

# Precision Medicine and Patient-Centered Outcomes Research: Toward tailored approaches to Healthcare Delivery

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**NATIONAL CANCER INSTITUTE**

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Patient Centered Outcomes  
Research Symposium

*No conflicts of interest to  
disclose*

# Presentation Roadmap

A little bit about me

Definitions: Patient-Centered Outcomes and Care, Precision Medicine

Investigating Tailored Care

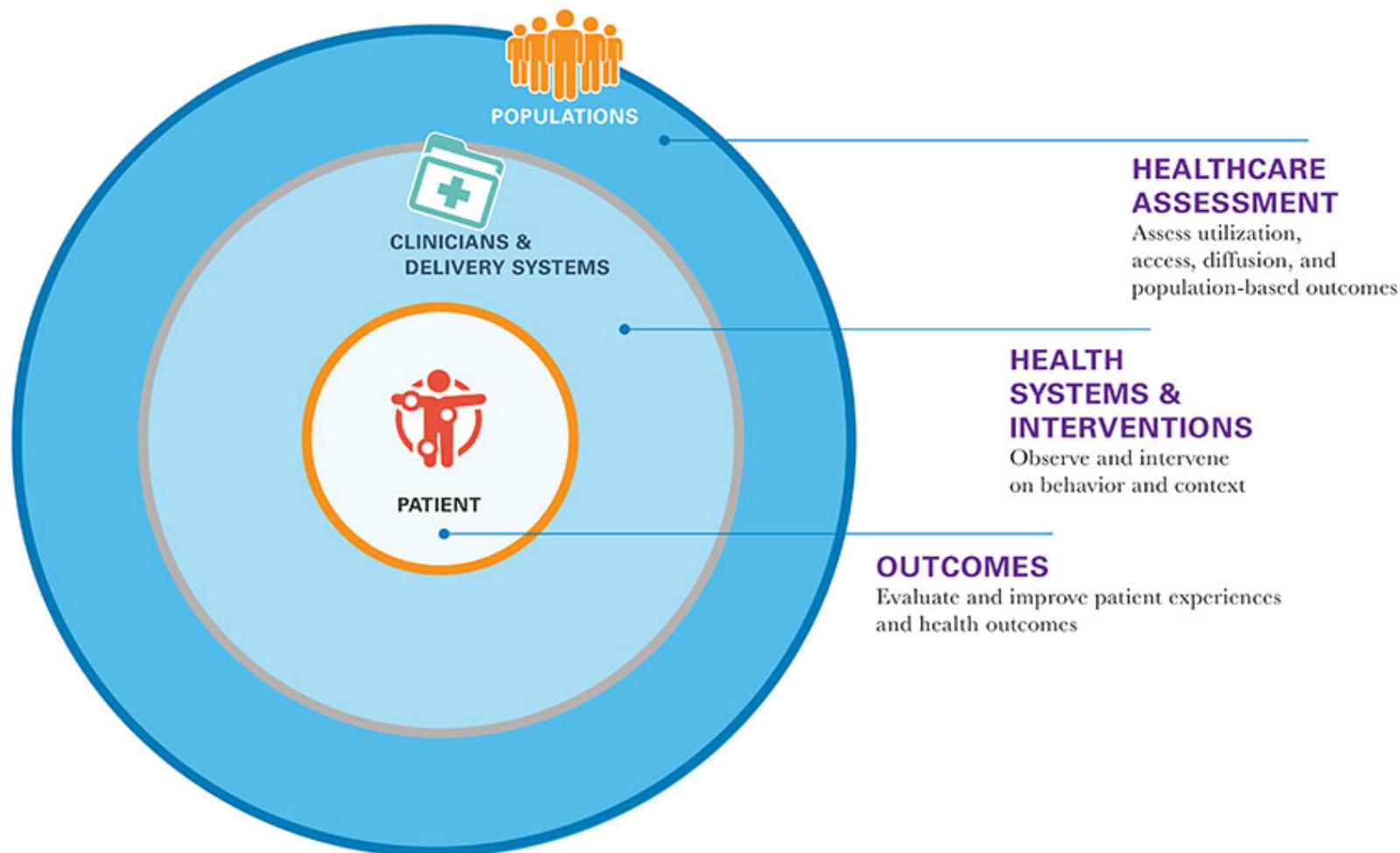
1. Precision Medicine Provider Survey
2. Experiences of Care
3. Patient Health Outcomes
4. Patient Values and Preferences

