Patient Perspective on Patient Preference Information Landscape

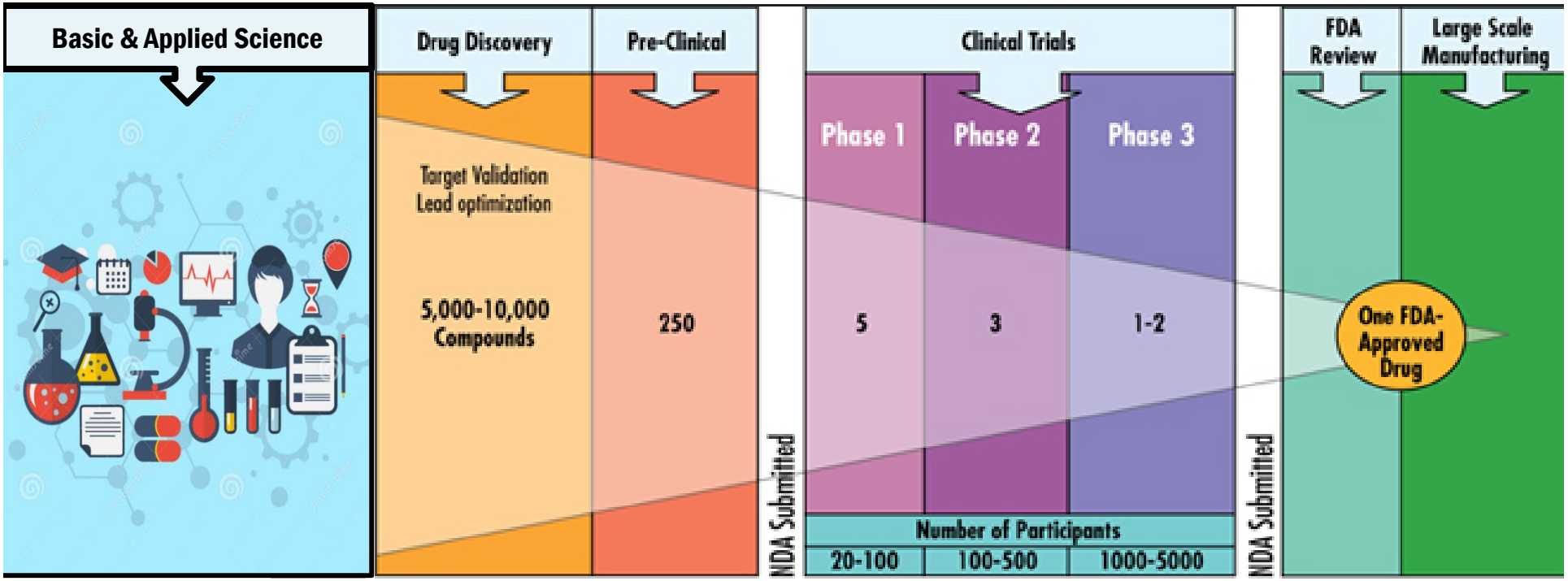
K. Kimberly McCleary
Acting Executive Director | Managing Director
FasterCures

CERSI Patient Preference Workshop
December 7-8, 2017

FasterCures
A CENTER OF THE MILKEN INSTITUTE

FasterCures is an "action tank" driven by a singular goal – to save lives by speeding up and improving the medical research system


lease Give Generousl



walk MS

WALK TO END ALZHEIMER'S alzheimer's association

Patient groups mobilize, advocate, fundraise & inform

Patient groups enlisted by sponsor/ CRO to help recruit for clinical trials

One patient representative appointed by FDA to advisory committee

FasterCures
A CENTER OF THE MILKEN INSTITUTE

FasterCures
A CENTER OF THE MILKEN INSTITUTE

Patient Group Engagement Across the Clinical Trial Continuum

Building a model to evaluate impact

- Direct funding and fund raising for research or product development
- Natural history database/registry support
- Help define eligibility criteria within the study protocol
- Feedback on meaningful clinical endpoints
- Assist in creating the informed consent form
- Advise on study recruitment
- Accompany sponsor to FDA to advocate study design

- Direct funding and fund raising for trial operations support
- Network recruitment / outreach
- Serve on a Data Safety Monitoring Board
- Report on patient feedback regarding sites, investigators, and study participant experience

