Empowering patient populations with physical or cognitive limitations through electronic clinical outcome assessments (eCOAs)

Participants

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Workshop outline

> eCOA is now a widely accepted method of data collection in clinical trials
> eCOA has been shown to reduce administrative burden, automate skip patterns and scoring, avoid secondary data errors and reduce missing data
> However, there is still some reluctance to implement eCOA in trials with certain populations
> Concerns may centre around physical functioning, cognitive functioning and technological familiarity
> Population specific modifications to eCOA setup can help to overcome these concerns and challenges and can provide opportunities to improve data collection in challenging populations

Workshop outline

- Developing eCOAs for use in patients with cognitive or physical limitations
- Testing eCOAs with patients, observers and staff members
- Implementing eCOAs in clinical trials
Examples
Population specific technical solutions

Paul O’Donohoe
Director of Health Outcomes
CRF Health
Dexterity Issues

> Certain conditions may mean patients struggle to provide responses on any modality
  - E.g. arthritis of the hand; diabetes
> Patients may have difficulties holding electronic devices and selecting responses using the touch screen
> Longer completion times may cause patients pain or discomfort in their hands

Dexterity Issues

> Technology gives us an opportunity to reduce the impact of some physical limitations
  - No need to hold pen or stylus
  - Knuckle tap
  - Large screens
  - Large answer buttons with even larger active areas so that accuracy isn’t critical
> Shorter completion times compared to paper makes eCOA more manageable
Elderly Patients

> Dexterity issues can be a significant in elderly populations
> Adjustable brightness and adjustable font size can help those with poor eye-sight
  - Interesting question about the impact such changes might have in comparability of data between patients
> Instructions and reminders leads the patient through data collection, from the time they should be completing questionnaires to the specific questions they should be answering
  - Don’t make users think
> Electronic data capture can remove the need for site visits
> With the growth of BYOD a provisioned solution is still vital, particularly for this population where saturation might be less than 20%

Elderly Patients

> Often a perception that elderly patients will be unable to use eCOA systems
> However typically one of our most compliant groups
> Training is key
> Careful design can also improve elderly patients experience using the system
Young Patients

> Range of considerations for ensuring paediatric populations can respond and engage

Psychiatric disorders

> Simplification of administration
Design Best Practices Cut Across Populations

> While some populations might have specific issues interacting with an electronic device e.g. the visually impaired, in general design best-practices should cut across all populations

> Variation in user needs within a trial arguably greater than that between trials

> How flexible should our systems be?

Web Content Accessibility Guidelines: the Worldwide Web Consortium

> For many years websites have been expected to be amenable to a huge range of accessibility challenges

> We could learn a huge amount about making our solutions as low-friction as possible
Lessons from Web accessibility?

> Raises very serious questions about the comparability of data captured from questionnaires delivered in wildly different ways

> We should always be designing our solutions from the very beginning to be as usable as possible, while considering any potential population specific challenges

Patient feedback and empowerment

Chloe Tolley
Senior Research Manager
Patient-Centered Outcomes
Adelphi Values
Understanding the patient experience

> **Usability (and feasibility) testing** of devices with the specific population of interest prior to implementation in a clinical trial can identify any issues

- Barriers to eCOA completion during the trial are alleviated prior to commencement
  - Patients find it easier and less burdensome or stressful to complete instruments using the devices in the trial
  - Thus, the quality of the data that is ultimately collected is increased

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**Understanding the patient experience**

**Usability testing**

- Are respondents from the target population are able to use the software and the device appropriately?
- e.g. are instructions easy to follow, is the text readable, is it easy to navigate between screens and can patients select their intended responses.
- Different to user acceptance testing (UAT)

**Feasibility testing**

- Can patients complete the COAs on the device in a real world setting, reflective of the trial experience?
- Usually includes pilot testing used to gain initial insights into compliance issues and device issues e.g. data sending and alarm functionality.

