NEST

National Evaluation System for health Technology

Coordinating Center

Rachael Fleurence, PhD

NESTcc Executive Director

September 19, 2017

SESSION OBJECTIVES

Successes in real-world evidence (RWE) collection and use

Explore how these examples could apply to future applications

The New York Times

BUSINESS DAY

F.D.A. Seeks to Tighten Regulation of All-Metal Hip Implants

By BARRY MEIER JAN. 16, 2013

After an estimated 500,000 patients in the United States have received a type of artificial hip that is failing early in many cases, the Food and Drug Administration is proposing rules that could stop manufacturers from selling such implants.

Under the proposal, which the agency is expected to announce on Thursday, makers of artificial hips with all-metal components would have to prove the devices were safe and effective before they could continue selling existing ones or obtain approval for new all-metal designs.

The Washington Post

The battle over Essure

Some people see a breakthrough in female contraception. Others see a dangerous medical device.

To Your Health

FDA toughens warning that uterine procedure can spread cancer

By Brady Dennis November 24, 2014

The Food and Drug Administration strengthened its warnings Monday against the use of a controversial uterine surgical technique, recommending that doctors avoid using laparoscopic power morcellators to remove uterine growths in the vast majority of women because of the risk of spreading hidden cancers.

DRUGS VS. DEVICES

NEST
National Evaluation System for health Technology
Coordinating Center

Regulatory and clinical context for devices is different from drugs with implications for availability and use of RWD/RWE.

MEDICATIONS

- Approval: At least two, very large (pivotal) RCT
- Adverse Events required in approval RCT
- Massive Post-Approval safety studies required
- National Drug Code (NDC) is universal in EHR and claims since 1972, permits surveillance of AE

MEDICAL DEVICES

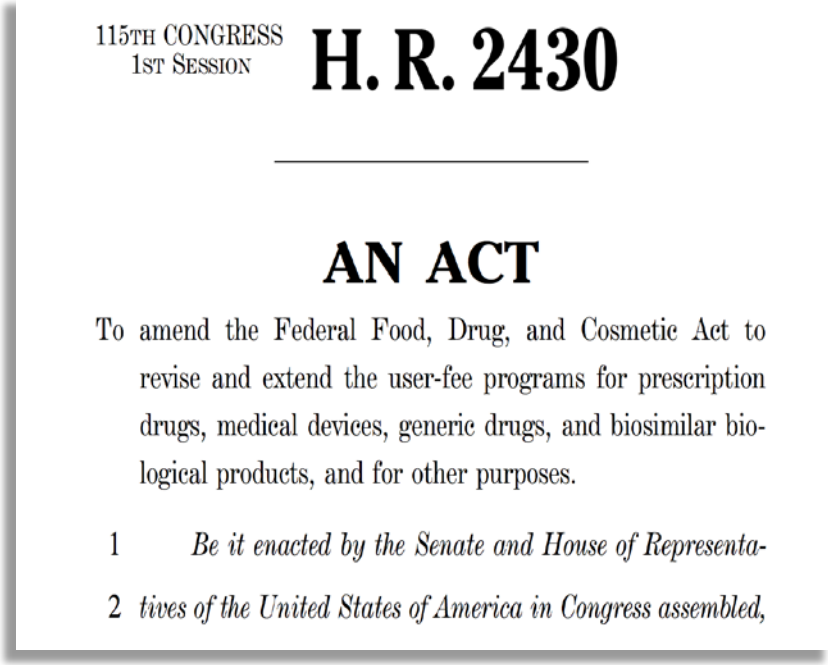
- Approval pathways: PMA and 5-10K
- Single (small) study required for PMA
- Variable requirements for Post Approval Studies (PMA only)
- Universal device identifier (UDI) implemented in 2015
- Impact of “Learning Curve” on device performance
- Current MDR system in need of overhaul (lack of denominator)

Source: Fred Resnic, Leahy Clinic; Faris&Shuren, NEJM 2017

NEST
National Evaluation System for health Technology
Coordinating Center

There is consensus among medical device ecosystem players that RWD/RWE can be transformational in the device space.

