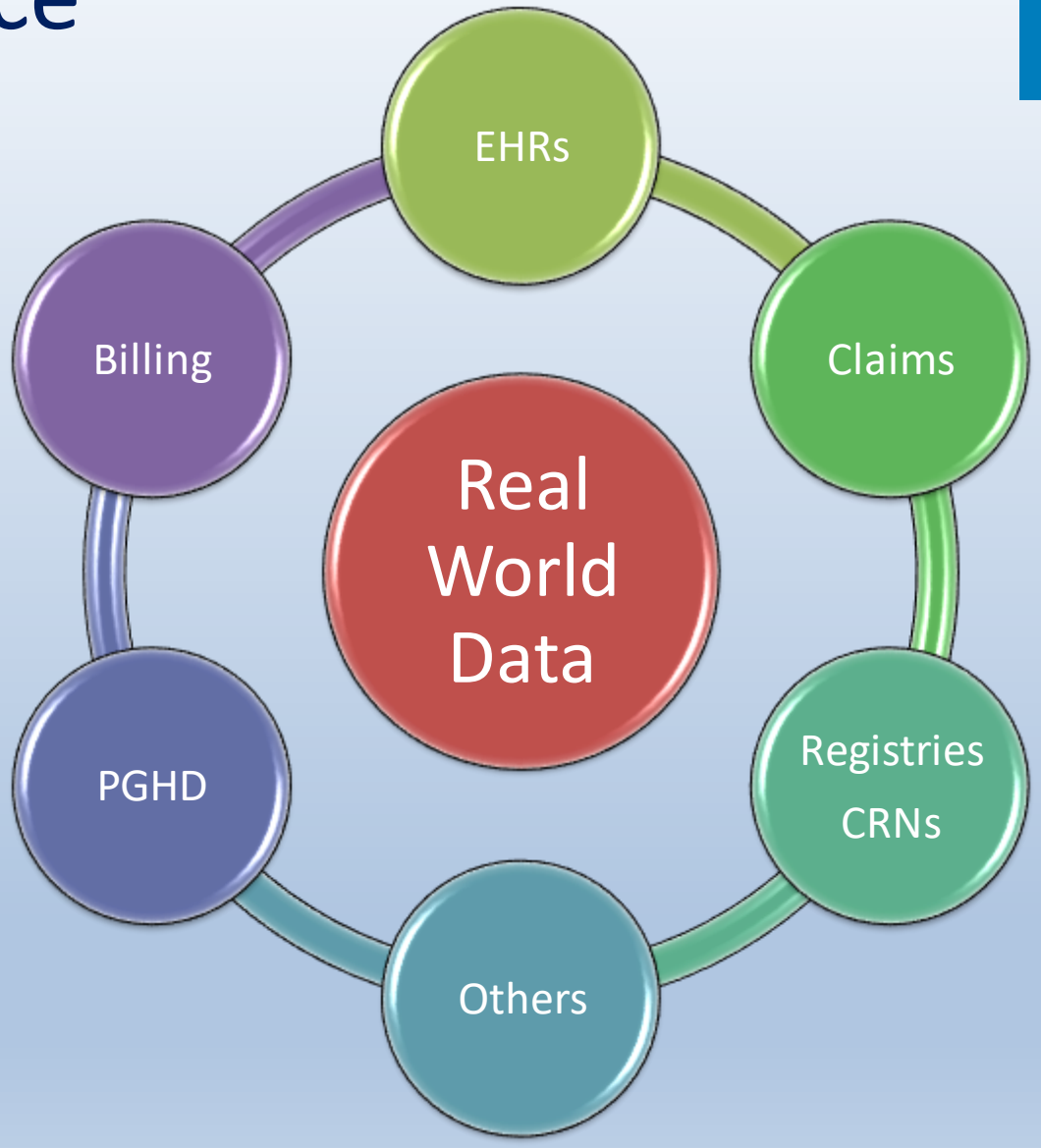
Uses of Real World Evidence

Erin Cutts
Center for Devices and Radiological Health (CDRH)
US Food and Drug Administration (US FDA)

