Patient Involvement in the Drug Development Process

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Who am I?

  - Symptoms my whole life
- Long history of severe fistulizing Crohn’s disease
  - Over 10 years of steroid-dependence
- IBD has had a profound impact on my life.
- I want to help other patients avoid the struggles I’ve gone through.
- I am an IBD advocate.
INFLAMMATORY BOWEL DISEASE

Cultivating therapies for an unseen condition
“Bowel disease in the internet age”
The Main Focus of My Work

- My work is based on the idea that with good information, IBD patients can make better decisions about their health and take a more active role in their healthcare.

- Through my website and social media outlets, I strive to help patients:
  - Empower themselves with good information
    - Report on the world of IBD
    - Original articles on topics that matter to patients
  - Think critically about new information related to their health
  - Avoid falling prey to pseudoscience
  - Make decisions about their health based on accurate information and consideration with their medical team – not fear.

- ...Before I was more informed and experienced, I struggled a lot more
My Clinical Trial Experience


CONSENT BY SUBJECT FOR PARTICIPATION IN A RESEARCH PROTOCOL

Protocol Number: 12787B

Name of Subject: Dan Sheas

Medical History Number: 

STUDY TITLE: A Phase III multi-national, multi-centre, double-blind placebo-controlled parallel group, 26 week study to assess the safety and efficacy of the humanized anti-TNF PEG conjugate, CDP870 400 mg sc, (dosed at Weeks 0, 2, 4 then 4-weekly to Week 24), in the treatment of patients with active Crohn’s disease.
My Health At The Time

- Severe Crohn’s Disease
- Long history of perianal disease
  - Fistulae
  - Fissures
  - Abscesses
- Nearly 10 years of steroid-dependence
- I had been struggling for a long time
- I was more than willing to try new or “experimental” treatments
  - First pediatric patient at my IBD center to try infliximab (1998)
- I wasn’t afraid, but I don’t recall feeling hopeful.
The Outcome (For Me)

- My health worsened throughout the trial
- Eventually, it was revealed that I had been taking the placebo
- I was switched to CDP870
  - My health continued to worsen
  - I was taken off CDP870
The Issues...

- I traveled 75 miles each way – typically anywhere from 3-4.5 hours in the car for each visit.
  - No easy feat with perianal disease!
- Most of the people involved in running the trial were great, but some were not.
  - I was treated rudely by some of the staff.
- I was sometimes made to wait for hours before receiving my injections.
- I did not feel like I was receiving care – I was merely a “subject”.

Issues surrounding my participation in the trial were a big part of why I moved on to find care elsewhere (2005-2006).

- A decision I came to regret in many ways

Given the same outcome of the trial:

- If I had felt like I was being cared for, and felt like my participation was valued more, I likely wouldn’t have left to find care elsewhere.

My desperation was a negative factor!

I would handle the situation differently now (more experience, knowledge, confidence)

I was still more than willing to participate in clinical trials, try new, “experimental”, or off-label treatments
Patient involvement in drug development

- Patients want to be involved!
  - If you offer them a voice at any step along the way, there will be patients ready to offer their time, experience, and insights.

- Patients are being involved!
  - Initiatives in place or being developed by:
    - Pharmaceutical companies
    - NIH
    - FDA
    - CCFA
    - Etc
  - How much of it is happening in IBD?

- Patients need to be well-informed in order to be involved in a meaningful way.
- Patients need to participate in clinical trials for any of this to matter.
The Clinical Trials Transformation Initiative (CTTI) is a public-private partnership to develop and drive adoption of practices that will increase the quality and efficiency of clinical trials.

To what extent IBD patients are currently involved, I’m not sure.
Educate Patients on Involvement

- PCORI's Initiative to Support Patient Involvement in Research (INSPIRE)
  - “The objectives of this project are to establish training, provide tools, and create a network to help researchers and patients better connect and collaborate in patient-centered outcomes research (PCOR).”

- The final phase of INSPIRE included the development of a “web portal of tools and resources to support patient-researcher partnerships”.

- Excellent educational resources: Basic research concepts, basic clinical trials information, what kinds of roles patients can take on in research, what makes a partnership successful, etc.
Give Patient’s a Voice

FDA’s Patient-Focused Drug Development initiative aims to more systematically gather patients’ perspectives on their condition and available therapies to treat their condition.

- What are the most impactful symptoms you experience?
- What needs are not being met?
- Patient’s views on currently available therapies
- Etc.