Funding Implementation of Patient Centered Care in Healthcare Delivery



## HEALTHCARE DELIVERY RESEARCH PROGRAM

Advancing innovative research to improve the delivery of cancer-related care



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# Creating the Foundation

## Patient-Centered Outcomes Research Symposium

**Symposium Theme:** Patient-Centered Perspectives on Precision Medicine and Precision Prevention

### Terminology

Outcomes Research

Patient-centered Outcomes Research

Patient-centered care

Precision Medicine



# Patient-centered outcomes and care

**Outcomes research** is the scientific field devoted to measuring and interpreting the impact of medical conditions and healthcare on individuals and populations (Lipscomb et al., 2005)

**Patient-centered outcomes research** helps patients and caregivers communicate and make informed healthcare decisions, allowing their voices to be heard in assessing the value of healthcare options. (PCORI)

- Patient generated health data (e.g., patient-reported outcomes and experiences of care, performance tasks, sensor-based data)

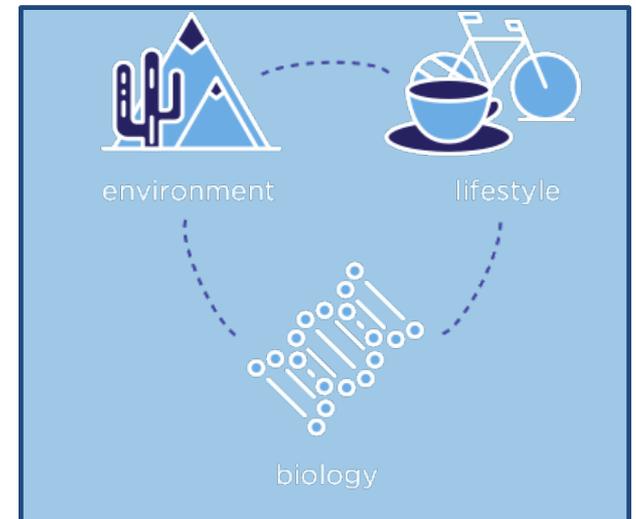
**Patient-centered care:** an individual's specific health needs and desired health outcomes are the driving force behind all health care decisions and quality measurements. Patients are partners with their providers, and providers treat patients from a clinical, and also, an emotional, mental, spiritual, social, and financial perspective. (NEJM)

# Precision Medicine and NIH

Precision medicine is an approach to disease prevention and treatment that takes into account individual variability in genes, environment and lifestyle to aid in the development of individualized care.

The NIH's Precision Medicine Initiative® is designed to generate the scientific evidence needed to move the concept of precision medicine into clinical practice.

The *All of Us* Research Program seeks to extend precision medicine to all diseases by building a national research cohort of one million or more U.S. participants.



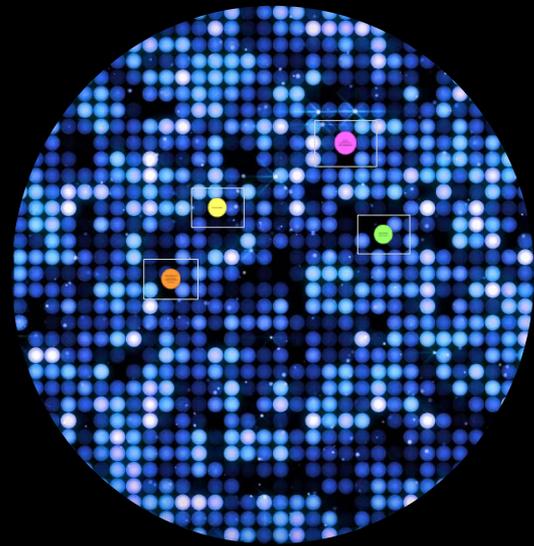
# Precision Medicine in Cancer Treatment

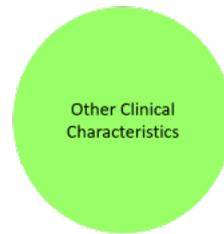
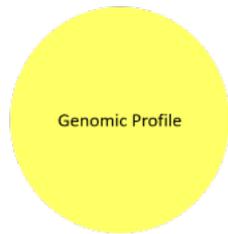
- In cancer, precision medicine uses specific information about a person's tumor (genes, protein) to help diagnose, plan treatment, find out how well treatment is working, or determine the prognosis.
- Examples: using targeted therapies to treat specific types of cancer cells, or using tumor marker testing to help diagnose cancer. Also called personalized medicine.
- How can we apply precision medicine in healthcare delivery?