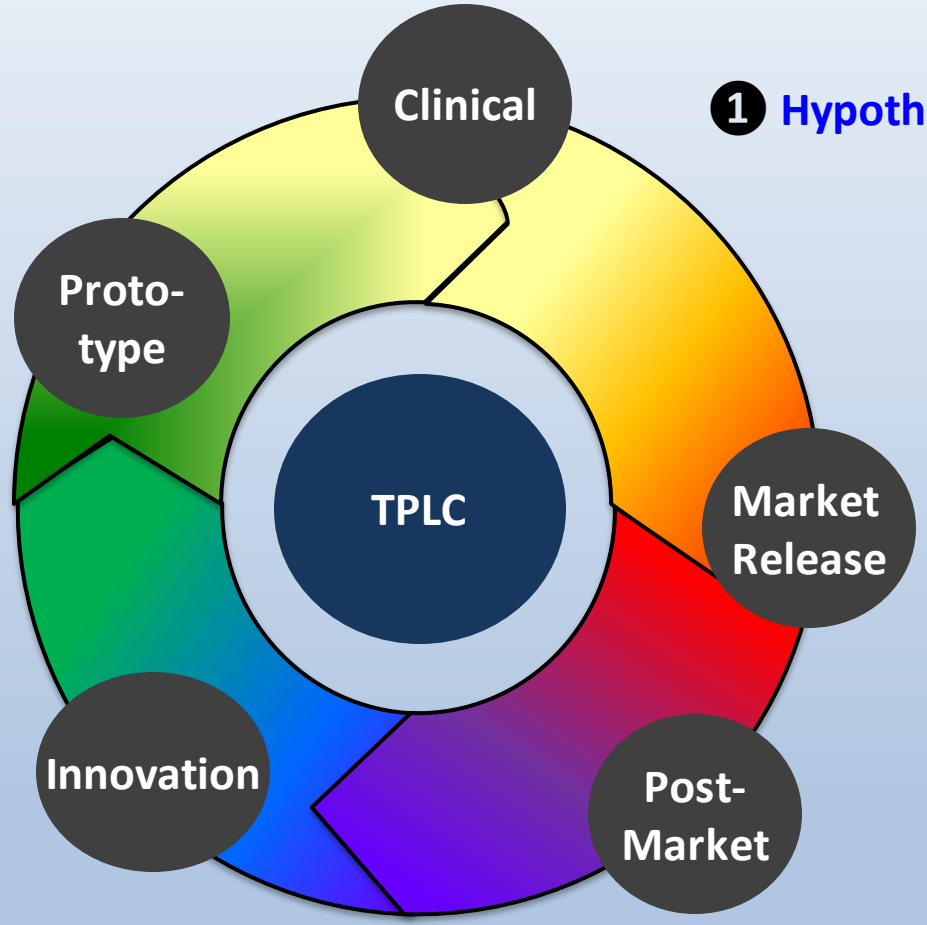
Real-World Data & Evidence

Real World Data *are data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources.*

Real World Evidence *is the clinical evidence regarding the usage, and potential benefits or risks, of a medical product derived from analysis of RWD.*



Potential Usages of RWE for Total-Product Life-Cycle Device Evaluation



1. **Hypothesis Generation** (e.g. treatment effect estimation for comparative studies)
2. **Inform prospective trial design**
3. RWE as a **control arm** for a clinical trial
4. Real-world data source as a **platform to support a clinical trial** (data collection / randomization)

