U.S. FOOD & DRUG ADMINISTRATION

Engaging Patients in Evidence Generation

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1

BIG DATA AND SUFFICIENCY

Sentinel Infrastructure

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Perspective

Mini-Sentinel and Regulatory Science — Big Data Rendered Fit and Functional

Bruce M. Psaty, M.D., Ph.D., and Alasdair M. Breckenridge, M.D.

N Engl J Med 2014; 370:2165-2167 | June 5, 2014 | DOI: 10.1056/NEJMp1401664

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In medicine, "big data" come in many forms. With the financial incentives provided by Medicare and Medicaid for the "meaningful use" of electronic health records (EHRs), the quantity of electronic medical data has expanded rapidly. Simultaneously, genomewide association studies funded by the National Heart, Lung, and Blood Institute have produced data sets with millions of genetic variants for each participant, encouraged the development of consortia with hundreds of thousands of study participants, and resulted in discoveries about the genetic origins of human health and disease.

What does "Big Data" Offer?

- **Breadth** – large numbers of individuals get us closer to the underlying source population – *potential reduction in selection bias*?
- **Depth** – increasing amount of data on each individual increases the chance that we will have measures of likely confounders – *potential reduction in information bias*?
- **Diversity** – different types of data offer the potential to "cross check" findings for any particular data source – *potential to enhance control for residual bias and/or improve generalizability*?

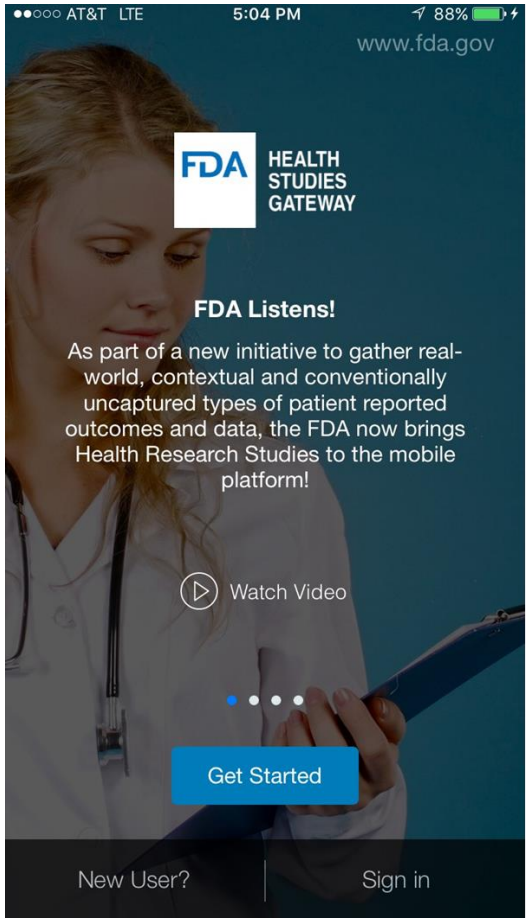
What is Sufficiency?

- Adequate data
  - Medical Product Exposure
  - Health Outcomes of Interest
  - Confounders
- Appropriate method
- To answer the question of interest
- To a satisfactory level of precision

