NEST Coordinating Center

An initiative of MDIC

ADEPT 6: NESTcc & Real-World Evidence

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THE NEED FOR A PEDIATRIC FOCUS

WHAT IS THE PROBLEM?

"Knowledge about device **usefulness, effectiveness, and safety** is largely based on the collective experiences of pediatric clinicians and **hand-me-down evidence from studies in adults**."[1]

DATA AS A SOLUTION

"The use of real-world data captured in electronic health records and other electronic clinical information systems, such as mobile apps, can **rapidly expand the evidence base for pediatric device effectiveness and safety**."[1]

[1] Strengthening the Evidence Base for Pediatric Medical Devices Using Real-World Data
Fleurence, Rachael L. et al.
The Journal of Pediatrics, Volume 214, 209 - 211

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Real-World Data (RWD)

Data generated in the routine course of care, rather than during pre-market research or clinical trials, that takes into account real patient experiences.*

Real-World Evidence (RWE)

Insights gleaned from Real-World Data (RWD) that have the potential to inform and empower patients, accelerate medical device innovation, and improve health care outcomes.*

*View additional information on RWE & RWD from the U.S. FDA here

WHAT HAPPENS TO YOUR DATA?

Healthcare Providers

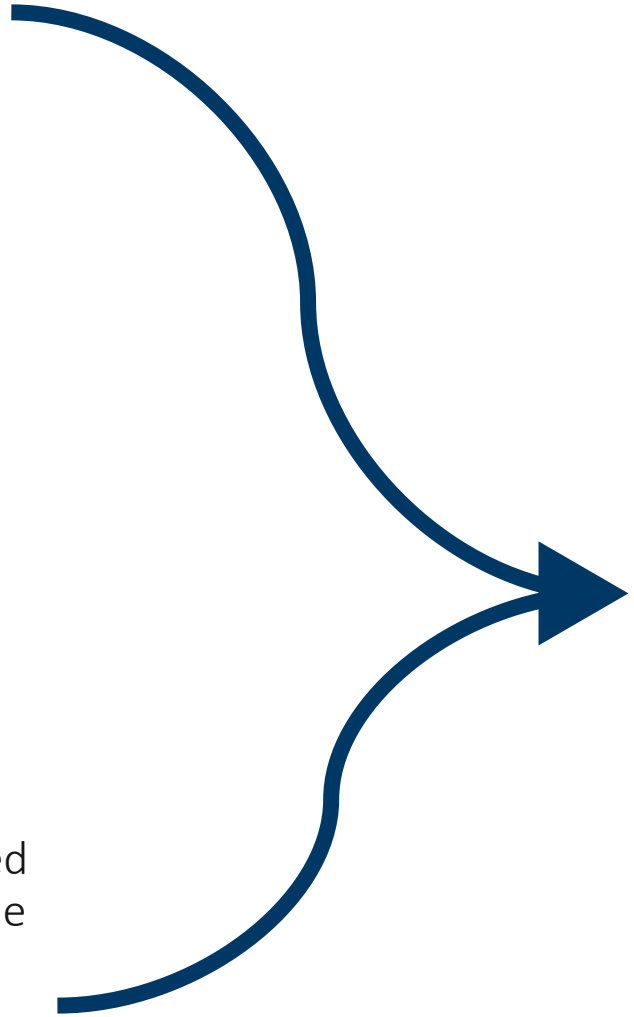
Doctors enter patient medical data into databases (EHR or Registry), such as which patients received a specific implantable device

Mobile Apps (PGD)

Phones and wearable devices track your data, such as steps, REM cycle, or blood sugar

Billing

Medical device data is captured through insurance claims in the billing process



How Is It Used?