- Support a **"pre-post-market shift,"** as confidence in availability of robust post-approval data on safety and effectiveness increases
- Improve the Medical Device Reporting system with implementation of **automated surveillance methods**
- Provide US patients with access to **innovative, safe, and effective** devices more quickly



115TH CONGRESS
1ST SESSION

H. R. 2430

AN ACT

To amend the Federal Food, Drug, and Cosmetic Act to revise and extend the user-fee programs for prescription drugs, medical devices, generic drugs, and biosimilar biological products, and for other purposes.

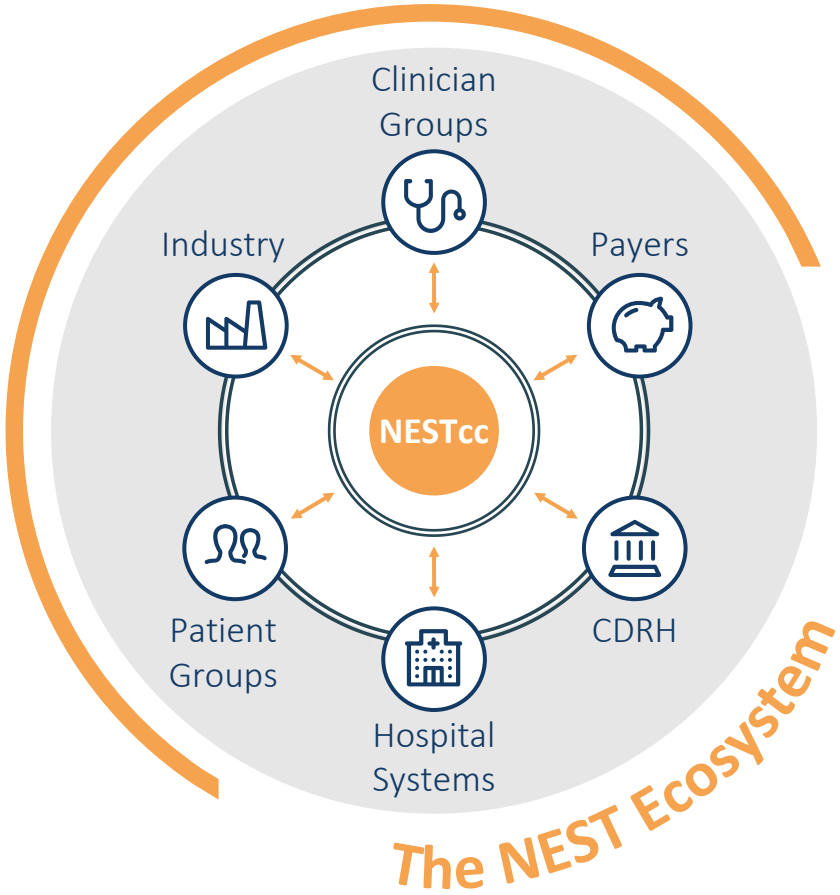
Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

FDA Reauthorization Act of 2017 (FDARA)

NESTcc'S ROLE IN THE ECOSYSTEM

NESTcc should serve as a catalyst to support the timely and reliable development of high-quality RWE.