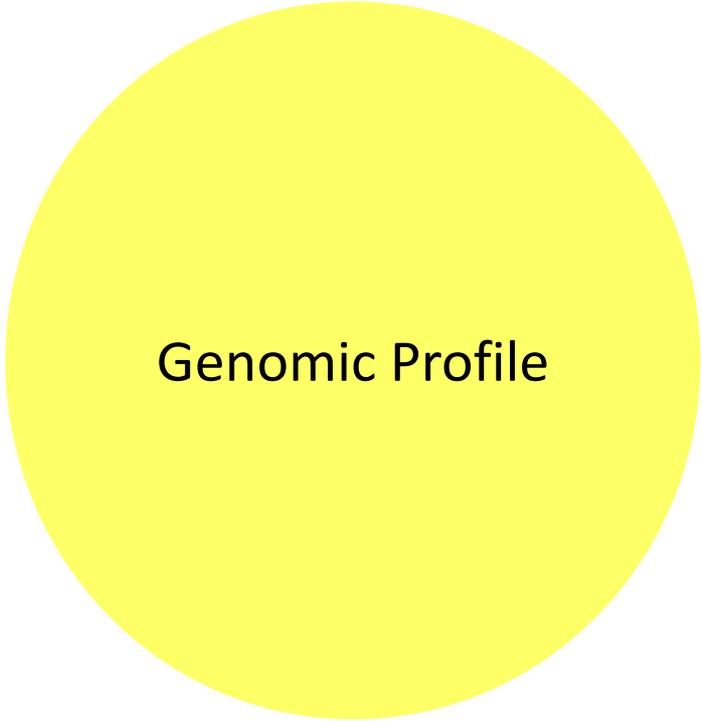
## NATIONAL CANCER INSTITUTE PRECISION MEDICINE IN CANCER TREATMENT

Discovering unique therapies that treat an individual's cancer based on the specific genetic abnormalities of that person's tumor.





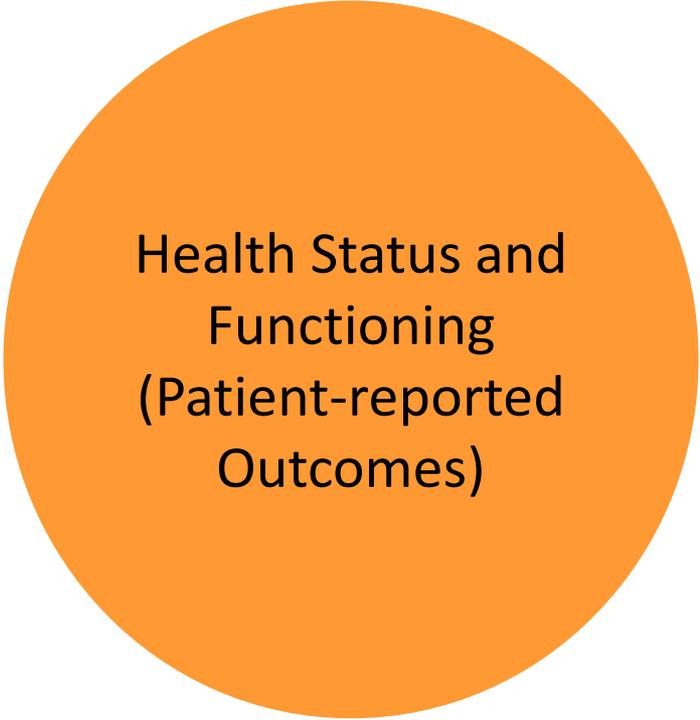




Genomic Profile



Other Clinical  
Characteristics

A large, solid orange circle is centered on the page. Inside the circle, the text "Health Status and Functioning (Patient-reported Outcomes)" is written in a black, sans-serif font, centered horizontally and vertically.