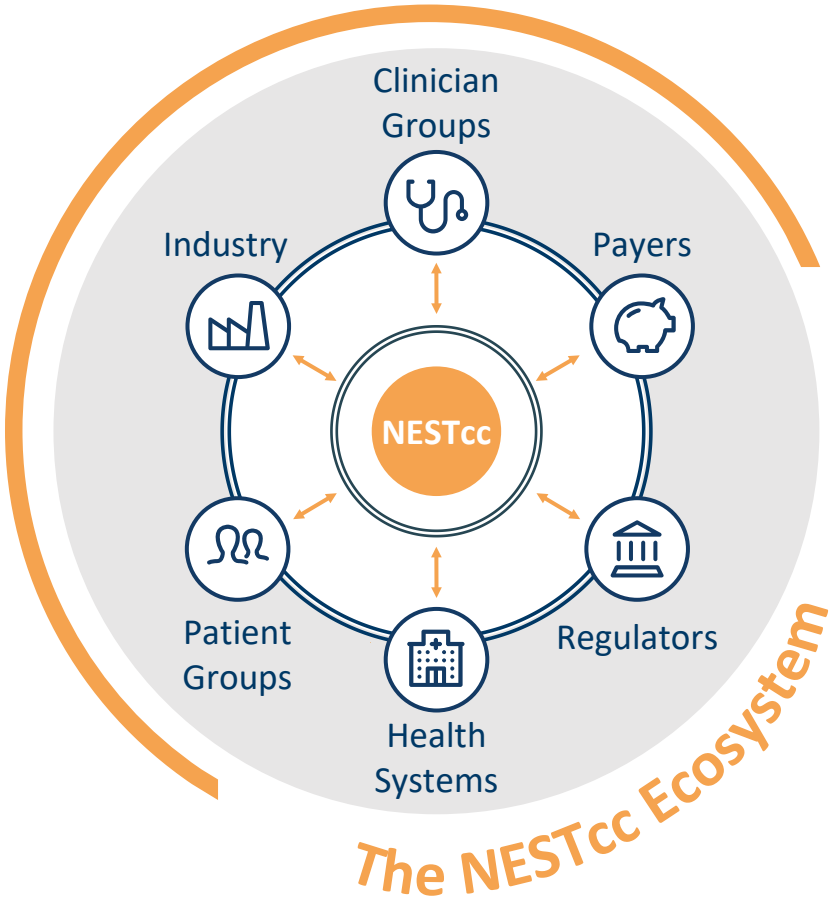
These types of data are studied to leverage current treatments and create new treatments for the pediatric population which are safe and effective

Mission

To accelerate the development and translation of new and safe health technologies, leveraging Real-World Evidence (RWE), and innovative research.

Vision

To be the leading organization within the health technology and medical device ecosystem for conducting efficient and timely high-quality Real-World Evidence (RWE) studies throughout the Total Product Life Cycle (TPLC).



Establishing the NESTcc Data Network

ESTABLISHING THE NESTcc DATA NETWORK

NESTcc has established relationships with 12 Network Collaborators to advance evaluation and use of high-quality Real-World Data (RWD). Additional Network Collaborators will be added in 2020 to increase the capacity and capabilities of the Data Network.

Data Network By-The-Numbers

12
Network Collaborators

195
Hospitals

3,942+
Outpatient Clinics

494M+*
Patient Records

@NESTccMedTech www.nestcc.org

*Does not account for duplicate records

Numbers reflect data as of February 2018

Utilizing the NESTcc Data Network

NESTcc TEST-CASES ADDRESS A RANGE OF DEVICE QUESTIONS

NESTcc's Test-Cases span a wide range of device classes, data sources, and disease areas.

Device Classes

Class I

Class II

Class III

Disease Area

Cardiovascular

Dental

Dermatology

Ear, Nose, & Throat

Mental Health

Oncology

Orthopedics

Respiratory

Surgery

Data Sources

Claims

Electronic Health Records (EHR)

Patient-Generated health Data (PGD)

Registries

The Test-Cases are being kicked off and are anticipated to present final results between January-December 2020.

CARDIOVASCULAR TEST-CASE – CARDIOVASCULAR DEVICE

Project Title	**Characterization and Utilization of Therapeutic Cardiac Devices in Children with Congenital Heart Disease**
Available Data Sources	EHR/Claims
Disease Area	Cardiovascular
Technology of Interest	Cardiovascular Device

Project Aims

- This Test-Case aims to determine if NESTcc could adequately capture a specific stent implantation in pulmonary arteries and systemic veins during congenital heart disease therapy. The stents being studied are of particular interest in pediatric patients.
- In addition, this project will characterize the range of pediatric stent use at participating PEDSnet sites and develop a scalable approach to investigating pediatric device use in PCORnet networks. This study would allow prevalence information to be collected across a consortium of multiple institutions.

