Innovation in Clinical Trials
Trends & Potential Futures
IQ Consortium Symposium - 21 October 2015

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Disclosure

Craig Lipset is an employee of Pfizer, a member of the Board of Directors of the Foundation for Sarcoidosis Research, MedStar Health Research Institute, and an Advisor to Blueprint Health.

The opinions expressed are those of the presenter and do not necessarily represent the employer or other affiliations.
future

time regarded as still to come
“Ideas are useless unless used. The proof of their value is their implementation. Until then they are in limbo.”

- Theodore Levitt, HBR August 2002
engaged patients
eSource
mobile
shared infrastructure
integrated health systems
Starting a weight loss plan and feeling better

May 24

I started the TRANSFORMS Fingolimod (now Gilenya) Phase III trial way back in August of 07, and one of the known side effects of the drug was a slight asthma-like breathing issue. While this wasn’t necessarily a given that I would have that happen, they did say they were going to do a pulmonary function test every three months.

This frightened me, an admitted hypochondriac, into quitting smoking cold turkey on the 4th of July. 2007 prior to my entering the study.

Why mention all this? Well because up until that time the only times I had been overweight in my life were the two times I was pregnant with each of my boys. But that didn’t really count. Not even the second time when it took more than a year to shed the "baby weight".

Then I quit smoking and the gradual change happened. It happen so gradually that it snuck right past me and packed the weight on my backside while I wasn’t looking. Well, if I had looked down at my gut I may have noticed, but I was in denial... right up until I went shopping for a new bathing suit since we got season passes to Wild Waters in Silver Springs, FL. After forking out that kind of dough I am not ABOUT to not go due to having no suite.
A surreal encounter between epatient and pharmaceutical exec

All this time I never knew that I was living the life of an “ePatient”. That’s what they are calling us. We are the new trend that’s been developing since the advent of the internet. Patients who no longer sit back and digest only what our doctors have to say or what we can learn from local support groups.

We are the patients who have gone electronic. The ePatients. We google, we surf, we join yahoo or Facebook groups, we tweet, we post, we reach out. We read everything we can on the topic that is nearest and dearest to our hearts. Our own health.

I started this blog in 2007 when I was wrestling with the idea of joining a clinical trial. My
Engage with research participants about social media

Craig H Lipset

A growing number of participants in clinical trials are sharing information about their health online. It’s time that the drug development community starts to examine how this social media use might compromise the integrity of research studies and how it might also offer new opportunities.

Not long ago, the likelihood of clinical trial participants socializing and sharing information was limited to the clinic waiting room. As such, the risk of conversations among patients leading to the unblinding of experimental treatments in research studies was generally viewed as minimal. Over time, this has changed. During the HIV/AIDS crisis of the 1980s and 1990s, patients to share health data to support their ability to select treatment options for optimal outcomes. In addition to sharing perceptions of efficacy and safety for approved products, patients can also track and share data for investigational medicines during clinical trials. PatientsLikeMe used data posted by patients with amyotrophic lateral sclerosis who participated
Researchers Fret as Social Media Lift Veil on Drug Trials

Online Chatter Could Unravel Carefully Built Construct of 'Blind' Clinical Trials

By AMY DOKSER MARCUS
July 29, 2014 10:30 p.m. ET

On her first day in a clinical trial for an experimental multiple sclerosis drug, Jeri Burtchell was convinced she was getting the new drug, not the standard therapy that some patients were randomly assigned to receive.

When she bumped into the trial's lead investigator in the elevator that day, she told him,
We made the big time! @WSJ #socialmedia #patientvoice @CraigLipset

So I get a call last night from Amy Marcus, the writer who penned the featured article about the dilemma that is raised by this very blog and others like it. The piece, "Researchers Fret as Social Media Lift Veil on Drug Trials", she said, was tentatively scheduled to go to press today.

I awaken to a tweet telling me I'm a rock star in the clinical trial world.

I click the link in the tweet to read the article and I hit a pay wall. How ironic that an article about my use of social media is hidden from me on the internet. Frustrated, I call all over this one horse town to find anyone that carries it in print.
“Gimme My Damn Data”

Medicine 2.0
September 18, 2009
### Evaluations from Patients who take Fingolimod clinical trial

**Category:** Others  

**Most Popular Types:** FTY720, TRANSFORMS CFTY720D2302, CFTY720D2309 (Show all)  

See also: Fingolimod  

#### Overview | Individual Patient Evaluations

5 patient evaluations for Fingolimod clinical trial

| Purpose: MS (Multiple Sclerosis) and Transplant rejection prevention (Started Oct 27, 2008) |
|---|---|---|---|---|---|---|---|
| Date | Dosage | MS (Multiple Sclerosis) Efficacy | Transplant rejection prevention Efficacy | Side Effects | Adherence | Burden |
| Aug 03, 2010 | 0.5 mg Daily | Moderate | | None | Always | Not at all |

**Advice** Aug 03, 2010  
I LOVE not having to do injections anymore. I am in my 2nd year and was told at my last visit that my visits will continue until drug approval or if I should have a major reaction that has been reported, such as macular degeneration. So far this has been wonderful!  
Cost: monthly

