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

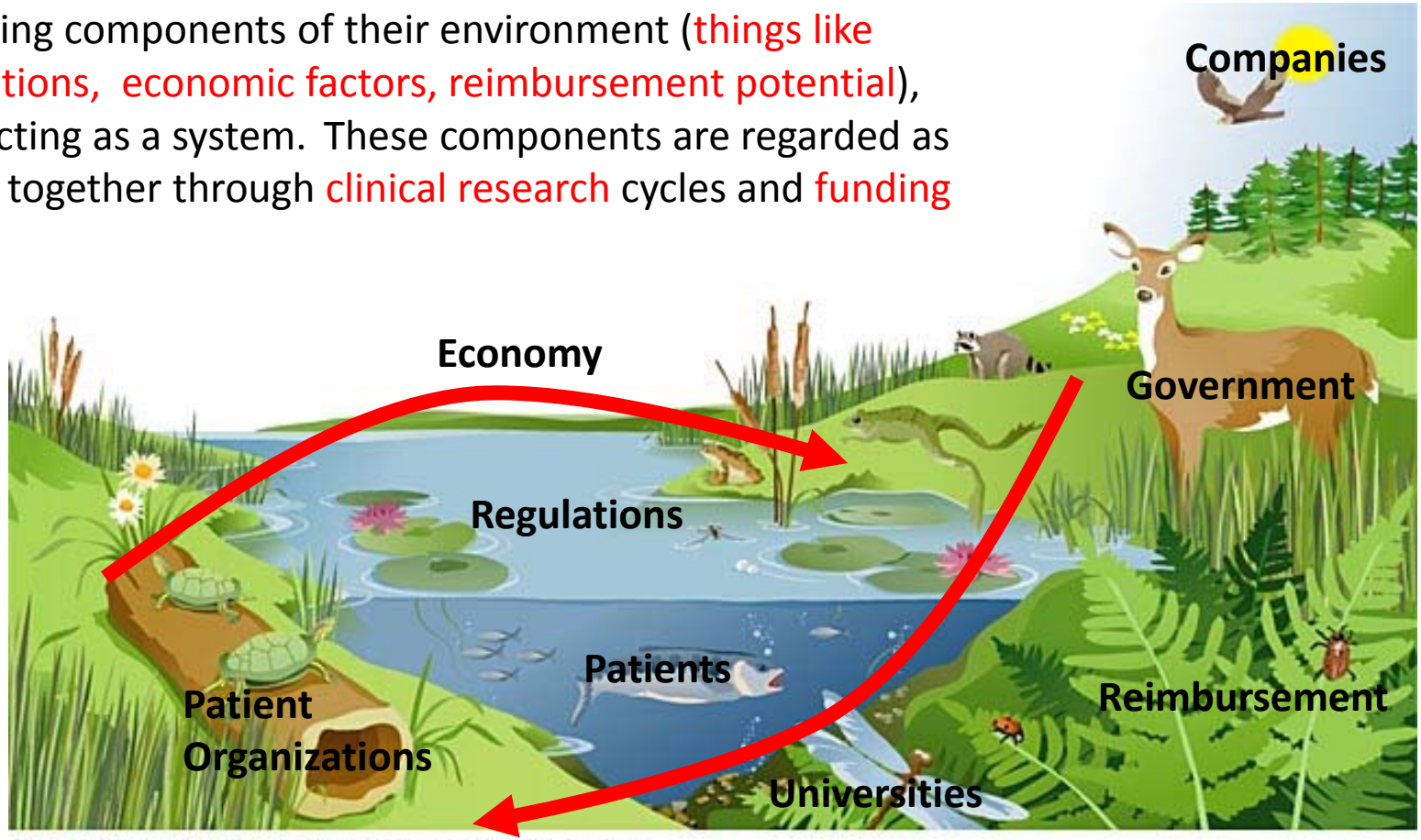


illustration by Jeff Grader / property of Delta Education

Everything important begins here

Connecting to Resources

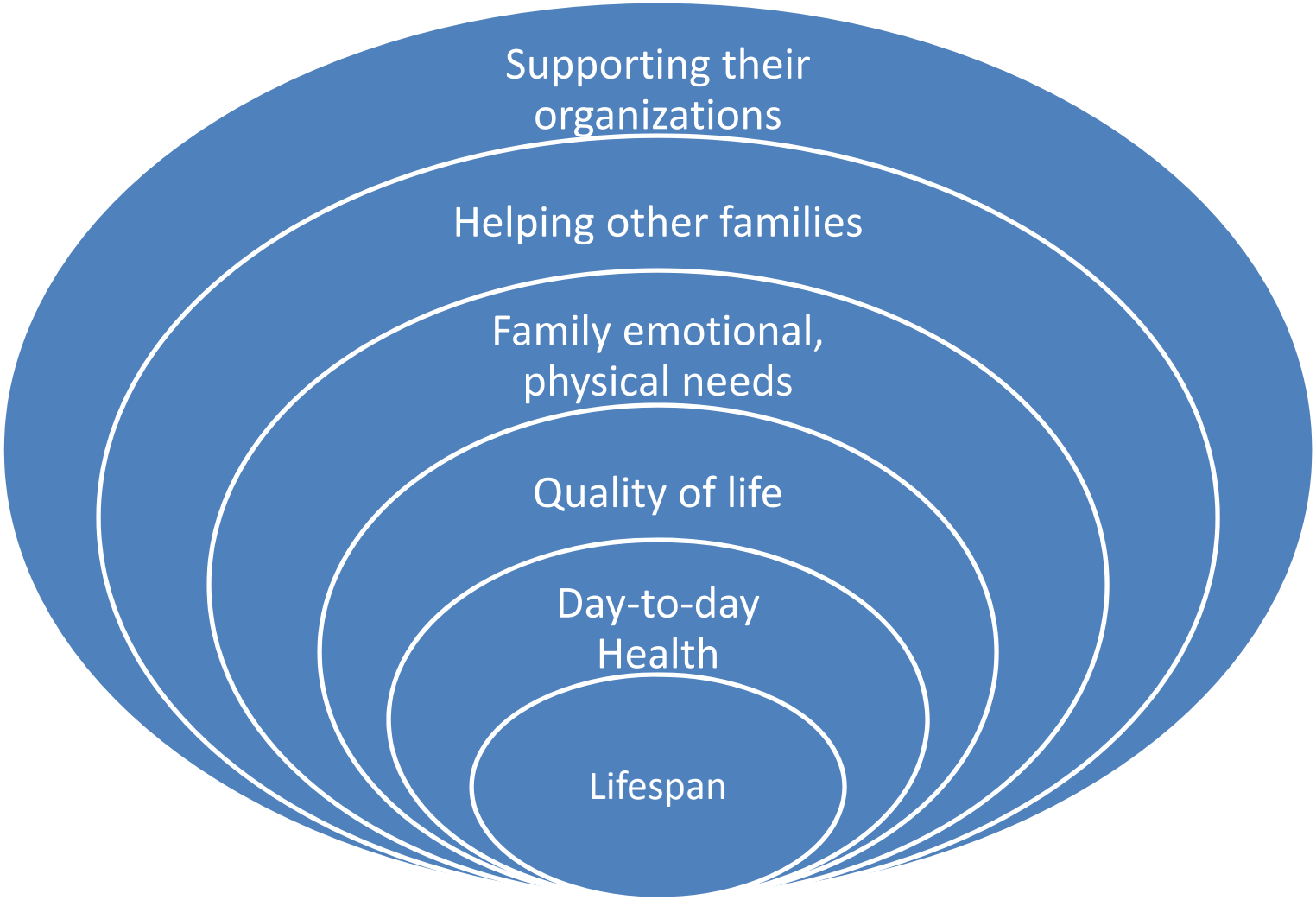
Sources of Information

- Societies
- Forums
- Facebook
- General online research
- Medical research
- Doctors

Connecting with Families

- Facebook
- Forums
- Blogs
- Conferences
- Get-togethers

Motivating Factors



Role Locus

Influencer

- Offers information to the community
- Opinion is sought by others
- Connects others together
- Often influencer inside and outside community

Participant

- Asks questions
- Answers others' questions
- Engages in the community
- Contributes to the overall body of knowledge

