Patient Engagement Advisory Panel

October 27, 2015

Washington, DC

pcori

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Agenda – October 27

9:00 a.m.	Recap of Day 1, Overview of Day 2
9:15 a.m.	Patient-focused benefit-risk: Drugs and Medical Devices
9:45 a.m.	Training Update
10:15 a.m.	Break
10:30 a.m.	Toolkit Discussion
11:00 a.m.	Pipeline to Proposals and Ambassadors Program Updates
11:30 a.m.	Engagement Awards Update
12:00 p.m.	Wrap-up, Next steps, and Reflections
12:30 p.m.	Meeting Adjourned

Recap of Day 1, Overview of Day 2

Sue Sheridan, MIM, MBA, DHL

Director of Patient Engagement

pcori

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Patient-focused Benefit-Risk: Drugs and Medical Devices

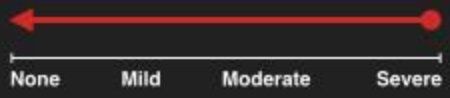
October 27, 2015

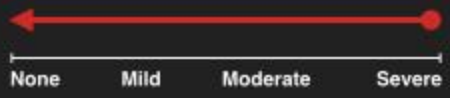
PCORI Patient Engagement Advisory Panel Meeting

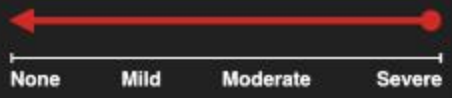
Bennett Levitan, MD-PhD

Department of Epidemiology

Janssen Research & Development, LLC

Outcome	Drug A	Drug B
Relief of pain		
Ability to perform work/school and social activities	No limitations	Cannot work, difficulty with chores and shopping
Annual chance of a heart attack	1 in 10,000	No chance
Which medicine would you choose if these were the only medicines available?	☐	☐

Outcome	Drug C	Drug B
Relief of pain		
Ability to perform work/school and social activities	No limitations	Cannot work, difficulty with chores and shopping
Annual chance of a heart attack	1 in 1,000	No chance
Which medicine would you choose if these were the only medicines available?	☐	☐

Outcome	Drug D	Drug B
Relief of pain		
Ability to perform work/school and social activities	No limitations	Cannot work, difficulty with chores and shopping
Annual chance of a heart attack	1 in 100	No chance
Which medicine would you choose if these were the only medicines available?	☐	☐

Endpoint	Study Drug/ 10,000 pt-yrs	Comparator Rate/ 10,000 pt-yrs	HR (95% CI)	Risk Difference / 10,000 pt-yrs
Primary efficacy (Stroke + embolism)	177	218	0.78 (0.65, 0.94)	-41 (-78, -5)
Secondary efficacy 1 (stroke, embolism + vascular death)	318	371	0.85 (0.73, 0.98)	-54 (-102, -6)
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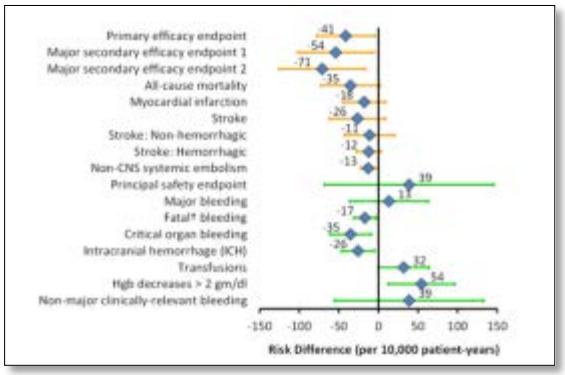
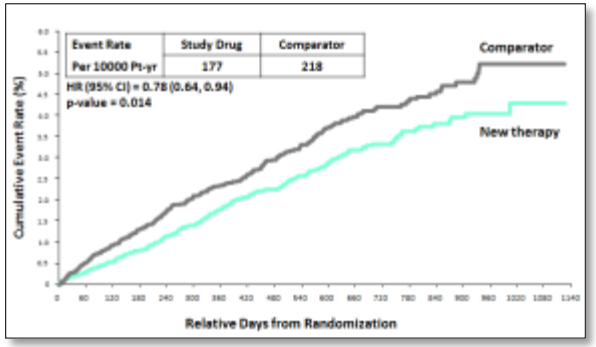
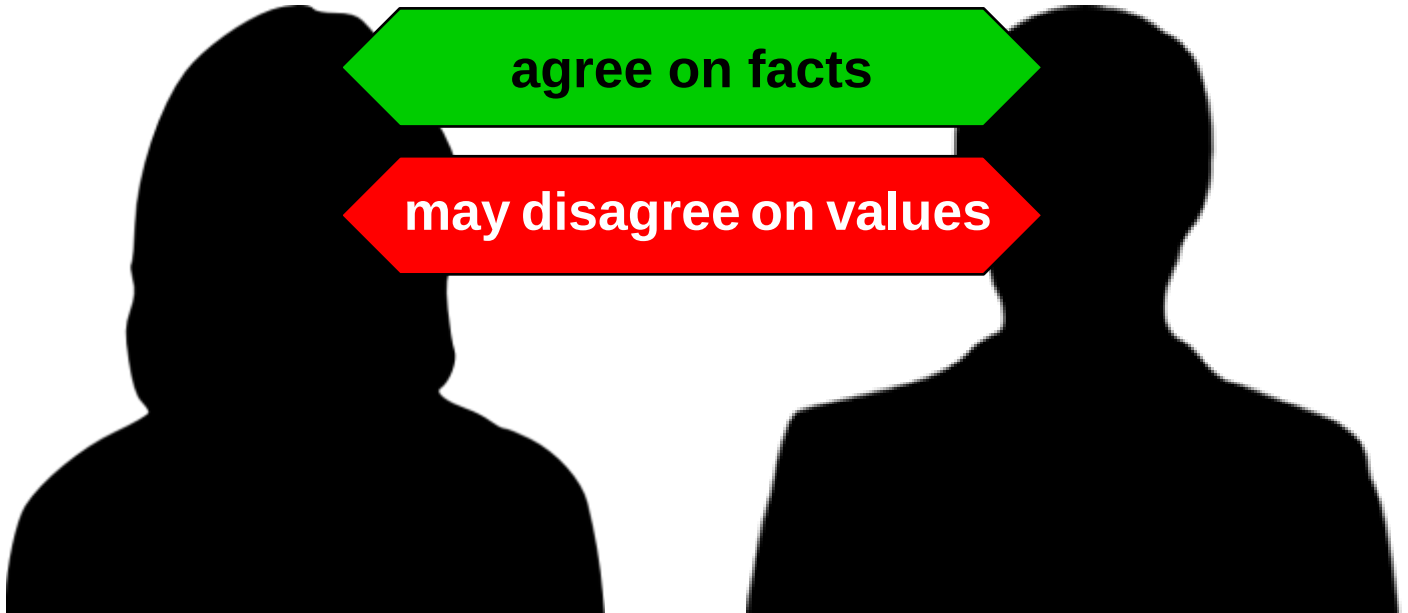




physician **patient**



What Did Migraine Patients Say?

Stated choice conjoint preference survey of 200 adult migraine patients

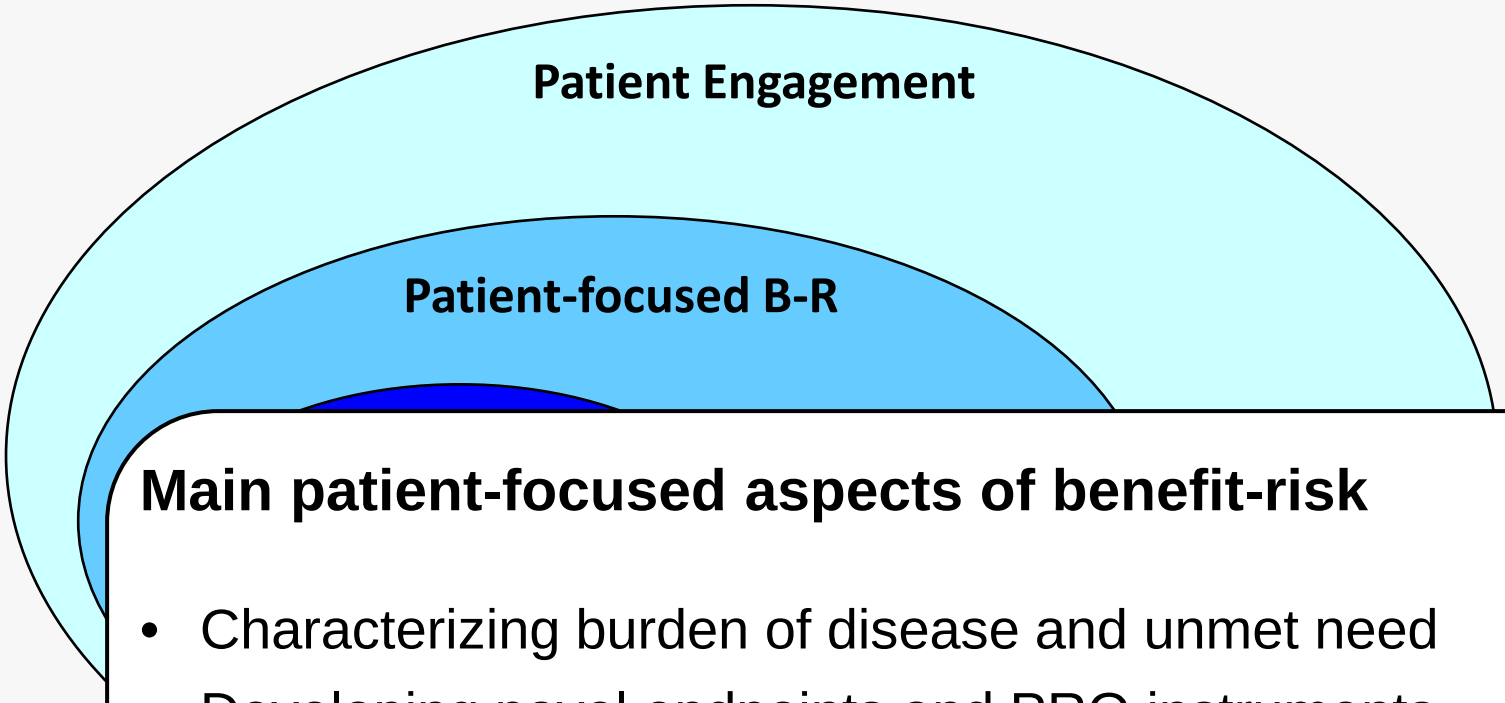
- **Relieving all functional limitations was twice as important as relieving all migraine pain**

Maximum Acceptable Risk = maximum level of treatment-related 1-year myocardial infarction risk patients would accept for a given improvement in migraine symptoms

- **Patients would accept up to a 2/1000 (95% CI 1.6 – 2.4) annual MI risk in exchange for restoring their ability to function during migraines.**

Gonzalez, Hauber, Levitan, Coplan, ISPOR 17th Annual International Meeting June, 2012.

