The National Health Council's Programs: An Introduction, and How You Can Become Involved

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Presenters

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Introduction

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Focus Areas and Focus Area Working Group

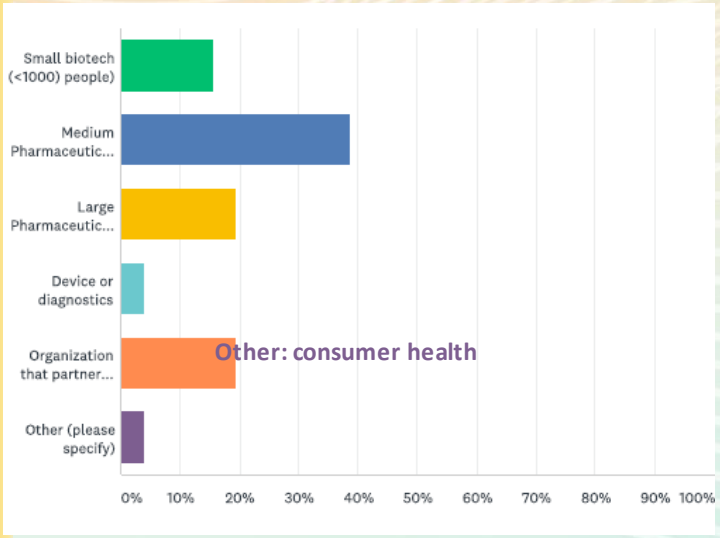
- Focus Areas (FA) are the **priority capabilities** that are strategically important for the successful implementation and maintenance of a high impact Medical Affairs organization.
- The Focus Areas will be managed and supported by a dedicated Focus Area Working Group (FAWG) which consists of individuals who will serve as **Subject Matter Experts** and be responsible for strategy development, gap identification, content creation and program implementation for their assigned Focus Area.

