EMA experience with the review of digital technology proposals in medicine development programmes

3rd Industry Stakeholder Platform on R&D support, 18 May 2018
EMA ROLE

- Remit
- Awareness
- Support innovation
- Support seamless decision making
- Guidance (within remit)

**Self-care ▶️ HC provider/patient relationship ▶️ Medicines authorisation**

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Possible Benefits of digital health in clinical trials

- Potential to streamline clinical investigations (rare diseases, health records)
- Patient functioning in real-world setting
- Off-site and remote data capture
- Enrol/monitor patients in distant locations
- Support Adherence?
- Decrease missing data?
- Potential for rapid response?

Currently:
- *Complement rather than substitute*
- Function
- Feel (?)
- Systems(?)

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mHealth technology data must be linked to meaningful clinical benefit (e.g., patient functioning)

- Identify Patient Population (Context of Use) & Concept of Interest for Meaningful Treatment Benefit
- Select or Develop Outcome Assessment Using Wearable Technology & Pilot Test
- Evaluate Measurement Properties
- Develop Meaningful Change Guidelines

Source: Patel, FDA, modified
Too much of a good thing?

Some real examples:

• Fast internet speed needed for data upload (eg 100 data points/sec in 3 axes)

• Interpretation of findings different from investigator rated scale (Variability of mobility patterns by time of day/region; Compliance of monitor wearing drop)

• Estimands for missing data? (missing times)
Selecting or Developing a Fit-For-Purpose Wearable Technology

- Safety and user acceptance?
- How well do we understand the wearable device performance characteristics in terms of its sensitivity, specificity, accuracy, precision, and other relevant performance characteristics?
- Is the wearable technology derived data reflective of a defined clinical benefit that’s relevant and important to patients daily life functioning?

Patient input is critical!

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EMA experience

- Development of novel outcome measures (e.g., COA, biomarker) for use in multiple drug development programs (e.g. Sarcopenia, Duchenne)
- Ingestible sensor for adherence measurement
- Scientific advices and ITF meetings on adherence / appropriate medication (chronic diseases: COPD, diabetes)
- Participation to IMI initiatives (e.g. SPRINT-T)
- GCP implications for RCTs (compliance, traceability...)

EMA is interested and open to innovation and engagement early and throughout clinical trial endpoint development
Challenges and opportunities

• Potential to **simplify and improve** data collection
• **Data** handling may depend on purpose (Local, Cloud, data protection, data integrity).
• Likelihood of multiple stakeholder responsibilities and **remit**.
• May be more straightforward to **validate** as PRO rather than clinical outcome.
• **HTA** involvement welcome – ADL and function
• Strengthen/forge link with **notified bodies (?)**
• Fine line between adherence and “promotion”?
Early regulatory engagement is essential, if dossier/proposal will be heavily reliant on these data.

Background information:

Guidance on qualification of novel methodologies
Ingestible sensor qualification opinion
Data privacy workshop
"Big data" workshop

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