Digital in Trials:
Improving Participation and Enabling Novel Endpoints

NIH Collaboratory Ground Rounds
26 July 2019

Craig H Lipset
@craiglipset
Linkedin.com/in/lipset
DISCLOSURE

The opinions expressed are those of the presenter and do not necessarily represent employers or other affiliations.
With what other industry could clinical researchers benchmark themselves?
Research Sponsor’s Outward Perception
Research Participant’s Inward Perception
Clinical Trials: Airline Travel

No one wants to be there, they just want to get to their destination.

Information online often coming from aggregators with much less content.

Execution relies on significant amount of shared infrastructure.

Experience shaped by many people not directly employed by the sponsor.

Each instance generates tremendous amounts of diverse data.

Significant reputation/perception issues.

Heavily regulated industries.

Significant priority and investment in safety.

Critical role of supply chain.

Increased focus on user experience.
### Start by Understanding the “User”

**Question:** Which missing service you expect and consider basic is causing the highest amount of frustration for you as a traveler? % share

<table>
<thead>
<tr>
<th>Service</th>
<th>% Share</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Changing flights</td>
<td>20</td>
<td>“Being able to change or cancel flights without needing to call a help desk”</td>
</tr>
<tr>
<td>Partner-airline link</td>
<td>17</td>
<td>“Check in easily when flying with a different airline”</td>
</tr>
<tr>
<td>Receiving updates</td>
<td>15</td>
<td>“Status updates. I frequently get them via TripIt before they arrive in the app”</td>
</tr>
<tr>
<td>App functionality</td>
<td>12</td>
<td>“The app constantly breaks down ... and I frequently need to log in again and type my email address and password”</td>
</tr>
<tr>
<td>Check-in</td>
<td>10</td>
<td>“Improve auto check-in to account for my previous travel preferences”</td>
</tr>
<tr>
<td>Seat selection</td>
<td>8</td>
<td>“Unable to view or change seat selection”</td>
</tr>
<tr>
<td>Issuing boarding passes</td>
<td>7</td>
<td>“Not having the electronic boarding pass on all my flights”</td>
</tr>
<tr>
<td>Frequent-flyer program</td>
<td>7</td>
<td>“Access to services on the frequent-flyer program is not simple and direct”</td>
</tr>
<tr>
<td>Delay prediction</td>
<td>2</td>
<td>“Delay prediction: no airline manages it”</td>
</tr>
<tr>
<td>Bag tracking</td>
<td>2</td>
<td>“Baggage tracking or missing-bag reports”</td>
</tr>
</tbody>
</table>

*McKinsey & Company | Source: Short internal survey of frequent flyers (n = 63), 76% percent of whom take more than 40 flights a year.*
Start by Understanding the “User”

Patient Group Engagement Across the Clinical Trial Continuum

Patient groups have potential to enhance the quality and efficiency of clinical trials by providing:

- Financial support for research
- Natural history data
- Input on relevance of research to patients
- Access to translational tools
- Help defining eligibility criteria
- Input on meaningful endpoints & PROs
- Advocacy for policy & funding issues
- Education to patient community

- Support to sponsors around key regulatory meetings
- Support preparing submissions for newborn screening for rare diseases
- Informing regulators on benefit-risk
- Public testimony at regulatory meetings

Discovery & Pre-Clinical
- Benefit-risk & patient-preference studies
- Protocol design & study feasibility input
- Study recruitment & retention strategy input
- Increased awareness about trials
- Participant feedback on trial experience
- Input on informed consent content & processes
- Peer advocates for participants
- Clinical trial networks
- Data Safety Monitoring Board members

Phase 1 - 3
- Phase 1-3 activities and...
- Support interpreting & disseminating study results
- Collaboration on post-marketing studies & surveillance initiatives
- Support developing access strategy & preparing for value or health technology review

Regulatory Review

Post-Approval

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THEMES FOR DIGITAL IN DEVELOPMENT

ACCESS

- access to participation
  - travel, virtual/remote, location-flexible

- access to information
  - pre-trial, during-trial, post-trial
  - sharing results & data

MEASURE

- improve existing measurement
- enable new endpoints

AUTOMATE

- process automation
- remove tasks
- improve quality
Will Digital Tools in Medicine Development enable…

**Improvement**,  
**Disruption, or**  
**Displacement?**
INCREMENTAL DIGITAL IMPROVEMENTS

**study planning**
- crowdsourcing
- data-driven
- AI-informed
- protocol-fueled
- automation

**patient engagement**
- data-driven matching
- electronic consent
- location-flexibility
- digital concierge support
- self-tracked & self-reported data
- patient aggregated data
- sharing results & data
- data ownership

**study conduct**
- remote monitoring
- digital/AI biomarkers
- electronically sourced data
- RWD/RWE
- synthetic controls
- process automation

**analysis & reporting**
- reporting automation deliverables to participants
DIGITAL AS A DISRUPTOR

- Patient information
- Study planning, design, feasibility

Consent
Compliant data capture
Novel endpoints

- Dose setting
- Patient selection & stratification
- Enhanced outcomes

Information Only
Data Capture
Digital Companion

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Example:
DIGITAL DISRUPTING LOCATION

Web-based trial to evaluate the efficacy and safety of tolterodine ER 4 mg in participants with overactive bladder: REMOTE trial

Contemporary Clinical Trials
Volume 38, Issue 3, 2014, Pages 196-197

M. Elf et al. / Contemporary Clinical Trials 38 (2014) 196–197

How are the data captured?

Data entered via an intermediary (e.g., a member of study team)
Mostly virtually w/ some human intervention (e.g., mobile phlebotomist)
Fully Virtually (e.g., all patient-generated collected via app/device)

Where are the data captured?

At a Site
Remote / Direct-to-Patient
Hybrid

How are the data captured?

Andy Coravos, CEO Electra Labs @ElektraLabs

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DIGITAL ENABLING DISPLACEMENT

DIY R&D

Patient-Led

Digital Twins
What stands in the way of realizing the impact of digital in medicine development?
WHAT IS MISSING?

Easy

Scale
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