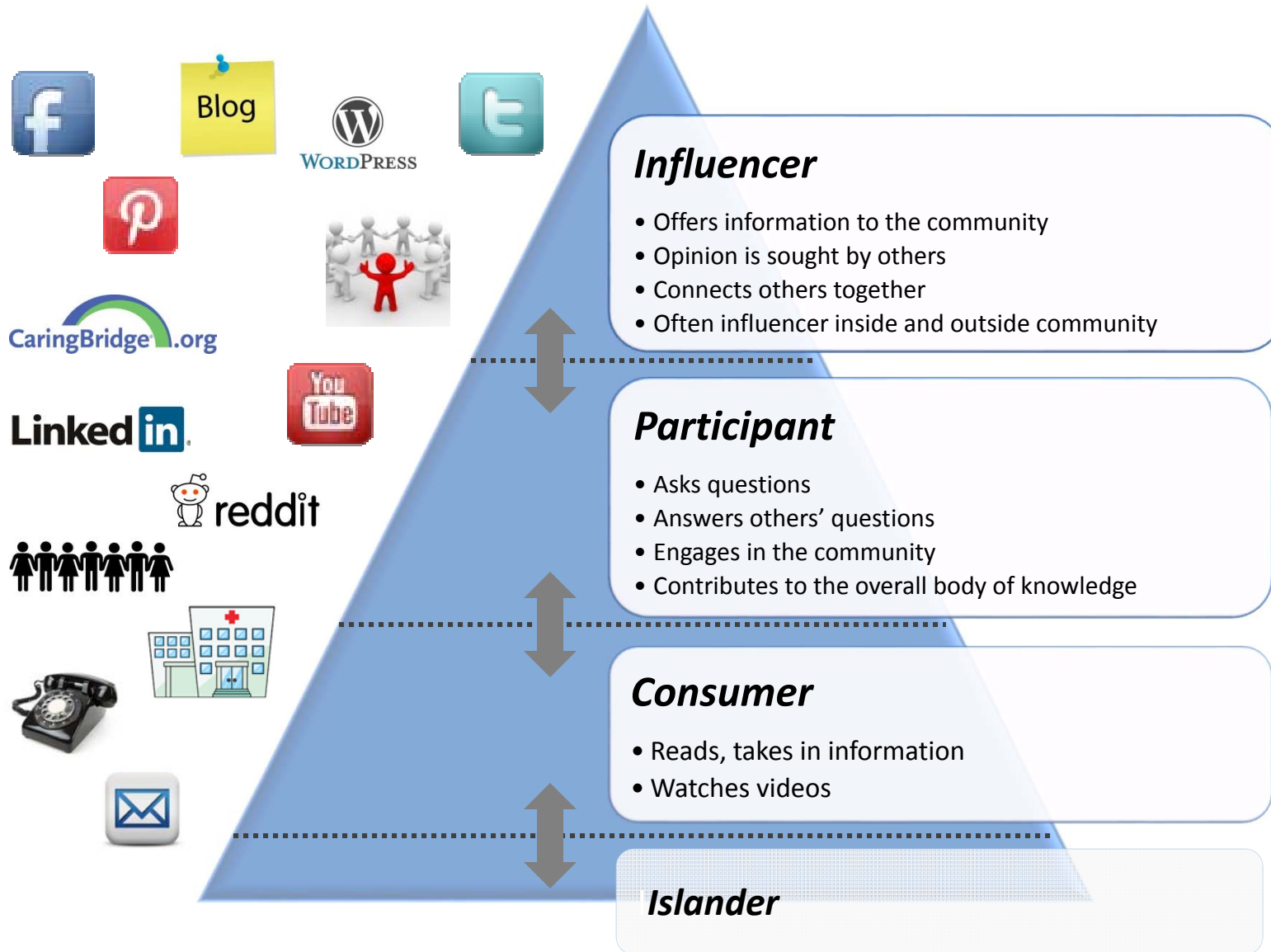
Connecting to Resources



Motivating Factors

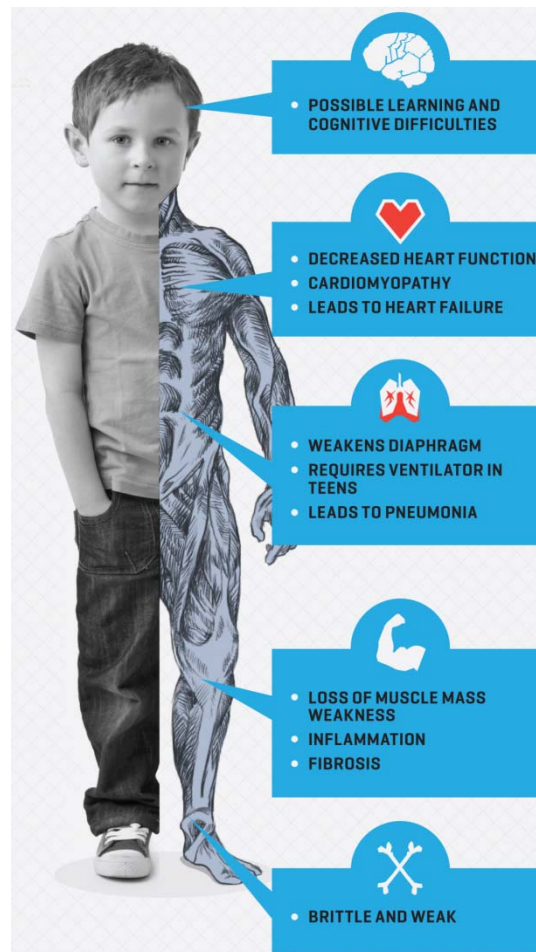


Role Locus



Similarities and Challenges

- RARE
- Genetic
- Multiple types
- Variable within affected family members
- Progressive
- Multi-system
- Complex care required
- Debilitating
- Family disease



- Rigorous Natural History
- Clinical Variability
- What to measure and how to measure it

What Is A Clinical Study?

- A study - A scientific procedure (experiment) undertaken to make a discovery, test a hypothesis, or demonstrate a known fact.
- Clinical – research in human volunteers (*sometimes called subjects, participants, patients*)
- Protocol – a highly specific written study plan
- Purpose: intend to add to medical knowledge.
- Types of clinical studies:
 - Clinical trials (interventional study, participants receive specific interventions (drug, device, procedure, behavioral modification) according to a study protocol.
 - Observational studies – health outcomes assessed according to a plan.

Adapted from <http://clinicaltrials.gov/ct2/info/understand#WhatIs>

You-The Participant (the rules)

- Clinical research cannot take part without participants
- Each participant makes a critical and necessary contribution to the acquisition of medical knowledge by participation in clinical trials
- These contributions are greatly appreciated – each bit of additional knowledge contributes to better understanding
- All trials and outcomes are valuable but different
 - Observational trials –refine previous knowledge, lead to improved study design over time
 - “Negative” trial - hypothesis not supported, for example, a drug does not work. This is disappointing but gives direction.
 - “Positive” trial – hypothesis supported, example, a drug works.