**Equivalency testing**

- Are patients responses on an electronic mode of administration comparable to their responses using paper administration? *

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*Coons et al 2009*
Best practice in understanding the patient experience

- Debriefing of device and guides
- Training
- Interviewer briefing
- Full process testing
- Testing with staff and observers
- Observations
- Thorough pilot testing

Psychiatric disorders

- 100% of patients reported that completing the instruments was a positive experience
- 100% of patients said the device was easy to use
- 100% of patients said that they found it easy to select their responses
- Patients were happy with the time it took to complete the instruments (three instruments were completed)
- Patients said they would be happy to complete the questionnaires on the device at multiple time points

“It’s much more easier. Than writing everything – instead of reading and write. It’s easier than reading and writing”


Psychiatric disorders

However...

- Completion time
- Dependence
- Repeated instructions
- Hesitation
- Fixation
- Incorrect recall

“I didn’t answer, like, 100% truthfully, but—not that it wasn’t 100% truthfully. It’s—just say I can’t—I couldn’t make up my mind on the answer.”

It was easy to read. It was—you know, it—it always had the little arrow things. So I—uh, at first, I didn’t realize what the arrow thing was the—on the screen for. But then I realized it was to push the arrow to continue. So that was helpful too.

Dexterity issues

- Patients who had OA in their dominant hand
- No differences in self-reported and observed ability to use the device, or completion times between patients with OA of the hand and those without
- Patients prefer to answer questions on a device than on pen and paper
- Patients were easily able go back and change responses

“Um, it was just simple. Like, eh—it’s like using a phone”

“It was a reasonable amount of time. A few minutes”
Dexterity issues

> This is in line with previous research by Bellamy et al and Tyser et al.

> However, some patients completing on a tablet device laid it flat on the table and said they sometimes struggled to read the text because of glare.

“So you’ve propped it up – the tablet on a water bottle here?” “Yeah. Just to take the glare of the light off of it. because the lights, you know, sort of like distort it a little bit”


Bellamy N, Wilson C, Hendrikz, J, Whitehouse S.L, Patel B, Dennison S, Davis T, for the EDC Study Group. WOMAC NRS 3.1 Osteoarthritis index delivered by mobile phone (m-WOMAC) is valid, reliable and responsive Journal of Clinical Epidemiology 2011 Feb;64(2)

Pediatrics

Images as response options are preferred by children and interpreted correctly on the whole

> Children find eCOA more engaging, especially if reward screens are used

> Children find it easy to use the devices

“Uh - it’s good to see because it tells you how much depending on how big the circles.” (8 year old boy)

“It was touch screen and it worked really easy. Um, like it responded really quickly, didn’t take that long.” (11 year old girl)

“If I didn’t have the alarm I probably wouldn’t have remembered so it was probably necessary.” (Parent of an 8 year old)

Parents find alarms a useful reminder

> Parents often report that they need to help their child complete the diaries for the first few days and then the child gets the hang of it

“Um, I like how simple it was. It kind of reminded me of like the Nintendo DS.” (12 year old boy)
Children don’t take a long time to complete the instruments on electronic devices

Elderly patients

In studies with elderly patients, while on some occasions they may take a little longer to get used to the device, there are no differences in patients’ ability to complete questionnaires on an eCOA device

“I thought it was simple and wasn’t a challenge and it didn’t interfere with our day or anything”
(Parent of an 11 year old)

“I thought it – I mean, even for me who doesn’t know computer anything, um, after I got going, I thought it was pretty simple.”
70 year old female (no touchscreen experience)

“Oh I kind of liked it yeah. It was simple. It was easy.”
80 year old female (no touchscreen experience)
Elderly patients

> There is no difference in the issues reported during at home completion by patients of differing age groups
> Patients find eCOA devices easy to use and intuitive to navigate
> Many of the patients have experience of using some form of computer or touch screen device, even if they do not own one themselves.