MAPS FAWG Charter 082020

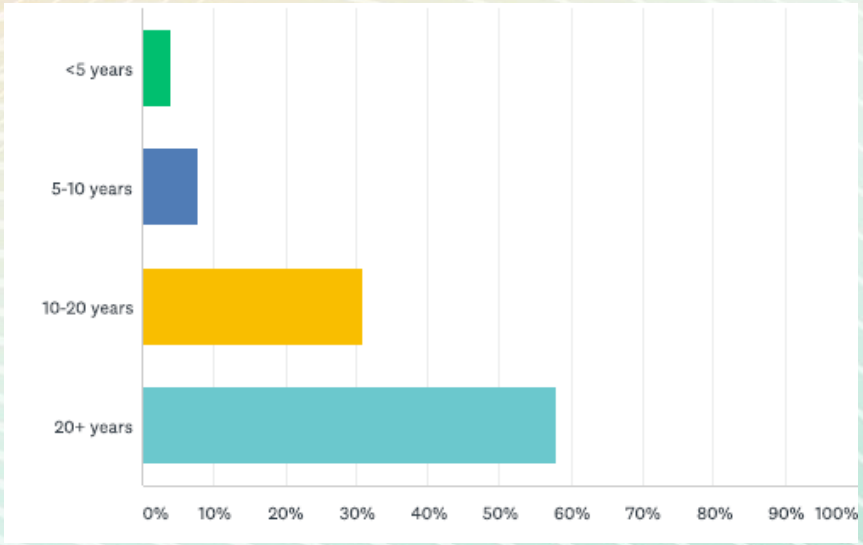
Patient Centricity FAWG

Needs Assessment MAPs Survey: Demographics

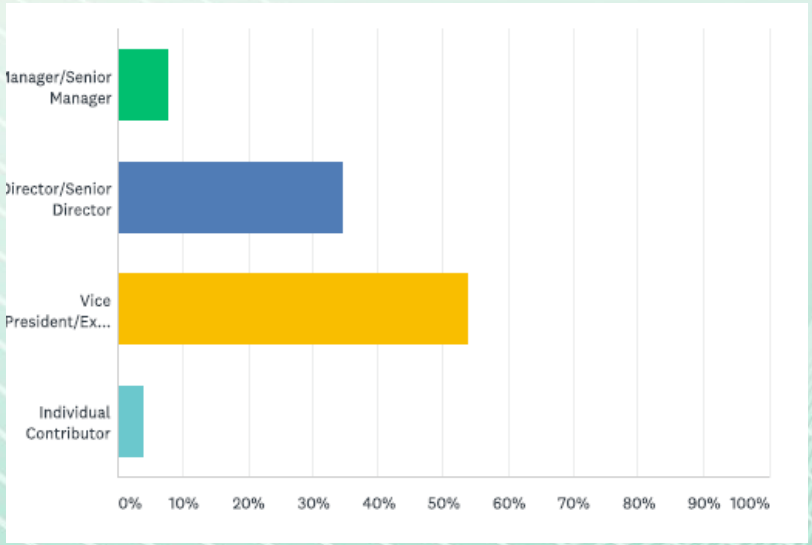
Company Type



Years of Experience

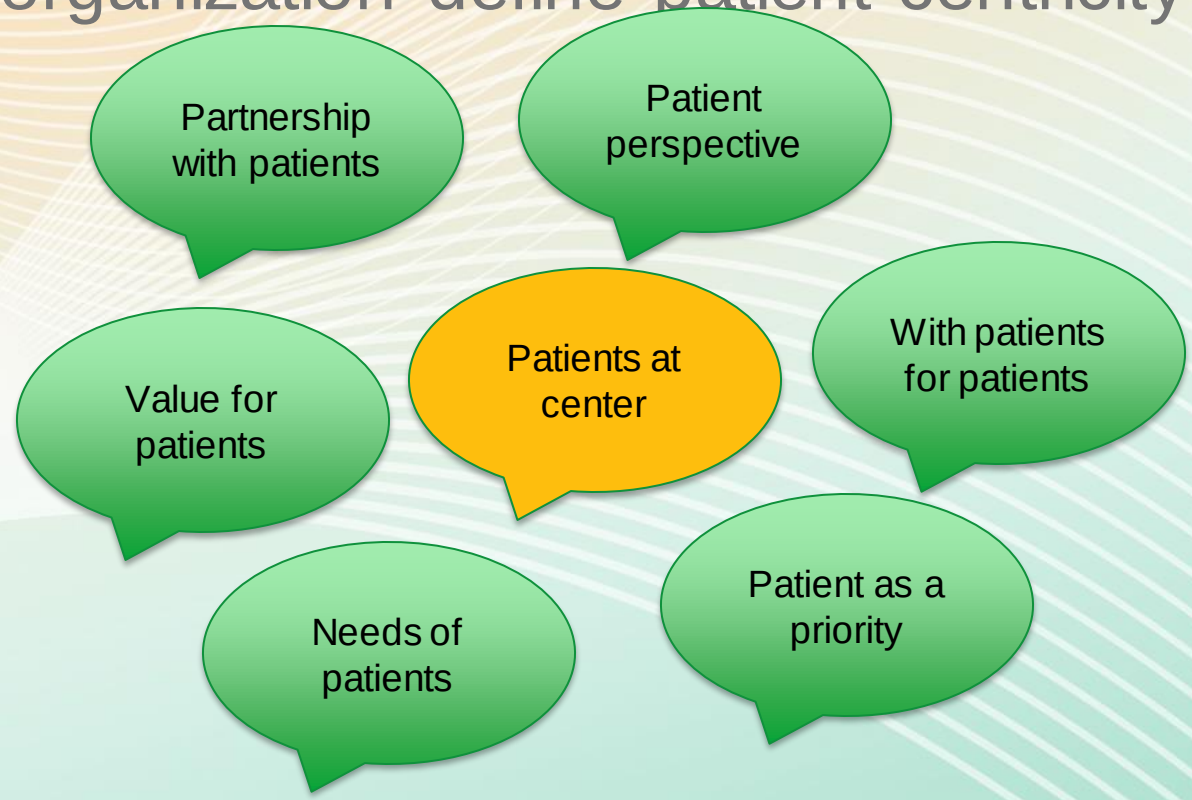


Professional Level



Key Insights from Needs Assessment

1. What is your definition of patient centricity; how do you/your organization define patient centricity?



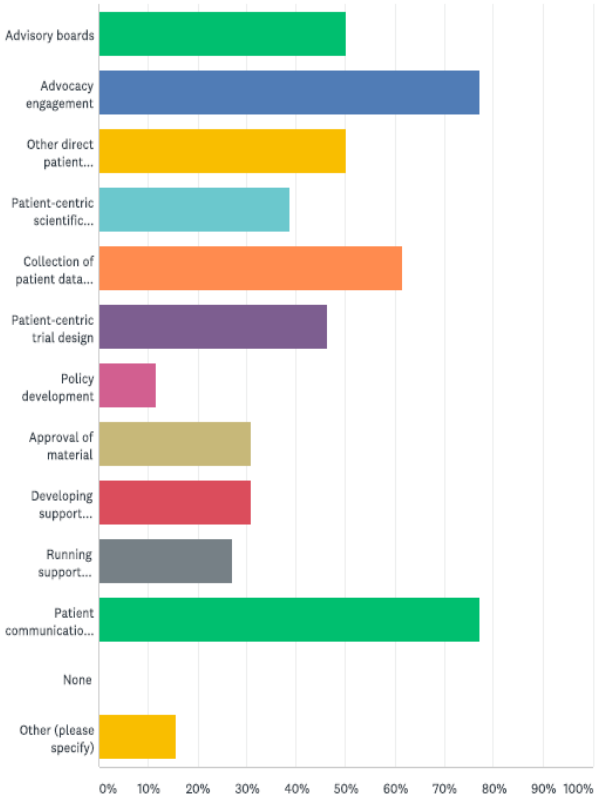
"As it relates to Medical Affairs and the pharmaceutical industry, patient centricity means ensuring that the patient (as well as the family/caregiver) is kept at the center of and/or engaged in the full spectrum of activities related to product development, from very early stages through product approval and beyond. The goal is to ensure that patients are kept informed and engaged in the many steps involved in drug/device development so that outcomes are improved and their overall burden reduced. In our day-to-day, patient needs and perspectives should be carefully considered and applied holistically in all activities."

Needs Assessment Survey

2. What aspects of patient centricity are you currently working on in your organization?

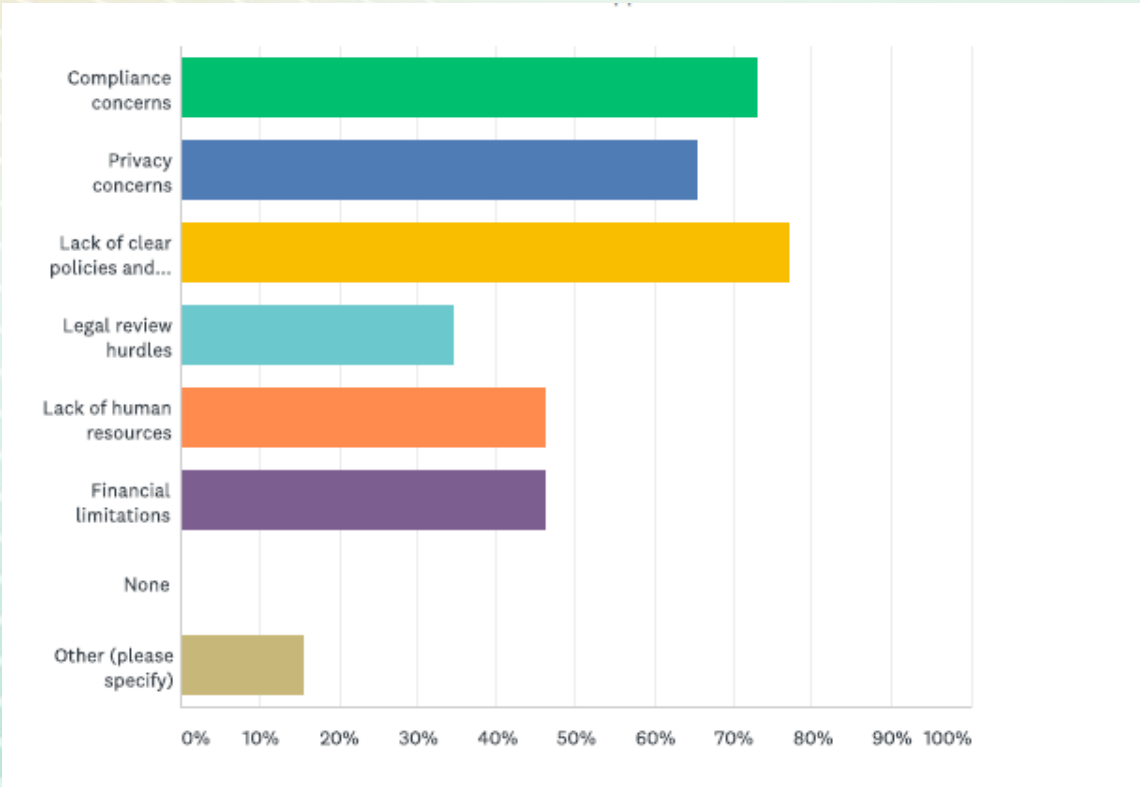
(Select all that apply)