Participating Network Collaborators

RESPIRATORY TEST-CASE – POSITIVE AIR PRESSURE (PAP) THERAPY

Project Title	**Structured interviews of Lived ExperiencE in Patients (SLEEP study) Obstructive Sleep Apnea and Central Sleep Apnea**
Available Data Sources	Claims; Electronic Health Records (EHR); Patient-Generated health Data (PGD)
Disease Area	Respiratory
Technology of Interest	Positive Air Pressure (PAP) Therapy

Project Aims

- The objective of this Test-Case is to conduct a prospective study of patient-reported outcomes (PROs), patient preferences, and patient experiences to gather PGD from patients with Obstructive Sleep Apnea (OSA) and Central Sleep Apnea (CSA).
- The study will aim to identify patients' preferences related to the benefits, challenges, risks, and side effects perceived when utilizing PAP management of OSA and CSA. Additionally, the study will assess PAP design or delivery characteristics that patients' perceive would potentially benefit them the most to improve therapy.

Participating Network Collaborators

Active Surveillance

NESTcc ACTIVE SURVEILLANCE ACTIVITIES

NESTcc received targeted funding from FDA and formed a Task Force to achieve real-time active surveillance.

Active medical device surveillance will better protect patients by continuously generating, accessing, and evaluating large data sets on device performance and clinical outcomes associated with device use in routine clinical practice.

The multi-stakeholder Active Surveillance Task Force represents Network Collaborators, providers, payers, industry, patient groups, and FDA.

A Roadmap is currently under development and will be issued for public comment in Spring 2020.

Ensuring High-Quality Data & Analysis Methods

ADVANCING DATA QUALITY & METHODS

NESTcc has established multi-stakeholder Data Quality and Methods Subcommittees to create Frameworks and govern high-quality data collection and analysis methods to **ensure device safety and effectiveness for patients, including pediatric populations**.

DATA QUALITY SUBCOMMITTEE

Charge:

Design a process by which NESTcc Network Collaborators can demonstrate their aptitude with the NESTcc Data Quality Framework.

METHODS SUBCOMMITTEE

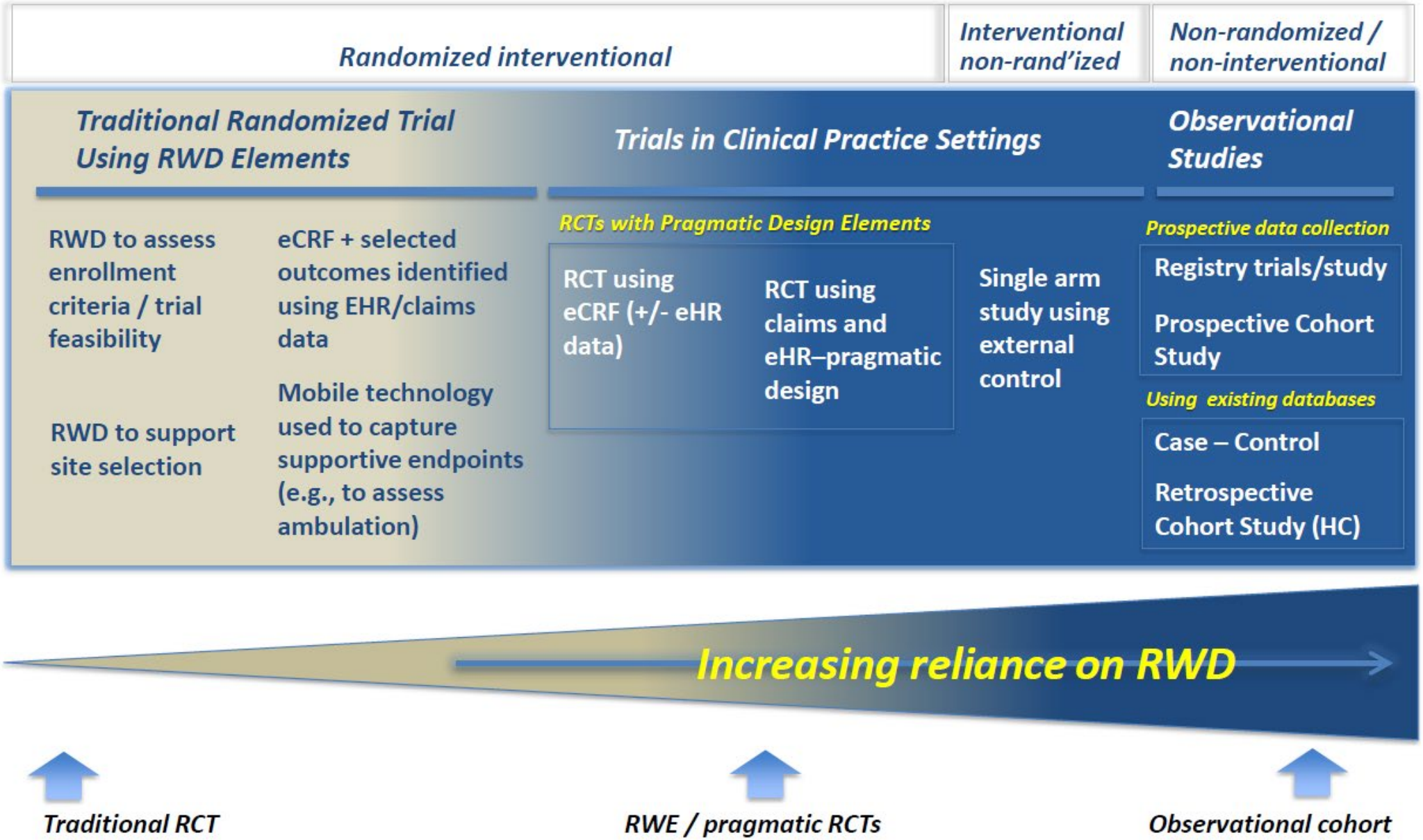
Charge:

Develop a framework for RWE protocol development and “best practices” in methodology for RWE device studies across the TPLC.

These subcommittees will complete initial versions of Data Quality & Methods Frameworks by November 2019.

Potential Use of RWD/RWE

POTENTIAL USE OF REAL-WORLD DATA



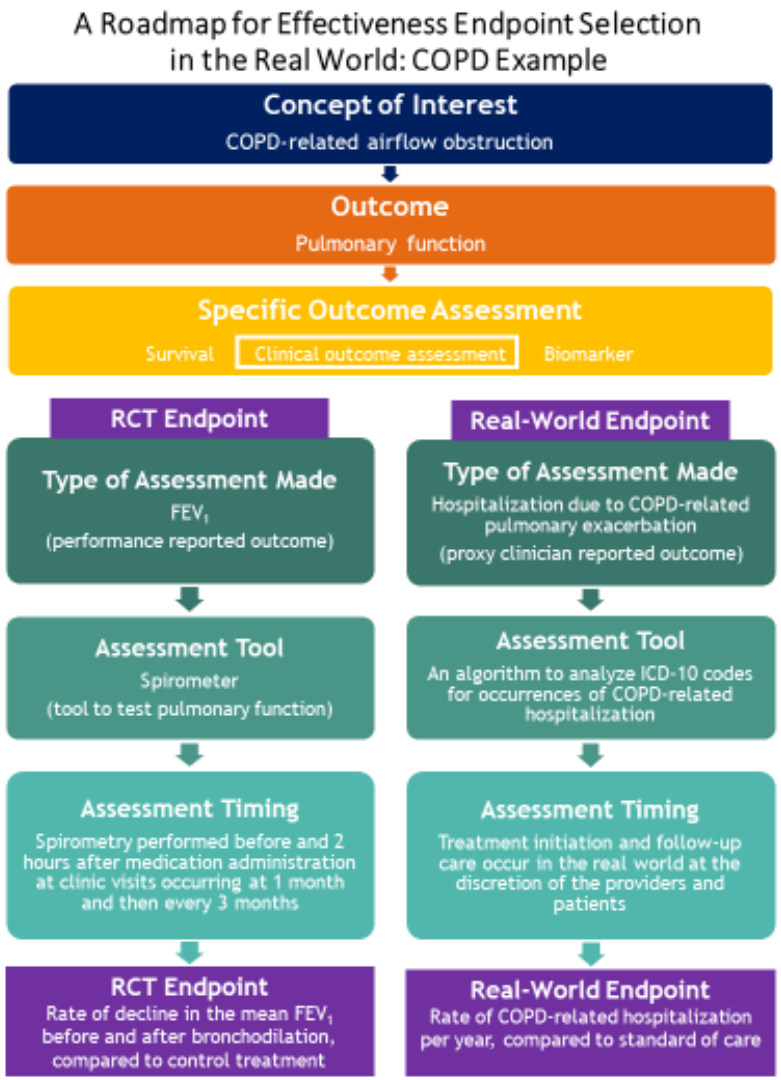
How do we develop novel endpoints?