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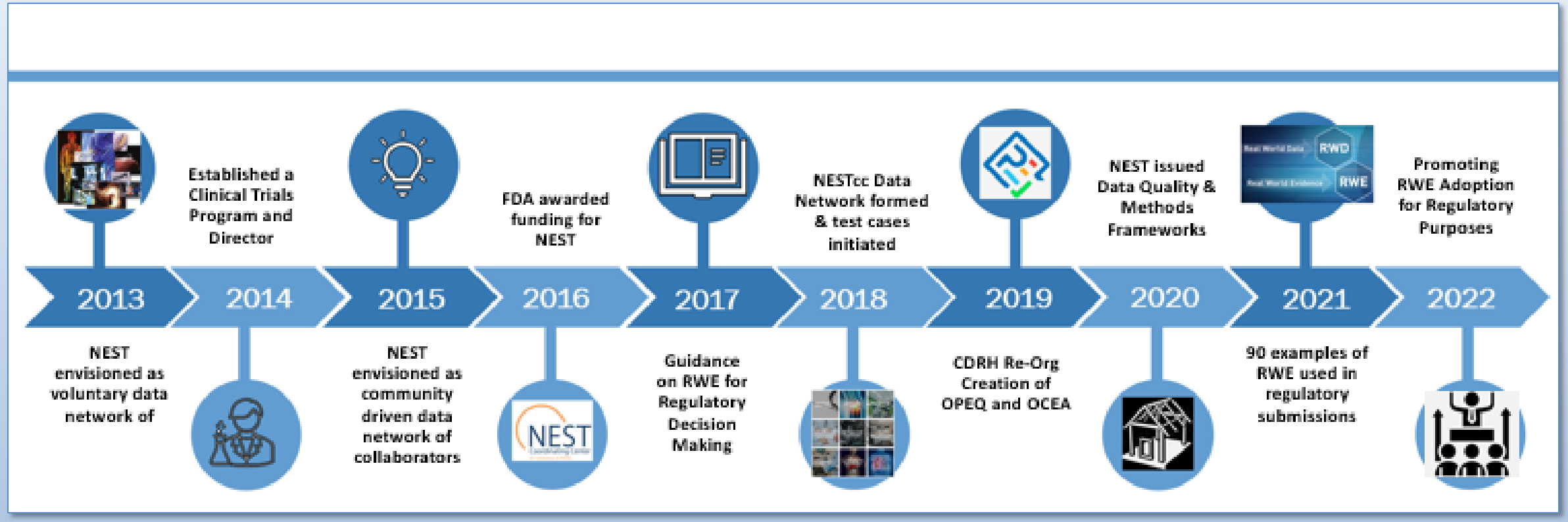
5. Data collection framework for **postmarket evidence generation (e.g. post-approval studies)**
6. Public health surveillance
7. Generate evidence to support **indication expansions** and **future innovation**

Benefits of Real-World Data Sources

- Understand device performance in real-world environment to inform benefit-risk
- Collect outcomes not always feasible in traditional trials
- Opportunities to partner w/patients in new ways
- Reduced time/cost to answer important questions
- Inform future device modifications and new technology development
- Better align evidence generation with innovation cycles

