



## **National Evaluation System for health Technology Coordinating Center (NESTcc) An Invitation to Submit Concepts for Targeted Test-Cases: Patient-Generated Health Data**

### **Call for Concepts**

The National Evaluation System for health Technology Coordinating Center (NESTcc), in partnership with the U.S. Food and Drug Administration's (FDA) Center for Devices and Radiological Health (CDRH), is soliciting concepts for test-cases that use patient-generated health data (PGD) to characterize and evaluate patient outcomes, perspectives, and preferences on the management and treatment of their condition. The solicitation for concepts is open to all medical device stakeholders, including manufacturers, health system providers, health payers, academia, and non-profits, including patient advocacy groups and should involve prevention, diagnosis, treatment, or surveillance with a medical device, imaging, or diagnostic technologies.

These test-cases will continue to assess the feasibility for key stakeholders to work with the NESTcc Data Network's Real-World Data (RWD) sources to support a range of studies and analyses, as well as address high priority areas of interest for NESTcc, including requirements outlined in the U.S. Food and Drug Administration (FDA) Reauthorization Act of 2017 (FDARA) and MDUFA IV. In addition to the priorities for NESTcc, this call addresses MDUFA IV commitments for CDRH's Patient Science and Engagement to advance patient input and involvement in the regulatory process.

### **Background**

In 2016, the FDA's Center for Devices and Radiological Health (CDRH) awarded a cooperative agreement to the Medical Device Innovation Consortium (MDIC) to establish the National Evaluation System for health Technology Coordinating Center (NESTcc). NESTcc's mission is to accelerate the development and translation of new and safe health technologies, leveraging Real-World Evidence (RWE), and innovative research.

NESTcc is being designed to support evidence generation for use-cases ranging from pre-market approval and clearances to expansion of indication, post-market safety and surveillance studies, and coverage decisions.<sup>1</sup> Both observational and interventional study designs may be appropriate depending on the question at hand. Beginning in 2017, NESTcc has initiated operations, establishing a multi-stakeholder Governing Committee and developing an initial [Strategic and Operational Plan](#) to build the foundation for NESTcc through four strategic priority areas:

1. To establish NESTcc governance;
2. To develop NESTcc's role;
3. To establish NESTcc's value; and

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<sup>1</sup> Shuren J, Califf RM. Need for a National Evaluation System for Health Technology. JAMA. 2016 Sep 20;316(11):1153-4. doi: 10.1001/jama.2016.8708

#### 4. To ensure NESTcc stakeholder engagement.

To begin the development of NESTcc's role, strategic data partnerships have been established with an initial group of Network Collaborators to build the foundation of the NESTcc Data Network. Experts have been selected to form the Data Quality and Methods Subcommittees to develop data quality and methodological standards. NESTcc launched its first round of [test-cases](#) to assess the capabilities of the NESTcc Data Network in late 2017. More information on NESTcc is available at: <http://www.nestcc.org>.

#### **Request for Test-Case Concepts**

Test-case concepts submitted to NESTcc should seek to understand the patient perspective on the health outcomes associated with prevention, diagnosis, treatment, or surveillance of their condition, that could constitute useful inputs into regulatory decision-making. While it is anticipated that test-case concepts will focus on specific diseases or devices, the learnings should contribute more generally both to the science of patient input and to the use of NESTcc's Data Network for these types of studies.

The goals of the test-cases are to:

- (1) Explore the feasibility of generating and using patient-generated health data for regulatory and coverage purposes using the NESTcc Data Network and identify the characteristics of the infrastructure necessary to support this.
- (2) Improve the understanding of the opportunities and challenges of using the NESTcc Data Network to support studies that capture and evaluate patient-generated data to identify and measure health outcomes that matter most to patients.
- (3) Provide learnings to advance patient input and involvement in the regulatory process.
- (4) Contribute to NESTcc's development of operational processes (e.g., contracting, IRB, data sharing agreements, publication policies).

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<sup>2</sup> (as appropriate for the study question): (1) patient-reported data, such as responses to questionnaires, symptom and behavior tracking, and validated patient-reported outcomes (PROs) (2) 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. six-minute walk test) (3) sensor data measuring a person's daily activities, mental state, or physiological status, from wearables and remote sensors (4) patient preference information (PPI) reporting patient valuations of benefit and risk related to relevant device types and specific illnesses and conditions, (5) patient experiences and general patient perspectives on their care.

It is envisioned that these test-cases will contribute to providing a more comprehensive understanding of health outcomes that matter to patients whose condition is managed or treated with medical devices and technologies and provide insights on how these studies could inform regulatory decision-making.

The test-case studies must require the use of patient-generated data and contribute to the continued development and testing of the NESTcc Data Network. After test-case concepts have been reviewed and

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<sup>2</sup> Mobilizing mHealth Innovation for Real-World Evidence Generation, Duke-Margolis Center for Health Policy (2017)

selected, NESTcc's Network Collaborators will be invited to participate in developing protocols to answer the test-case concept question in collaboration with the entity that has submitted the concept.

These test-case concepts must be useful to medical device manufacturers and other stakeholders, including patient stakeholder communities. In addition, the test-cases will inform the understanding of the type(s), quality, and quantity of patient-generated data to be considered; the potential for use of PGD in regulatory submissions; and the qualities of the infrastructure necessary to capture, analyze, and optimize the utility of PGD.

### **Details and Requirements**

NESTcc is interested in receiving a wide range of test-case concepts that reflect the diversity of the medical device ecosystem. Evidence generated by the test-cases may support regulatory and coverage use-cases across the Total Product Life Cycle (TPLC), although not all use-cases are expected to be relevant:

*Pre-Market (i.e., PMA, 510(k), De Novo):* Using RWE to inform pre-market development of incremental improvement of medical devices.

