Possibility of mHealth in Clinical Development

Takeru Yamamoto
Managing Director of Asian Pacific Region
Medidata Solutions
Faculty Disclosure

In compliance with ANCC Guidelines, I hereby declare:

I do not have financial or other relationships with the manufacturer(s) of any commercial service(s) discussed in this educational activity.

Takeru Yamamoto

Managing Director of Asian Pacific Region
Medidata
Agenda

• Who we are
• What’s mHealth
• Why mHealth
• Our Experience
• Summary
WE ARE

A cloud-based technology company…

…transforming clinical research…

…for life sciences companies and the patients who depend on them.
Medidata Value

Resource Efficiency

Faster Time to Market

Risk Mitigation
Scientific Planning

Business Management

CRO

Physicians & Nurses

Sponsor

Patients
Expanding from EDC to Platform

Study Conduct
- Rave, Coder, Safety Gateway
  - Balance

Patient Engagement
- mHealth
- ePRO

Analytics & Benchmarks
- Insights

Study Planning
- Grants Manager
- CRO Contractor
- Study Design Optimization

Site Engagement
- CTMS
- Risk-based Monitoring

Consistent with typical progression of market-leading cloud companies
• Who we are
• What’s mHealth
• Why mHealth
• Our Experience
• Summary
mHealth – what is it?

- It is an abbreviation for “mobile health”
- Practice of medicine and public health supported by mobile devices.
- Patients are equipped with sensors or wearables, such as wristbands, bracelets, patches or any other devices.
- It enables you to…
  1. gather clinical health data directly from patients
  2. deliver healthcare information to practitioners, researchers and patients.
  3. monitor patients vital signs in real-time
- It opens the door to more remote patient participation
mHealth devices produce new data

- Objective
- Real world
- eSource
- Remote
- Real time
- Continuous

BP Cuff
Glucometers
Oximeter
Spirometer
Wearable Sensors

Wearable Sensor: a device that can be worn by or attached to a human to provide remote measurements of the wearer’s physiology, actigraphy, and/or environment.

Wearables include devices that track:

- Vital signs (ECG, HR, RR, EEG, EMG, temperature etc)
- Activity, step count, posture, fall detection, gait analysis
- Energy expenditure, sleep, water retention,
- Air quality, elevation, pollen count, amount of sunlight, skin hydration, speech rhythm
- The list is rapidly expanding…….

Forms:

- Patches
- Wrist bands, watches
- Clip ons, earrings, necklaces, jewelry
- Woven into garments
Accurate, objective measurement of outcomes

2. MODERATE ACTIVITIES, such as moving a table, pushing a vacuum cleaner, bowling, or playing golf:
   - Yes, Limited A Lot (1)
   - Yes, Limited A Little (2)
   - No, Not Limited At All (3)

3. Climbing SEVERAL flights of stairs:
   - Yes, Limited A Lot (1)
   - Yes, Limited A Little (2)
   - No, Not Limited At All (3)
• Who we are
• What’s mHealth
• Why mHealth
• Our Experience
• Summary
Stark Reality of Clinical Development

Pervasiveness of subjective patient data
It’s subject to environment

And to biases
It’s subject to interpretation, recall & judgment
In-clinic data alone...
mHEALTH: INSTRUMENTING PATIENTS... may not tell the whole story.
A New Paradigm

“Bring the Patient to the Trial”

Bricks and mortar data collection

“Bring the Trial to the Patient”

Virtual trials w/ remote monitoring – real world, real time physiological data w/ FDA-cleared, medical grade wearable biosensors
Life sciences sponsors are looking for better phenotypic data and surrogate endpoints - 1

<table>
<thead>
<tr>
<th>Potential expansion and improvement in surrogate endpoints</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Current</strong></td>
</tr>
<tr>
<td>Pain</td>
</tr>
<tr>
<td>Pain intensity scales (PRO), e.g., visual analog scale, brief pain inventory</td>
</tr>
<tr>
<td>Stroke</td>
</tr>
<tr>
<td>NIHSS (National Institutes of Health Stroke Score) or Rankin Scale</td>
</tr>
<tr>
<td>Parkinson's</td>
</tr>
<tr>
<td>Unified Parkinson's Disease Rating Scale (UPDRS)</td>
</tr>
</tbody>
</table>
Life sciences sponsors are looking for better phenotypic data and surrogate endpoints - 2

<table>
<thead>
<tr>
<th>Potential expansion and improvement in surrogate endpoints</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Current</strong></td>
<td><strong>Possible surrogates</strong></td>
</tr>
<tr>
<td>COPD</td>
<td></td>
</tr>
<tr>
<td>6-minute walk test</td>
<td>Daily steps for trial duration</td>
</tr>
<tr>
<td>Asthma</td>
<td></td>
</tr>
<tr>
<td>PRO surveys on number of night awakenings</td>
<td>Number of night awakenings detected by wearable device</td>
</tr>
<tr>
<td>Obesity</td>
<td></td>
</tr>
<tr>
<td>Weight, blood pressure and hypoglycemic events</td>
<td>Active minutes / steps walked</td>
</tr>
</tbody>
</table>
mHealth Value Proposition