What Should The “Participant” Know?

- Participation is always completely voluntary
 - Informed consent is a process, not a piece of paper
- Standard treatment of DM1/DM2 will continue whether or not someone chooses to participate in a clinical trial
- A clinical trial may offer an experimental therapy
 - The experimental therapy may not make DM better
 - The experimental therapy may make DM worse
 - Not every participant in a trial may receive the same dose of experimental therapy
 - Not every participant in a trial may receive the experimental therapy (placebo group)

How Do You Find Out About Clinical Trials?

- Clinical trials are highly visible
- Sources include advocacy groups web sites, blogs, Facebook pages
- All interventional trials in the U.S.A. must list the trial at:

www.clinicaltrials.gov

Trial sites (locations) are listed

- Observational trials usually are listed on www.clinicaltrials.gov . Listing is optional.
- European trials can be found at <https://www.clinicaltrialsregister.eu/>

Trial record **1 of 104** for: Duchenne Muscular Dystrophy

[Previous Study](#) | [Return to List](#) | [Next Study](#)

Heart Disease in Duchenne Muscular Dystrophy and Becker Muscular Dystrophy (REVERSE-DBMD)

Participant recruitment is suspended.

(Suspended by the DSMB.)

Moser Research Institute at Kennedy Krieger, Inc.

University

provided by (Responsible Party):

Principal Investigator: Hugo W. Moser Research Institute at Kennedy Krieger, Inc.

ClinicalTrials.gov Identifier:

NCT01168908

First received: July 22, 2010

Last updated: February 4, 2013

Last verified: February 2013

[History of Changes](#)

View

Tabular View

No Study Results Posted

[Disclaimer](#)

[How to Read a Study Record](#)

How Does One Participate In A Clinical Trial?