Health Status and  
Functioning  
(Patient-reported  
Outcomes)



Patient  
Experiences of  
Care, Preferences

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# Precision Medicine in Cancer Treatment



Key organizations (NCCS, ACSO, BCBS) have issued guidelines for specific genomic tests and related treatment

Cost of testing, cost of treatment, variable insurance coverage



Limited data about how providers use genomic testing in the context of cancer care



# National Survey of Precision Medicine in Cancer Treatment

Assess a US representative sample of medical oncologists on:



**National Survey of Precision Medicine in Cancer Treatment**

A survey of the  
NATIONAL CANCER INSTITUTE  
NATIONAL INSTITUTES OF HEALTH  
U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES

In collaboration with the  
NATIONAL HUMAN GENOME RESEARCH INSTITUTE, NIH  
and the AMERICAN CANCER SOCIETY

**Please Note:**  
This survey is about genomic testing for cancer treatment, also known as precision or personalized medicine. It is intended for oncologists who have treated or evaluated patients with cancer, including hematologic malignancies and solid tumors. If you have NOT treated or evaluated any patients with any type of cancer in the past 12 months, please check the box below and return the blank survey in the envelope provided.

I have not treated or evaluated cancer patients in the past 12 months.

- Use of genomic tests in treatment decisions
- Perceptions of barriers, and drivers that affect use of genomic tests in clinical practice
- Confidence in knowledge of genomics, ability to explain concepts to patients, and use genomic information to guide treatment
- Understanding of guidelines and evidence supporting use of genomic testing
- Physician, practice, and system factors influencing adoption of genomic testing in clinical care
- What next?

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**Understanding patient's health from patients**



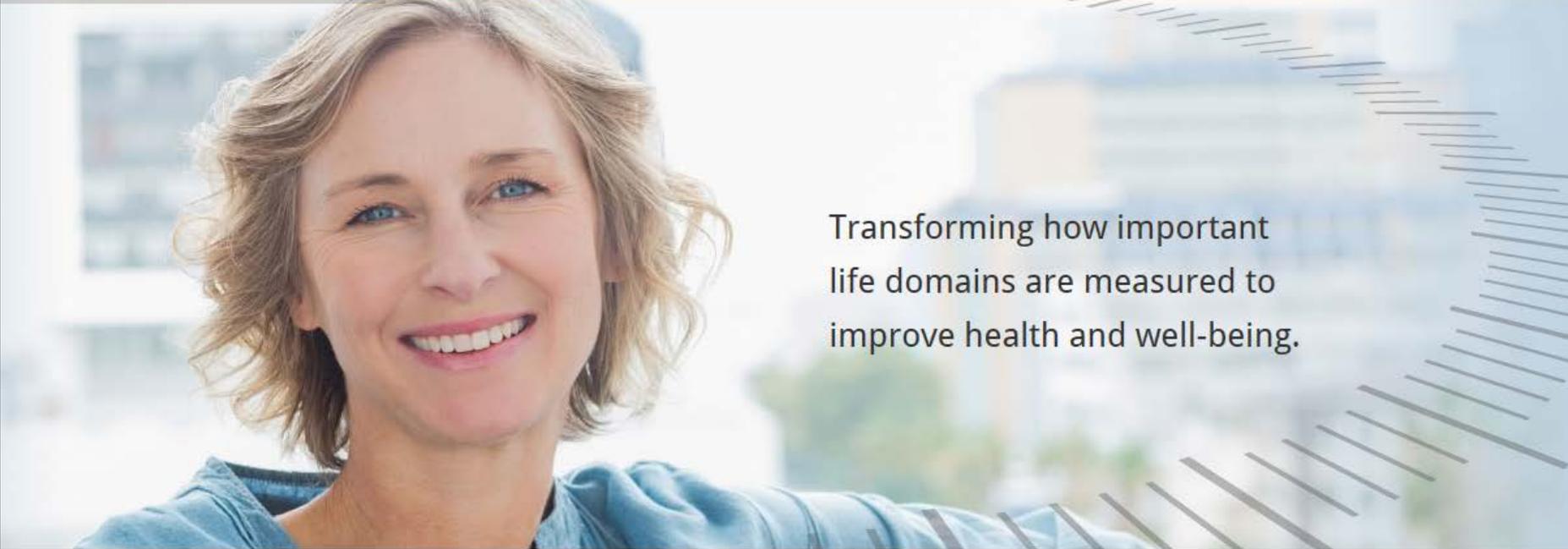
# NIH supported research resource

[www.healthmeasures.net](http://www.healthmeasures.net)



**HealthMeasures**  
TRANSFORMING HOW HEALTH IS MEASURED

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Transforming how important  
life domains are measured to  
improve health and well-being.

