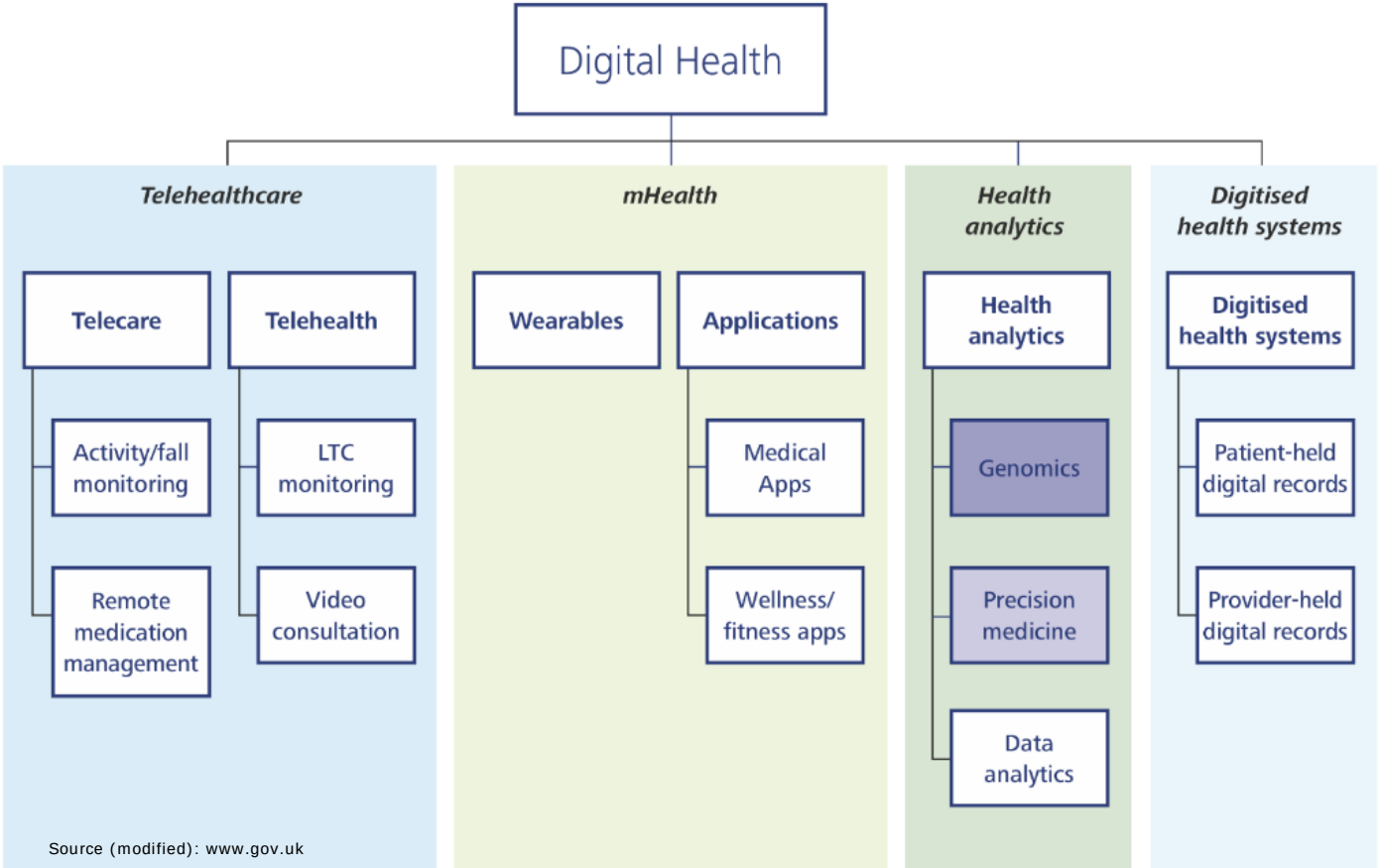
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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

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

Francesca Cerreta , Scientific Advice



EMA ROLE

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

Self-care ◊◊◊ HC provider / patient relationship ◊◊◊ Medicines authorisation

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(?)**

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)

Concept of Interest for Meaningful Treatment Benefit

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!

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:

right click and open hyperlink

Guidance on qualification of novel methodologies

Ingestible sensor qualification opinion

Data privacy workshop

"Big data" workshop