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NESTcc Call For Concepts Public Webinar

Targeted Real-World Evidence Test-Cases:
Leveraging Patient-Generated health Data

July 31, 2018

CALL FOR CONCEPTS WEBINAR AGENDA



Welcome

NESTcc Overview and Current Activities

Review of Call for Concepts for Targeted Test-Cases



Resources:

*Open Opportunity for RWE Test-Cases
Leveraging Patient-Generated health Data
[announcement](#) and [submission form](#)*

TERMINOLOGY DEFINITIONS

NESTcc Data Network: A network of organizations to help determine current capabilities, gaps, and priority areas of Real-World Data (RWD) and Real-World Evidence (RWE) for the medical device landscape.

NESTcc Network Collaborator: A range of organizations, companies, and collaborators that will be a resource of high-quality Real-World Data that include but are not limited to: Health Systems, Health Payers, Registries, Patient- or Device-Generated Data, Analytics Offerings, etc. These partnerships will help set data quality and methods standards for clinical studies due to their access to EHR and private claims data as well as linkage data with coverage information.

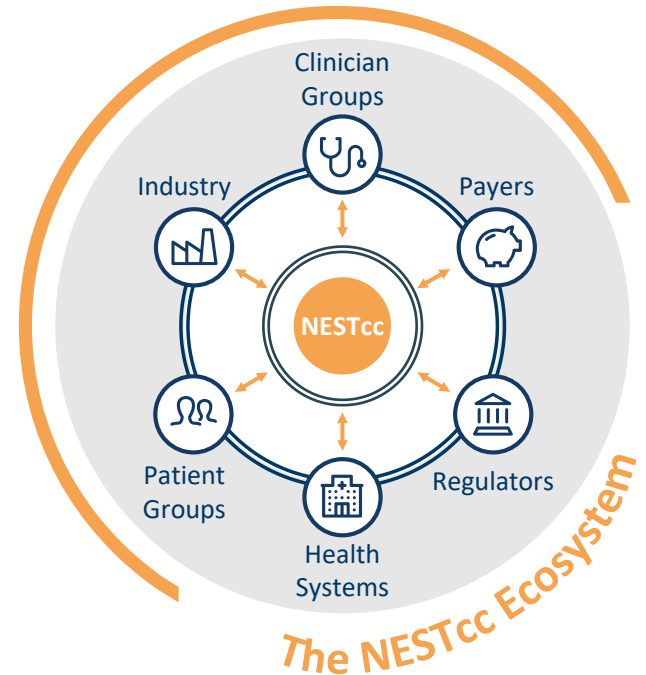


NESTcc Mission Statement

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

History of NESTcc

- 2015** NEST envisioned as a **voluntary data network** of collaborators by Brookings Planning Board and Medical Device Registry Task Force
- 2016** FDA awarded grant for NESTcc to **Medical Device Innovation Consortium (MDIC)**
- 2017** NESTcc multi-stakeholder **Governing Committee** selected
- 2018** Initial **NESTcc Data Network** formed and testing initiated
- 2018** NESTcc **Data Quality** and **Methods Committees** formed
- 2018** NESTcc announces **Second Round of RWE Test-Case Call for Concepts**, and a targeted call for RWE test-cases leveraging **Patient-Generated health Data (PGD)**



NESTcc DEVICE NETWORK TIMELINE 2018

- Establish initial **NESTcc Data Network** with 11 Network Collaborators
- Implement Test-Cases** with manufacturers and NESTcc Network Collaborators
- Establish **Data Quality** and **Methods standards** as well as initial **NESTcc operating processes**
- Identify **optimal uses of NESTcc Data Network and gaps** (e.g. UDI) that need addressing
- Expand NESTcc Data Network** to include additional Network Collaborators



**CURRENT DATA NETWORK
ACTIVITIES**

BUILDING NESTcc'S DATA NETWORK

NESTcc has established relationships with Network Collaborators to advance evaluation and use of high-quality RWD from various sources.

