*EMA – Big Data for Regulatory Purposes*

# FDA Guidance on Real-World Data

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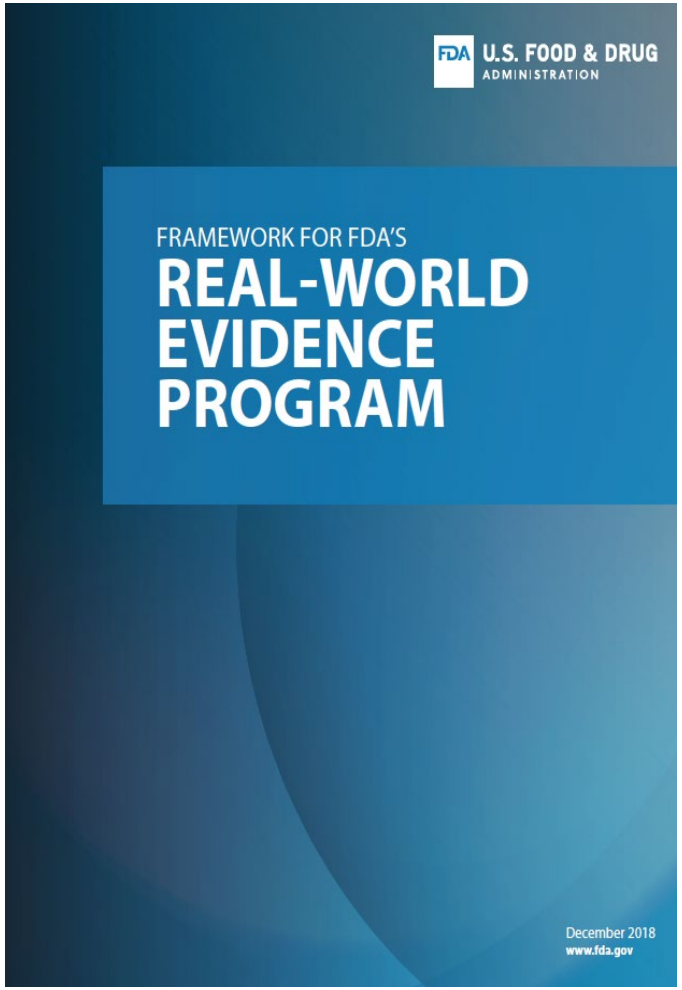
# 21st Century Cures Act (2016)


- **FDA shall establish a program *to evaluate the potential use* of real-world evidence (RWE) to:**
  - **Support new indication for a drug approved under section 505(c)**
  - **Satisfy post-approval study requirements**
- **Draft framework issued in Dec 2018:**
  - **Describe sources of RWE, challenges, pilot opportunities, etc.**
- **Draft guidance for industry issued in Sep, Oct, and Nov 2021**
- **Standard for *substantial evidence* remains unchanged; commitments under Prescription Drug User Fee Act (PDUFA) VI**

# FDA RWE Framework (2018)




- Applies to Center for Drug Evaluation and Research (CDER) and Center for Biologics Evaluation and Research (CBER)
- Multifaceted program to implement RWE:
  - internal processes
  - external stakeholder engagement
  - demonstration projects
  - guidance development

# RWE Draft Guidance: EHR/Claims Data

## Real-World Data: Assessing Electronic Health Records and Medical Claims Data To Support Regulatory Decision-Making for Drug and Biological Products

Guidance for Industry

*DRAFT GUIDANCE*

**September 2021**
**Real World Data/Real World Evidence (RWD/RWE)**

# EHR/Claims Data Guidance (cont'd)

**Focus of draft guidance:**

- **selection of data source(s) to appropriately address the study question**
- **development and validation of definitions for exposures, covariates, outcomes**
- **data provenance during accrual, curation, analysis**

**Note: choice of study design and method of statistical analysis are outside of guidance scope**

# RWE Draft Guidance: Data Standards

# Data Standards for Drug and Biological Product Submissions Containing Real-World Data

## Guidance for Industry

***DRAFT GUIDANCE***

**October 2021**
**Real-World Data/Real-World Evidence (RWD/RWE)**

# Data Standards Guidance (cont'd)

**Focus of draft guidance:**

- **processes for managing RWD**
- **conforming RWD to FDA data standards**
- **mapping RWD to FDA submission standards**
- **considerations for data transformations**

**Note: this guidance applies regardless of the 'type' of RWD**

# RWE Draft Guidance: Registry Data

# Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products

***DRAFT GUIDANCE***

# Registry Data Guidance (cont'd)

## TABLE OF CONTENTS

# CDER-CBER RWE Guidance Series

## CDER Guidance Agenda
## New & Revised Draft Guidance Documents
## Planned for Publication in Calendar Year 2021[1]

### CATEGORY – Real World Data/Real World Evidence (RWD/RWE)[2]

- Considerations for the Use of Real-World Data and Real-World Evidence to Support Regulatory Decision-Making for Drug and Biological Products
- Data Standards for Drug and Biological Product Submissions Containing Real-World Data
- Real-World Data: Assessing Electronic Health Records and Medical Claims Data to Support Regulatory Decision-Making for Drug and Biological Products
- Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products

[1] Final guidance documents planned for publication in calendar year 2021 are not included on this list. CDER is not bound by this list of topics, nor required to issue every guidance document on this list. We are not precluded from developing guidance documents on topics not on this list.

[2] New category added since the January 2021 posting

# New RWE Demonstration Projects

**Funding Opportunity Title ('U01'): Exploring the use of Real-World Data to Generate Real-World Evidence in Regulatory Decision-Making**

**N=31 applications received; n=4 applications funded**

| Number | Applicant | Project Title |
|---|---|---|
| 1 U01FD007213-01 | Brigham and Women's Hospital | Enhancing evidence generation by linking RCTs to RWD |
| 1 U01FD007206-01 | Genentech-UNC | Applying novel statistical approaches to develop a decision framework for hybrid RCT designs, combining internal control arms with data from RWD sources |
| 1 U01FD007172-01 | Verantos, Inc. | Transforming RWE with *U*nstructured and *S*tructured data to advance *T*ailored therapy (TRUST) |
| 1 U01FD007220-01 | Critical Path Institute | Advancing standards and methodologies to generate RWE from RWD through a neonatal pilot project |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