#### 1 helpful mark

| Purpose: Participate in clinical trial (Started Oct 29, 2007) |
|---|---|---|---|---|---|---|
| Date | Dosage | Efficacy | Side Effects | Adherence | Burden |
| Aug 03, 2010 | 0.5 mg Daily | Moderate | None | Always | Not at all |

By shenannigans99  
See shenannigans99's full Fingolimod clinical trial history

| Purpose: MS (Multiple Sclerosis) (Started Nov 18, 2006) |
|---|---|---|---|---|---|---|
| Date | Dosage | Efficacy | Side Effects | Adherence | Burden |
| Sep 01, 2009 | Daily | Can't tell | Severe | Always | Not at all |

By cobeu8  
See cobeu8's full Fingolimod clinical trial history

| Purpose: Other (Started Apr 08, 2003) |
|---|---|---|---|---|---|---|
| Date | Dosage | Efficacy | Side Effects | Adherence | Burden |
| Jun 01, 2009 | 1 mg Daily | None | Moderate | Always | Not at all |

Latest side effects:  
- Slowed heart rate

By Singer  
See Singer's full Fingolimod clinical trial history
Waiting for p < 0.05 (A publication of PLM)

11/06/2012
By: Robert A. Goldstein

<table>
<thead>
<tr>
<th></th>
<th>Lithium carbonate</th>
<th>NP001</th>
<th>KNS-760704 (dextramipexole)</th>
<th>Sodium chlorite</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Total N of reported trial from ClinicalTrials.gov</strong></td>
<td>Various</td>
<td>105</td>
<td>943</td>
<td>N/A</td>
</tr>
<tr>
<td><strong>N of patients/matched controls meeting our data criteria on PatientsLikeMe</strong></td>
<td>78 / 390</td>
<td>28 / 280</td>
<td>29 / 319</td>
<td>17 / 85</td>
</tr>
<tr>
<td><strong>Duration of PatientsLikeMe observation window</strong></td>
<td>12 months</td>
<td>6 months</td>
<td>12 months</td>
<td>2.5 months</td>
</tr>
<tr>
<td><strong>Baseline rate of decline for all patients</strong></td>
<td>0.91 (0.04)</td>
<td>0.93 (0.07)</td>
<td>0.96 (0.05)</td>
<td>0.87 (0.30)</td>
</tr>
<tr>
<td><strong>Change in rate of decline for patients reporting intervention or study participation</strong></td>
<td>-0.02 (0.10)</td>
<td>-0.21 (0.21)</td>
<td>-0.13 (0.16)</td>
<td>0.69 (0.67)</td>
</tr>
<tr>
<td><strong>Placebo effect [fixed offset for any report &lt;= 2 months]</strong></td>
<td>-0.45 (0.17)</td>
<td>-0.54 (0.17)</td>
<td>-0.44 (0.26)</td>
<td>-0.38 (0.29)</td>
</tr>
<tr>
<td><strong>Model confidence that treatment slows progression to a clinically significant degree across all study participants [combination of treatment/placebo arms]</strong></td>
<td>5%</td>
<td>55%</td>
<td>36%</td>
<td>12%</td>
</tr>
<tr>
<td><strong>Estimated effect size if PatientsLikeMe has same balance between treatment/placebo arms as actual trial</strong></td>
<td>-0.02 (0.10)</td>
<td>-0.32 (0.26)</td>
<td>-0.27 (0.23)</td>
<td>0.69 (0.67)</td>
</tr>
<tr>
<td><strong>Model confidence that treatment slows progression to a clinically significant degree, for patients in treatment arm[s] using estimate from above</strong></td>
<td>5%</td>
<td>69%</td>
<td>64%</td>
<td>12%</td>
</tr>
</tbody>
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Internet entitled “Waiting for p < 0.05” on Abbott Lab’s website, presented the results of the first meeting in Florida in April 2012. The meeting was publically available at http://figshare.com/articles/Waiting_for_p%3C0.05/96802.

The following editors encourage a robust debate on early disease stages, such as ALS. The early disease stages can be cumbersome, slow and expensive. Complete the trials in which a single arm is given placebo, unless the data supports the benefits of the medications, could be a much more efficient approach for those patients and researchers alike. The data indicates initial results being made...
A Clinical Drug Trial Via Phone, Computer

By JENNIFER CORBETT DOOREN
June 7, 2011

Pfizer Inc. is conducting a drug trial in which patients participate from their homes using computers and smartphones rather than visiting a clinic.

Sanofi-backed virtual trial gets green light in Europe

February 16, 2016 | By Nick Paul Taylor

A Sanofi ($SNY)-backed consortium has won clearance to run a virtual diabetes trial in Europe. The regulatory nod reportedly marks the first time European authorities have green-lit a clinical trial with full electronic informed consent.

