Developing Novel Endpoints Generated by Mobile Technology for Use in Clinical Trials -- The Use Case Approach

Jennifer Goldsack, CTTI
Elements of a Use Case

In writing each use case, our goals are to:

- Identify the steps required to develop a novel endpoint for use in a clinical trial
- State the requirements at each of these steps
- Recognize any challenges that exist
- Propose solutions to these challenges
Scope and Assumptions

Assumptions

- The technology generating the data for each use case is assumed to produce data that is reliable, valid and sensitive.

Scope

- Developing data standards are out of scope for this work.
- The impact of the use case outcomes on survival are out of scope for this work. Work on these use cases are focused on the treatment benefit these outcomes can demonstrate in the present time.
- The use case outcomes are to demonstrate treatment benefit, not disease prevention. i.e. the patients in studies for which these endpoints will be used have been diagnosed with the stated disease.
Key Operational Definitions

- Novel Endpoints
- Biomarker
- Clinical Outcome
- Clinical Outcome Assessment (COA)
- Concept of Interest (COI)
- Context of Use (COU)
- Outcome Assessment
- Performance Outcome (PerfO)
- Treatment Benefit

Sources
Target Deliverables

For each use case
- Technical document
- Process map
- Peer reviewed publication

Will inform the development of recommendations on the development of novel endpoints, generated from data captured from mobile devices, for use in clinical trials.
Importance of the Use Cases

**USE CASES:**
1. Physical activity and gait / Parkinson’s disease / accelerometer
2. Physical activity / heart failure / accelerometer
3. Blood sugar level / diabetes / CGM
4. Physical activity / muscular dystrophy / accelerometer
Group & Room Assignments

Technology Review Sessions

- **Accelerometer**
  - *Pinnacle Grand Ballroom*
  - Parkinson’s disease, heart failure and muscular dystrophy use case teams

- **Continuous Glucose Monitor**
  - *Connection Room (Mezzanine Level)*
  - Diabetes use case team
Group & Room Assignments

Use Cases

- Parkinson’s disease
  - Collaboration Room, Level 2

- Heart Failure
  - Leadership Room, Level 2

- Diabetes
  - Connection Room, Mezzanine Level

- Muscular Dystrophy
  - Pinnacle Grand Ballroom, Level 2 (Remain in general session)
<table>
<thead>
<tr>
<th>PARKINSON’S DISEASE</th>
<th>HEART FAILURE</th>
<th>DIABETES</th>
<th>MUSCULAR DYSTROPHY</th>
</tr>
</thead>
<tbody>
<tr>
<td>Collaboration Room</td>
<td>Leadership Room</td>
<td>Council Room</td>
<td>Pinnacle Grand</td>
</tr>
<tr>
<td>(Level 2)</td>
<td>(Level 2)</td>
<td>(Mezzanine Level)</td>
<td>(Remain in Session)</td>
</tr>
<tr>
<td>Jen Goldsack</td>
<td>Martin Landray</td>
<td>Brian Perry</td>
<td>Annemarie Forrest</td>
</tr>
</tbody>
</table>

Amy Delozier
Aristide Merola
Cheryl Grandinetti
Christopher Miller
Daniel Karlin
Gerald Podskalny
Diane Stephenson
Gary Ostroff
Jeremy Wyatt
Kun Jin
Lauren Bataille
Miriam Rafferty
Ray Dorsey
Robert DiCicco
Sean Khozin
Selena Daniels
Tristan Massie
Walter Maetzler

Ashish Narayan
Dharmesh Patel
Evan Muse
John Alexander
Jonathan Seltzer
Leonard Sacks
Mary Jane Lapinski
Matthew Heasley
Michelle Campbell
Robert Temple
Stephen Carlson
Thomas Birkner
Tracy Bergemann
William Herrington

Adam Aten
Anna McCollister-Slipp
Campbell Hutton, JDRF
Courtney Lias
Craig Kollman
Elektra Papadopoulos
Jennifer Clark
John Shin
Julie Schulman
Kaveeta Vasisht
Nicole Jelesoff
Rav Shankar
Scott Komo
Shashi Amur
Stephen Coons
Steve Griffen
William Chong

Abby Bronson
Billy Dunn
Christopher Leptak
Chul Ahn
Lee Sweeney
Kathi Kinnett
Ken Skodacek
Komathi Stem
Mary Smith
Leslie Jacobsen
Marc Walton
Michael Binks
Nick Kozauer
Nikunj Patel
Nirav Sheth
Pat Furlong
Sonya Eremenco
Theresa Strong
Thank you.

Jen Goldsack, CTTI

Jennifer.goldsack@duke.edu