- **Advisory boards**
- **Advocacy engagement**
- **Other direct patient engagement activities**
- **Patient-centric scientific exchange**
- **Collection of patient**
- **Patient-centric trial design**
- Policy development
- Approval of material
- Developing support programs
- Running support programs
- **Patient communications (medical information, lay trial summaries, publications)**
- Other
- None
- Click all that apply



Needs Assessment Survey

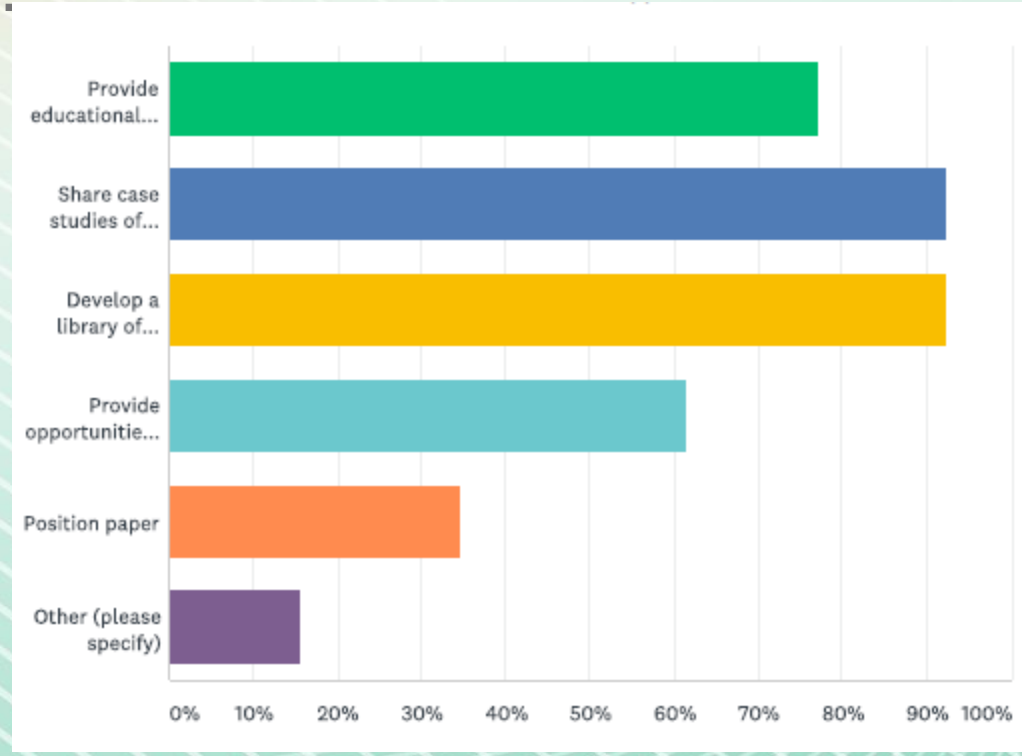
3. What challenges or barriers do you face (or anticipate facing) as you execute on patient-centric initiatives in your organization?
   - **Compliance concerns**
   - **Privacy concerns**
   - **Lack of clear policies and/or guidance documents**
   - Legal review hurdles
   - Lack of human resources
   - Financial limitations
   - Other:



Needs Assessment Survey

4. What can MAPS do to help you to overcome these challenges and barriers, either real or perceived?

- **Provide educational programs and/or resources (eg, webinars or white papers from key experts) on existing and newly emerging industry guidance around patient centricity**
- **Share case studies of successful patient-centric approaches across the spectrum of Medical Affairs activities**
- **Develop a library of useful resources related to industry best practices for patient centricity and patient engagement**
- **Provide opportunities for exchange of information and experiences related to implementation of patient-centric approaches**
- **Position paper**
- **Other: if other please list**



Promoting Best Practices in Patient Engagement

Elisabeth M. Oehrlein, PhD, MS
Assistant Vice President, Research and Programs
National Health Council

Session Overview

This session will provide a learning opportunity for our audience by providing information about:

- The National Health Council
- Understand how MAPS members can use NHC tools
- Terminology & Foundational Work
- Ongoing Initiatives
- Getting Involved!

A Diverse Membership

- **Voluntary Health Agencies**
  Defined as 501(c)(3) organizations composed of individuals, both lay and professional, voluntarily and democratically organized.

- **Professional and Membership Associations**
  Health-related associations of individuals or institutions, including alliances and coalitions.

- **Nonprofit Organizations with an Interest in Health**
  Organizations engaged in or concerned with health issues that support the mission of the NHC.

- **Business and Industry**
  For-profit companies engaged in the sale of health-related products or services such as pharmaceutical, generic drug, insurance, biotechnology, and medical device companies.

- **Associate Members**
  Organizations that are considered to be primarily vendors. They have all member privileges except they are not allowed to be represented on the NHC Board and cannot vote at meeting of the entire membership.