- Natural history database / registry support
- Provide feedback on how the patient community views results
- Help return study results to participants
- Write newsletter articles or blog about results
- Co-present results
- Serve on post-market surveillance initiatives

Pre-Discovery → Pre-Clinical → Phase 1 → Phase 2/3 → FDA review & approval → PAS/Outcomes

- Interest of research question to patient community
- Provide data on unmet need and therapeutic burden
- Direct funding and fund raising for research or product development
- Understanding mechanisms of action relevant to disease and symptom burden

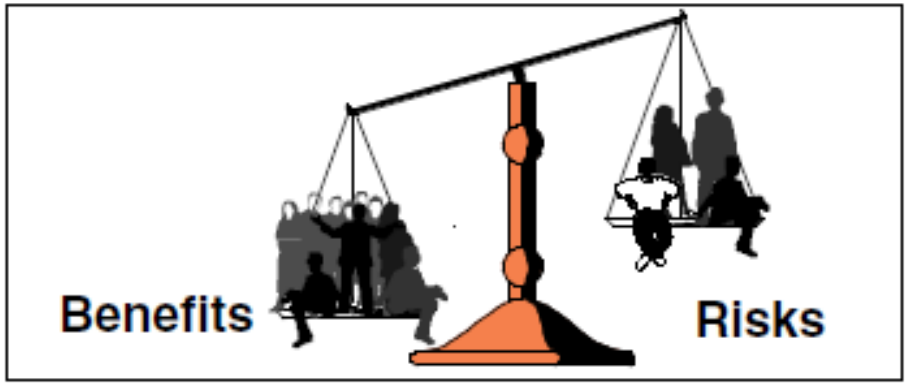
- Network recruitment / outreach
- Direct funding and fund raising for research or product development
- Infrastructure support
- Provide input on study design (barriers to participation)
- Support trial awareness and recruitment
- Peer advocate during informed consent procedure

- Serve on FDA advisory committees
- Provide testimony at FDA hearings
- Feedback on meaningful clinical endpoints

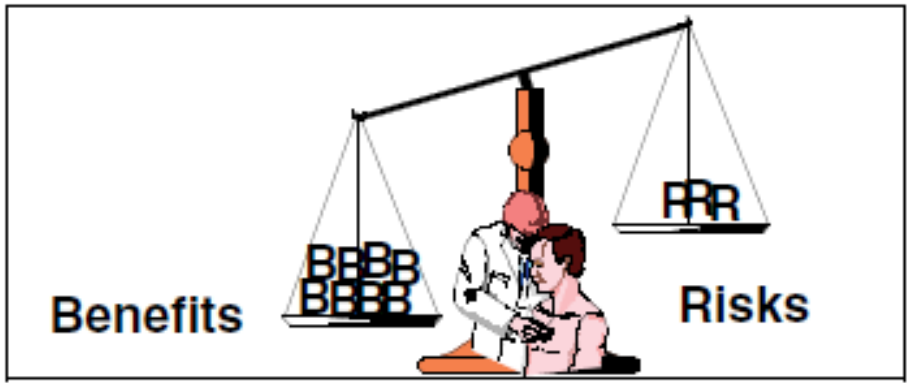
**Adapted from Parkinson's Disease Foundation materials for CTTI's Patient Groups & Clinical Trials Project*

CTTI

FDA
evaluates
benefits and risks
for the population



Provider
evaluates
benefits and risks
for a patient



Patient
evaluates
benefits and risks
in terms of
personal values

"Understanding the Benefits and Risks of Pharmaceuticals: A Workshop Summary"
Institute of Medicine, 2006

FasterCures
A CENTER OF THE MILKEN INSTITUTE

Contains Nonbinding Recommendations

Factors to Consider Regarding Benefit-Risk in Medical Device Product Availability, Compliance, and Enforcement Decisions

Guidance for Industry and Food and Drug Administration Staff

Document issued on December 27, 2016.

The draft of this document was issued on June 16, 2016

For questions about this document regarding CDRH-regulated devices, contact the Office of Compliance at 301-796-5900.