2

EXPANDING THE DEPTH AND DIVERSITY OF BIG DATA

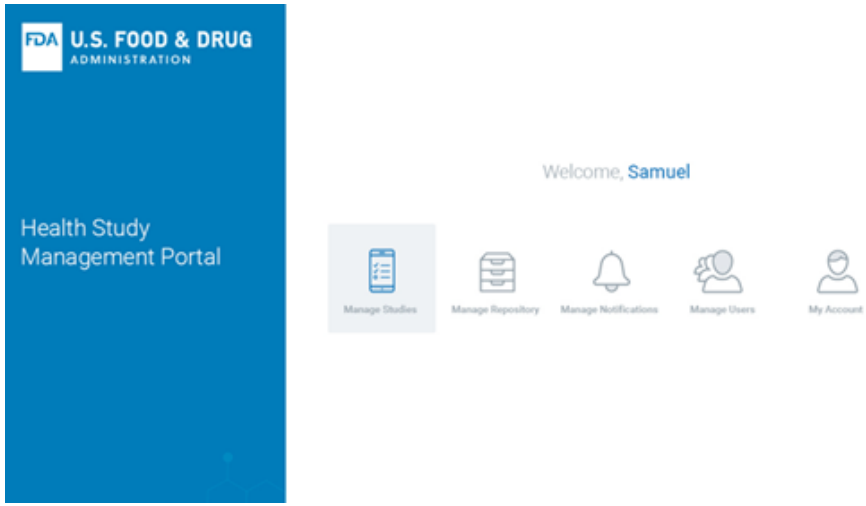
FDA Health Studies Gateway

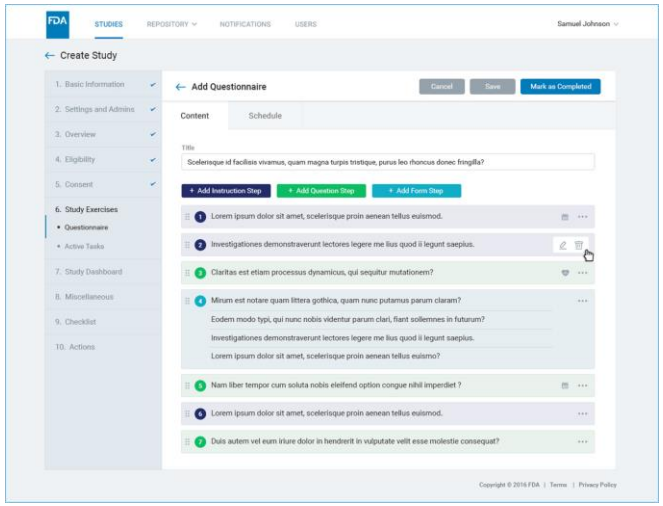


- First effort to link patient-reported data from a mobile platform to the Sentinel Infrastructure
- Study Mobile apps built using Apple ResearchKit and ResearchStack (Android)
- Initial use case will be medication safety during pregnancy
- Collaborators include Harvard Pilgrim Healthcare Institute, Group Health Research Institute, LabKey, Boston Technology Corporation, and University of California San Diego

Note: App is not currently active. Wireframes are samples and will be altered before launch.

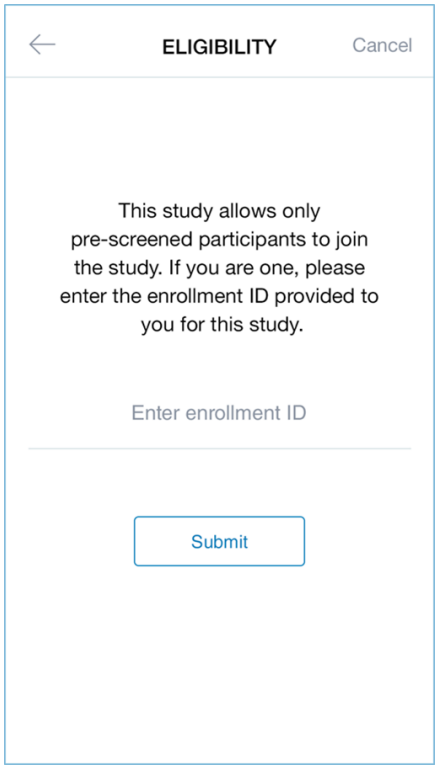
Create

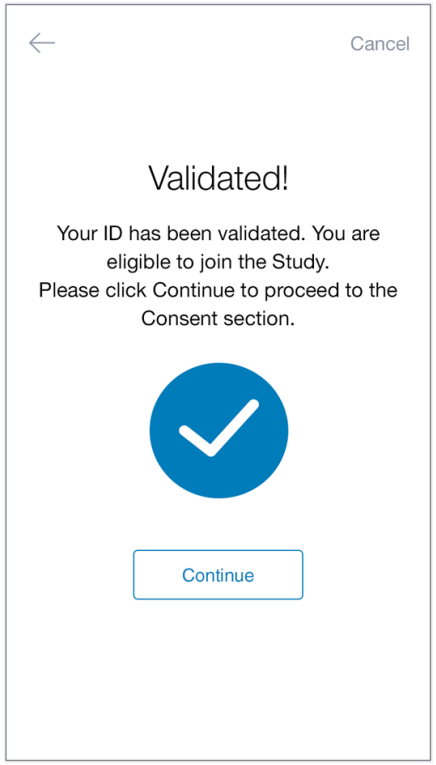


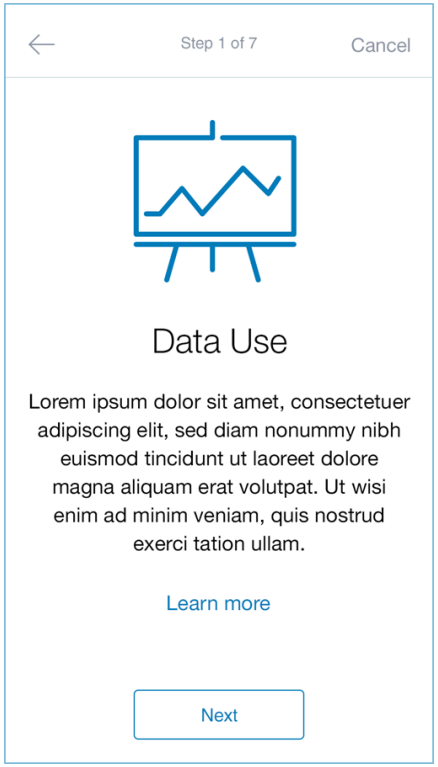


- Configure Study Elements (including questions and active tasks)
- Create patient enrollment tokens and map them to patient IDs