Patient Engagement, Patient-Focused B-R and Patient Preferences

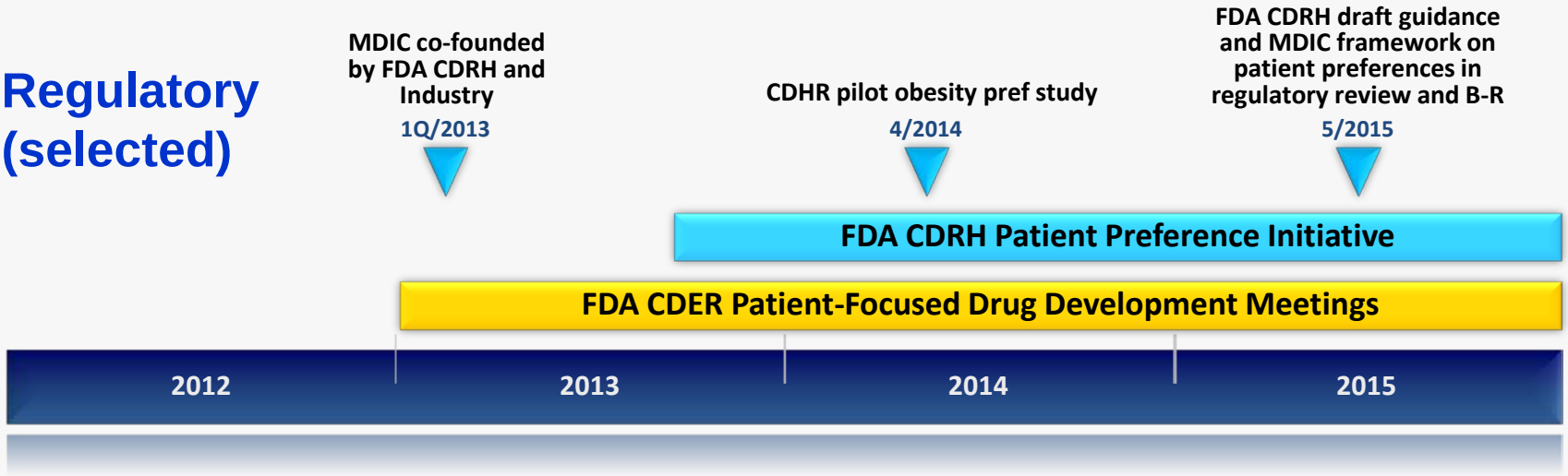


Main patient-focused aspects of benefit-risk

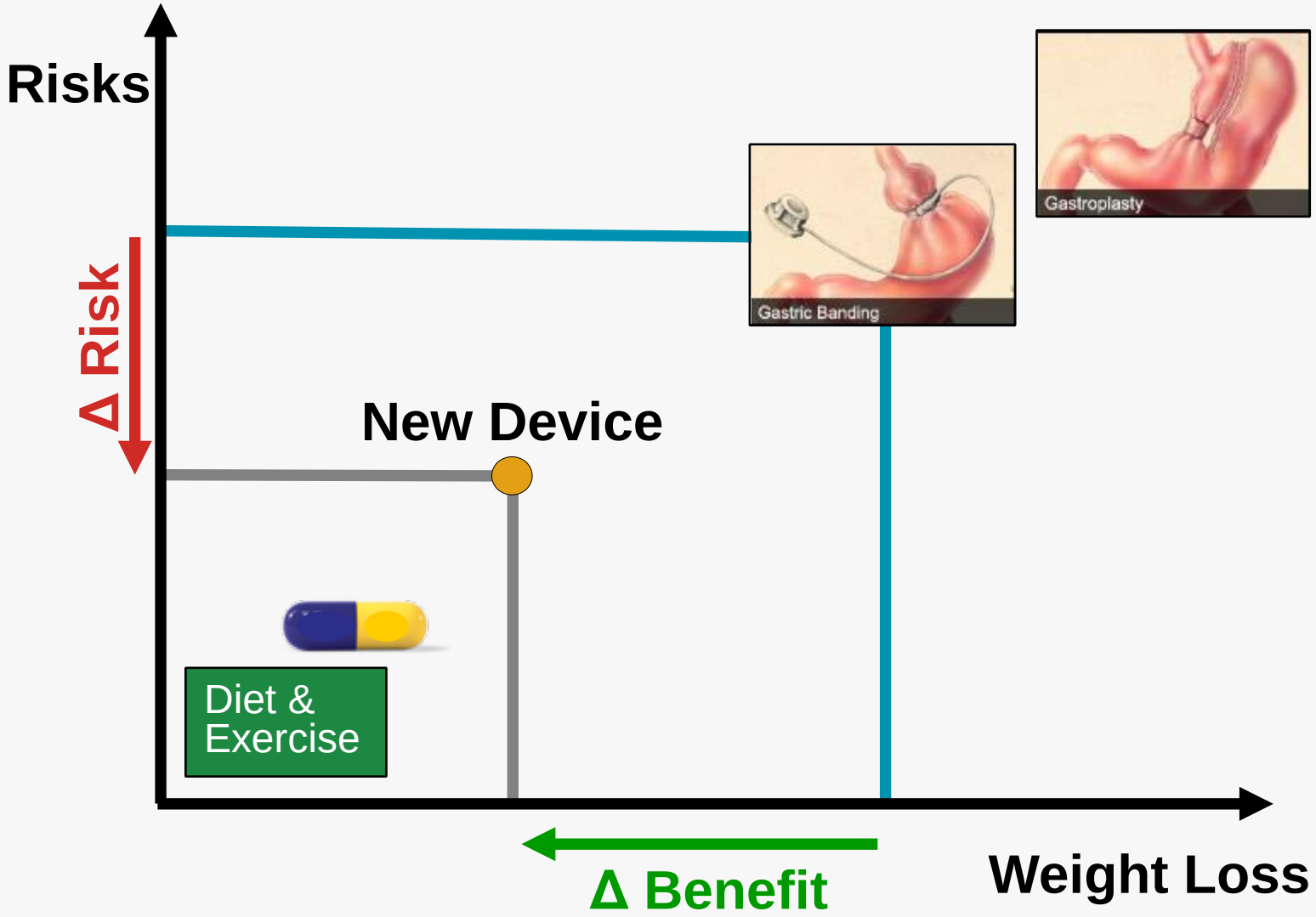
- Characterizing burden of disease and unmet need
- Developing novel endpoints and PRO instruments
- Determining which endpoints/rates/levels are most important
- Assessing preference tradeoffs between endpoints
- Identifying preference heterogeneity and subgroups

Growing Regulatory Momentum for Patient-Focused Drug Development / B-R

Regulatory (selected)



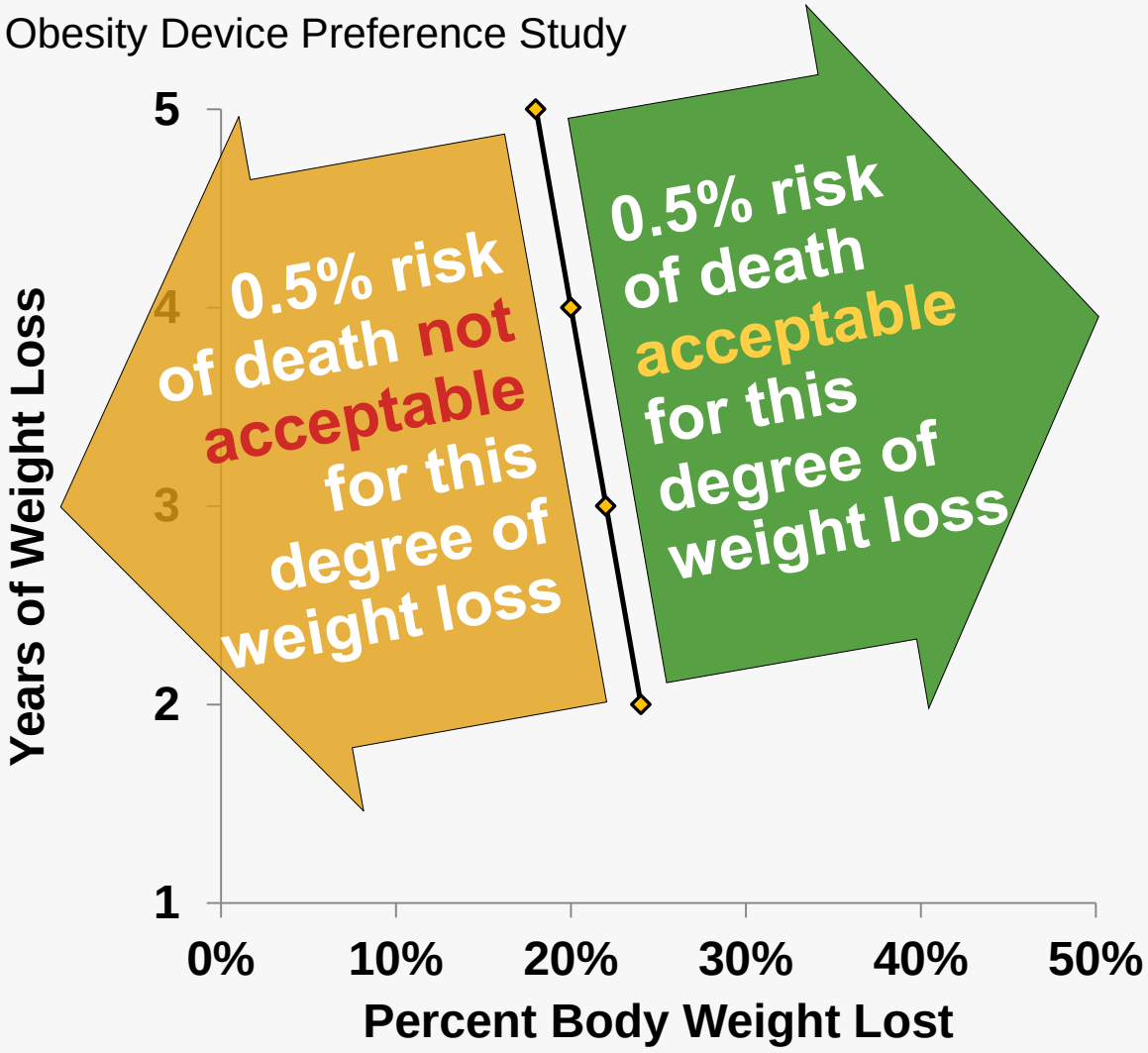
FDA CDRH Obesity Device Preference Study