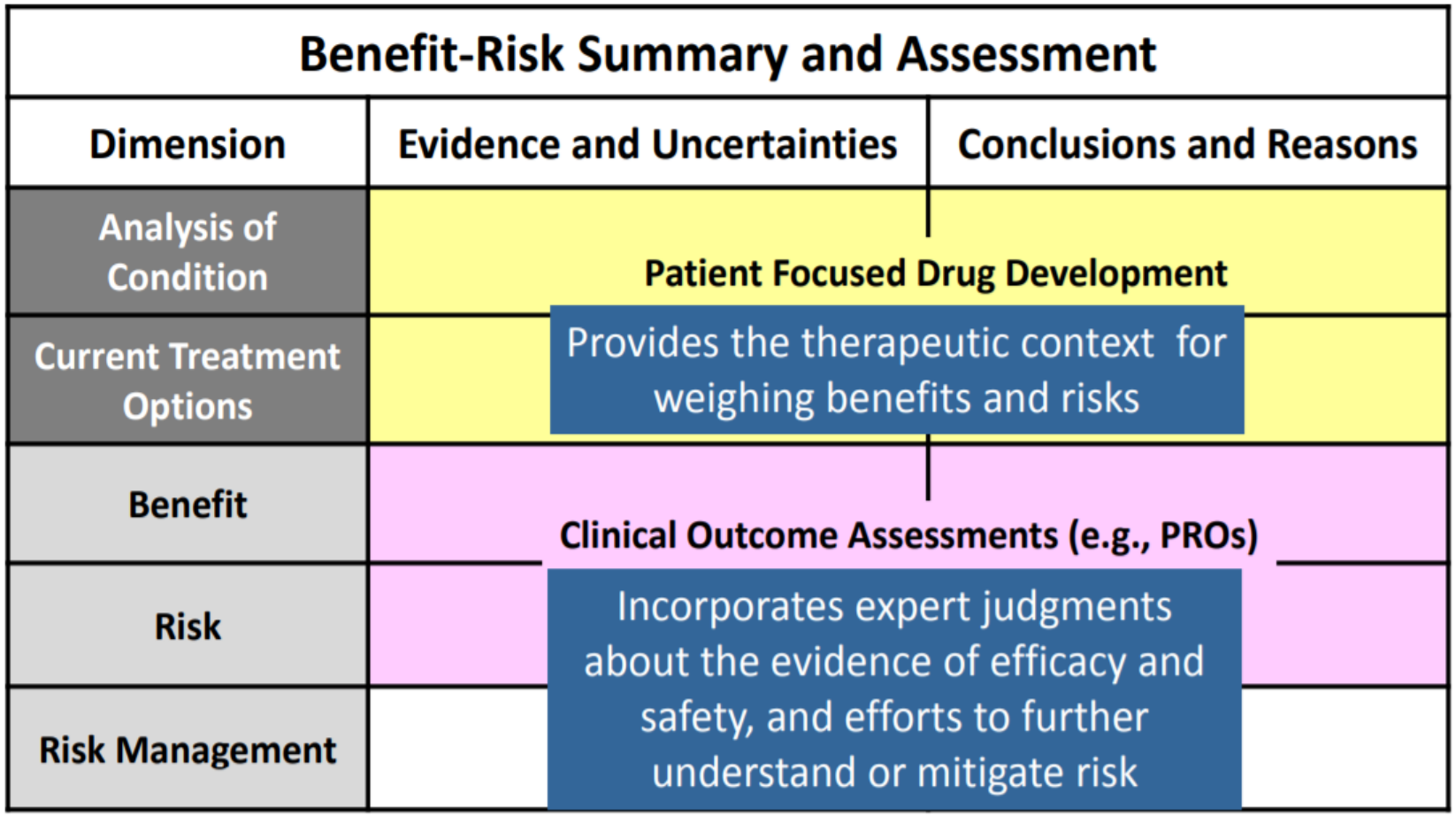
FDA U.S. FOOD & DRUG ADMINISTRATION
CENTER FOR DEVICES & RADIOLOGICAL HEALTH

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Devices and Radiological Health

Patient perspective on benefits	What is the severity of the disease state? Is this a chronic disease? If chronic, can the illness be managed with other treatments or therapies? How long do patients live with the disease? Even if the benefit is in a small portion of the population, do those patients who would experience the benefit value it? Is the duration of the benefit achieved of value to patients? How much do patients value this treatment? Does the treatment improve overall quality of life? Are the benefits of the medical device well understood? Is communication regarding change in benefit realistic?	What is the severity of the disease state? Is this a chronic disease? If chronic, can the illness be managed with other treatments or therapies? How long do patients live with the disease? Even if the benefit is in a small portion of the population, do those patients who would experience the benefit value it? Is the duration of the benefit achieved of value to patients? How much do patients value this treatment? Does the treatment improve overall quality of life? Are the benefits of the medical device well understood? Is communication regarding change in benefit realistic?
Patient tolerance of risk	What level of concern do patients have regarding the risks? Even if the risk is in a small portion of the population, do those patients who would experience the risk understand it? Are patients willing to take the risk of this treatment to achieve the benefit? How well are patients able to understand the risks of the treatment?	What level of concern do patients have regarding the risks? Even if the risk is in a small portion of the population, do those patients who would experience the risk understand it? Are patients willing to take the risk of this treatment to achieve the benefit? How well are patients able to understand the risks of the treatment?