“*It’s easy to read*”
70 year old female

Didn’t seem to take any time. I don’t know what the time is, but it didn’t seem to take any time. (laughter)
77 year old male

“Well, if I could get through it, practically anybody should.”
77 year old male (no touchscreen experience)

Elderly patients

> Recent reports found 74% of those aged 65+ in US own a mobile phone (up from 69% in 2013), and 82% ownership for those aged 65-74 in the UK.
> Studies have shown that elderly populations are actually one of the most compliant when it comes to eCOA completion at home

<table>
<thead>
<tr>
<th>Age</th>
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<th>Average Compliance</th>
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<tbody>
<tr>
<td>Adult</td>
<td>59,004</td>
<td>87.2</td>
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<tr>
<td>Children</td>
<td>5,040</td>
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<td>Elderly</td>
<td>6,251</td>
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<tr>
<td>Infant (caregiver)</td>
<td>11,807</td>
<td>95.3</td>
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<tr>
<td>Teenage</td>
<td>8,242</td>
<td>90.3</td>
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</tbody>
</table>
Improving collection of eCOA data in trials

> When eCOA are designed appropriately, most populations interviewed had minimal issues in completing instruments on an eCOA device
> In addition populations tend to prefer completing electronically to pen and paper
  > eCOA offers solutions to challenges associated with pen and paper completion
> Usability and feasibility testing helps to identify and resolve issues that could become barriers to eCOA completion within a trial
  > Device modifications
  > Trial design

Experience in pediatric clinical trials

Jessica Abel
Senior Manager
Global Health Economics and Outcomes Research
Allergan
Collecting patient-reported outcome data in pediatric clinical trials: Unique challenges

> Challenges associated with completion of patient-reported outcome (PRO) measures by pediatric populations
  - Limitations on ability to recall and report on symptoms within a specified timeframe
    - Twice daily item completion likely necessary for conditions for which patients must provide average symptom severity or report events over 24 hours (i.e. symptom-driven conditions)
      - Completion on paper not practical for duration of study
      - Event-driven reporting not feasible due to school restrictions on use of devices or embarrassment among peers
  - Shorter attention spans
    - May find measures burdensome to complete

Collecting patient-reported outcome data in pediatric clinical trials: Unique challenges

> Children may also have difficulty:
  - Reading (on paper questionnaires) or understanding/processing (via IVRS) questions
    - More challenging for pediatric patients when they can only hear the questions in comparison to seeing the questions and response options together
    - Typically respond better to visual images
  - Remembering to complete questionnaires (e.g. twice daily or daily)

> Challenges associated with administration of PROs on an eCOA platform
  - Cultural differences in ability to use and familiarity with devices
Potential solutions for successful eCOA implementation in pediatric clinical trials

- Development of one instrument for use across age groups with option for different reporters to accommodate variability in learning/cognition across narrow age ranges
  - Self-administered
  - Interviewer-administered
  - Observer-completed

- Completion requirements at more appropriate times (e.g. twice daily—morning and evening) to minimize burden on daily routine and potential embarrassment among peers

- Shortened recall periods

- Appropriate training for patients and caregivers on device completion
  - Site-based training
  - Quick-reference guides
  - At home training

Administering PROs via eCOA platforms in pediatric populations: Advantages

- Use of smaller, handheld devices (similar to iPhone) allows for more convenient use and completion by children

- Simple and intuitive designs
  - Visual response options
  - Skip patterns
  - Instruction screens with images to help focus children on correct recall period
  - Reminders and alarms for children and caregivers
  - Reward screens

- Faster completion than on paper

- Use of games and pictures make it fun for children to complete
Use of eCOAs in pediatric IBS-C/FC clinical trials: A case study – What works?