## Welcome to HealthMeasures!

Here you'll discover the latest life domain measurement tools to improve patient outcomes and strengthen research quality. Self-reported patient assessments evaluate symptoms, well-being, and life satisfaction; as well as physical, mental, and social health. Examiner-administered assessments measure sensory, motor, and cognitive functions. Concise and cost-effective, these NIH-funded initiatives include PROMIS<sup>®</sup>, NIH Toolbox, Neuro-QoL, and ASCQ-Me.

Brief and psychometrically sound patient-centered health tools for ages 3 to 85  
 Flexible administration – computer adaptive tests (CATs), short forms, and interactive tests  
 For use in clinical research, clinical practice, quality improvement, and educational settings



- Self- and parent-report
- Global, physical, mental, and social health
- Adults and children
- General population and those living with a chronic condition

### NeuroQoL

- Self- and proxy-report
- Physical, mental, and social health
- Adults and children
- Those living with a chronic condition

### ASCQ-Me

- Self-report
- Physical, mental, and social health
- Adults
- Those living with sickle cell disease



- Performance tests
- Cognitive, motor, sensory function, emotion
- Adults and children
- General population, those living with a chronic condition

[www.HealthMeasures.net](http://www.HealthMeasures.net)

[www.healthcaredelivery.cancer.gov/healthmeasures](http://www.healthcaredelivery.cancer.gov/healthmeasures)



NeuroQoL

ASCQ-Me<sup>SM</sup>  
Adult Sickle Cell Quality of Life Measurement Information System



HealthMeasures  
 TRANSFORMING HOW HEALTH IS MEASURED



# PROMIS<sup>®</sup> : Patient Reported Outcomes Measurement Information System<sup>®</sup>

**PRO system:** brief, precise, valid, reliable fixed or tailored tools for patient-reported health status in physical, mental, and social well-being for adult & pediatric populations

**Advantages:** Disease-agnostic, Flexible, Adaptable, low burden, Static forms and Computer Adaptive Testing (CAT)

**Standardized:** Item Response Theory (IRT) for construction; One metric (T-score, Mean=50, SD=10; ref=US population)

**Available for use in Research and Clinical Settings:** pdfs, API, iPad App, Research Platforms (e.g., REDCap), EHR vendors and interface (Epic, Oberd, others to be added)

# PRO Challenges for Clinical Settings

## Need to address:

- Collection, application, integration/combining data
- Interpretation at the individual level
- Clinic flow
- Varied Content

## Challenges with:

- Response Burden
- Shared Electronic Platforms
- Multiple Settings
- Aligning with Care



*"Your appointment's been cancelled. You took too long filling out those forms."*

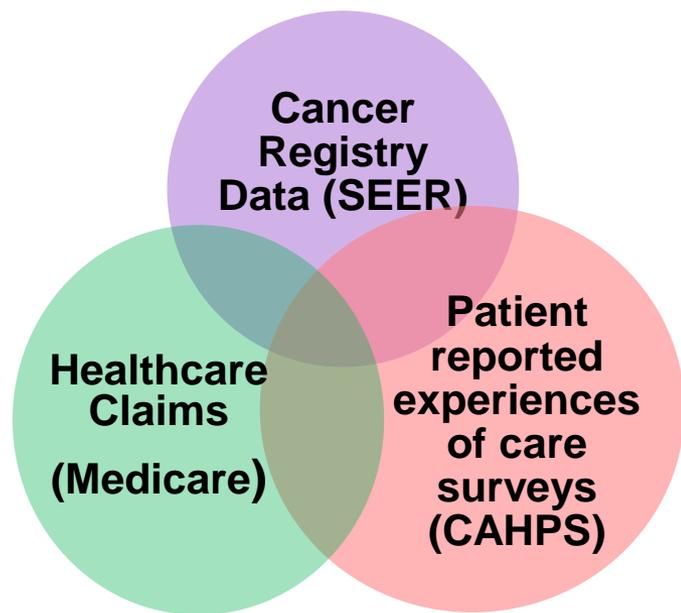
# Patient Experience Of Care

- Encompasses the range of interactions that patients have with the health care system, including from doctors, nurses, and staff in hospitals, physician practices, and other health care facilities.
- Includes domains that patients value highly when they seek and receive care, such as:
  - getting timely appointments,
  - easy access to information
  - good communication with health care providers.