- Better data
- Improved patient experience
- More efficient trials
Visualization and analytics: data quality

- Does the data fall within the parameters defined by the device specifications?
- Is the data physiologically appropriate?
- Does the data fall within plausible ranges?
- Is the data of a quality that will allow clinical analysis?
- Does the data have an appropriate level of inter-subject variability?
- How does the data compare with 'clinical standard' measurements?
Visualization and analytics: subject compliance

- Is the subject wearing the device?

- Is the subject wearing it correctly / is the device operating correctly?

- Is the subject wearing the device or is something else submitting data on their behalf?
Visualization and analytics: clinical insights

- What is the relationship between the mobile health data and currently accepted clinical endpoints and/or health outcomes?

- Are there insights in the data that provide an indication of subpopulations that are most responsive or least responsive to therapy?
# Outcomes focusing on socioeconomic value

<table>
<thead>
<tr>
<th></th>
<th>Drug A</th>
<th>Drug B</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Efficacy</strong></td>
<td>Extends Overall Survival by an average of 1 year</td>
<td>Extends Overall Survival by an average of 1 year</td>
</tr>
<tr>
<td><strong>Safety Profile</strong></td>
<td>Good</td>
<td>Good</td>
</tr>
</tbody>
</table>
### Outcomes focusing on socioeconomic value

<table>
<thead>
<tr>
<th></th>
<th>Drug A</th>
<th>Drug B</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Efficacy</strong></td>
<td>Extends Overall Survival by an average of 1 year</td>
<td>Extends Overall Survival by an average of 1 year</td>
</tr>
<tr>
<td><strong>Safety Profile</strong></td>
<td>Good</td>
<td>Good</td>
</tr>
<tr>
<td><strong>Patient “ Territory”</strong></td>
<td>Average of ~10 m²</td>
<td>Average of ~10000 m²</td>
</tr>
</tbody>
</table>
Value for Patient / surrogate endpoints

Patient Territory (m²)

Drug A
Drug B

Duration of Therapy
1 year
Improve Patient Experience

Reduce patient burden
- Fewer time for Reporting
- Fewer site visits

Increase the communication
- Advices by investigators timely manner with captured data

Easier to recruit, easier to retain
Higher engagement by Patients
The Human Factor: Shift in Patient Role from Subjects to Partners
Challenges exist

• Technical
  • Must be able to handle disparate data and new data types
  • Wireless device (phone) provisioning and management can be challenging

• Operational
  • New paradigms, new workflows, new skills and expertise needed
  • New methods, new capabilities, new mindset from traditional IT to information systems and solutions
  • Need science / evidence to show new data streams are useful
  • Can be cost/complexity adder for first few trials while going through learning curve

• Regulatory
  • Legal and regulatory issues – realtime data, responsibility to act, cross boarder issues
  • Privacy/security of data in transit and at rest
• Who we are
• What’s mHealth
• Why mHealth
• Our Experience
• Summary
Medidata sponsored to help us understand the opportunities & challenges w/ instrumenting patients
Successfully instrumented patients

- Patients equipped w/ Fitbit Flex & smartphone
  - 90% compliant with wearing activity trackers
- Subjects enrolled via their phones
- Subjects completed questionnaires via Patient Cloud ePRO
Successfully integrated mobile health data with traditional clinical data

- Medidata’s Data Science team identified correlations
  - For example, correlation between activity & pain
  - For example, connection between vigorous activity and weight loss
Experiences to be published via microsite
• Who we are
• What’s mHealth
• Why mHealth
• Our Experience

• Summary
Cost, Efficiency, and Logistics Opportunities

- Reduced patient burden – easier to recruit, easier to retain
  - Passive monitoring – reduced reporting burden
  - Reduced travel burden – fewer site visits
  - Reduced time
  - Reduced site cost to capture data
- Recruit from wider geographic range
- Reliable, objective data vs subjective reporting = improved data quality
- Continuous data vs. snapshots in time
- Reduced data collection costs
New Insights Opportunities

Know More, Know Sooner, Respond Faster:

• Access to unprecedented amounts of real world, continuous, real-time biometric patient data
• Objective data to support/verify subjective data
• Enable adaptive trials
• Fail fast product development – know sooner if a therapy has issues
• Ability to better differentiate a therapy
• Potentially increased safety through near-time monitoring
Challenges exist

- Technical
- Regulatory
- Operational
Faster, lower risk, more cost effective clinical trials

More value for life sciences companies

Faster treatment access for patients