Ho, M. P., et al. (2015). "Incorporating patient-preference evidence into regulatory decision making." Surg Endosc.

Detailed Thresholds for Maximum Acceptable Risk: Can Inform Development Strategy and Regulatory Requirements

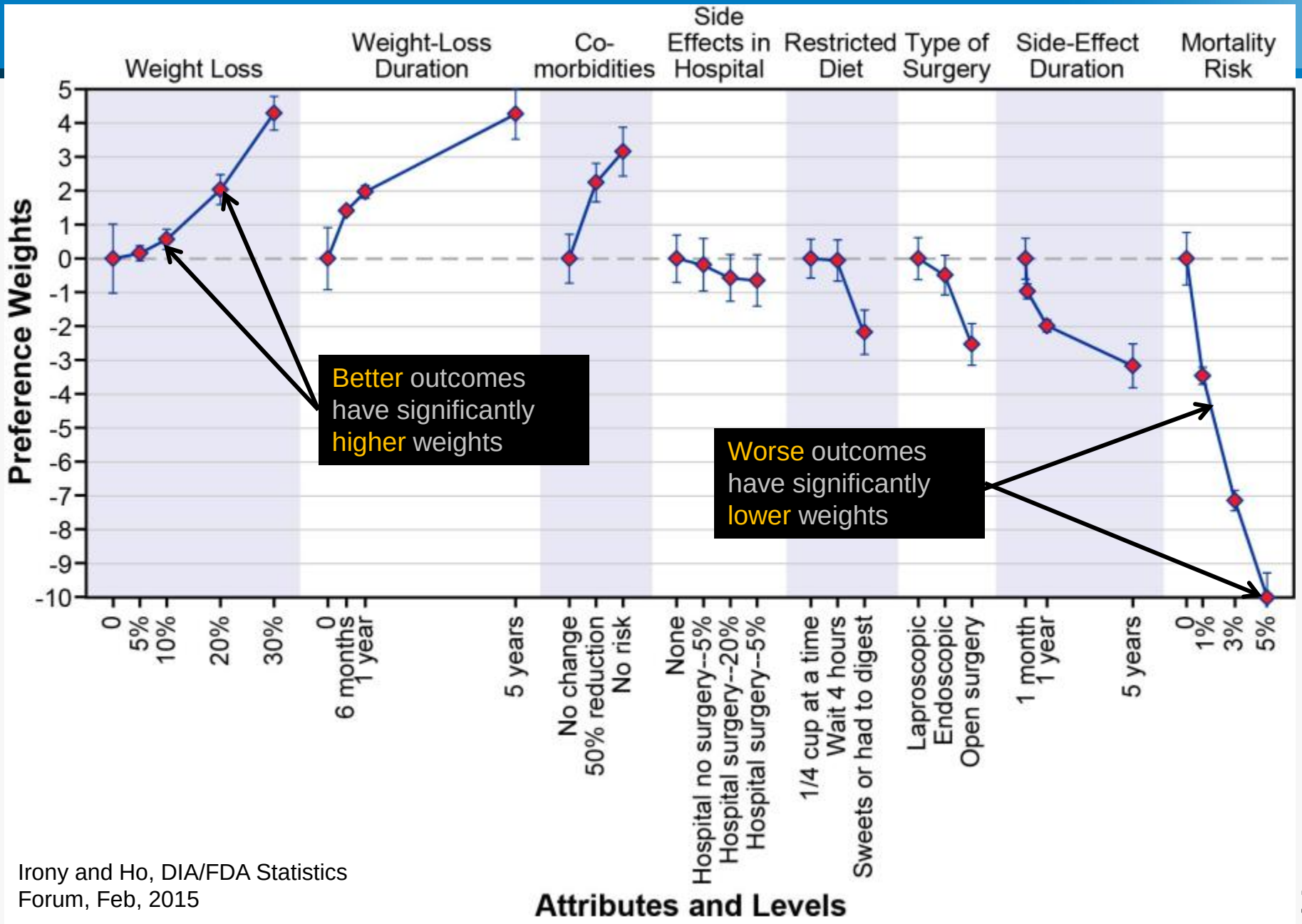
FDA CDRH/RTI Obesity Device Preference Study



Irony and Ho, DIA/FDA Statistics Forum, Feb, 2015

Results for average obese patient (243lbs and 5'10")

Mean Preference Weights



Irony and Ho, DIA/FDA Statistics Forum, Feb, 2015

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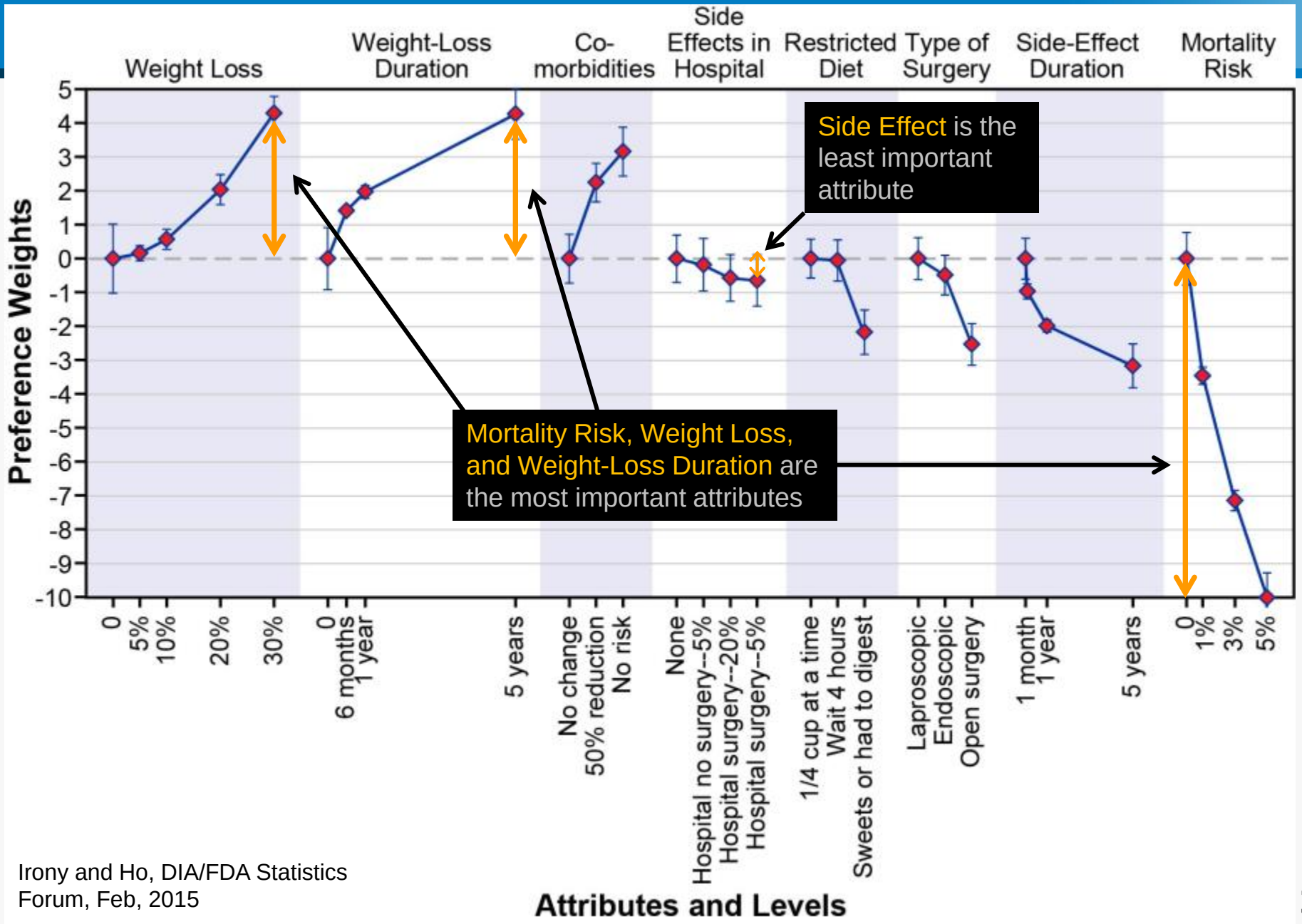

