Participation Matters
Pat Furlong
An ecosystem is a community of living organisms (plants, animals and microbes) in conjunction with the nonliving components of their environment (things like air, water and mineral soil), interacting as a system. These components are regarded as linked together through nutrient cycles and energy flows.

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.

Why are we here?
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

- Lifespan
- Health
- Day-to-day
- Quality of life
- Family emotional, physical needs
- Helping other families
- Supporting their organizations
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/
Heart Disease in Duchenne Muscular Dystrophy and Becker Muscular Dystrophy (REVERSE-DBMD)

ClinicalTrials.gov Identifier:
NCT01168908

First received: July 22, 2010
Last updated: February 4, 2013
Last verified: February 2013

History of Changes

No Study Results Posted
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