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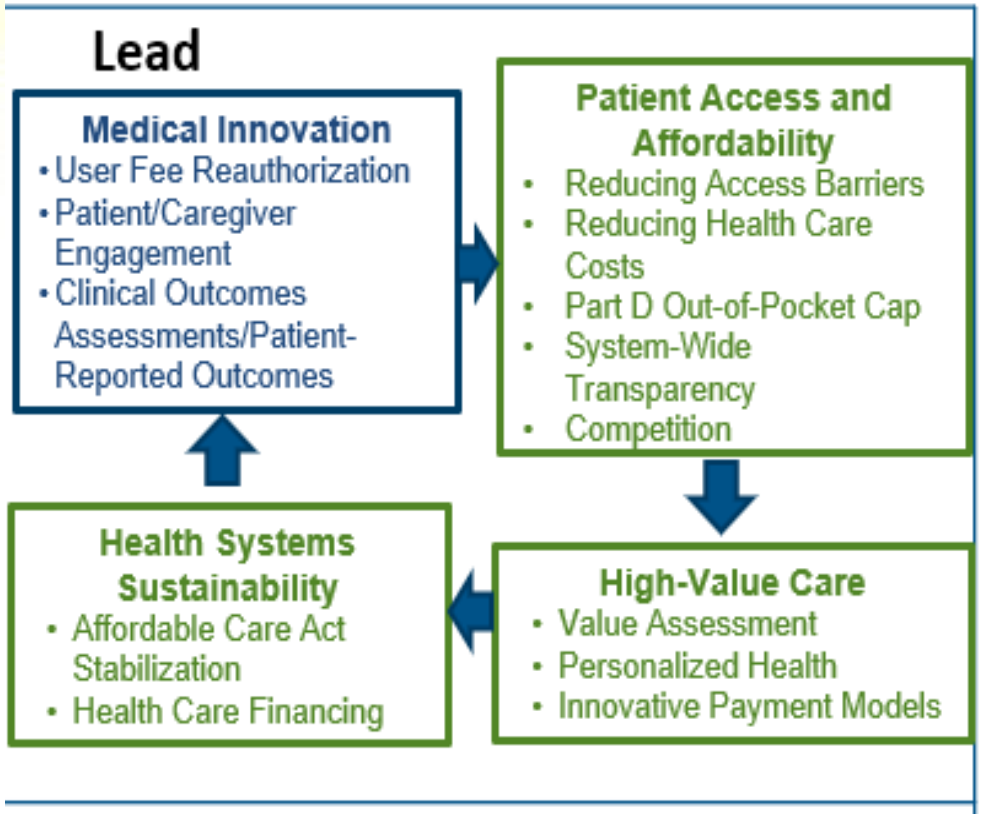
Policy Matrix "Lead" Box



2020 Programs

PE/Medical Innovation
- Patient Engagement
  - Patient Journey Mapping
  - Fair-Market Value and legal agreements
  - Health Equity*
  - Real-World Evidence
  - Patient Community Training
  - Patient-centered RWE

Patient Access and Affordability
- Telehealth
  - Patient-centered telemedicine
- Health Equity*

High-Value Care/Value Assessment
- Value Assessment
  - Patient-centered methods
  - Employer engagement
  - Communications

Health Systems Sustainability
- Innovative Payment Models
- Health Equity*

* Health Equity is embedded in all our work

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Patient-Centricity

Any process, program, or decision focused on patients in which patients play an active role as **meaningfully engaged participants,** and the central focus is on optimizing use of patient-provided information

Patient centered means doing things

WITH patients –

not **FOR** *or* **TO**

– patients

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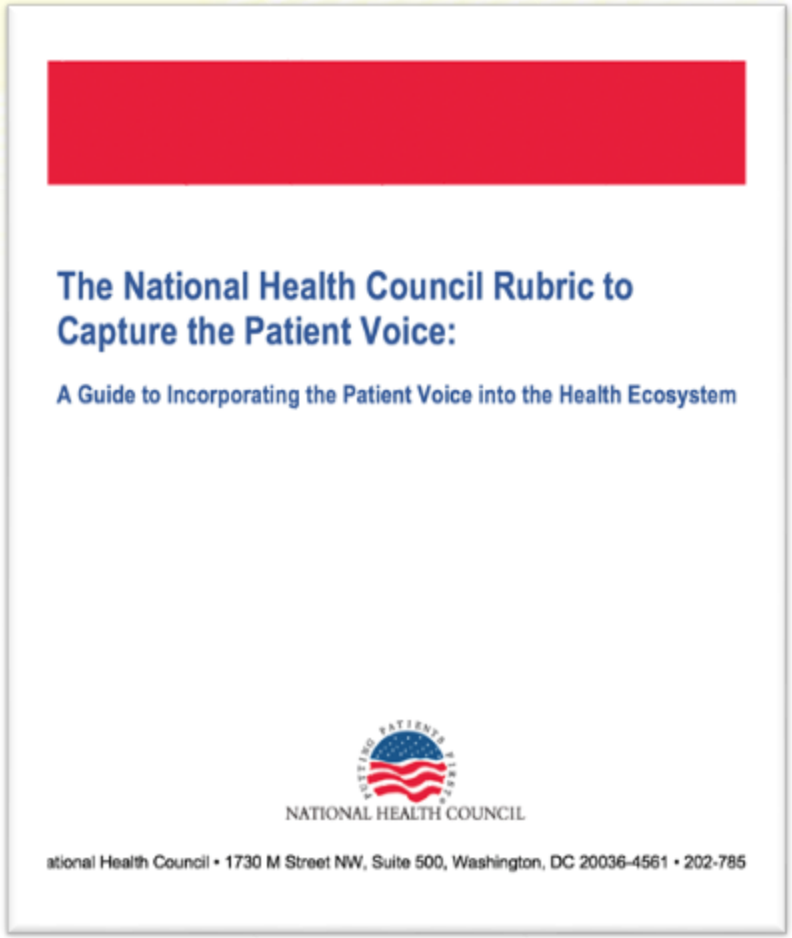
NHC Rubric to Capture the Patient Voice



- To provide a tool the patient community or any other stakeholder can use to evaluate attributes of patient centeredness and to guide them on meaningful patient engagement throughout any activity they might undertake

- It can be used as a general blueprint for any setting where patient centricity is desired:
  - Research
  - Medical-product development
  - Health-care delivery
  - Real-world analyses
  - Health policy