NCI is facilitating research to understand and improve patients' experiences of their care

# SEER-CAHPS Linked Data Resource

Surveillance, Epidemiology and End Results – Consumer Assessment of Healthcare Providers and Systems



## Examines topics such as:

- Doctor Communication
- Getting Needed Care
- Getting Care Quickly
- Care Coordination

- Over **205,000 cancer** respondents
- More than **724,000 non-cancer**
- Medicare **claims** allow examination of aspects of healthcare utilization

Rich data source on experiences of care from perspective of cancer patients

# SEER-CAHPS Linked Data Resource

Surveillance, Epidemiology and End Results – Consumer Assessment of Healthcare Providers and Systems

## Sample **Research Questions** When Using SEER-CAHPS data

### Patient/Physician Communication

- How do perceptions of physician communication vary by cancer type?
- Do ratings of physician communication predict healthcare utilization patterns?

### Care Coordination

- Does perception of care coordination vary based on cancer phase (diagnosis, treatment, last year of life)? By cancer treatment modality?

### Access to Care

- Is perceived access to care associated with time between diagnosis and first course of cancer treatment?
- Does type of physician (primary care vs. specialist) predict perceived timeliness?

### Patient Perceptions of Care Quality

- Do patient experiences with quality predict subsequent healthcare

# Bringing the Patient Voice to the Value Conversation

- Examine context and goals of importance to patients
- *A priori* and ongoing conversations about needs and prioritization of treatment options and services in context of whole person
- Evaluate costs and financial burden on patients
- Context Matters: Priorities can and will evolve

## THE CANCER CONTROL CONTINUUM



# Addressing Patient Values, Preferences and Priorities: **Goals and Gaps**

## Goals

- Determine patients' perspectives on their health and care
- Combine different sources of data and interpret together
- Develop cohesive and clear understanding of patient health and needs
- Integrate in real time for decision-making

## Gaps

- Ways to make patient priorities visible in clinical record
- Evidence of the effects of goal-setting on relevant health and other outcomes
- Effective tools to assess patient values and priorities are lacking
- **Understand need to assess priorities, few are doing it**

# Mayo PROQOL instrument

which of the following, if any, represents your  
**single biggest concern**  
 right now...

 <p><b>personal relationships</b></p> <ul style="list-style-type: none"> <li>• Family</li> <li>• Friends</li> </ul>	 <p><b>monitoring health</b></p> <ul style="list-style-type: none"> <li>• Testing blood sugars</li> <li>• Checking feet</li> </ul>	 <p><b>emotional health</b></p> <ul style="list-style-type: none"> <li>• Sad</li> <li>• Anxious</li> <li>• Other emotional concerns</li> </ul>	 <p><b>money</b></p> <ul style="list-style-type: none"> <li>• Cost of medicine or supplies</li> <li>• Paying for care</li> </ul>	 <p><b>health behaviors</b></p> <ul style="list-style-type: none"> <li>• Diet</li> <li>• Exercise</li> <li>• Sleep</li> </ul>
 <p><b>medicine</b></p> <ul style="list-style-type: none"> <li>• Taking medication</li> <li>• Managing side effects</li> </ul>	 <p><b>getting health care</b></p> <ul style="list-style-type: none"> <li>• Finding a provider to talk to</li> <li>• Scheduling appointments</li> </ul>	 <p><b>work</b></p> <ul style="list-style-type: none"> <li>• Schedule</li> <li>• Environment</li> <li>• Managing your health condition at work</li> </ul>	 <p><b>physical health</b></p> <ul style="list-style-type: none"> <li>• Pain</li> <li>• Fatigue</li> <li>• Physical difficulties</li> </ul>	 <p><b>something else</b></p>

## \$ money

Have you recently had any of the following problems or concerns?  
 (Check all that apply)

- Problems paying your medical bills
- Problems paying for all the care you needed
- Problems paying for all the medicines or supplies that you needed
- Not maintaining health insurance coverage
- Skipped a recommended test or medical treatment
- Not filled a prescription for a medicine
- Cut pills in half or skipped doses of medicine
- Skipped dental, vision or mental health care visit because of cost
- Put off or postponed getting health care you needed
- Something else



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# Implementation of Systematic Symptom Management: Beau Biden Cancer Moonshot<sup>SM</sup> Initiative

**GOAL:** Accelerate progress in preventing, diagnosing, and treating cancer to accomplish a decade's worth of work in 5 years