- For a treatment benefit to be meaningful, it must decrease negative symptoms that matter most to patients (type of benefit AND magnitude of benefit)
- Treatment benefit is evaluated through an "endpoint" (a precisely defined variable intended to reflect an outcome of interest that is statistically analyzed to address a particular research question)

- Reliable and well-defined endpoints include:
  - Type of assessment (what are you measuring?)
  - Assessment tool (what are you measuring with?)
  - Timing of assessment
  - Other relevant details (e.g. how multiple assessments are to be combined)

- Real-World studies often aim to operationalize the same endpoints as clinical trials:
  - Feasibility
  - Relevant for use in selected population (RCTs vs. routine clinical care)

Duke-Margolis – "Establishing Guideposts for Developing Real-World Endpoints"



How can RWD be used to develop novel endpoints?

- RWD complements RCT data in that it may be collected in a broader, more representative population and over an evolving standard of care
- Instead of only relying on established endpoints used in RCTs, an alternative approach to selecting endpoints in real-world studies is to harness the value of RWD:
  - E.g. assessment tools (e.g. PGD) that are used in the real world can be leveraged to develop novel endpoints
  - Opportunity to generate evidence on clinical concepts/outcomes that are more meaningful to patients (and providers) that may not be studied in RCTs or can be studied over a longer time frame compared to RCTs
  - Real-World endpoints should always be tested and validated!

Duke-Margolis – "Establishing Guideposts for Developing Real-World Endpoints"

Engage with NESTcc

The NESTcc Quarterly Newsletter is distributed on the first Tuesday of each quarter.

- The Newsletter contains upcoming dates and links to news items and publications from the previous quarter.
- The newsletter is available to the public and can be subscribed to here.

NESTcc Quarterly Newsletter

January - March 2019

Mark Your Calendars

Tuesday, May 28 - Friday, June 14, 2019: Public Comment Period for NESTcc Data Quality & Methods Frameworks

Wednesday, July 31, 2019: Final Version 1 of NESTcc Data Quality & Methods Frameworks

We are excited to launch our first Quarterly Newsletter, reflecting on key NESTcc news and announcements from the previous quarter and highlighting important upcoming dates. Any feedback is welcome and can be sent to nestcc@mdic.org.

January

NESTcc: A Look Ahead at What's Next for NESTcc in 2019

CONNECT WITH NESTcc

Explore opportunities to connect with NESTcc online with the following resources:

NEST
Coordinating Center
An initiative of MDIC

www.nestcc.org

@NESTccMedTech

nestcc@mdic.org

Extra Slides

Electronic Health Records (EHR)

Patient data entered by healthcare providers in a range of healthcare delivery settings for routine care purposes.

Claims

Encounter data from a range of healthcare sites (inpatient hospital, outpatient hospital, emergency room, physician's office, surgery center, etc.) describing diagnoses, treatments, and billed and paid amounts for reimbursement purposes.

Registry

An organized system that uses observational study methods to collect uniform data (clinical and other) to evaluate specified outcomes for a population defined by a particular disease, condition, or exposure, and that serves a predetermined scientific, clinical, or policy purpose(s).

Patient-Generated health Data (PGD)

Data that is generally captured outside of the clinical setting through patient reported outcomes (PRO), in-home or point-of-care monitoring devices, wearable technologies, fitness trackers, etc.

NESTcc TEST-CASES

TOTAL-PRODUCT LIFE CYCLE (TPLC) ALIGNMENT	DISEASE AREA	TECHNOLOGY OF INTEREST	DATA SOURCES
Pre-market Submission	Dermatology	Wound Closure Devices (topical skin adhesives, staples, sutures)	Claims; Electronic Health Records (EHR)
Label Expansion	Vascular	Endovascular stent	EHR; Registry
Label Expansion	Cardiology	Catheters used in Rx of Cardiac Arrhythmias	EHR
Label Expansion	Cardiology	Mechanical Aortic Heart Valves	EHR; Registry
Label from General to Specific Indication	Surgery	Microwave Ablation Device	EHR
Post-market Surveillance	Orthopedics	Total Knee Arthroplasty	Claims; Registry
Post-market Surveillance	Dental	Craniomaxillofacial Bone Distractors	EHR
Post-market Surveillance	Orthopedics	Intervertebral Lumbar Body Fusion Devices	Claims; EHR