TO DATE, MEMORANDA OF UNDERSTANDING (MOUs) HAVE BEEN SIGNED WITH 12 NETWORK COLLABORATORS:



BUILDING NESTcc'S DATA NETWORK

NESTcc surveyed its Data Network to determine current capabilities, gaps, and priority areas.



Completed Capabilities Surveys

Duke University Health System • HealthCore • Lahey Clinic • Kaiser Permanente • Mayo Clinic • MDEpiNet • Mercy • OneFlorida • PEDSnet • Vanderbilt University • Weill-Cornell Medical Center • Yale New Haven Health System

Survey respondents represent:



150
Hospitals



3,042+
Outpatient Clinics

Patient data represents:



469M+

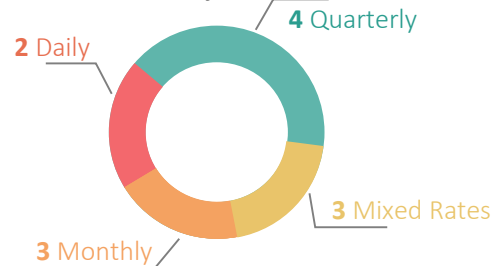
Patient Records

Common data models:

- ✓ I2b2
- ✓ OMOP
- ✓ PCORnet
- ✓ Sentinel

Network Collaborators report regular data

refreshes:



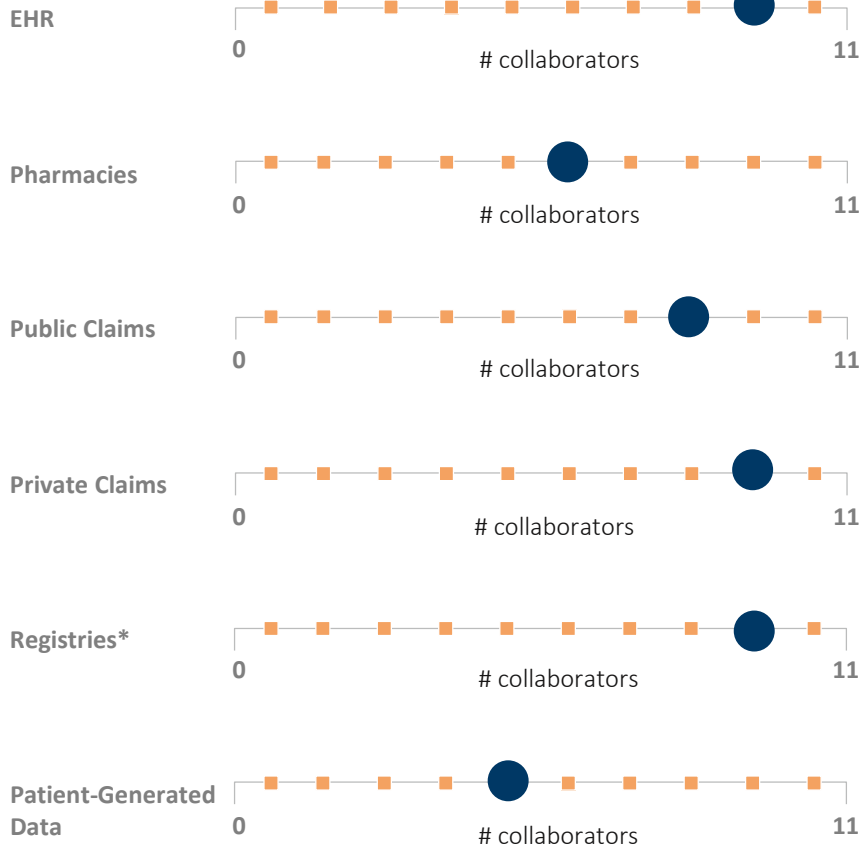
Most cited expertise:

- ✓ Cardiovascular and Cardiac Surgery
- ✓ Women's Health
- ✓ Neurosurgery
- ✓ Gastroenterology
- ✓ Orthopedic

CLOSER LOOK AT NESTcc'S DATA SOURCES

The collaborators comprising the NESTcc Data Network have access to a range of available data sources, including those listed below.