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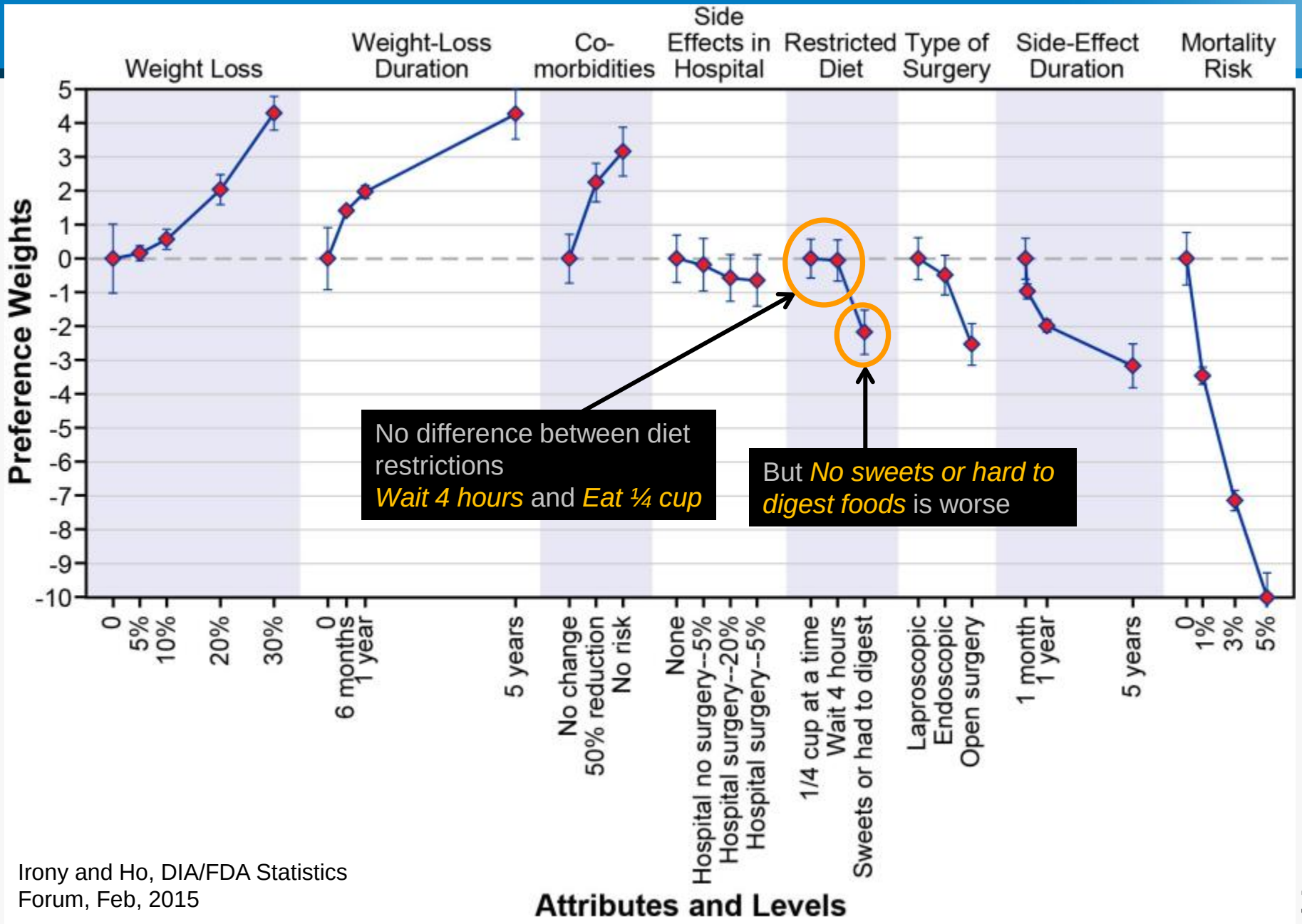


Irony and Ho, DIA/FDA Statistics Forum, Feb, 2015

Can These Studies Make a Difference?

FDA Weighs Patients' Risk Tolerance in Approving Obesity Device

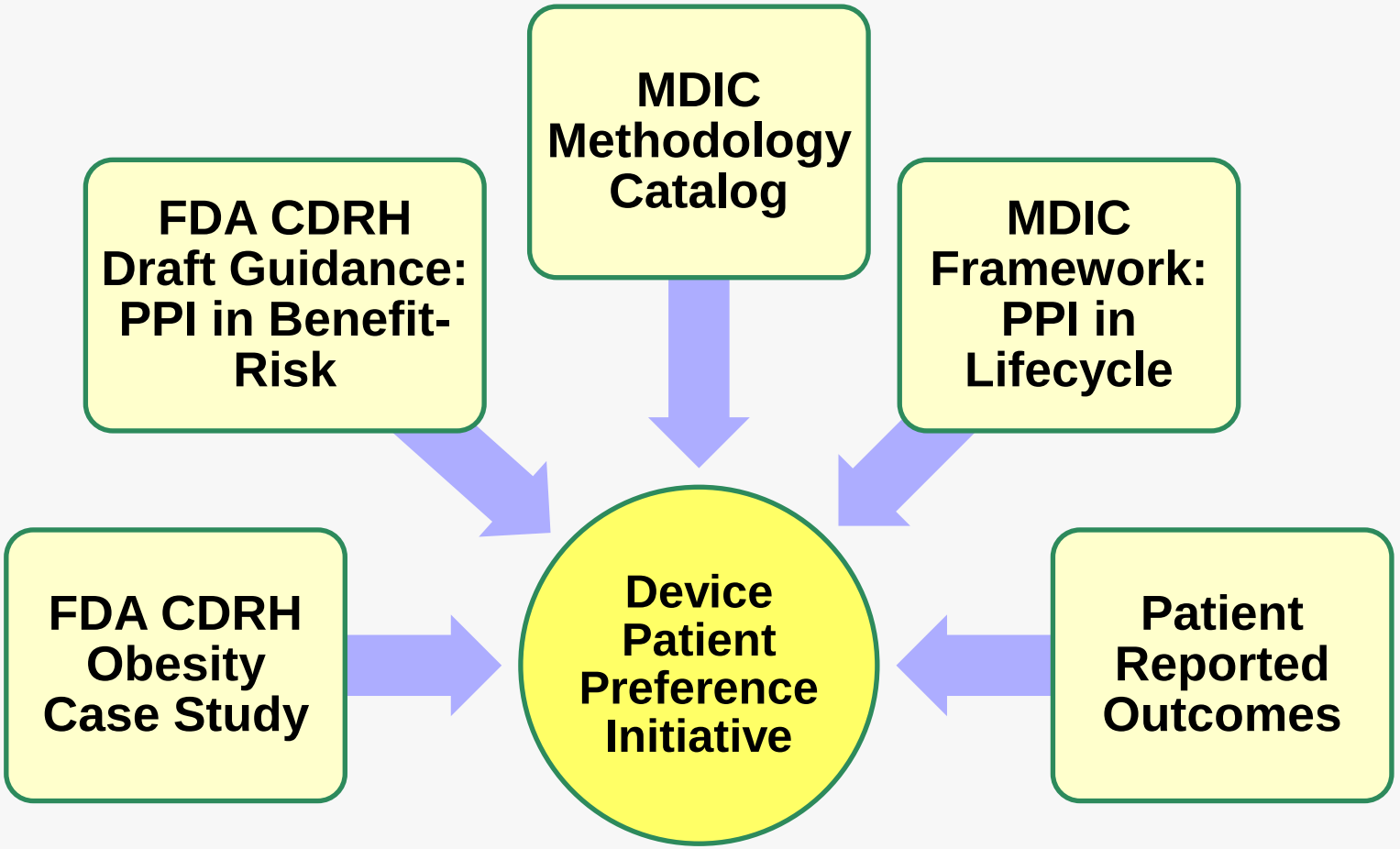
By Ferdous Al-Faruque / Email the Author / View Full Issue

The agency approved EnteroMedics' *Maestro* neuromodulator to treat obesity despite the device not meeting endpoints in its pivotal trial. The agency relied in part on a survey that found obese patients willing to take more risks in exchange for weight loss.

In making the decision the agency took into consideration patients' willingness to accept higher potential risk of the device which failed to meet its co-primary endpoints in a pivotal study. It is the first approval to result from CDRH's pilot program to formally incorporate patient preference into risk-benefit determinations for obesity devices, and it is the first new obesity device approved by FDA since 2007.

Pink Sheet 1/20/15

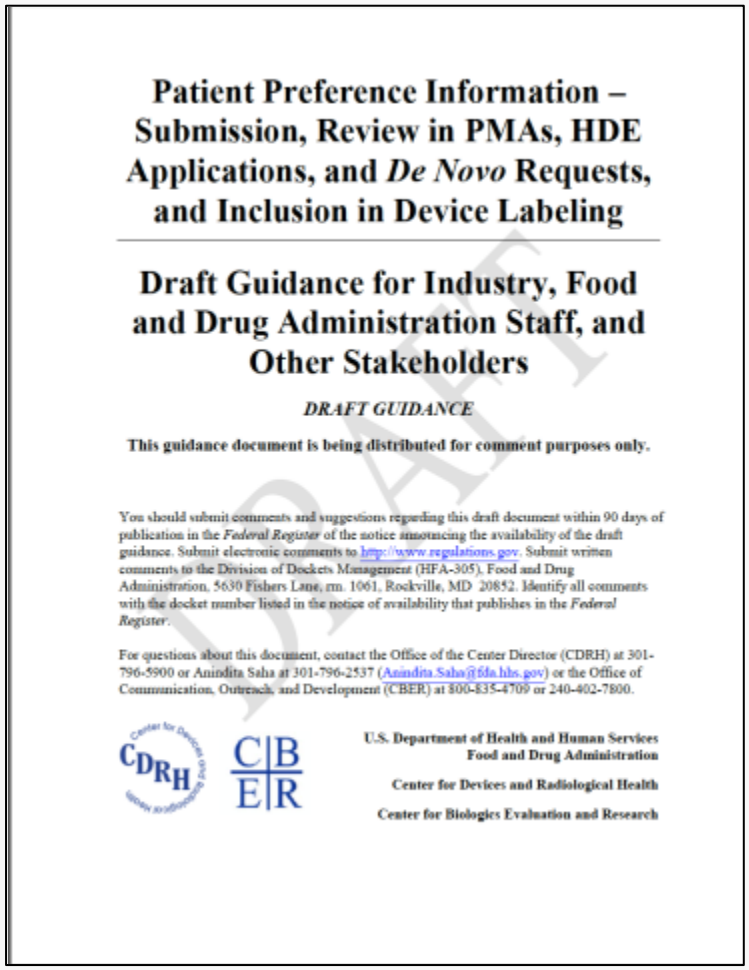
FDA CDRH Patient Preference Initiative: Collaborative Building Blocks