- Establish **partnerships** with a range of organizations, companies, and collaborations that provide data and analytics solutions
- Set **data quality and methods standards**, provide certifications, and conduct evaluations
- Offer **products and services** of value to key stakeholders in the ecosystem to support a sustainable NESTcc



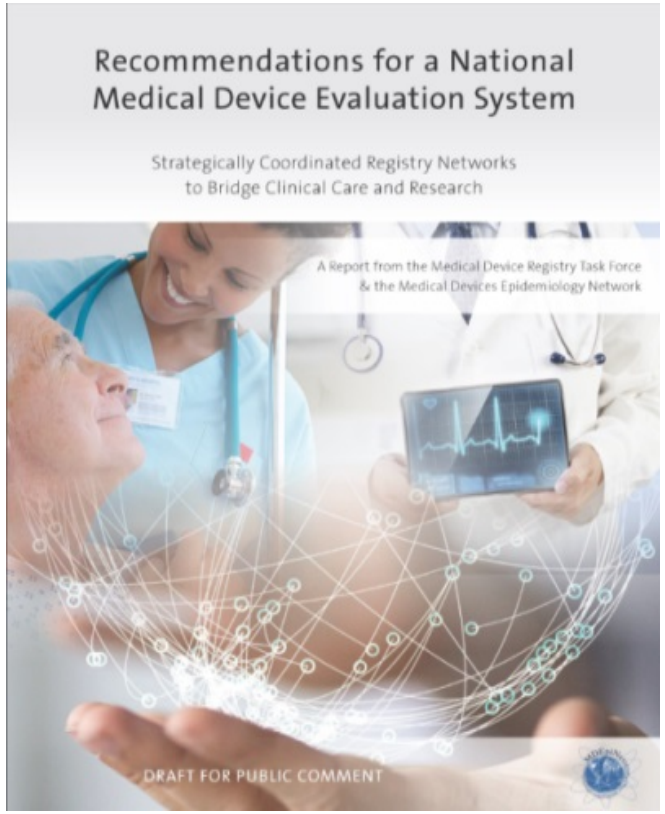
THE ROLE OF REGISTRIES

Registries have historically played an important role in the regulatory space for medical devices.

Examples of high-quality registries:

- Transcatheter Valve Therapy Registry (TVT-R), American College of Cardiology and the Society of Thoracic Surgeons
- National Cardiovascular Disease Registries, American College of Cardiology
- Vascular Quality Initiative (VQI), Society of Vascular Surgeons (SVS)
- International Consortium of Orthopedic Registries (ICOR)

New developments in **Coordinated Registries Networks** linking existing registries with claims data, EHRs have started, including international registries



Source: MDTRF Task Force Report, August 2015

USE OF RWD/RWE IN DECISION-MAKING

Use of RWD/RWE in device registries has already demonstrated success.

- Provide high-quality, fit-for-purpose data
- Support observational and randomized interventions at lower costs (e.g., TASTE trial)
- Deploy algorithms for automated safety surveillance (e.g., DELTA study)

Registries are currently the main source of RWE decisions by FDA-CDRH.

National Registries in CDRH "RWE" decisions (2017):

15 Post-Approval Studies

1 Continued Access Study

8 Pre-Market Studies (including labeling expansion)

7 Post-Market Surveillance Studies (522)

International Registries are being leveraged for:

3 Post-Approval Studies

Source: FDA-CDRH Staff

Source: TASTE Trial, Fröbert, NEJM 2013; DELTA, Resnic NEJM 2017

CHALLENGES

Device-Specific Challenges

- High cost of developing and maintaining registries
- Registries cannot be developed for all devices and all disease areas
- Limited availability of Unique Device Identifier in EHRs or claims
- Impact of operator characteristics and learning curve

Ecosystem-Wide Challenges

- Data quality issues and lack of standard data capture
- Methods issues
- Data linkage issues
- Lack of data
- Administrative issues
- Privacy and security concerns

WHAT DOES THE FUTURE HOLD?

There is no question that the future lies in the use of RWD and generating robust RWE. How far off that future lies is the question.

Tremendous progress and learnings in the past decade from key initiatives:

Sentinel
Patients Like Me
PCORnet
All of Us initiative
Salford Lung Study
PCORnet Adaptable Trial
NEST

Many strides in technical (EHR "adoption", cloud) and cultural aspects (patient involvement) of the transformation of health care

Current barriers are still real—how quickly we overcome them will be a factor of time, resources, and leadership