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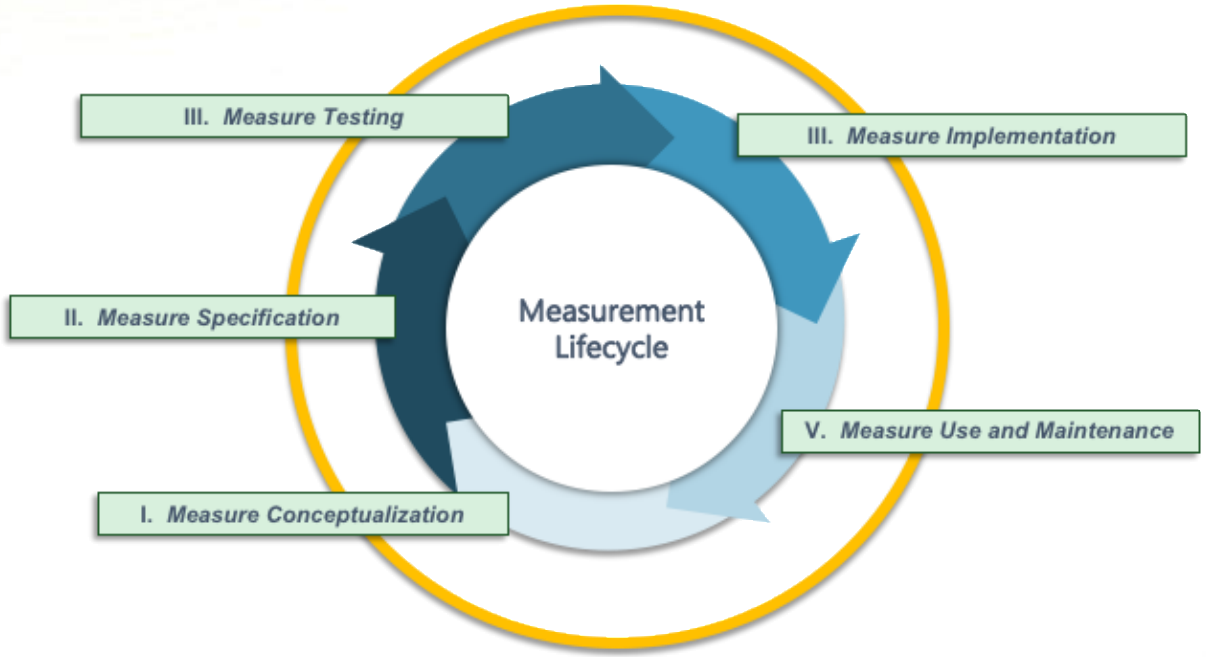
Rubric is Complementary to Partner Resources

Evaluating Meaningful Engagement within Quality Measure Development

Person and Family Engagement

PQA
Pharmacy Quality Alliance



Source: Pharmacy Quality Alliance, https://www.pqaalliance.org/assets/PQA-Patient-Engagement-Rubric.pdf

Patient-Focused Medicine Development's (PFMD) Quality Guidance

Source: Patient-Focused Medicines Development, https://patientfocusedmedicine.org/the-patient-engagement-quality-guidance/

Domains of Patient-Centricity

- **Patient Partnership** - Patients should be involved in every step of the process, including planning and dissemination.
- **Transparency** - All activities should be conducted in an open way, and assumptions, inputs, processes, and results need to be disclosed to patients in plain language and a timely fashion
- **Representativeness** - Sufficient number and types of people are included in the engagement activity to ensure that those engaged can speak on behalf of the target population.
- **Diversity** – The activity should consider differences among patients, including patient subpopulations, trajectory of disease, and stage of a patient's life
- **Outcomes Patients Care About** - Whether the activity is research, policy, or care delivery oriented, the outcome(s) being measured should include those that patients state are important to them
- **Patient-Centered Data Sources and Methods** – A variety of credible sources can facilitate timely incorporation of new information and account for the diversity of patient populations and patient-centered outcomes
- **Timeliness** - Engagement happens at the appropriate time(s) throughout the process so that it is useful, effective, or relevant to meet the objective of the activity

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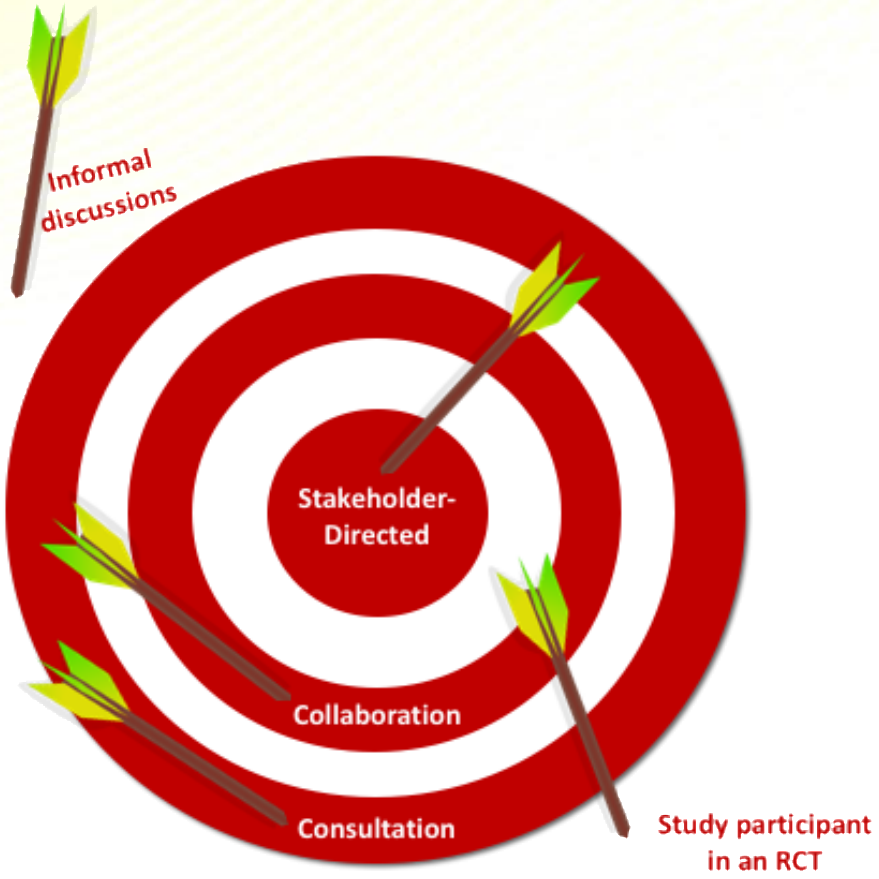
Meaningful Engagement



- Refers to direct relationships and partnerships that are bi-directional, reciprocal, and continuous
- Communications are open, honest, and clear
- Engagement goals, participants, methods, desired impacts, and actual impacts are clearly outlined and transparent