Real-World Evidence Program in CDRH



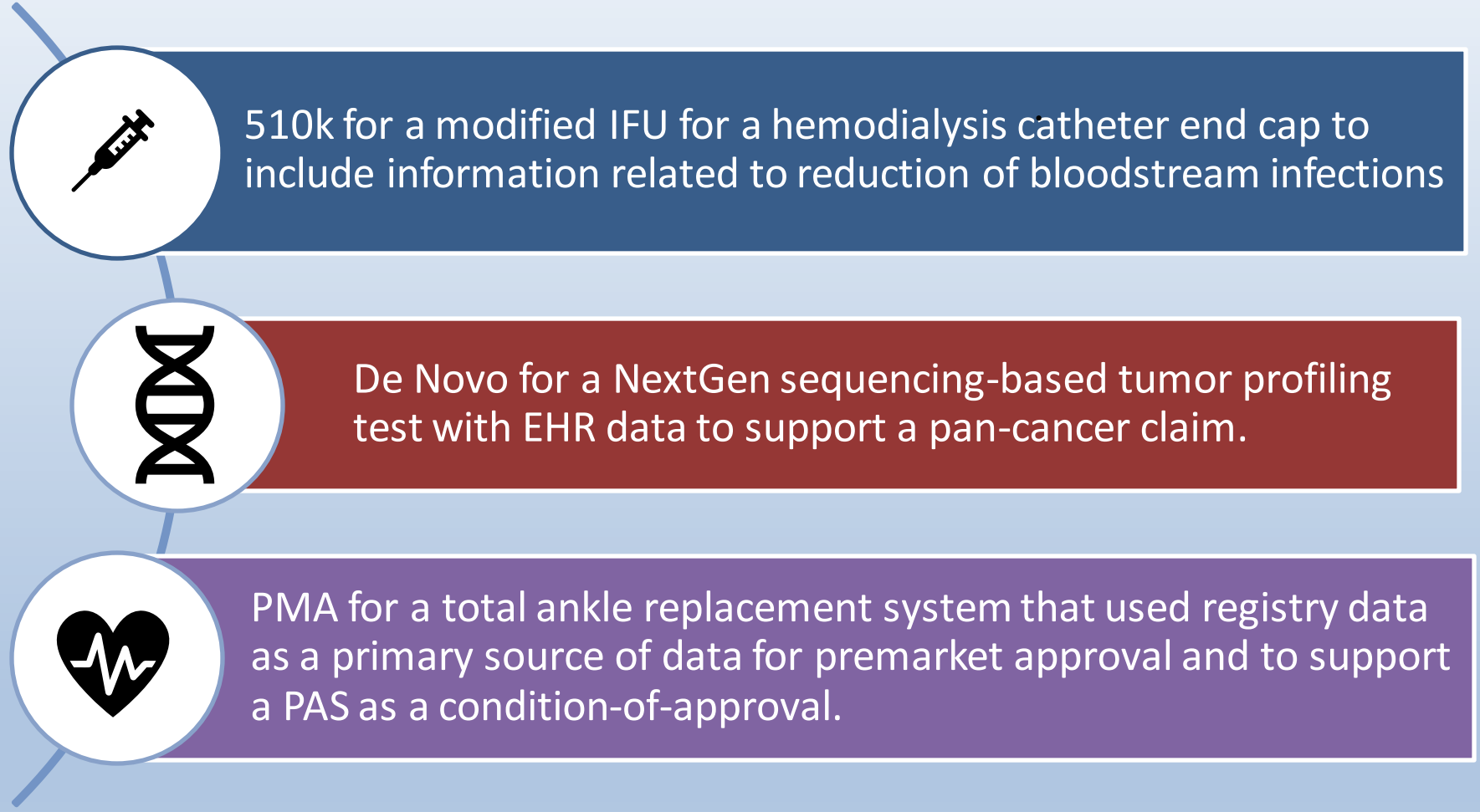
Leading the evolution of the clinical evidence landscape through:

- Optimizing Infrastructure to Develop Real-World Evidence (RWE)
- Promoting RWE Adoption and Use for Regulatory Purpose

Promoting RWE Adoption and Use for Regulatory Purposes: *Achievements*

- RWE Guidance for Medical Devices
  - Potential uses of RWD
  - Characteristics of RWD
    - Relevance
    - Reliability
  - Examples
- Compiled and published 90 publicly available, illustrative examples of RWE used in regulatory submissions FY ’12-’19
  - Variety of submission types, data sources, purposes, & TPLC stage
- Continuous staff training on RWE

A Few RWE Case Examples



CDRH Commitment to RWD/RWE

MDUFA PERFORMANCE GOALS AND PROCEDURES, FISCAL YEARS 2023 THROUGH 2027

F. Real World Evidence (RWE)

The Agency will use user fee revenue for the continued development of Real-World Data (RWD) and RWE methods and policies to advance regulatory acceptance for premarket submissions, including expanded indications for use and new clearance/approval of new devices, and clarify related reporting requirements.