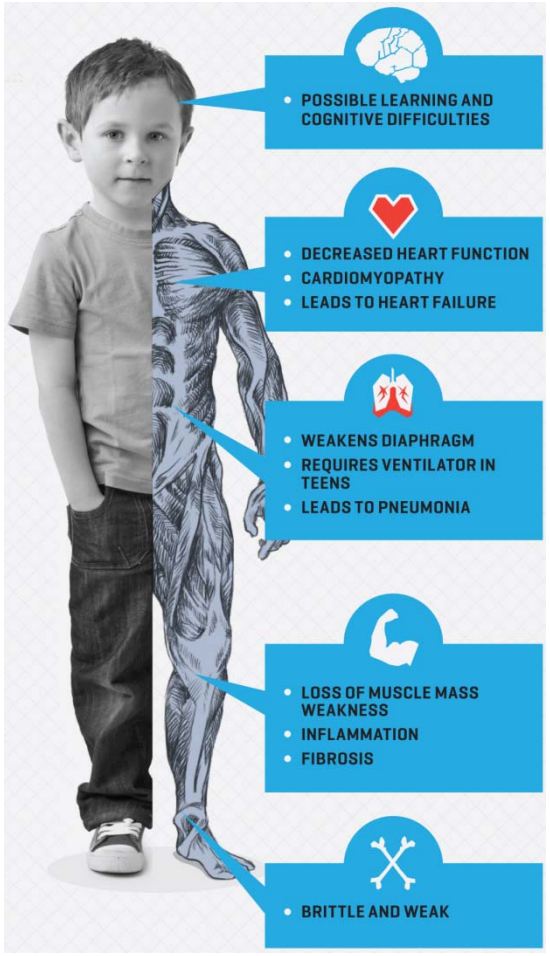
Consumer

- Reads, takes in information
- Watches videos

Islander

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:

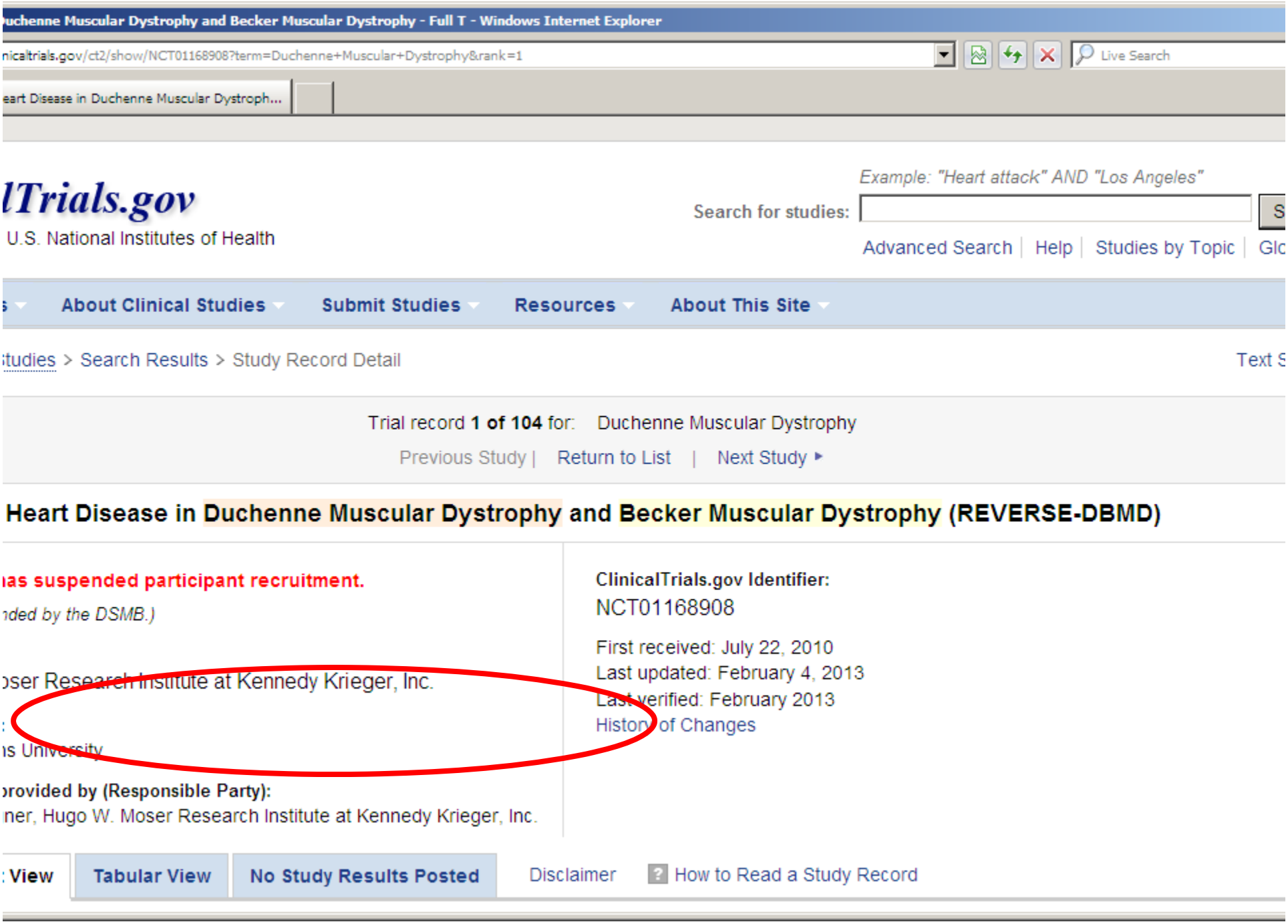
  www.clinicaltrials.gov

  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/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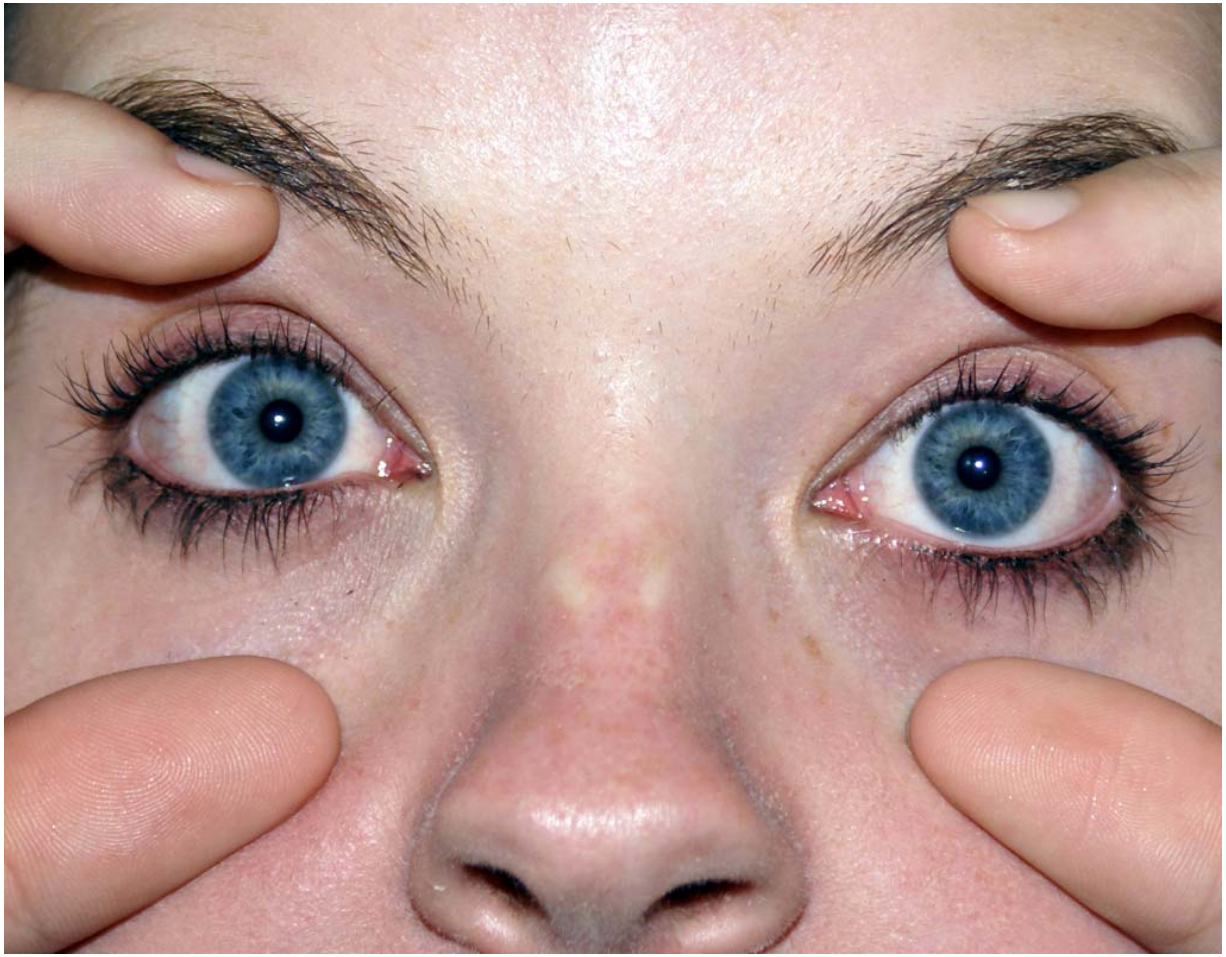
ner, Hugo W. Moser Research Institute at Kennedy Krieger, Inc.
ClinicalTrials.gov Identifier:
NCT01168908
First received: July 22, 2010
Last updated: February 4, 2013
Last verified: February 2013
History of Changes
View
Tabular View
No Study Results Posted
Disclaimer
How to Read a Study Record