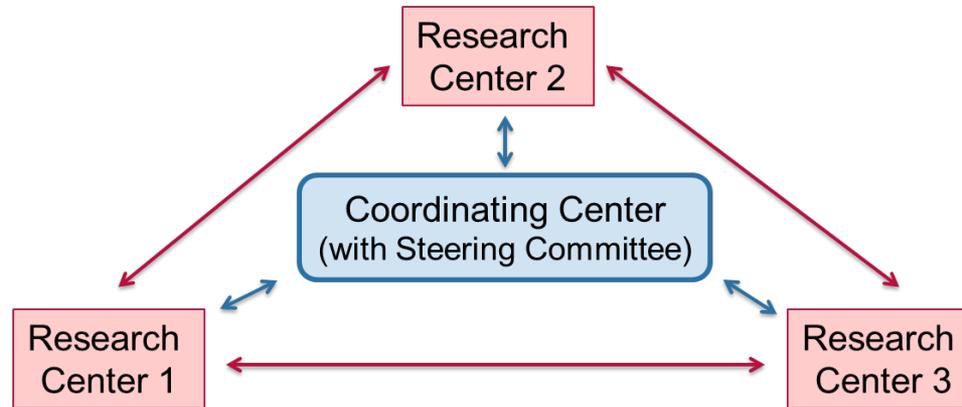


## RECOMMENDATION F:

**Minimize cancer treatment's debilitating side effects**  
Accelerate the clinical adoption of integrated systems to monitor patient-reported symptoms and provide decision support using implementation science approaches and evidence-based symptom management guidelines.

Improving Management of Symptoms during And following Cancer Treatment (IMPACT) Research Consortium

# IMPACT Research Consortium



- Develop scalable, transferable, sustainable models to monitor and address common cancer symptoms in clinical practice
- Rigorously examine impact on symptom control, functioning, treatment delivery, and healthcare utilization
- Using network-wide data, evaluate effects across:  
Symptoms, Cancer continuum, Minority and medically underserved
- Produce findings and materials for wider implementation

## Examples of Current Healthcare Delivery Research Program Funding Opportunity Announcements (FOA)

Title	FOA Number (Mechanism)	Brief Description	Expiration Date	HDRP Contact
<b>Using IT to Support Systematic Screening and Treatment of Depression in Cancer</b>	PA-18-493 (R01) PA-18-492 (R21)	Encourages research on new IT-enabled delivery models to improve management of depression in cancer	May 8, 2021	Gurvaneet Randhawa 240-276-6940 <a href="mailto:Gurvaneet.randhawa@nih.gov">Gurvaneet.randhawa@nih.gov</a>
<b>Oral Anticancer Agents: Utilization, Adherence, and Health Care Delivery</b>	PA-18-004 (R01) PA-18-014 (R21)	Funds development of models and strategies to improve safe and effective delivery of OAA to optimize clinical outcomes	January 8, 2020	Kathleen Castro 240-276-6834 <a href="mailto:castrok@mail.nih.gov">castrok@mail.nih.gov</a>
<b>Intervening with Cancer Caregivers to Improve Patient Health Outcomes and Optimize Health Care Utilization</b>	PAR-18-246 (R01) PAR-18-247 (R21)	Funds interventions that support the success of informal cancer caregivers	April 12, 2019	Michelle Mollica 240-276-7621 <a href="mailto:michelle.mollica@nih.gov">michelle.mollica@nih.gov</a>
<b>Linking the Provider Recommendation to Adolescent HPV Vaccine Uptake</b>	PAR-18-008 (R01) PAR-16-337 (R03) PAR-18-019 (R21)	Encourages research on how delivery system enhances or inhibits effectiveness of a provider's recommendation	July 6, 2019 (R01) July 17, 2019 (R03, R21)	Sarah Kobrin 240-276-6931 <a href="mailto:kobrins@mail.nih.gov">kobrins@mail.nih.gov</a>

For a comprehensive list of current DCCPS FOA, visit: <https://cancercontrol.cancer.gov/funding.html>  
<https://healthcaredelivery.cancer.gov>

# Bringing it all together: Toward Tailored Healthcare

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**Goal:** Collect and act upon targeted information from and about patients that can be incorporated into clinical care that takes into account the whole patient

**What:**

- Combine clinical information with patient values, functioning, symptoms (Hint: may change over time)

**How:**

- Utilize current technology and innovative approaches to individualize care
- Engage patients to address their unique needs and ensure the best outcomes

# Discussion

[Ashley.Smith@nih.gov](mailto:Ashley.Smith@nih.gov)



NATIONAL  
CANCER  
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