AVAILABLE DATA SOURCES



UDI IMPLEMENTATION



*Registries Include (but are not limited to):

- Anesthesia Quality Institute's National Anesthesia Clinical Outcomes
- Cardiac Catheterization
- Cardiogenic Shock
- Immunization
- Implant registries
- Integrated tumor
- International Consortium Lower-GI
- American College of Surgeons National Surgical Quality Improvement Program
- Oncology
- Pediatric Cardiomyopathy
- Prostate Ablation-Related Energy Devices
- Robotic Surgery
- Society of Thoracic Surgeons National Database
- Society for Vascular Surgery
- Thalassemia Clinical Research Network - Thalassemia Registry
- Vital Records (Birth and Death)

ROUND 1 RWE TEST-CASES: SUMMARY OF PROJECT SLATE

High-level concepts of each test-case currently in development are summarized below:

TOTAL-PRODUCT LIFE CYCLE (TPLC) ALIGNMENT	PRODUCT(S)	AREA
Label Expansion	Stent graft component product	Vascular
Pre-market Submission	Topical Skin Adhesive	Dermatology
Post-market Surveillance	Knee replacement	Orthopedics
Post-market Surveillance	Bone Distractor	Orthopedics
Post-market Surveillance	Spinal decompression device	Orthopedics
Move from General to Specific Indication	Device used for soft tissue ablation	Surgery (Oncology)
Label Expansion	Catheters used in Rx of Atrial Fibrillation	Cardiovascular
Patient Management Clinical Guidelines	Anti-coagulation dosage post mechanical heart valve (MHV) replacement	Cardiovascular

**TARGETED RWE TEST-CASE
CALL FOR CONCEPTS**

CONCEPTS FOR TARGETED TEST-CASE ANNOUNCEMENT

NESTcc is interested in receiving recommendations from industry, health system providers, health payers, academia, and non-profits for test-cases using PGD that could be executed with NESTcc's Network Collaborators.

MISSION OF THE TEST-CASES

Test-case concepts should seek to understand the patient perspective on the health outcomes associated with the treatment of their condition. The evidence generated should constitute useful inputs into regulatory decision-making. The goals of the test-cases include:



Explore the feasibility of generating and using patient-data for regulatory and coverage purposes using the NESTcc Data Network and designing the infrastructure necessary to support this.



Improve understanding of the opportunities and challenges of using the NESTcc Data Network to support studies that capture and evaluate PGD to identify and measure health outcomes that matter most to patients.



Provide learnings to advance patient input and involvement in the regulatory process.



Contribute to NESTcc's development of operational processes (e.g., contracting, IRB, data sharing agreements, publication policies).



CONCEPT DETAILS AND REQUIREMENTS

NESTcc is interested in receiving a broad range of test-cases. The following eligibility criteria were informed by the first round of test-cases submitted.

ELIGIBILITY CRITERIA



Concepts may be submitted by:

- ◆ Medical device, imaging, and diagnostics technology manufacturers
- ◆ Multi-industry collaborations (submitting through an organization or association as a third-party convener)
- ◆ Health system providers
- ◆ Health payer groups
- ◆ Academia
- ◆ Non-profits, including advocacy groups



Projects that are not currently underway



Projects leveraging PGD



SOURCES OF PGD FOR TEST-CASE ANNOUNCEMENT

Test-cases will provide a more comprehensive understanding of the health outcomes that matter to patients and provide insights into how these studies could inform regulatory decision-making.

SOURCES OF PATIENT-GENERATED DATA

Test-case concepts will inform the development of studies that collect and analyze patient-generated data, using quantitative and/or qualitative methods, and that will include one or several of the following categories (as appropriate for the study question):

- ✓ Patient-reported data, such as responses to questionnaires, symptom and behavior tracking, and validated patient-reported outcomes (PROs)
- ✓ Task-based measures collecting objective measurements of a person's mental and/or physical ability to perform a test consisting of a defined task or set of tasks (e.g. 6-minute walk test)
- ✓ Sensor data measuring a person's daily activities, mental state, or physiological status from wearables and remote sensors
- ✓ Patient preference information (PPI) reporting patient valuations of benefit and risk related to relevant device types and specific illnesses and conditions
- ✓ Patient experiences and general patient perspectives on their care



EXAMPLE OF A POTENTIAL PGD TEST-CASE

The following is an example of a potential test-case concept:



For patients having undergone knee replacement surgery, the study will identify which outcomes matter most to patients.