PPI = Patient Preference Information

Objectives of FDA CDRH Draft Guidance on Patient Preference Information

- Encourage voluntary submission of patient preference information
- Outline recommended qualities of patient preference studies for valid scientific evidence
- Provide recommendations for collecting patient preference information to FDA
- Provide recommendations for including patient preference information in labeling for patients and healthcare professionals



Patient Preference Information – Submission, Review in PMAs, HDE Applications, and *De Novo* Requests, and Inclusion in Device Labeling

Draft Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

DRAFT GUIDANCE

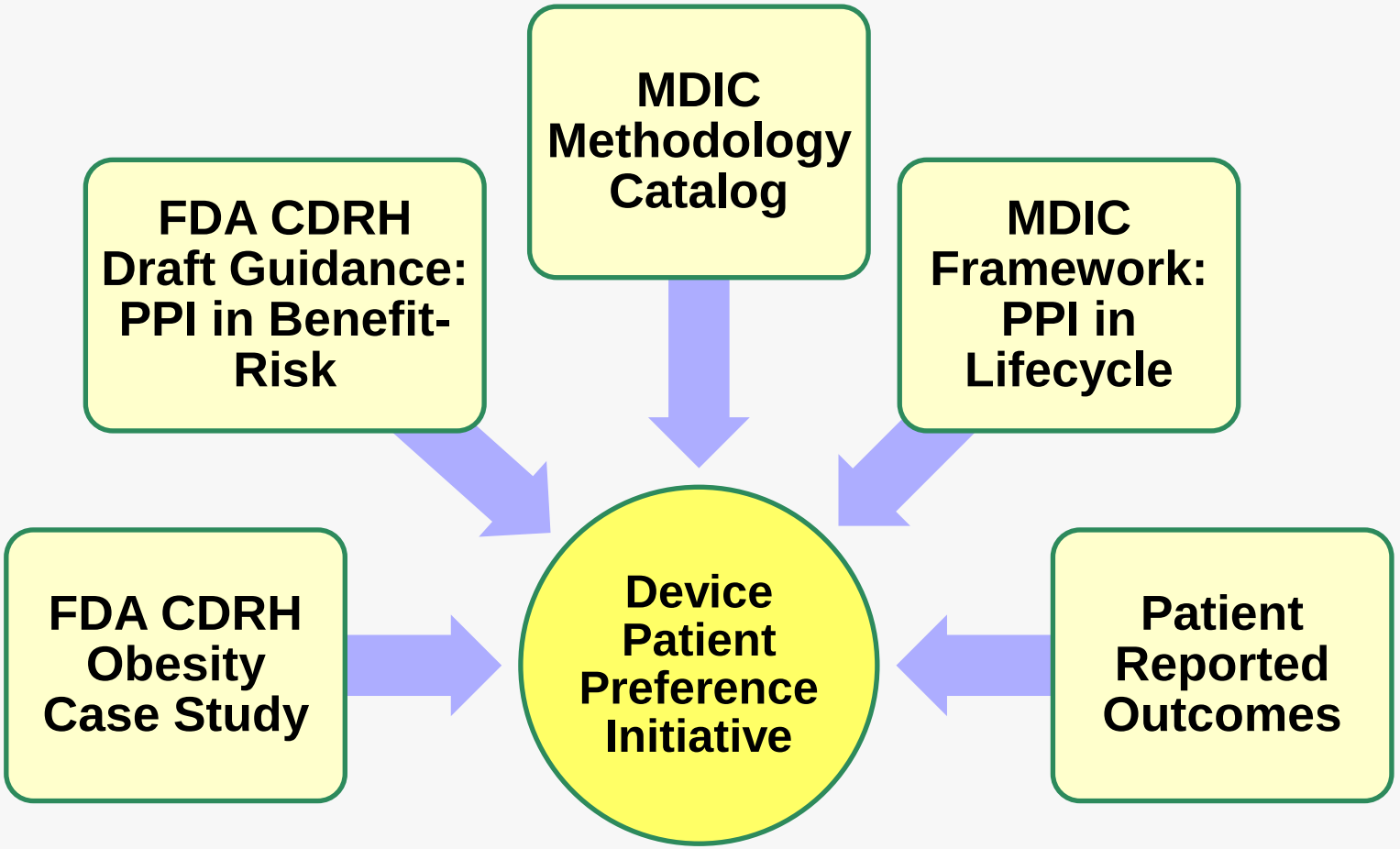
This guidance document is being distributed for comment purposes only.

You should submit comments and suggestions regarding this draft document within 90 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to http://www.regulations.gov. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. Identify all comments with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions about this document, contact the Office of the Center Director (CDRH) at 301-796-5900 or Anindita Saha at 301-796-2537 (Anindita.Saha@fda.hhs.gov) or the Office of Communication, Outreach, and Development (CBER) at 800-835-4709 or 240-402-7800.

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

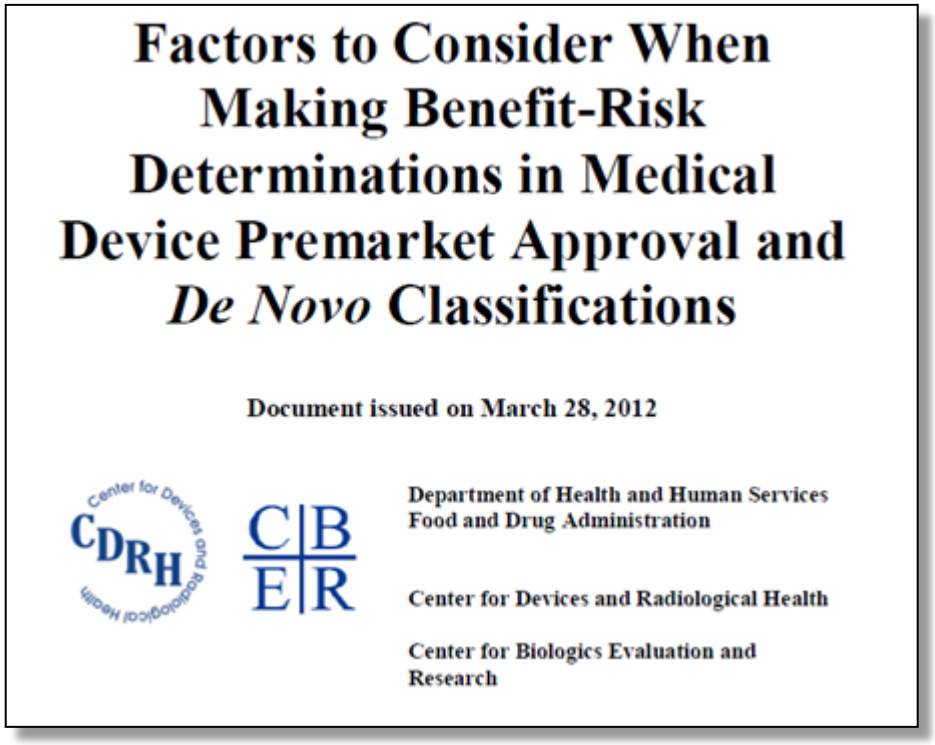
FDA CDRH Patient Preference Initiative: Collaborative Building Blocks



PPI = Patient Preference Information

Regulatory Guidance in Benefit-Risk Assessment for Medical Devices

- FDA CDRH 2012 guidance on factors to consider for B-R assessment in devices
- Landmark regulatory policy statement on benefit-risk
- Impetus for MDIC patient-centered B-R project



Factors to Consider When Making Benefit-Risk Determinations in Medical Device Premarket Approval and *De Novo* Classifications

Document issued on March 28, 2012

Department of Health and Human Services
Food and Drug Administration

Center for Devices and Radiological Health

Center for Biologics Evaluation and Research

Patient-Centered B-R Assessment

- **FDA CDRH guidance recognizes that patients will vary in how they value benefits and tolerate risks**
  - "FDA realizes that some patients are willing to take on a very high risk to achieve a small benefit, whereas others are more risk averse."
  - "FDA would consider evidence relating to patients' perspective of what constitutes a meaningful benefit when determining if the device is effective, as some set of patients may value a benefit more than others."