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NHC Programs Snapshot

Patient Engagement

- **"Moving the Field" Forward**
  - Telemedicine
  - Fair-Market Value & Contract Templates
  - Patient Experience Mapping Toolbox
- **Capacity Building**
  - Patient-Focused Medical Product Development Webinar Series
  - Case Examples
- **Science of Patient Engagement Symposium**

Real-World Evidence

- **"Moving the Field" Forward**
  - Recommendations for Applying Patient-Provided Information to RWD-based analyses
- **Capacity Building**
  - Building Capacity for Patient Engagement in RWE (PCORI funding; Duke Margolis as partner)

High-Value Care

- **"Moving the Field" Forward**
  - Value-based arrangements
  - Exploring alternative methods: multi-criteria decision analysis (with NPC)
  - HTAi Work Group: Statement of Information for Patients
- **Capacity Building**
  - Online training and resources
  - Value Work Group
  - Value Assessment Communications Toolkit

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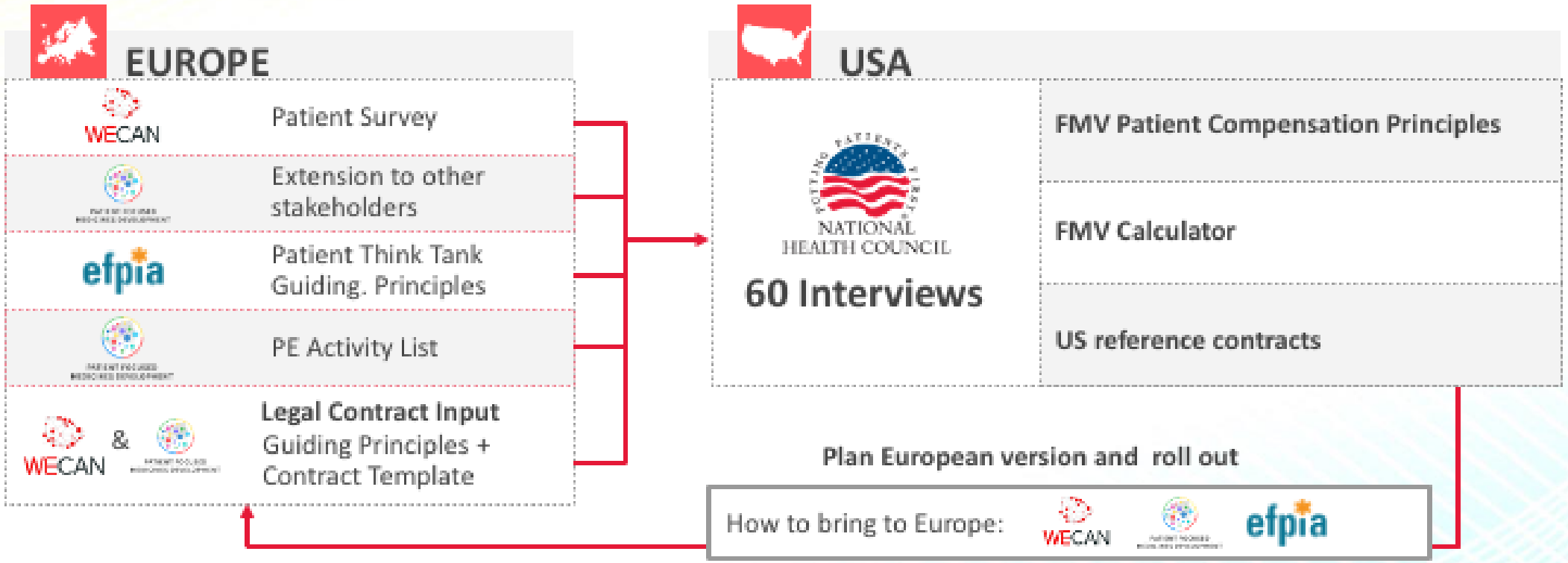
National Health Council's Fair-Market Value Calculator and Related Resources



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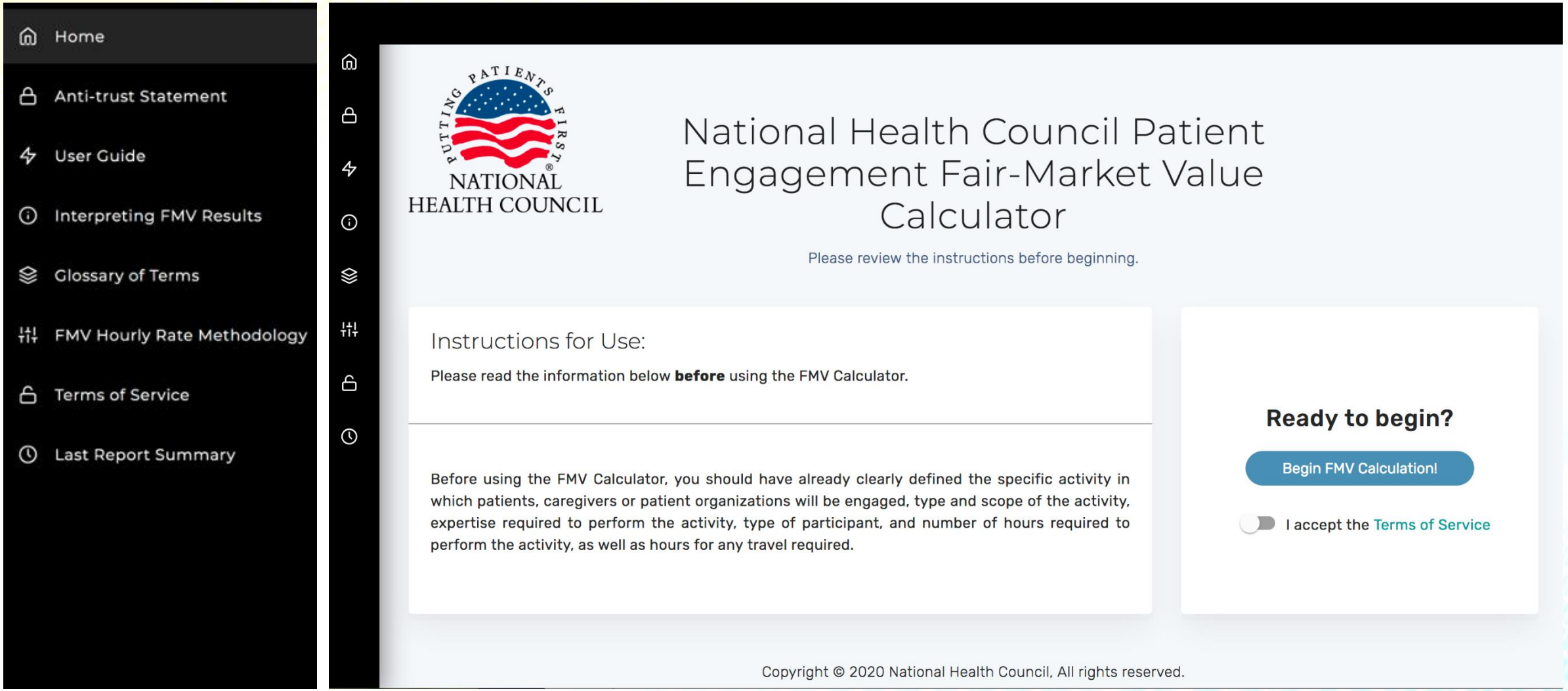
National Health Council's Fair-Market Value Calculator and Related Resources



