Leveraging Electronic Health Records:
Expanding Opportunities for Evidence Generation

Paul G Kluetz, MD
Deputy Director, Oncology Center of Excellence
U.S. Food and Drug Administration

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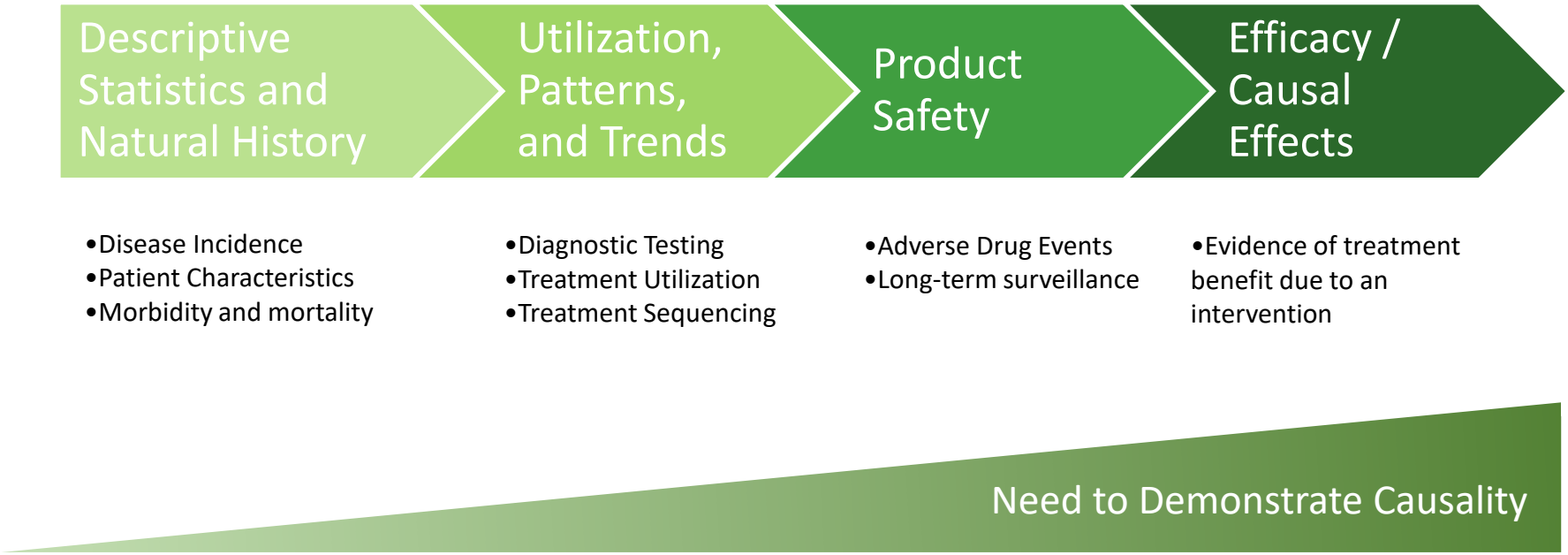
Disclosures

- I have no financial relationships to disclose

The FDA Perspective –

- FDA's mission is to assure the safety, efficacy and security of drugs and biologics
- Science based- data driven decisions
- Substantial evidence of efficacy to approve an indication for a drug or biologic is only ONE use of data for regulatory decision making

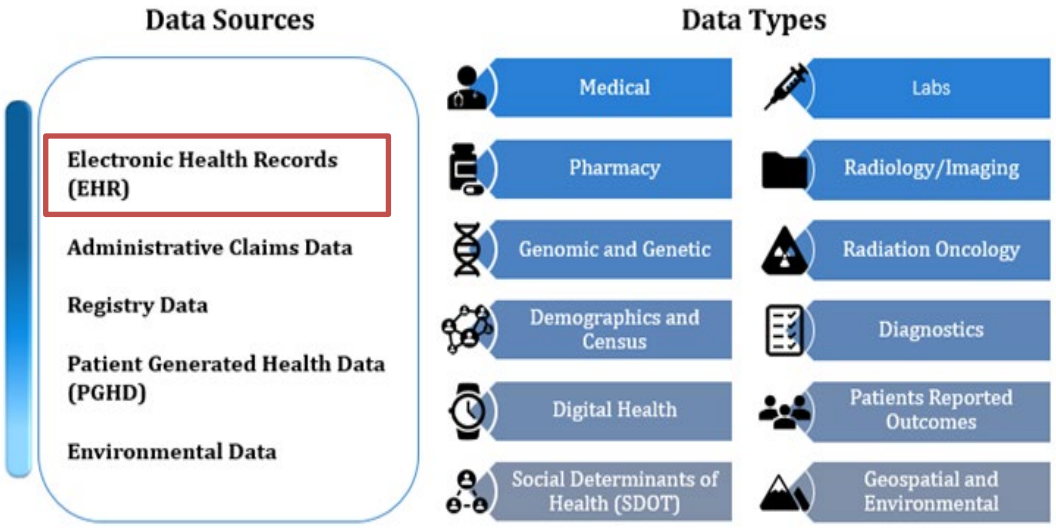
Data Can be Used for Many Objectives



Data Sources and the FDAs Real World Evidence Framework

Real-World Data (RWD)
data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources

Real-World Evidence (RWE)
*the clinical evidence about the usage and **potential benefits or risks** of a medical product derived from analysis of RWD*



FDA does not endorse one data source over another or seek to limit the possible sources of data that may be relevant to answering study questions