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

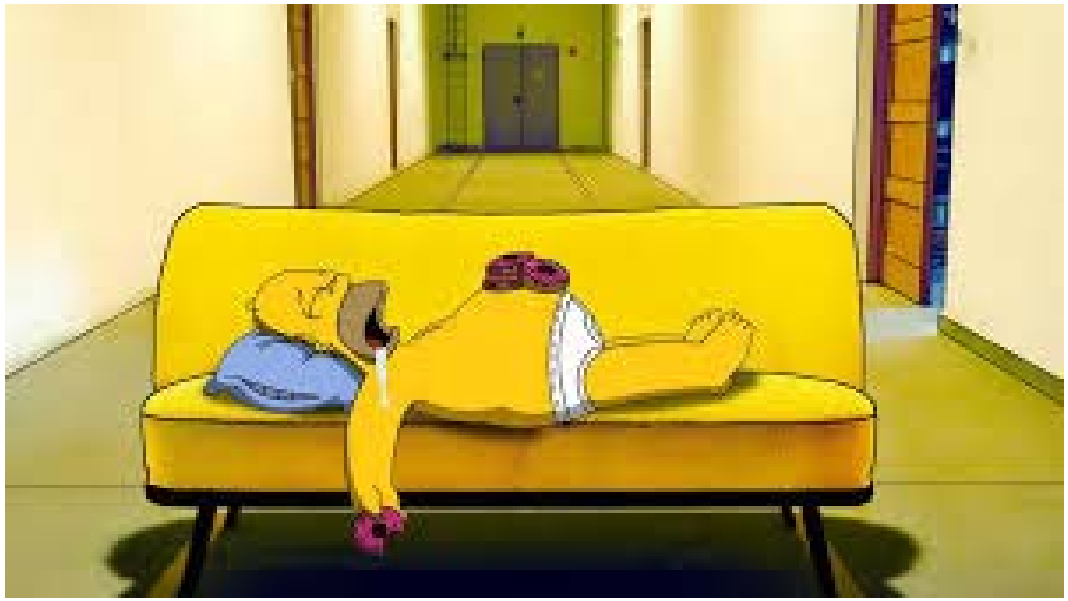


Clinical Trials: reality check

Expectations and Hope

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

Trials impact the family



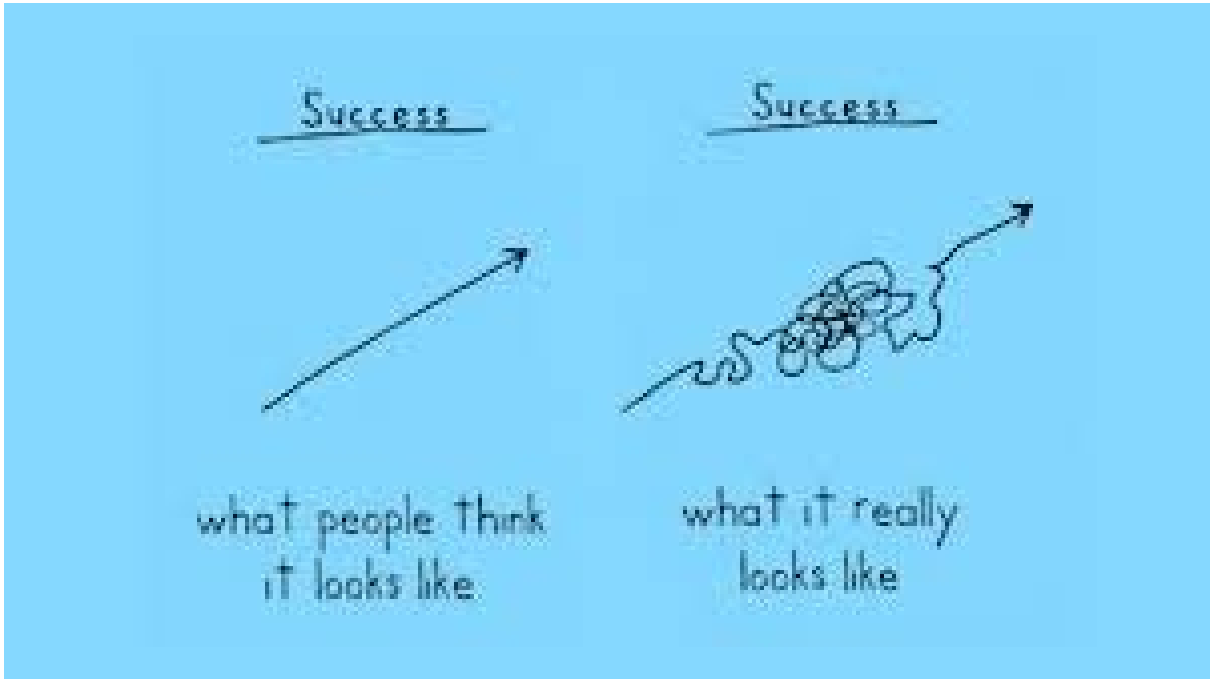
Burden of Participation

- 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

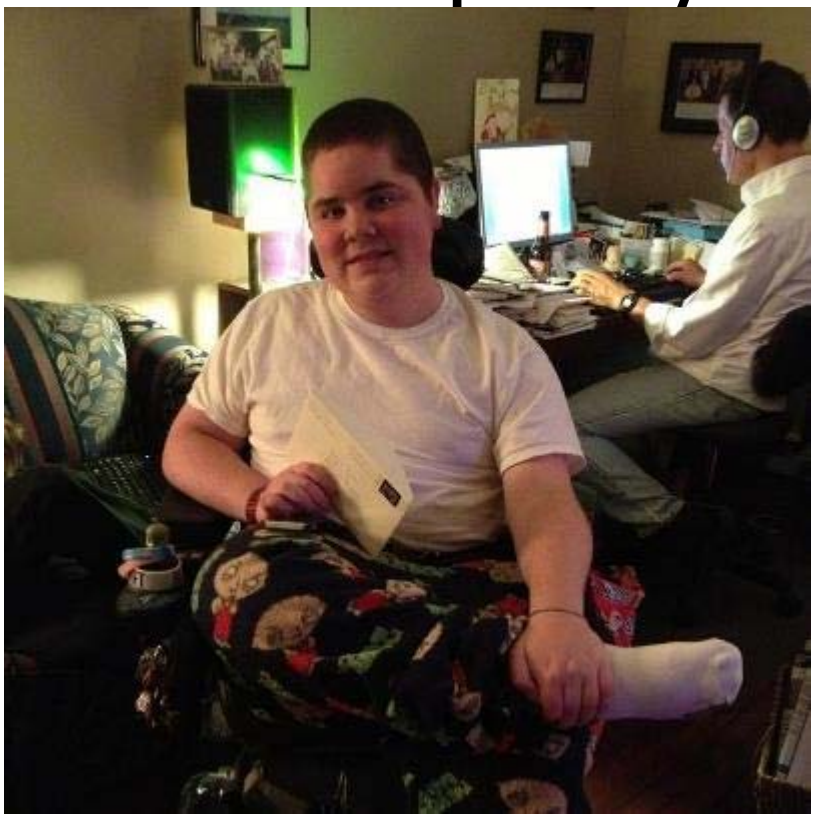
- Potential CT are: social isolation within DM community as a result of participation (e.g. 'being chosen' to be included in the trial, or placebo status, or individual's perceived improvement or decline).
- Simultaneously, not being technically allowed to talk about the trial with other individuals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

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