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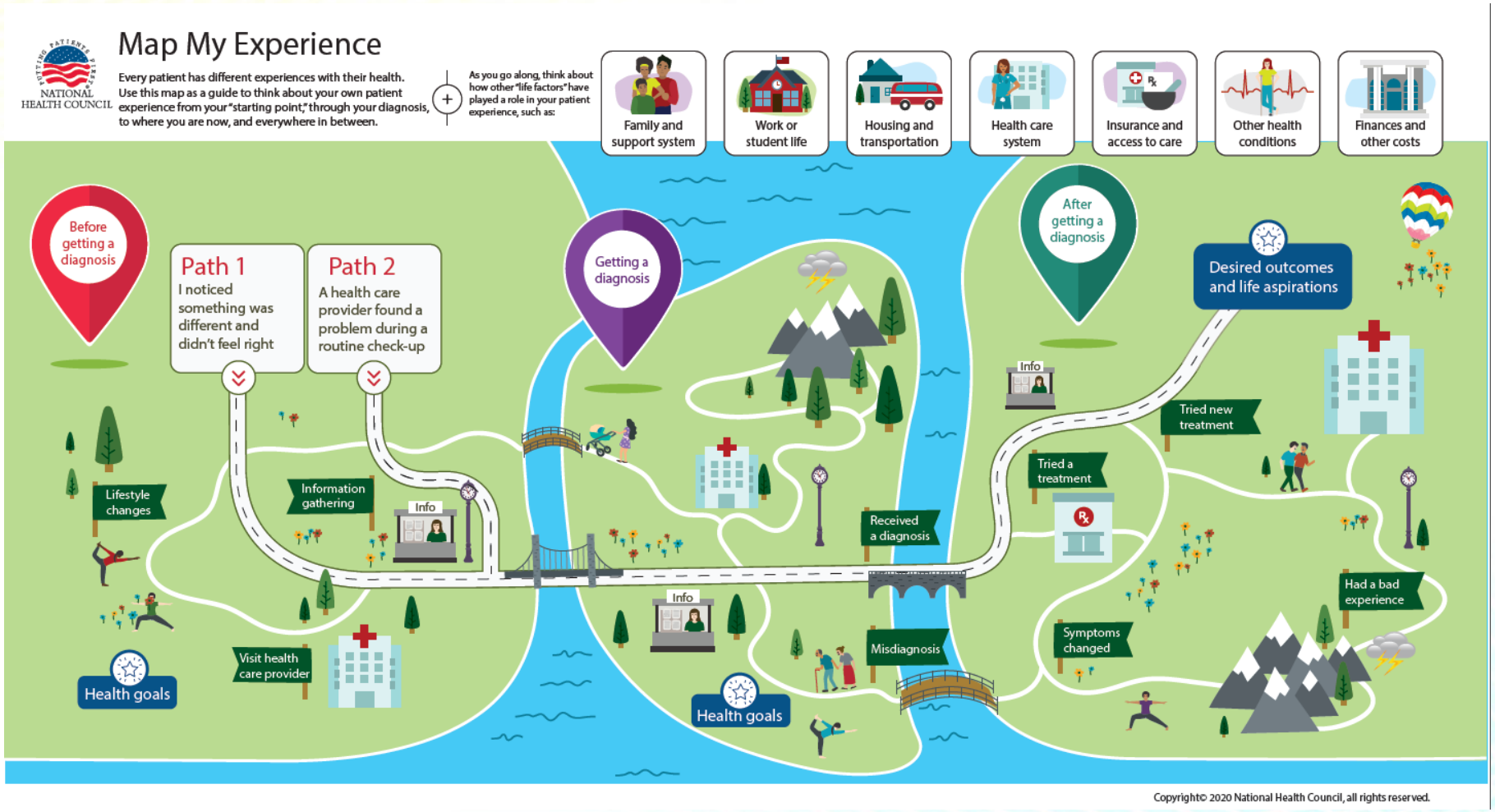
National Health Council's Patient-Experience Mapping Toolbox



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National Health Council's Patient-Focused Medical Product Development Webinar Series

November 2018 - Present

- Patient-Reported Outcomes and Patient-Centered Outcomes
- FDA's Roadmap to Patient-Focused Outcome Measurement in Clinical Trials
- Untangling the Terms: Endpoints, Items, Outcomes, PROs, PROMs, PRO-PMs
- It all starts with the "concept of interest" and "context of use"
- What do We Mean by Validation of a Measure?
- Measuring Patient Experiences: Distinguishing Between Patient-Reported Outcomes and Patient Preferences
- Lessons Learned From a Patient Group's Experience Developing a PRO
- What is the Difference Between Health-Related Quality of Life (HRQoL) and Patient-Reported Outcomes?
- Introduction to Core Outcome Sets

- What are Clinician-Reported Outcomes (ClinROs)?
- Clinical Outcome Assessments: Build New or Adapt Old?
- 21st Century Cures: Requirement on Patient Experience Data and Related Information
- A Look at Rare Disease Listening Sessions with NORD and the FDA
- Observer-reported or Proxy-reported: Is One Better than the Other?
- What is the Role of Patient-Reported Data in Outcomes-Based Contracts?
- Patient-Focused Medical Product Development: Real-World Case Examples
- Health Literacy Throughout Drug Development: Why It Matters to Pharma and to Patients
- **Upcoming:** Webinar on recent publication regarding Clinical Trials Transformation Initiative's work on "Optimizing Mobile Clinical Trials by Engaging Patients and Sites."