➔ **Guidance suggests that FDA would consider patient perspective and preferences on benefits and risks**

But it did not say how…

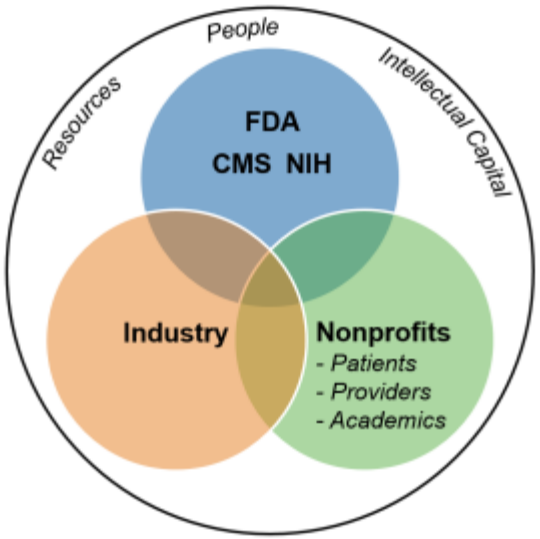
Medical Device Innovation Consortium

- **48 Members**
- **5 Projects**

Case for Quality | Clinical Trial Innovation & Reform | Clinical Diagnostics
Computer Modeling & Simulation | Patient-Centered Benefit-Risk Assessment

A 501(c)3 - Public-Private Partnership collaborating on Regulatory Science to make patient access to new medical device technologies faster, safer, and more cost-efficient

MDIC
MEDICAL DEVICE INNOVATION CONSORTIUM



Align Resources

Accelerate Progress

Achieve Results

WORKING COOPERATIVELY with FDA to re-engineer pre-competitive technology innovation

REDUCING TIME and resources needed for new technology development, assessment, and review

HELPING PATIENTS gain access to new medical technologies sooner

ALIGN | ACHIEVE | ACCELERATE

www.mdic.org

Vision for Patient-Centered Benefit-Risk Project

To establish a credible framework for assessing patient preferences regarding the probable benefits and risks of a proposed medical device and for incorporating this patient preference information into pre-market and post-market regulatory submissions and decisions

MDIC PCBR Steering Committee

- **Robert Becker, MD, PhD, FDA, Center for Devices and Radiological Health, Office of In Vitro Diagnostics and Radiological Health**
- **Randall Brockman, MD, FDA, Center for Devices and Radiological Health, Office of Device Evaluation**
- Stephanie Christopher, Medical Device Innovation Consortium; MDIC PCBR Program Manager
- Jessica Foley, PhD, Focused Ultrasound Foundation
- Jim Gardner, MD, MBA, Cook Group, Inc.
- Andrew J. Greenfield, MBA, AbioMed
- Arieh Halpern, Simulia
- **Martin Ho, MSc, FDA, Center for Devices and Radiological Health, Office of Surveillance and Biometrics**
- **Telba Irony, PhD, FDA, Center for Devices and Radiological Health, Biostatistics and Office of Device Evaluation**
- Ross Jaffe, MD, Versant Ventures and National Venture Capital Association (NVCA); MDIC Board Champion, PCBR Project
- Alethia Karkanis, WL Gore
- Richard Kuntz, MD, MSc, Medtronic
- Jack Lasersohn, JD, The Vertical Group and National Venture Capital Association (NVCA)
- Bennett Levitan, MD, PhD, Janssen R&D LLC, Johnson & Johnson
- Barry Liden, JD, Edwards Lifesciences
- Bryan Luce, PhD, MBA, Patient-Centered Outcomes Research Institute (PCORI)
- Kim McCleary, FasterCures
- **Mimi Nguyen, FDA, Center for Devices and Radiological Health, Office of the Center Director**
- **Kathryn O'Callaghan, FDA, Center for Devices and Radiological Health, Office of the Center Director**
- Bryan Olin, PhD, Cyberonics
- Anindita Saha, FDA, Center for Devices and Radiological Health, Office of the Center Director
- Diana Salditt, Medtronic
- Peter Saltonstall, National Organization for Rare Disorders (NORD)
- Heather Watson, Exponent

Committee Advisors

- Marc Boutin, JD, National Health Council
- Scott Braithwaite, MD, MS, FACP, Department of Population Health, NYU School of Medicine
- Brett Hauber, PhD, RTI Health Solutions
- Bray Patrick-Lake, MFS, Clinical Trials Transformation Initiative (CTTI)
- Sean Tunis, MD, MSc, Center for Medical Technology Policy

MDIC Patient-Centered Benefit-Risk Project

Framework

- Framework for Patient-Centered Benefit-Risk Assessment

Catalog

- Catalog of Patient Preference Assessment Methods

Future Work

- Agenda for Future Research in Patient Preferences

MDIC Patient-Centered Benefit-Risk Project

Framework

- Framework for Patient-Centered Benefit-Risk Assessment

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Future Work

- Agenda for Future Research in Patient Preferences

Key Components of Framework

- Definitions and core concepts
- When is collecting patient preference information potentially valuable for B-R assessment?
- Use and value of patient preference information throughout the lifecycle
- How patient preference information may be useful in the regulatory process
- Potential value of patient preference information beyond the regulatory process
- Methods for preference assessment and factors to consider in their use

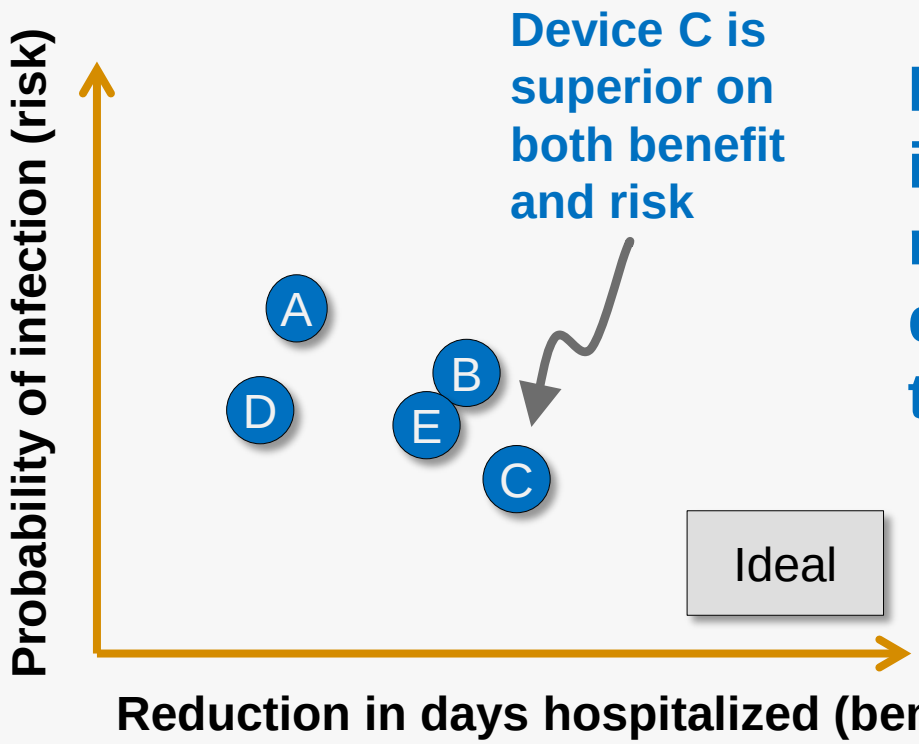
What are “Preferences”?

Qualitative or quantitative statements of the relative desirability or acceptability of attributes that differ among alternative health interventions

Definition applies equally well to preferences of caregivers, physicians, payers, and regulators.

* Medical Device Innovation Consortium Patient-Centered Benefit-Risk Framework, http://mdic.org/wp-content/uploads/2015/05/MDIC_PCBR_Framework_Web.pdf

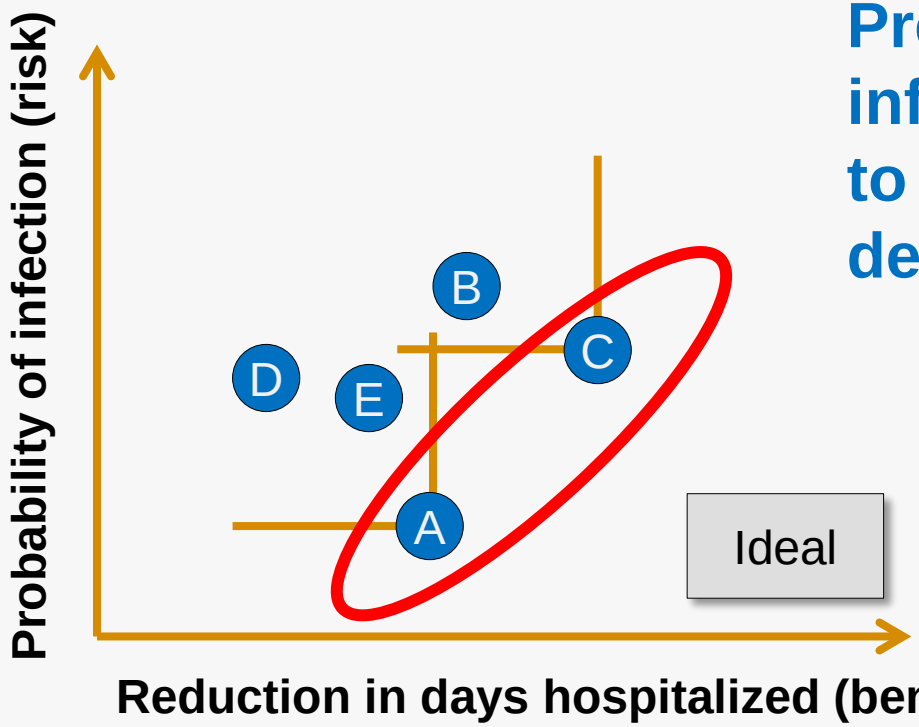
Which Treatment Is Best?



Preference information is not needed to determine the best treatment

Medical Device Innovation Consortium Patient-Centered Benefit-Risk Framework, http://mdic.org/wp-content/uploads/2015/05/MDIC_PCBR_Framework_Web.pdf

Now Which Treatment Is Best?