Test-Case Example:

This study could involve the collection of patient-reported outcomes (e.g. knee pain, overall satisfaction with surgery results, work status, health related quality of life).

If relevant to the understanding of patient perspectives, the study could also collect clinical outcomes (e.g. mortality, readmission rates, reoperation and revision rates).

The study could seek to evaluate patient preferences with respect to benefits and risks of the intervention.



SUBMISSION, REVIEW, AND SELECTION PROCESS

Submission Process

Phase I September

- Organization **submits a test-case concept** to NESTcc.
- Test-case information **will be kept confidential.**

Review Process

Phase II September - January





- Each concept will be reviewed by two to four reviewers from MDIC staff and the FDA.
- Network Collaborators will assess feasibility and interest through an opt-in/opt-out process.
- NESTcc staff will use responses to match concepts and Network Collaborators for feasible projects.
- Full proposals and budgets will be developed through a collaborative process between the submitting group and the matched Network Collaborators.

Selection Process

Phase III February (Anticipated)

- NESTcc will **select up to 4** test-case concepts.
- The recommended projects will be presented to the **NESTcc Selection Team and the MDIC Board** for approval.

ADDITIONAL CONSIDERATIONS

-  NESTcc may request additional information from submitters to facilitate its matching process.
-  Funds from NESTcc will be available to support the selected test-cases. Funding will go to Network Collaborators participating in the projects.
-  NESTcc will request full project proposals and budgets at later date.
-  NESTcc anticipates providing \$250-550k per project, depending on the award structure and number of participating Network Collaborators participating in each project.

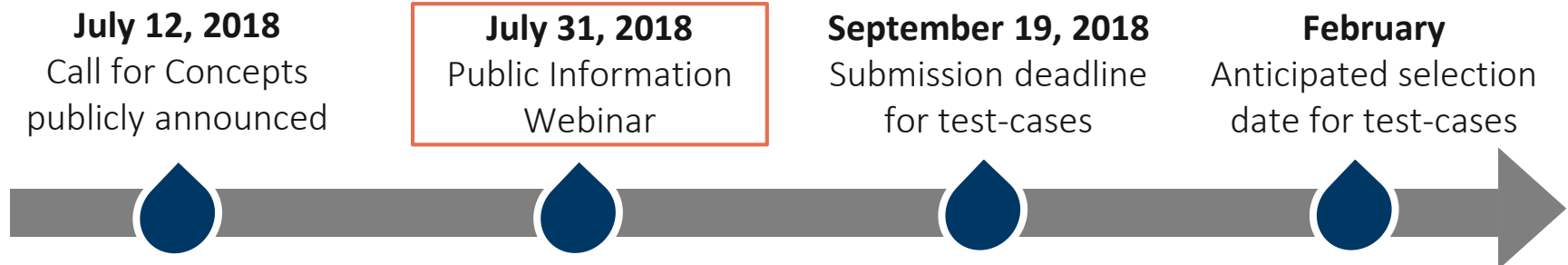


KEY DATES AND REMINDERS

SUBMISSION REMINDERS

- Submissions for test-case concepts should be submitted to NESTcc Project Manager, Tiffany Abushaikha, at NESTcc@mdic.org.
- The deadline for submitting concepts is 5p.m. EDT on September 19, 2018.
- A required submission template is available [here](#), and can also be found at <https://nestcc.org/opportunities/>.
- The concept submission must not exceed two (2) pages.
- Please direct all questions to the NESTcc team at NESTcc@mdic.org.

OPPORTUNITY TIMELINE





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