Pediatric Test-Case

NESTcc TEST-CASES

TOTAL-PRODUCT LIFE CYCLE (TPLC) ALIGNMENT	DISEASE AREA	TECHNOLOGY OF INTEREST	DATA SOURCES
Pre-Market Submission	Oncology	Lung Cancer Diagnostic	Electronic Health Records (EHR); Registry
Pre-Market Submission	Cardiovascular	Electrode Renal Denervation System	EHR
Pre-Market Submission; Label Expansion	**Cardiovascular**	**Cardiovascular Device**	**Claims**
Pre-Market Submission; Post-Market	Orthopedics	Annular Closure Device	Claims
Pre-Market Submission; Label Expansion; Surveillance	Orthopedics	Objective Performance Criteria (OPC) for Knee and Hip Implants	Claims; Registry
Post-Market; Surveillance; Coverage	Cardiology	Apple Watch Diagnostic + mHealth	EHR; Patient-Generated health Data (PGD)
Pre-Market Submission	**Ear, Nose, and Throat**	**Ear Tubes**	**Claims; EHR**
Post-Market; Surveillance	Cardiovascular	Cardiac Device Leads	Claims; EHR
Surveillance	Stress Urinary Incontinence	Synthetic Mesh Sling	EHR; Registry
Surveillance	Stress Urinary Incontinence	Urinary Mesh Software mHealth	PGD; Registry
Surveillance; Coverage	Mental Health	mHealth for Insomnia	EHR; PGD
Coverage	**Respiratory**	**Positive Air Pressure, PAP Therapy**	**Claims; EHR; PGD**

Pediatric Test-Case

DERMATOLOGY TEST-CASE – WOUND CLOSURE

Project Title	Comparative Effectiveness of Alternative Approaches for Wound Closure
Data Sources	Claims; Electronic Health Records (EHR)
Disease Area	Dermatology
Technology of Interest	Wound Closure

Project Aims

- There are three methods for closure of wounds that result from surgeries or trauma: sutures, staples, and skin adhesives.
- The purpose of this project is to skin closure approaches in terms of the types of skin wounds and the patient populations they are used for, along with short-term outcomes, such as need for additional procedures, wound dehiscence, and health services use.

Participating Network Collaborators

DENTAL TEST-CASE – CRANIOMAXILLOFACIAL DISTRACTORS

Project Title	**Developing Capacity to Conduct Proactive Post Marketing Safety Surveillance of Craniomaxillofacial Distractors Using Electronic Health Record Data**
Data Sources	Electronic Health Records (EHR)
Disease Area	Dental
Technology of Interest	Craniomaxillofacial Distractors

Project Aims

- This Test-Case will assess the feasibility of using Real-World Data (RWD) captured through the NESTcc Data Network to conduct proactive post-market surveillance for safety with devices used in pediatric populations.
- This Test-Case seeks to determine the feasibility of using RWD captured through PEDSnet, a NESTcc Data Network Collaborator, to conduct proactive post-market surveillance for safety and effectiveness for CMF distractors.

Participating Network Collaborators

PEDSnet
A Pediatric Learning Health System

ORTHOPEDIC TEST-CASE – INTERVERTEBRAL LUMBAR BODY FUSION DEVICES

Project Title	**Developing Capacity to Conduct Proactive Post Marketing Safety Surveillance of Intervertebral Body Fusion Devices Using Electronic Health Record Data**
Available Data Sources	Claims; Electronic Health Records (EHR)
Disease Area	Orthopedic
Technology of Interest	Intervertebral Body Fusion Devices

Project Aims

- This Test-Case will assess the feasibility of using Real-World Data (RWD) captured through the NESTcc Data Network to conduct proactive post-market surveillance for safety for Class II medical devices.
- Specifically, this Test-Case will conduct proactive post-market surveillance for safety and effectiveness of lumbar interbody systems captured within Lahey Hospital and Medical Center (Lahey), a NESTcc Data Network Collaborator.

Participating Network Collaborators

PEDSnet
A Pediatric Learning Health System

Lahey Hospital & Medical Center

EAR, NOSE, & THROAT TEST-CASE – EAR TUBES

Project Title	**Pediatric Clinical and Health Services Outcomes following Tympanostomy Tube Insertion**
Available Data Sources	Claims; Electronic Health Records (EHR)
Disease Area	Ear, Nose, & Throat (ENT)
Technology of Interest	Ear Tubes

Project Aims

- The purpose of this project is to describe the clinical and health services outcomes of tympanostomy tube insertion (TTI) with and without tonsillectomy and adenoidectomy.
- This study will be the largest ever conducted on TTI, providing the most in-depth evaluation of the clinical and health services outcomes for this procedure. The study's findings are anticipated to be of great interest to patients and their families, primary care physicians, ENT surgeons, and health system leaders who deal with the real-world issues related to ear infections and their management.

Participating Network Collaborators