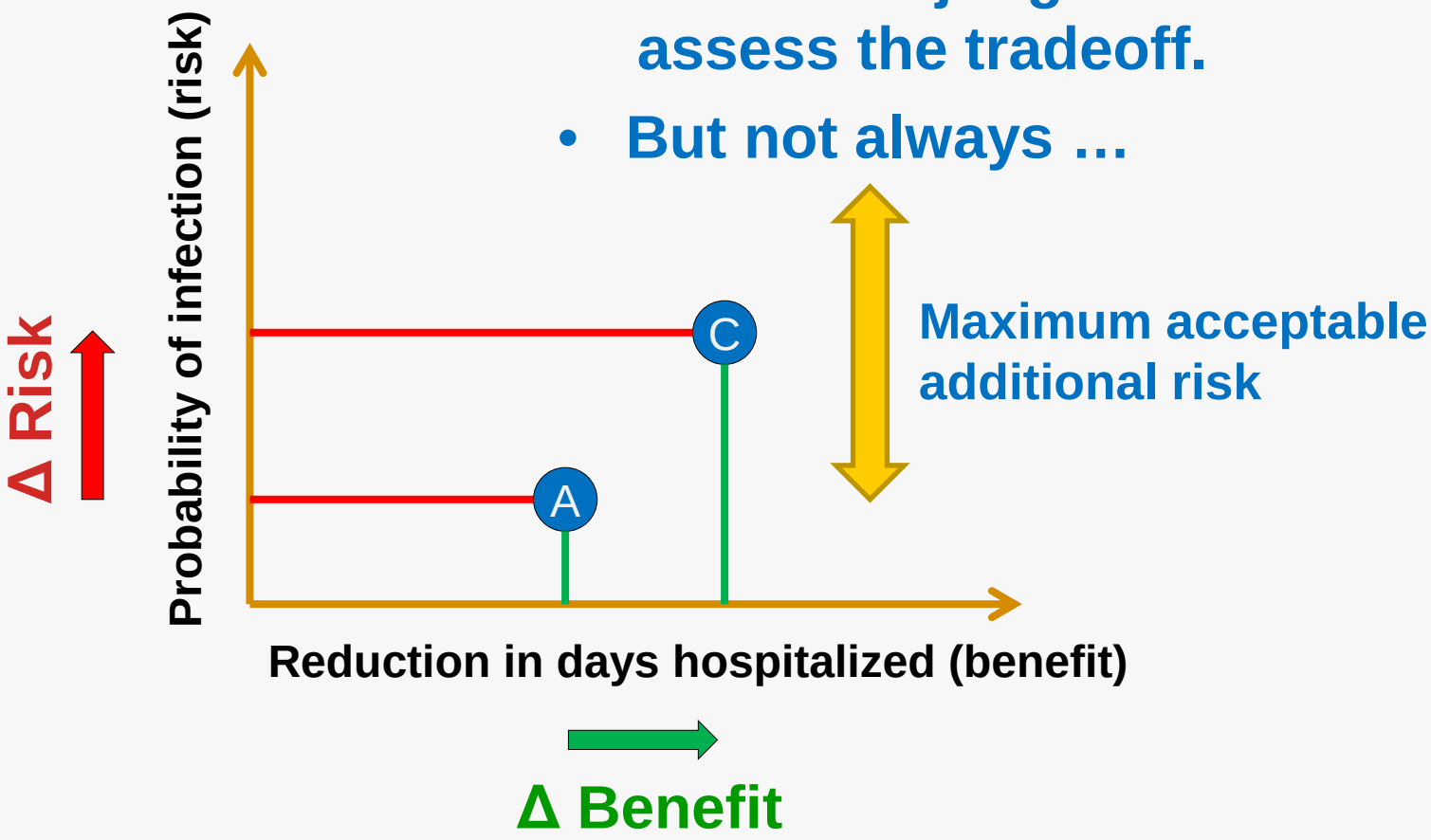


Preference information is needed to choose between device A and C

This is a “Preference Sensitive Decision”

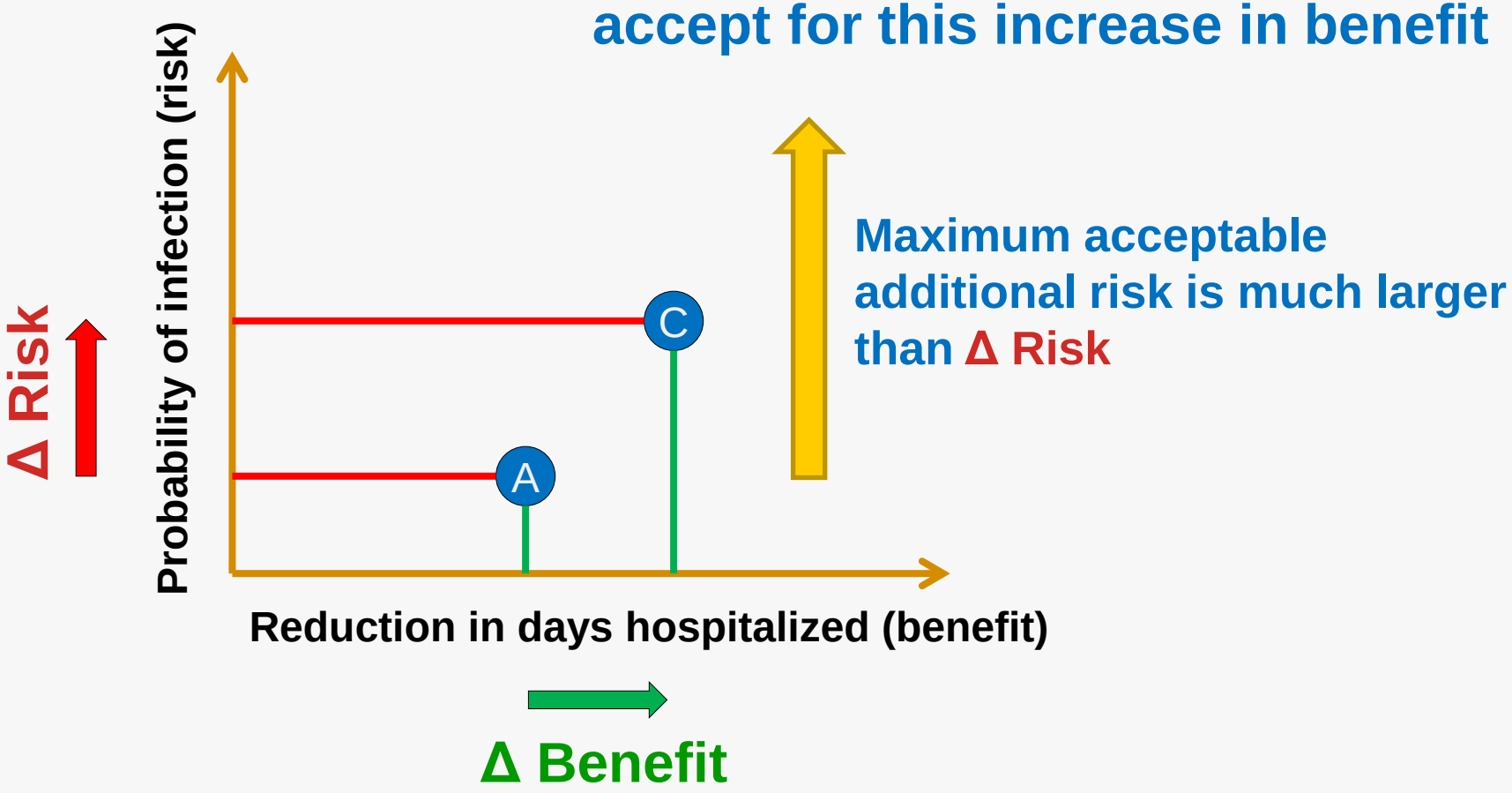
How Do Preferences Help Us Choose?

- In many cases, the decision is clear over a plausible range of preferences → clinical judgment is sufficient to assess the tradeoff.
- But not always …



How Do Preferences Help Us Choose?

- **Preference studies give the maximum additional risk that <u>patients</u> would accept for this increase in benefit**



When Is Patient Preference Information Potentially Valuable?

- **Factors related to the patient's perspective**
  - Patients willing to accept a different degree of risk than regulators
  - Important differences in the preferences of subgroups of patients
  - Understanding the clinical experience requires considerable familiarity with the disease (e.g., highly subjective endpoints, lifestyle indication, rare diseases)
- **Factors related to benefit-risk tradeoffs (preference sensitive)**
  - Clear benefit with rare serious risks compared to alternatives
  - Modest benefit but considerably less risk than alternatives
  - Harms occur early/benefits occur later (e.g., Tx to delay onset of a disease)
  - Considerable uncertainty on whether a patient will realize the benefit or risks
- **Factors related to novelty**
  - New mechanism of action or technology with which patients are unfamiliar
  - Lack of precedent in indication or technology

Medical Device Innovation Consortium Patient-Centered Benefit-Risk Framework, http://mdic.org/wp-content/uploads/2015/05/MDIC_PCBR_Framework_Web.pdf

MDIC Patient-Centered Benefit-Risk Project

Framework	Catalog	Future Work
• Framework for Patient-Centered Benefit-Risk Assessment	• Catalog of Patient Preference Assessment Methods	• Agenda for Future Research in Patient Preferences