FasterCures
A CENTER OF THE MILKEN INSTITUTE

Patient Perspective Can Inform BR Assessment at Multiple Levels

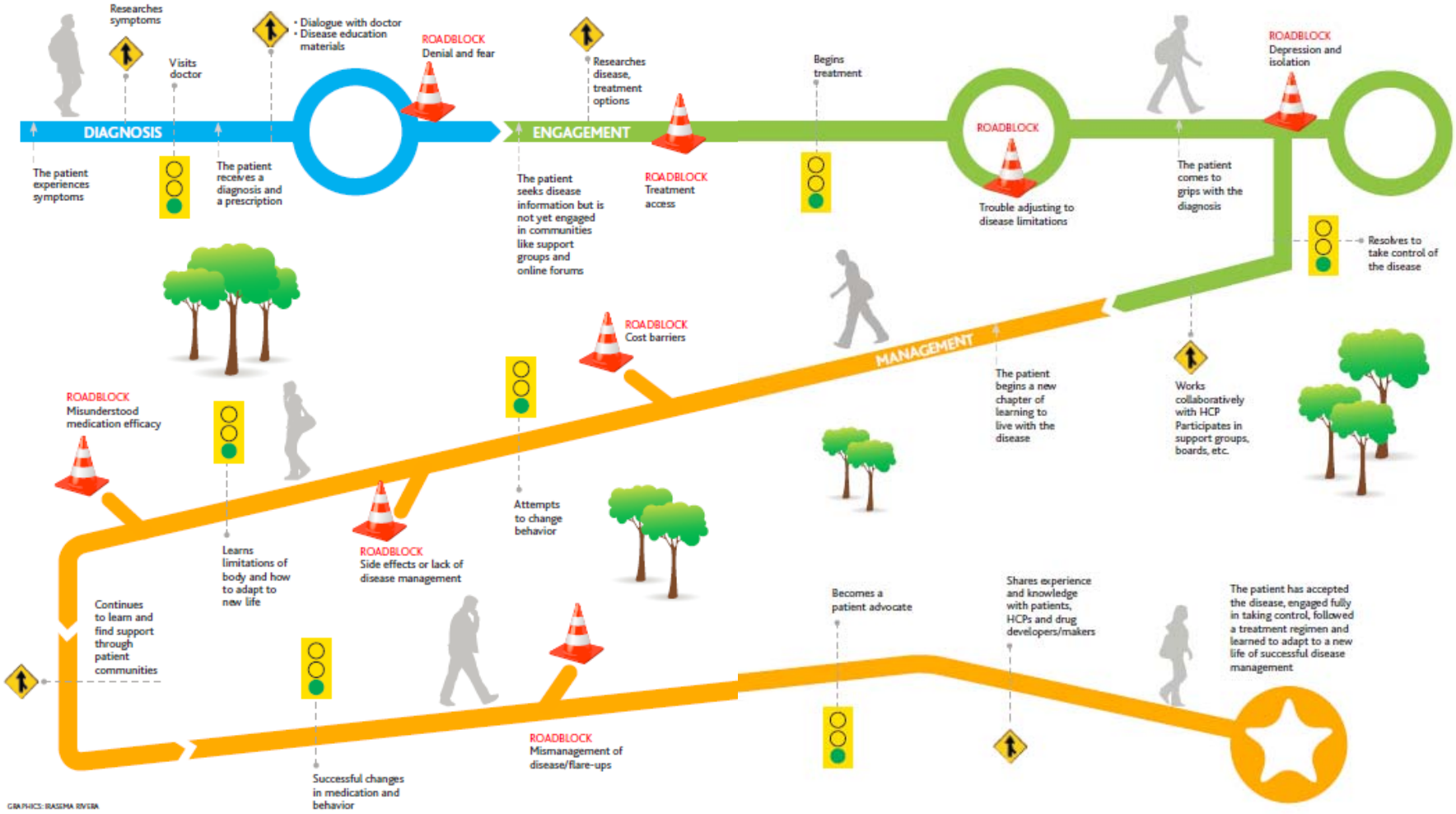


From Theresa Mullin, "Informing Benefit-Risk Assessment with Patients' Perspectives, September 18, 2017