> Pediatric irritable bowel syndrome with constipation (IBS-C) and functional constipation (FC) are chronic gastrointestinal disorders characterized by infrequent bowel movements and abdominal symptoms
  - Rome III diagnostic criteria differ between IBS-C and FC in children and adolescents (4-17 years old)
  - However, qualitative research among both populations suggests the core signs/symptoms are the same, with varying degrees of frequency/severity
  - Key symptoms to assess include:
    ▪ Frequency of bowel movements
    ▪ Stool form/consistency
    ▪ Abdominal pain severity

> Symptoms can only be assessed through direct patient report

> If a patient is unable to report due to age or limitations in ability to read or understand PRO items, a parent/caregiver must be asked to rate associated observable behaviors

Use of eCOAs in pediatric IBS-C/FC clinical trials: A case study – What works?

> Electronic diary developed for children aged 6-17 years

> Advantages of leveraging eCOAs in pediatric populations:
  - Private
  - Fun and engaging for children
  - Clear and simple presentation of content
  - Reduced respondent burden through use of skip-patterns, branching logic
Use of eCOAs in pediatric IBS-C/FC clinical trials:
Twice daily completion & shortened recall periods

Morning completion: requires recall of previous night

Evening completion: requires recall over that day

Use of eCOAs in pediatric IBS-C/FC clinical trials:
Visual response options

> Visual response options aid in patient comprehension of response scales
  - Preferred by children
  - Help in interpretation of response options (vs. text-only response options)
Use of eCOAs in pediatric IBS-C/FC clinical trials: Instructions and reward screens

Use of eCOAs in pediatric IBS-C/FC clinical trials: Lessons learned

> Knowledge of unique challenges inherent in pediatric eCOAs varies among internal cross-functional stakeholders
  - Necessitates expert input/guidance from PRO team on eCOA implementation in the clinical trial to ensure key scientific integrity of the instruments is maintained
    - Specification of inclusion criteria for primary respondent/caregiver, patient reading levels, diary compliance, etc.
    - Testing of eCOA device

> Issues with patient compliance may impact study enrollment
  - Necessary to track patient diary completion in real-time and follow-up with patients at home to encourage compliance
  - Consistent compliance with eCOA completion is critical to capturing high quality data throughout the trial
Summary and conclusions

Conclusions

> While challenges exist in the use of eCOAs in certain populations, there are a number of solutions for successful implementation of eCOA devices.

> eCOAs provide opportunities to engage patients and make it easier for them to complete questionnaires than is possible via pen and paper.

> Key learnings from examples where similar issues have been resolved in the past (e.g. web accessibility) can be utilized to address eCOA issues across patient populations with physical or cognitive limitations.

> Usability studies allow for identification and resolution of any issues specific to that population prior to implementation in clinical trials, reducing barriers to eCOA completion.

> Feedback from patients indicates that the majority find eCOA devices easy to use, prefer them to paper, and are compliant when completing them at home.

> Experiences in the use of eCOAs in clinical trials highlights the importance of population specific device and protocol design and consistent monitoring of compliance to ensure the collection of high quality data.
Questions?

Thank you!
Examples

**Patient Global Assessment (PGA)**

Please indicate by tapping on the horizontal line your answer to the following question relating to the past **TIMEFRAME**:

Considering all the ways your **HEALTH PROBLEM** affects you, how would you rate the way you feel over the past **TIMEFRAME**?

0 Excellent: 100 Poor

**Your usual activities TODAY** (e.g. work, study, housework, family or leisure activities):

- I have no problems doing my usual activities
- I have slight problems doing my usual activities
- I have moderate problems doing my usual activities
- I have severe problems doing my usual activities
- I am unable to do my usual activities
1. Overall, how severe was your psoriasis-related itching over the past 24 hours?

- 0: No itching
- 10: Itching as bad as you can imagine

FAMILY LIFE / HOME RESPONSIBILITIES
The symptoms have disrupted your family life / home responsibilities:

- Not at all
- Mildly
- Moderately
- Markedly
- Extremely

0 1 2 3 4 5 6 7 8 9 10