Information obtained can be used to influence regulatory decision-making and future drug development.
Externally-led Patient-Focused Drug Development Meetings

- Official meetings planned (2013-2017) do not include IBD.
- Fortunately, the FDA “welcomes patient organizations to identify and organize patient-focused collaborations to generate public input on other disease areas, using the process established through Patient-Focused Drug Development as a model”.
- An externally-led meeting for IBD would:
  - Provide valuable information to all parties involved in drug development.
  - Give IBD patients a voice to influence future drug development and research.
- Well-designed, well-conducted meetings in the DC Metro area would help to encourage FDA involvement.
Unmet Needs in IBD

- Novel treatments for pain
  - Lack of safe options
  - Cannabis use is prevalent
- Scarring
- Dysmotility
- How to make biologics more durable?
- How to address fatigue more effectively?
  - 40-48% of IBD patients in remission (clinical remission?) still experience fatigue
IBD Fact #39:
fatigue makes it hard to finish anything

@dansharpibd
Understanding Patients’ Preferences

- Oral / SubQ / IV
- Citrate-free injectables?
- Dose frequency
- Biologics vs. small molecule drugs
Clinical Trial Enrollment is Crucial

- Enrollment is low!
- Lack of enrollment means:
  - Sponsors lose money
  - Patients miss out on opportunities to find treatments that work for them
  - Progress in medicine is slowed – fewer unique treatments available to patients
“Treatment X is so new! They have no idea how it works, or what it is doing to our bodies! We are their guinea pigs!”
There is a lack of awareness about clinical trials.

There is a fear of the unknown.

This can be changed with education and through direct experience.

Without enough information (or experience), patients may be afraid of clinical trials

- Concerns about their safety
- The feeling of being experimented on
- Working with medical professionals other than their physician
- Not being able to continue the treatment after trial
The majority of volunteers would participate in a clinical trial study again.

In a 2013 CISCRP survey of 5,701 volunteers, 88% said they would participate again!

Clinical trials, once experienced, no longer represent the potentially frightening unknown.

Source: CISCRP (https://www.ciscrp.org/our-programs/research-services/charts-and-statistics/)
Top Ways that People Report Finding out About Clinical Trials

<table>
<thead>
<tr>
<th>Method</th>
<th>Percent Mention</th>
</tr>
</thead>
<tbody>
<tr>
<td>Internet</td>
<td>46%</td>
</tr>
<tr>
<td>The Media (TV, Radio, Newspapers)</td>
<td>39%</td>
</tr>
<tr>
<td>eMail</td>
<td>32%</td>
</tr>
<tr>
<td>Research Center Ads</td>
<td>28%</td>
</tr>
<tr>
<td>My Physician or Nurse</td>
<td>23%</td>
</tr>
<tr>
<td>Mail</td>
<td>21%</td>
</tr>
<tr>
<td>Family/Friends</td>
<td>13%</td>
</tr>
</tbody>
</table>

Source: CISCRP, 2013; N=5,701 people worldwide

Source: CISCRP (https://www.ciscrp.org/our-programs/research-services/charts-and-statistics/)
Clinical Trial Recruitment: What Not To Do

- There have been cases of companies taking quotes and pictures from IBD advocates (without authorization), then posting it on their sites in a way that makes it seem as though a given advocate is involved with them or “endorses” them somehow.

- One such company had a poster at DDW on their use of social media to enroll clinical trial participants.
  - The abstract stated that they used social media to “educate and engage patients leading to participation in this study”.
  - There was no mention of their use of IBD advocates’ work to generate traffic to their social media pages, or to give the impression of the various advocates’ involvement.
  - Their social media timelines contained very little educational material relating to their trial.
They took a quote from Sara Ringer (Inflamed and Untamed) out of context and made it seem as though she had submitted it to them as part of their campaign.

Sara is a well-known and trusted figure in the IBD patient community.

False impression of association.

Clinical trial recruiter or patient? Who is this person?
CCFA’s Clinical Trials Community

- Searchable directory of clinical trials for IBD patients
- Clinical trial awareness campaigns
- Educational tools
- Peer-to-peer support program
- An example of patient involvement
- Ultimately, patients will be able to help shape the way clinical trials are done!
Short, well-produced, engaging video

Features (and is narrated by) an actual clinical trial patient

- He shares his experience as a volunteer in one of U of M's trials
- Describes talking to Dr. Higgins several times before joining
- He was not a U of M patient!
- “It might not be for you, it might be for you, but come to talk ‘em. A little bit of time out of your day might make a world of difference.”
Awareness & Normalization

- “IBD Patient News” – newsletter
  - Every issue contains information on clinical trials that are ongoing or currently enrolling
  - Copies available in all outpatient GI clinic rooms, waiting room, as well as online
  - Normalizes clinical trials
    - Clinical trials represent real options to consider, not an act of desperation.
- IBD center patient education events
  - University of Chicago’s patient education nights (e.g. “Why Haven’t We Cured Crohn’s Disease & Ulcerative Colitis?”)
  - The importance of clinical trials emphasized
In Conclusion

- Patients want to be, need to be, and (to some extent) are involved in the drug development process
  - Patients need to be educated to truly be involved
  - Initiatives need to be supported, and utilized in the IBD world
    - It will help patients and all parties invested in development – insight on unmet needs, patient preferences, clinical trial design, etc
- Clinical trial awareness and education is important
  - Normalize the idea of clinical trials
    - Clinical trials represent new options, not simply a “last resort” taken out of desperation.