FasterCures
A CENTER OF THE MILKEN INSTITUTE

The Patient Journey

Illuminating gaps and opportunities in the case of a rheumatoid arthritis (RA) patient



The Patient Journey | Medical Marketing & Media

FasterCures
A CENTER OF THE MILKEN INSTITUTE

Relationship of Benefit-Risk Tradeoffs to Health Objective

Disease Prevention
- safe
- minimally invasive
- durable effect

Acute life-saving
- effective
- immediate
- manageable AEs

Chronic treatment
- durable
- minimal AEs
- convenience for desired activity level

End of Life
- minimal AEs
- based on desire for hospice, or home care

Chronic disease delay
- durable
- manageable AEs
- convenience for desired activity level

Curative
- targeted
- durable
- manageable AEs

Frank Rockhold, PhD | Duke & GSK

FasterCures
A CENTER OF THE MILKEN INSTITUTE

Evaluating Evidence & Options

- Age
- Gender
- Race
- Stage of disease
- Severity of disease
- Subtype (pharmacogenetics, others)
- Comorbidities
- Prior medical history
- Prior treatment history
- Prognosis
- Family history/genetics
- Lifestyle factors
- Risk tolerance

How closely did study participants resemble me?

How well do potential benefits meet my needs?
What harms do I risk?

FasterCures
A CENTER OF THE MILKEN INSTITUTE

Insurance coverage

Reimbursement

Out of pocket costs

Disability vs. employment

Geographic proximity

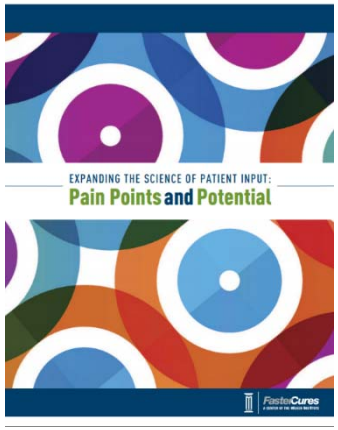
Support system

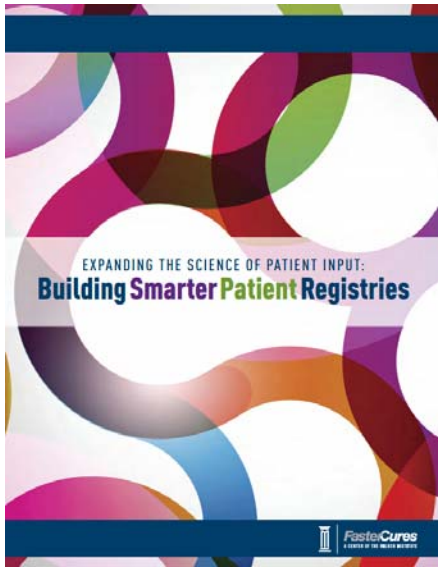
FasterCures
A CENTER OF THE MILKEN INSTITUTE

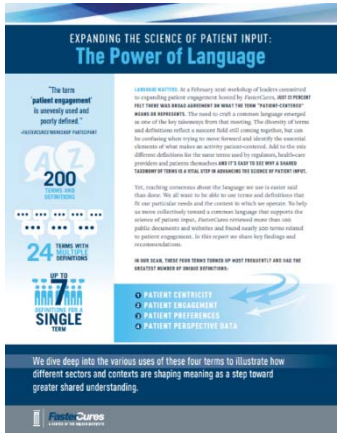
Precision Medicine 2050?

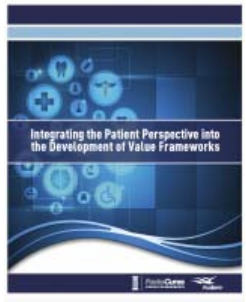
Patients Count
THE SCIENCE OF PATIENT INPUT

Learn more at fastercures.org/programs/patients-count









FasterCures
A CENTER OF THE MILKEN INSTITUTE