Generating Evidence From Clinical Trials and Clinical Care

EHR Data Can Play an Important Role Throughout the Continuum

RCT	Decentralized	Pragmatic	Observational
Prospective	Prospective	Prospective	Often Retrospective
Randomized	Randomized	Randomized	Non-randomized
Systematic Site-Based assessments	Systematic REMOTE assessments	Less systematic assessments	Routine Clinical Care
Highly Monitored / Site-Based	Highly Monitored / Site or REMOTE	More Selective Monitoring	Generally Unmonitored
Narrow Population	Broader Population	Broader Population	Broadest Population

Acceptability of various evidence sources is context dependent

Electronic Health Records (EHR)- Challenges

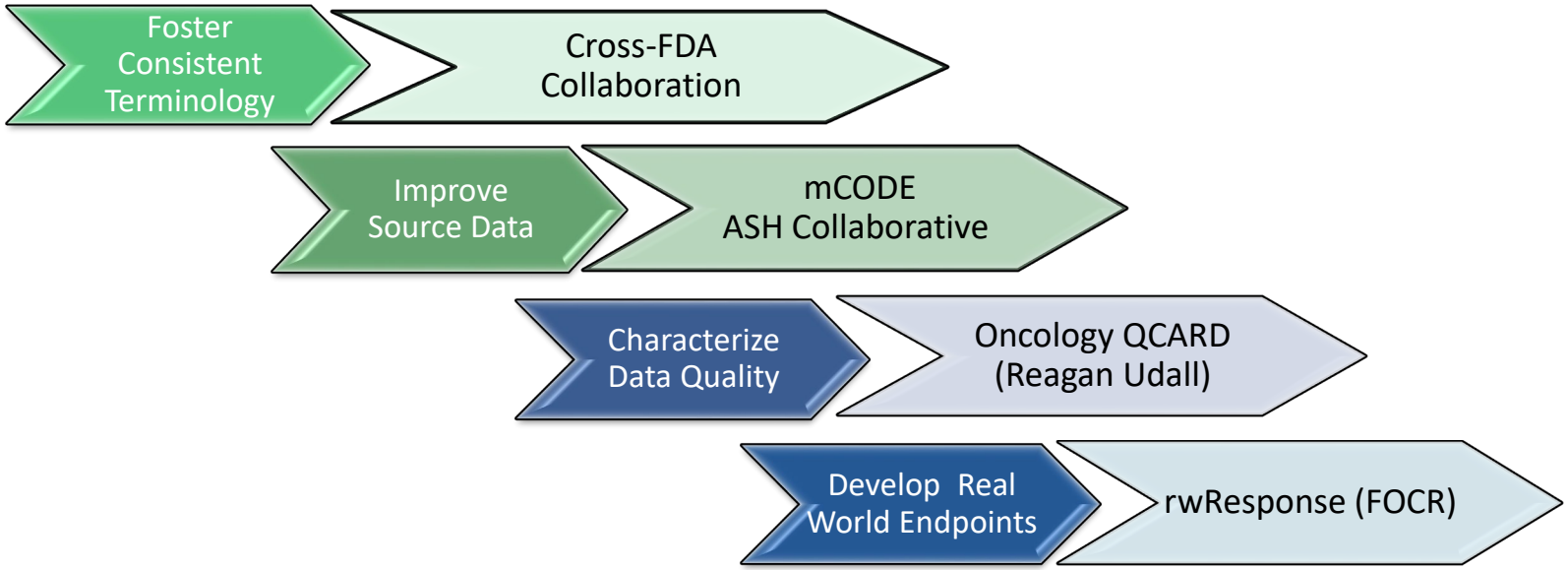
Challenges for use of retrospective EHR cohorts to Generate Evidence in Oncology

- Lack of Randomization for Time to Event Endpoints
- Lack of structured data and need for extensive curation
- Inability to collect Tumor Based Endpoints (Response)
- Completeness of Capture
- Data Quality and Missing Data

FDA Oncology Real World Evidence Program

Collaboratively advance the appropriate use of real-world evidence in oncology product development to facilitate patient-centered regulatory decision-making.

Current Collaborative Efforts



https://www.fda.gov/about-fda/oncology-center-excellence/oncology-real-world-evidence-program

Conclusion

- Electronic Health Records data is an increasingly available source of data that could be used more effectively in cancer research

- Challenges include unstructured data, lack of standard data elements and incomplete capture among others

- FDA has multiple ongoing efforts to advance the utility of EHR and other RWD sources to improve its utility for regulatory use

FDA Resources and Guidance to Industry

FDA's Real World Evidence Program
https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence

FDA Oncology Center of Excellence – RWE
https://www.fda.gov/about-fda/oncology-center-excellence/oncology-real-world-evidence-program

Dec 2018 Framework for FDA's Real-World Evidence Program
https://www.fda.gov/media/120060/download

Jul 2018 Use of Electronic Health Records (EHR) Data in Clinical Investigations
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/use-electronic-health-record-data-clinical-investigations-guidance-industry

Sep 2021 Assessing EHR and Medical Claims Data to Support Regulatory Decision-Making for Drug and Biologic Products
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/real-world-data-assessing-electronic-health-records-and-medical-claims-data-support-regulatory

Dec 2021 Considerations for the Use of Real-World Data and Real-World Evidence To Support Regulatory Decision-Making for Drug and Biologic Products
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/considerations-use-real-world-data-and-real-world-evidence-support-regulatory-decision-making-drug

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