*Label Expansion:* Using RWE in a regulatory submission to support an expanded indication for use of medical devices already on the market.

*Post-Market Approval Studies (PAS):* Using generated RWE to track medical device's safety and effectiveness as part of its condition of approval.

*Surveillance:* Using generated RWE to track and document medical device safety and effectiveness for products on the market.

*Coverage:* Using generated RWE to support coverage and reimbursement decisions by public and private payers, and health systems.

NESTcc is interested in devices subject to approval or clearance pathways, Class II and Class III devices, as well as imaging and diagnostic technologies (IVDs), and technologies used in cancer. Submitted concepts should be new projects that are not currently underway.

### **Test-Case Example**

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

For patients having undergone knee replacement surgery, the study will identify which outcomes matter most to patients. 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, etc.). If relevant to the understanding of patient perspectives, the study could also collect clinical outcomes (e.g. mortality, readmission rates, reoperation and revision rates, etc.). The study could seek to evaluate patient preferences with respect to benefits and risks of the intervention.

### **The Role of a Network Collaborator**

NESTcc has executed Memoranda of Understanding (MOUs) with 12 organizations to participate as Network Collaborators with NESTcc in the establishment of the NESTcc Data Network. These Network Collaborators include both institutions and consortia and together represent more than 150 hospitals,

3,042 outpatient clinics, and 108 Million patients. The Network Collaborators have access to over 469 million patient records and available data sources include EHRs, pharmacies, public and private claims, registries, and patient-generated data. The NESTcc Data Network is being expanded to include new Network Collaborators. As new Network Collaborators are eligible to participate in test-case projects, they will be listed as Network Collaborators on the [NESTcc website](#).

## **The Role of NESTcc**

MDIC staff of NESTcc will work collaboratively with the submitting organizations and the Network Collaborators to streamline processes and facilitate efficient discussion and contracting throughout the test-case phases – from solicitation through full project proposal development.

## **Process**

### *Eligibility*

This opportunity is open to medical device, imaging, and diagnostic technology manufacturers, health system providers, and health payers, academia, and non-profits, including patient advocacy groups. NESTcc also encourage test-case concepts on behalf of medical device manufacturers who are submitting through an organization or association as a third-party convener (i.e., medical device associations and multi-manufacturer initiatives).

### *Submission*

Test-case concepts must be submitted to [NESTcc@mdic.org](mailto:NESTcc@mdic.org) in the required template (available [here](#)) by 5p.m. EST on September 19, 2018. The concept submission **must not exceed two (2) pages**.

As part of the application, NESTcc will also request that the submitters describe the availability of in-house data sources that could be used in the study (e.g. device-generated data, list of procedures using the device of interest, etc.). Actual data should not be shared with NESTcc at the time of the concept submission.

All submissions will remain confidential among MDIC staff of NESTcc. Information on the test-case submissions will be kept confidential. Submission concepts will not be shared externally unless they are selected as a viable test-case through the review and selection process and an agreement is signed between participating parties.

Please contact NESTcc ([NESTcc@mdic.org](mailto:NESTcc@mdic.org)) if you have any questions or would like to discuss ideas prior to submitting.

### *Review*

Each concept will be reviewed by two to four reviewers from MDIC and the FDA through objective evaluation criteria. Comments will be used by the NESTcc Executive Director to recommend a slate of test-cases to a Selection Team (a subset of the NESTcc Governing Committee). The Selection Team will complete conflict of interest disclosures in accordance with [MDIC conflict of interest policy](#).

### *Preliminary Selection*

NESTcc will select up to four test-cases using criteria of patient-centeredness, feasibility, timeliness, cost, scalability and generalizability of results. Priority may also be given to concepts addressing high-priority

areas of interest. NESTcc will work jointly with medical device manufacturers and Network Collaborators to execute the test-cases in an efficient and equitable manner. NESTcc will also ensure the FDA remains informed and engaged with these specific projects.

After preliminary selection takes place in September 2018, NESTcc will facilitate discussions between the organization submitting the test-case concept and the matched Network Collaborator(s). Through those consultation steps, Network Collaborators will determine whether they want to opt-in or opt-out of proposed concepts and will confirm preliminary feasibility to move forward with a collaborative effort to complete preliminary project information forms. Pending approval to move forward, NESTcc may then approve project teams to proceed to submit a full project proposal—including a budget, list of deliverables, and timeline—which will undergo a formal review by NESTcc to be selected for eligibility to receive NESTcc funding. The slate of recommended projects will then be presented to the NESTcc Selection Team and the MDIC Board for approval.

### **Timeline**

- Posting Date: July 12, 2018
- Public Information Webinar: July 31, 2018
- Due Date: September 19, 2018

Please send proposals or questions to MDIC NESTcc Project Manager Tiffany Abushaikha, [NESTcc@mdic.org](mailto:NESTcc@mdic.org). Deadline for proposals is September 19, 2018, 5p.m. EST.

### **About MDIC**

The Medical Device Innovation Consortium (MDIC) is the first public-private partnership created with the sole objective of advancing medical device regulatory science throughout the total product life cycle. MDIC's mission is to promote public health through science and technology and to enhance trust and confidence among stakeholders. We work in the pre-competitive space to facilitate development of methods, tools, and approaches that enhance understanding and improve evaluation of product safety, quality, and effectiveness. Our initiatives improve product safety and patient access to cutting-edge medical technology while reducing cost and time to market.

For more information visit: <http://www.mdic.org>