Genentech Pushes The Clinical Trial Envelope

By Ed Miseta, Chief Editor, Clinical Leader
Follow Me On Twitter @outsourcedpharm

Having spent more than 11 years at Genentech, Joling Mew has heard a lot of industry discussion about patient-centric trials. Mew is development excellence leader in product development and also works closely with the Strategic Innovation Group. Her responsibilities include leading cross-functional, strategic business initiatives with the goal of driving innovations that will transform the drug development process. In response to the patient-centric buzz, Genentech is testing a new model for clinical trials. And Mew believes
Enabling Data Donation

1. Patients Have Unprecedented Access to their Electronic Health Data

2. 90%+ Patients Will Share Their Health Data to Support Research*

3. R&D Teams Are Voracious for Diverse & Contemporary Data

Provide Patients With The Ability to Donate Their Diverse Health Data for Research


Makovsky Health -- survey of 1,001 adults indicates 90% would share their health data to support research -- [http://bit.ly/datadonor_makovsky](http://bit.ly/datadonor_makovsky)

JAMA Internal Medicine -- survey of 3,336 indicates top consideration when sharing electronic clinical data was that data is used for research -- [http://bit.ly/datadonor_publicpref](http://bit.ly/datadonor_publicpref)

engaged patients

mobile

shared infrastructure

eSource

integrated health systems

Gelsinger
The Mobile Tipping Point

There are now more gadgets on Earth than people

Humanity has reached a milestone on our journey toward the inevitable confrontation with Skynet. For the first time ever, there are now more active mobile devices on planet Earth than people.

by Eric Mack / October 6, 2014 8:21 PM PDT

Mobile apps overtake PC Internet usage in U.S.

Unique subscribers

3.6bn
4.6bn
4%
50%
59%
2014 - 2020
Apple ResearchKit

- Open source
- Access Apple Health data & hardware sensors
  - Barometer, Three-axis gyroscope, Accelerometer, Proximity sensor, Ambient light sensor, Magnetometer & GPS
- Three initial modules
  - Consent
  - Surveys
  - Active Tasks
  - Three initial modules
- MyHeart Counts reached 11,000 downloads in 24 hours
deep “digital phenotyping”
patient selection -> new endpoints -> Improve outcomes
Opportunities for Digital Disruption

Support Study Conduct
- Patient information
- Study planning, design, feasibility

Collect Data for Efficacy & Safety
- Consent
- Compliant data capture
- Novel endpoints

Companion Dx or Tx
- Dose setting
- Patient selection & stratification
- Enhanced outcomes
Regulators are leading the way...
engaged patients
eSource
mobile
shared infrastructure
integrated health systems
Leveraging Today’s Healthcare Infrastructure

- Integrated EMR
- aligned physicians
- multiple hospitals
- millions of lives
- ACOs & triple aims
The Salford Lung Study protocol: a pragmatic, randomised phase III real-world effectiveness trial in chronic obstructive pulmonary disease.


Please note:
Please note the Salford Lung Study is only available to patients registered at participating GP sites in Salford, Trafford, Stockport or South Manchester.

The Salford Lung Study.
Researching treatment of chronic obstructive pulmonary disease and asthma in Salford.

If you have COPD
Recruitment is now closed

If you have asthma
Please Click Here

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RF/RESP/0281/14
Integrated research partnerships build momentum

Research function provides infrastructure, leverages large pool of health systems’ patients and data

By Karyn Korieth

Anticipating the convergence of healthcare and clinical research to form learning health systems, a handful of organizations—some originating within the dedicated site network community, others within academia and the Veterans Affairs system—are partnering with community hospitals and large healthcare systems to

![Growing proportion of community-based principal investigators worldwide]

<table>
<thead>
<tr>
<th>Year</th>
<th>University/hospital/government clinics</th>
<th>Independent physicians</th>
</tr>
</thead>
<tbody>
<tr>
<td>2006</td>
<td>64%</td>
<td>36%</td>
</tr>
<tr>
<td>2008</td>
<td>59%</td>
<td>41%</td>
</tr>
<tr>
<td>2010</td>
<td>52%</td>
<td>48%</td>
</tr>
<tr>
<td>2012</td>
<td>47%</td>
<td>53%</td>
</tr>
</tbody>
</table>

Source: FDA’s Bioresearch Monitoring Information System File (BMIS)
engaged patients
eSource
mobile
shared infrastructure
integrated health systems

Geisinger
Developing a Clinical Trials Infrastructure in the United States
Paul Eisenberg, Amgen, Inc.; Petra Kaufmann, National Institute of Neurological Disorders and Stroke; Ellen Sigal, Friends of Cancer Research; and Janet Woodcock, U.S. Food and Drug Administration
April 13, 2012

GAP and IMI to Sign MOU to Accelerate Alzheimer's Drug Development
GAP, initiated by the New York Academy of Sciences and Global CEO Initiative, moving one step closer to reaching its goal of establishing a global, trial-ready platform for Alzheimer's disease.
Posted 3/19/2015

Antibiotic Trial Network Could Emerge From FDA/NIH Workshop
Master trial protocols aimed at patients with resistant organisms could streamline product development once sponsors are ready to "pay to play."
engaged patients actively participating

empowered by mobile tools

with data captured from electronic sources

conducted within healthcare settings

evaluating multiple medicines with shared infrastructure
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