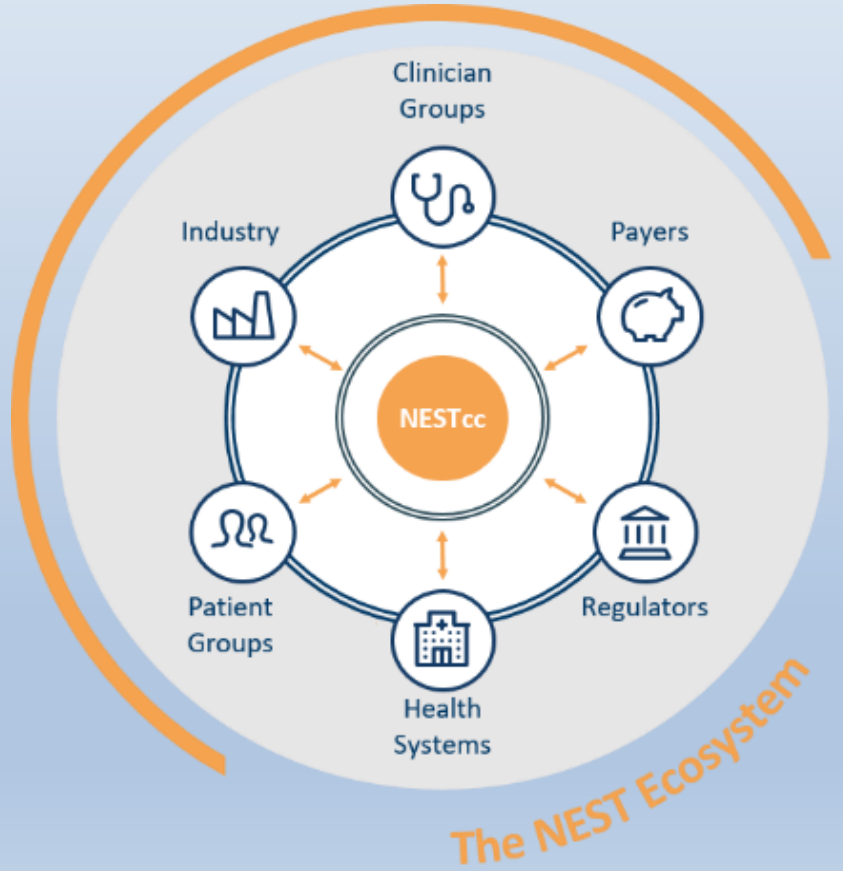
1. FDA will update the 2017 guidance document Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices to provide more clarity on:
    a. Least burdensome general expectations on what is needed to demonstrate the "Fit-for-Purpose of RWD" for premarket regulatory purposes, including expanded indications for use and new clearance/approval of new devices;
    b. More information, including generalized examples, on previously used and accepted methodologies; and
    c. Best practices for RWE review.
2. FDA will continue to advance CDRH's RWD/RWE Training program for FDA review teams including the medical review staff. Topics will include best practices for RWE review and when to engage with CDRH RWE subject matter experts.
3. FDA will provide transparent program development updates and financial accounting of User Fee revenue specifically intended for the activities in this section.
    a. FDA will update stakeholders on the RWE program activities at two or more open public meetings during the course of MDUFA V.
    b. FDA hiring of internal experts to support the review of RWD/RWE-related submissions will be tracked.
    c. If any portion of the user fee funding is distributed to the National Evaluation System for health Technology (NEST), the funding should be used to transparently:
        i. Support the development of RWD resources to facilitate appropriate access for research studies;

National Evaluation System for health Technology (NEST)

A voluntary data network of collaborators able to efficiently consolidate Real-World Evidence (RWE) from clinical registries, electronic health records, medical billing claims, patient-mediated data, and other sources to inform medical device development and evaluation, and to support regulatory decision-making throughout the total product lifecycle (TPLC).

- 21 Test Cases Conducted
  - Explored feasibility
  - Identified areas where NESTcc could reduce costs
  - Independent assessment of Test Cases revealed lessons learned

- Premarket Implementation Cases Ongoing
  - Multistakeholder involvement to develop RWE through the NEST ecosystem to support a premarket submission



Medical Device Active Surveillance System

- Request for Information (RFI)
  - Published in Feb. 2023
  - Inform the next evolution of the medical device active surveillance system
  - Understand the safety of medical devices as used within clinical practice, by achieving:
    - *Better* data capture
    - *Detection* of potential safety signals
    - Timely *assessment* leading to actionable findings

CDRH Fosters the Development and Use of High-Quality Real-World Evidence

- Collaborating with MDIC and NEST on framework documents
  - Active Surveillance Roadmap
  - Active Surveillance Methods
  - Data Quality Framework
- CDRH engages with 12 National CRNs and 4 International Registry Consortia
  - Include over 100 national or regional registries from 45 countries

Support Total Product Life Cycle Reviews

- Experts within CDRH provide support and training in Good Clinical Practice, Data Quality, Study Design, Analytic Methodology, and knowledge of specific RWD sources
- Leverage high-quality RWD sources to replace traditional post-approval studies and efficiently address postmarket questions
- Advance active surveillance to improve device safety

U.S. FOOD & DRUG ADMINISTRATION

Thank you!