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National Health Council's Patient-Focused Medical Product Case Examples

1. **Pediatric Assent**

Lilly worked directly with iCAN (International Children's Advisory Network) and UC-Berkeley to improve its approach to the typical pediatric/adolescent assent form. When a child is too young to sign a consent form, the parent provides the consent. However, the children can also give their assent to participate in the clinical trial. Lilly researchers worked alongside patients in the iCAN program to create assent templates for children/adolescents that tell the story of what it's like to participate in a clinical trial. Similar to a picture book, the colorful template follows kids as they experience blood draws, X-rays, and other procedures. Here is a more detailed description the program.

Key point: Lilly researchers updated assent forms to better engage young patients in clinical trials by working with young patients to create them and incorporating their ideas into the updated version.

2. **JAKAFI® (ruxolitinib)**

Jakafi (ruxolitinib) was approved by the FDA in 2011 for the treatment of intermediate or high-risk myelofibrosis. One of the endpoints used to support FDA approval was demonstrated improvement in myelofibrosis-related symptoms. Symptoms were measured by a novel patient-reported outcome (PRO) measure, the modified Myelofibrosis Symptom Assessment Form (MFSAF) version 2.0 diary. The MFSAF 2.0 was the result of additional qualitative patient interviews, cognitive debriefing, and validation testing conducted by the sponsor in order to meeting the requirements outlined in FDA's 2009 PRO guidance. See Acquadro and Regnault.

The FDA uses this as an example of successful Clinical Outcome Assessment development.

16 examples published on website

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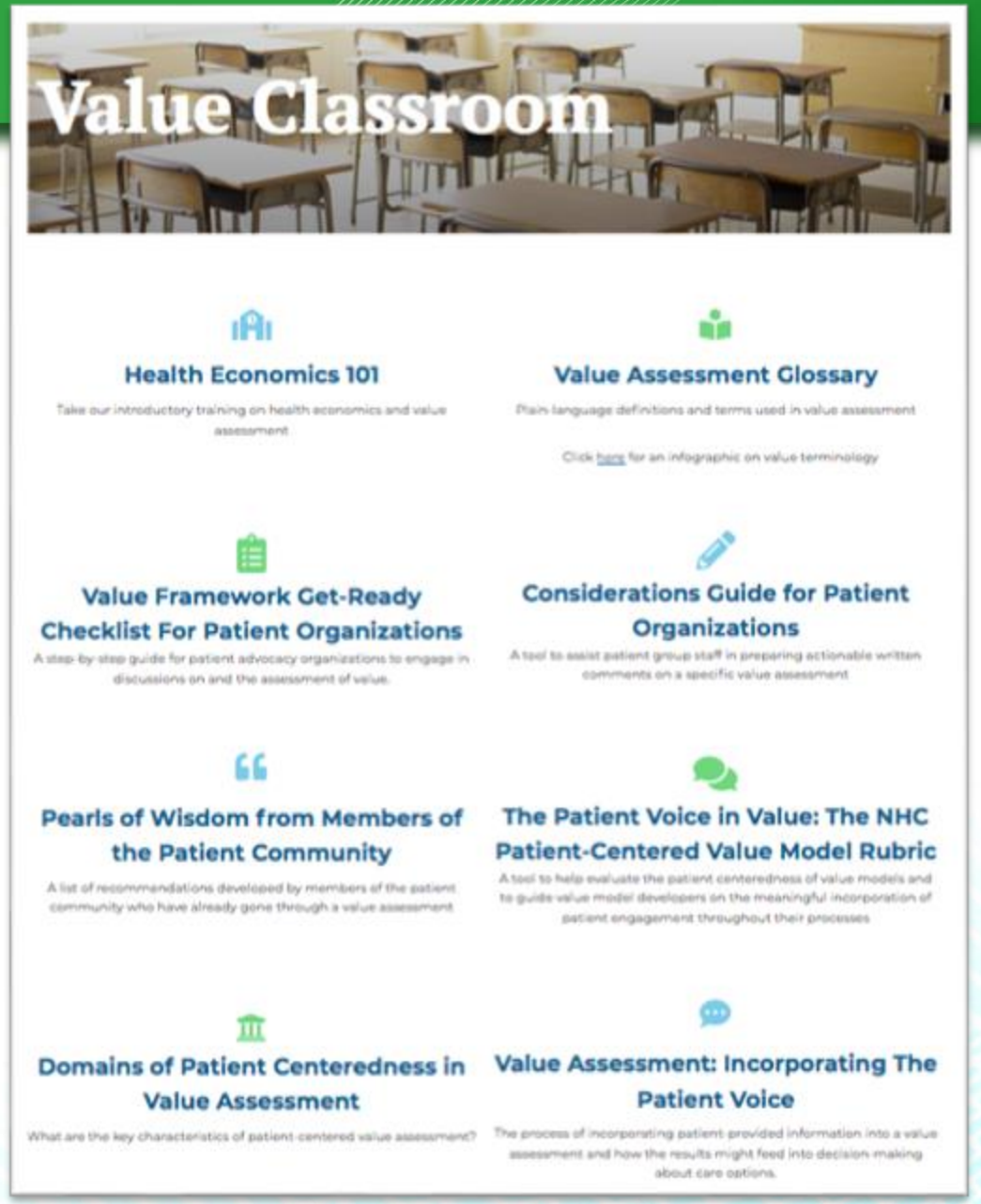
National Health Council's Annual Science of Patient Engagement Symposium

National Health Council's Value Program

- Advancing Patient Engagement and Patient-Centered Methods



PAVE

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https://nationalhealthcouncil.org/education/value-classroom/

National Health Council's Real-World Evidence Program

Resources for Research Community

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Opportunities for Engagement

- Advisory Board membership
  - Program Sponsorship

- Education/Networking
  - Science of Patient Engagement Symposium
  - Medical Innovation Action Team
  - Regular webinars

- Consulting

Thank you!

Questions?