Enroll

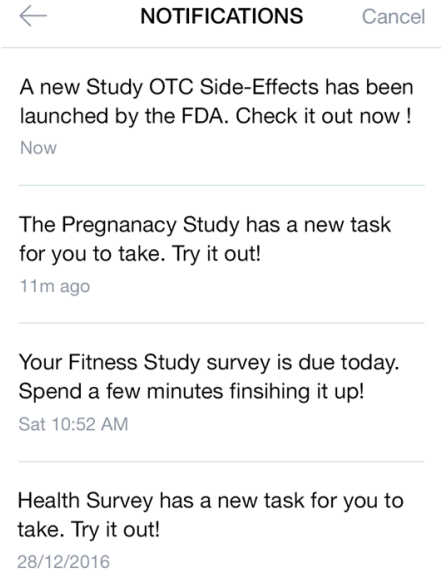


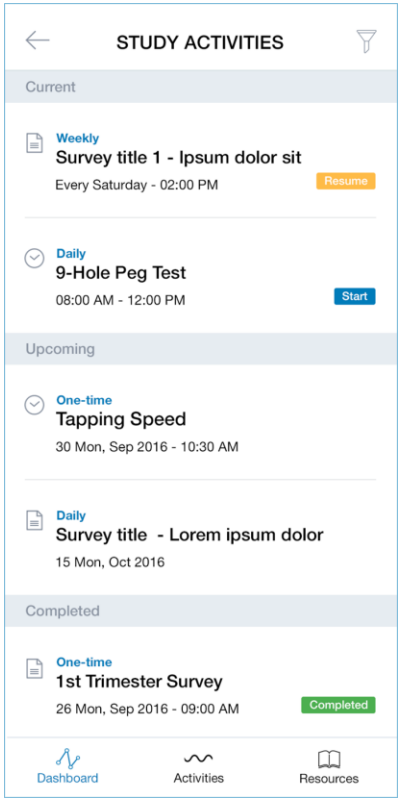


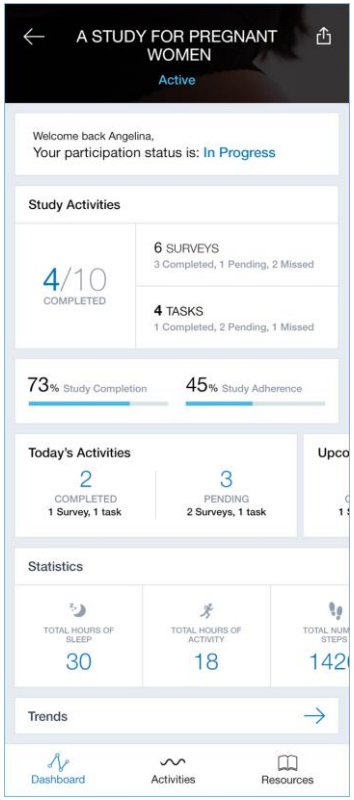


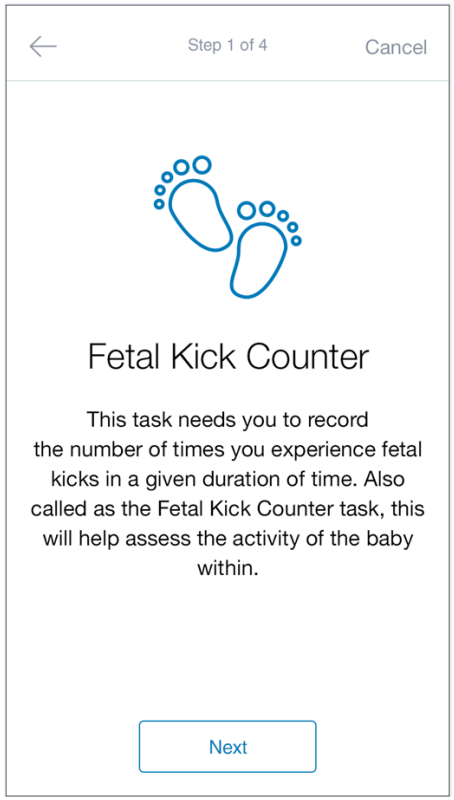
- Select a cohort and distribute enrollment tokens
- Participants download the app in iOS or Android app stores