- You or your family member's doctor finds out about a trial
 - You looks up the eligibility criteria for the trial
- Examine the Eligibility criteria of the trial to see if it is appropriate
 - Go to clinicaltrials.gov and find the listing inclusion/exclusion criteria
 - Inclusion criteria – things the participant must have to be in the trial
 - Exclusion criteria – things that will prevent the participant from being in the trial
 - Participation in previous clinical trial may be an exclusion
- Contact a trial site to determine if you are eligible
- Review the information about the trial (informed consent)
- Determine if you are able/want to participate in the trial
 - The extra time, doctor's visits, and additional tests are a major commitment
 - Only you can determine if participation in a trial is right for you

Eyes Wide Open



Clinical Trials: reality check

Expectations and Hope

- Impact of Advocacy
- Exposure to good ideas, targets, possibilities over time
- ? Unrealistic expectations –clinicians and patients

Trials impact the family



Burden of Participation

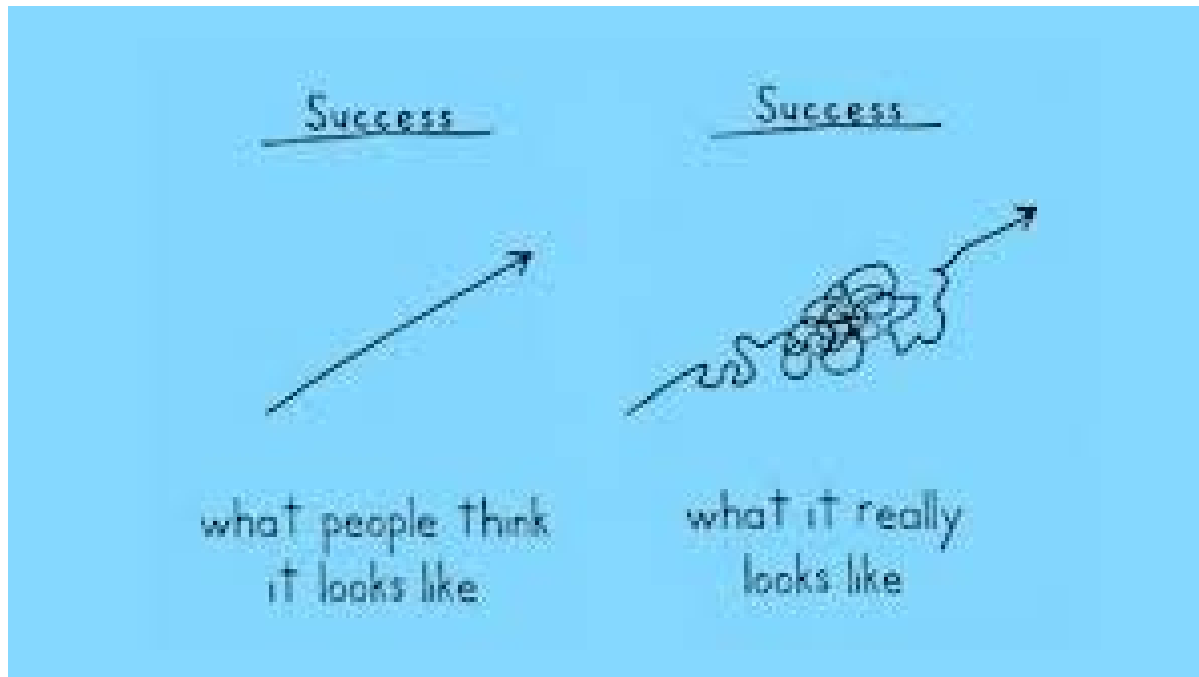
- Time requirements for patients and families
- Rigid and impractical processes
- Travel demands
- Dealing with Contract Research Organizations (CROs)

There's more...

- financial burden (reimbursement is often slow, carry several thousand dollars on credit card, time off work, child care...)
- physically and/or emotionally burdensome to the patient (varies from minimal to significant) and to the family

Clinical Trials

the only path to success



Challenges

- Placebo group as a threat to expected benefits of trial
- Progressive debilitating disease -progressing as a threat to hopes for better outcome;
- Lack of or insufficient communication from sponsor;
- "promises" for access that may not be met; patients (families) trying to evaluate if the individual is getting benefit coupled with not receiving study data; deciding whether to stay in a trial
- May take longer than a 48 week study to fully understand the full impact of a drug

Impact of social networks

- Potential CT are: social isolation within DM community as a result of participation (e.g. 'being chosen' to be included in the trial, or placebo status, or individual's perceived improvement or decline).
- Simultaneously, not being technically allowed to talk about the trial with other individuals or families
- . Managing and tempering hopes/expectations related to benefit and to logistics of CT.

Therapeutic Dose of HOPE



Recommendations/customer service

- Include patients/families in discussions around trial design (early and often)
- Communication plan
 - Timeline for communication
 - Individual results
 - Expanded access –yes/no?
- “warm line” – 24/7



*The goal is:
Customer Congruency: When what we promise and what the customer receives are thought to be the same.*

Small things make big difference in the quality of our lives



I didn't know I could still do this.

[Patrick M. Denger](#)
[16 hours ago](#) [iOS](#)