Engage



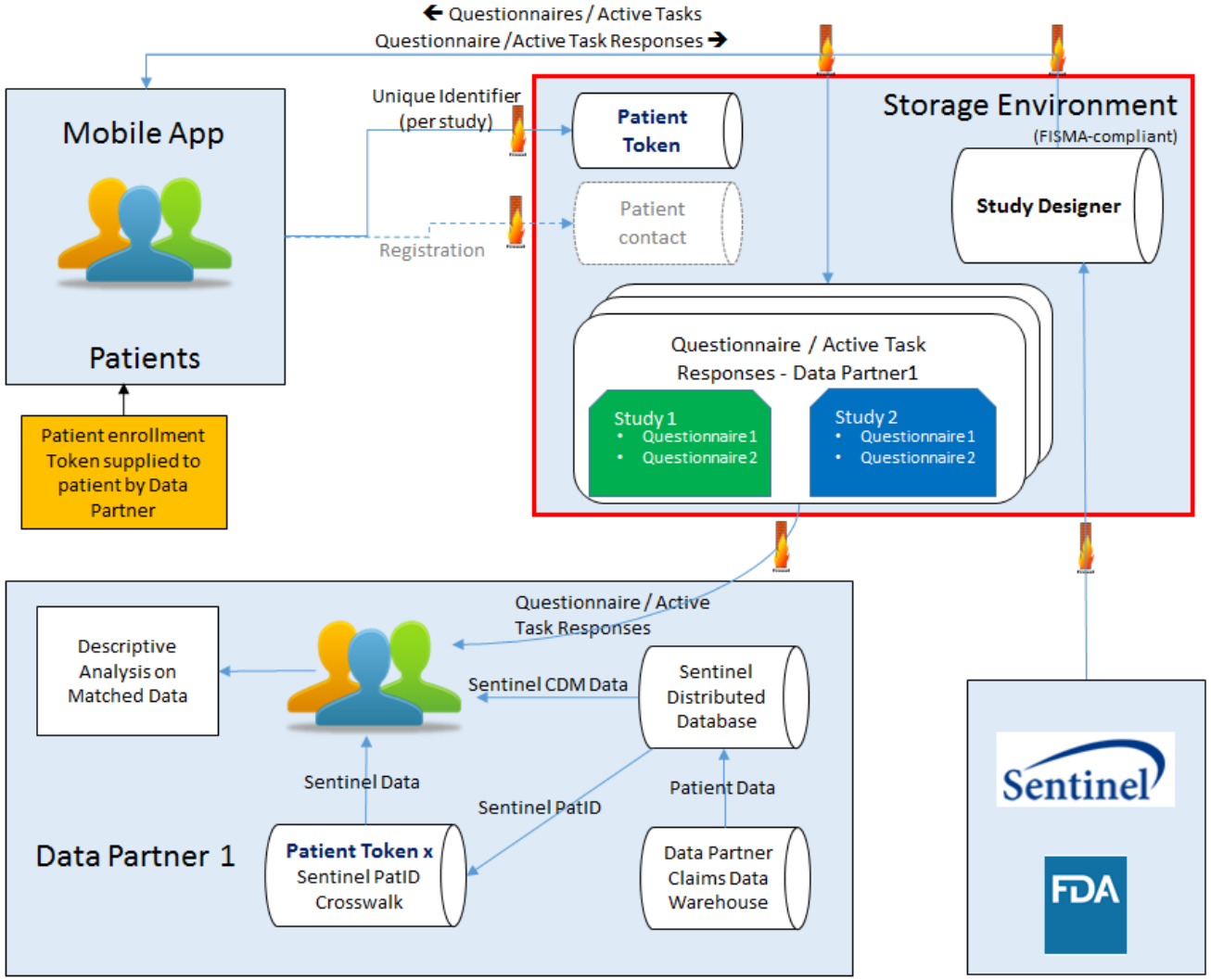






- Participants respond when they choose within the study schedule
- Study Dashboard displays progress as well as highlights from data collection

Link Primary and Secondary Data



3

TAKEAWAYS FROM TRANSDISCIPLINARY COLLABORATION

Adapt existing concepts and infrastructure

- Establish partnerships with study managers, epidemiologists and clinicians who have been working in the domain
- Leverage existing networks and technology
- Evaluate the strengths and limitations of the mobile approach vs. traditional clinical research processes

Listen to patients

- Including but not limited to:
  - “Research subject” burden
  - Meaning and Logic of questions
  - Important topics
  - Sensitive topics
  - Mobile technology use patterns
  - App usability

Provide flexibility so others can innovate

- Start with the most parsimonious approach that will still meet decision making needs
- Include capacity to scale for broad-based evidence generation by multiple stakeholders
- Embed the ability to alter the new infrastructure
- Iterate
- Groom “early adopters”

Acknowledgement

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