MDIC Patient-Centered Benefit-Risk Project

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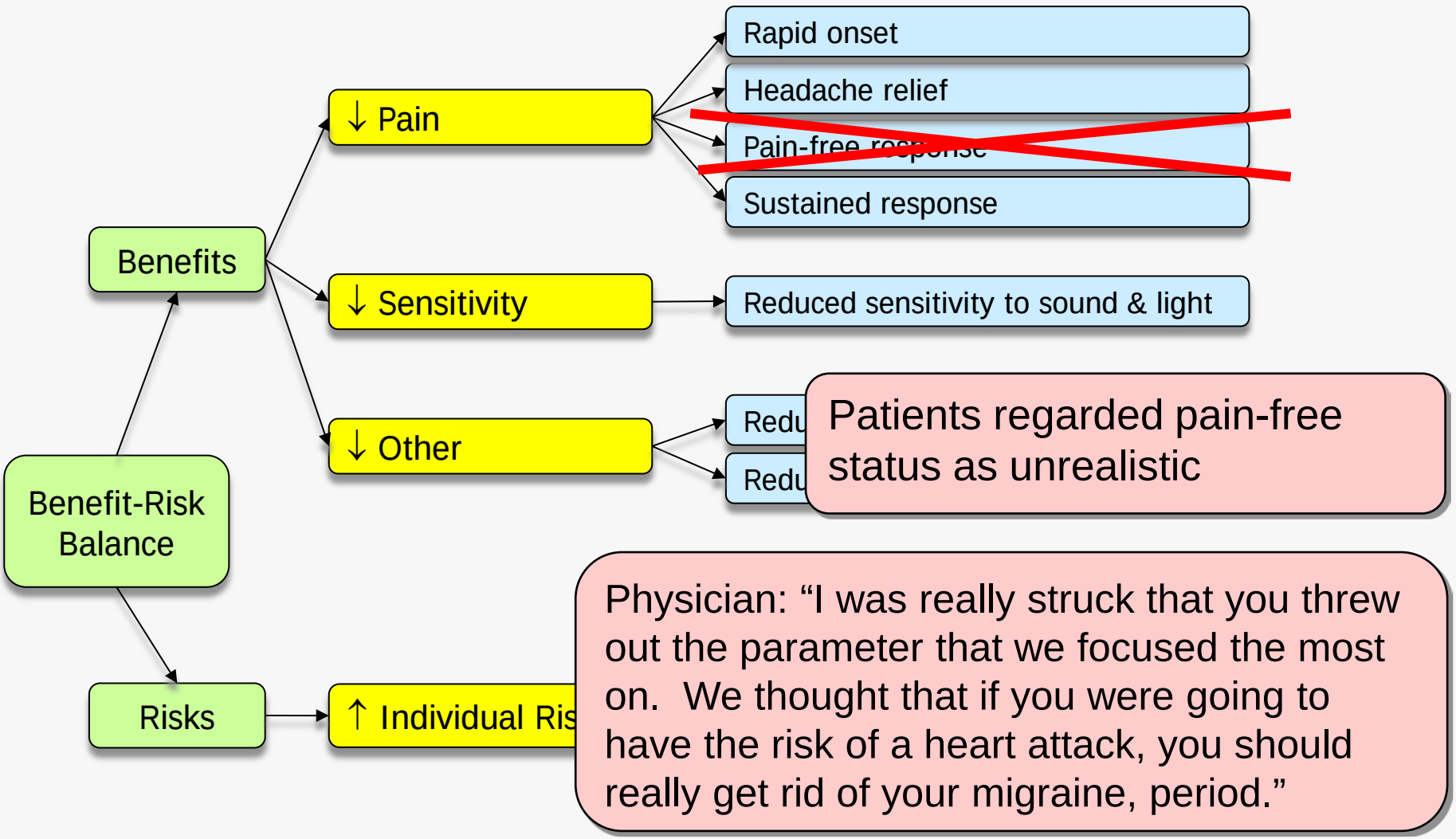
Roles for Patient Preferences in Regulatory Review and Post-Approval

Patient Perspective: Determining Which Endpoints Are Most Critical

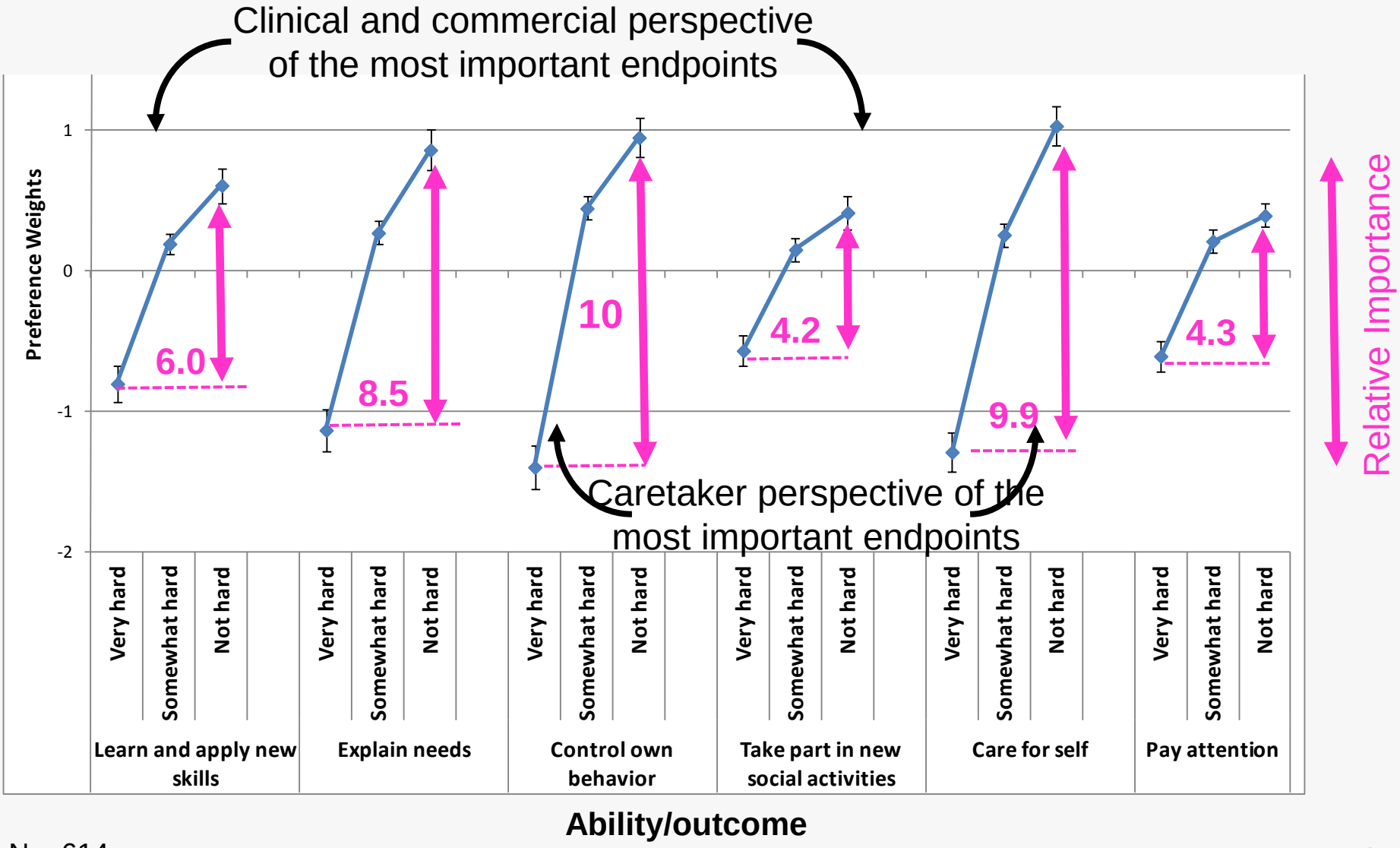


Levitan, Phillips, Walker, TIRS, **34** 564 – 573, (2014)

Fragile-X Syndrome

- **Rare genetic condition impacting development**
  - Learning and intellectual disabilities, cognitive impairment, behavioral challenges (ADHD, autism, social anxiety), physician features
  - No cure – educational, therapeutic support
- **Preference study conducted to prepare for Phase 3 study**
  - Intent was to identify which endpoints or components of existing instruments were most important to patients
  - Survey administered to family members, given patient cognitive limitations

Preference Survey Identified Large Gap Between Clinician and Patient Caretaker Beliefs on Endpoint Importance

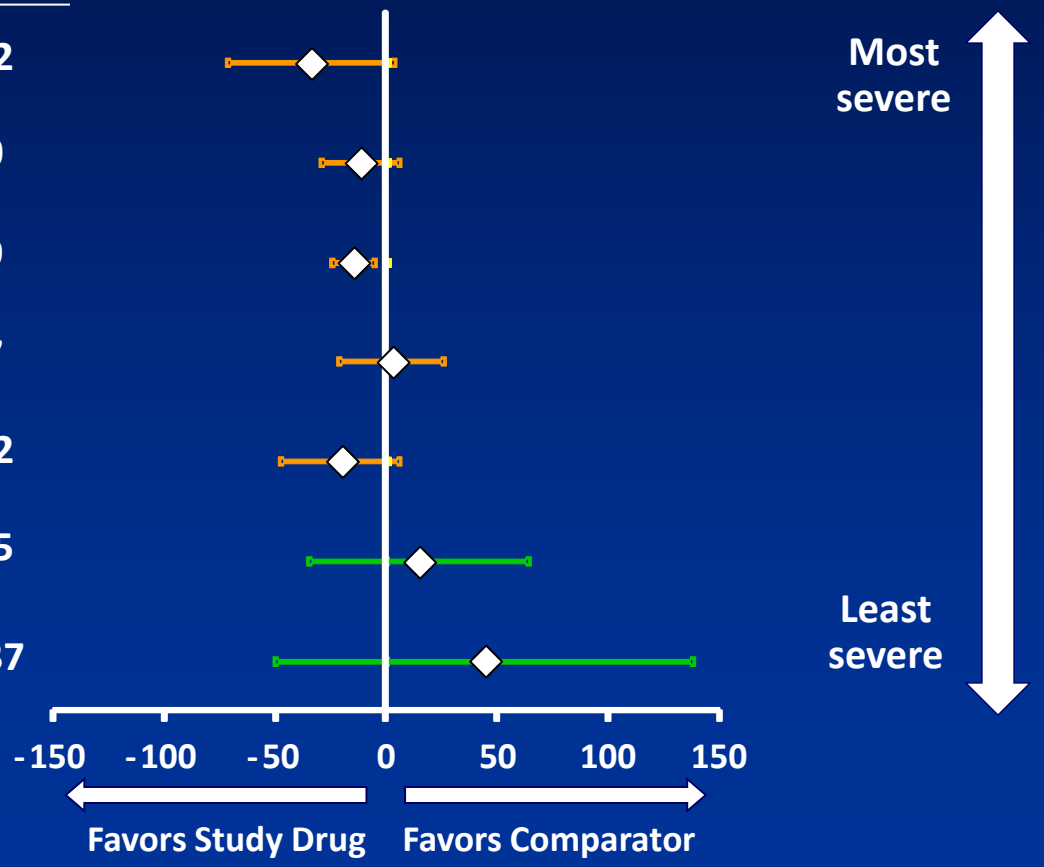


N = 614

J. Cross, CNS Summit, Nov, 2012

Risk Differences by Clinical Severity/Impact†
Atrial Fibrillation Example

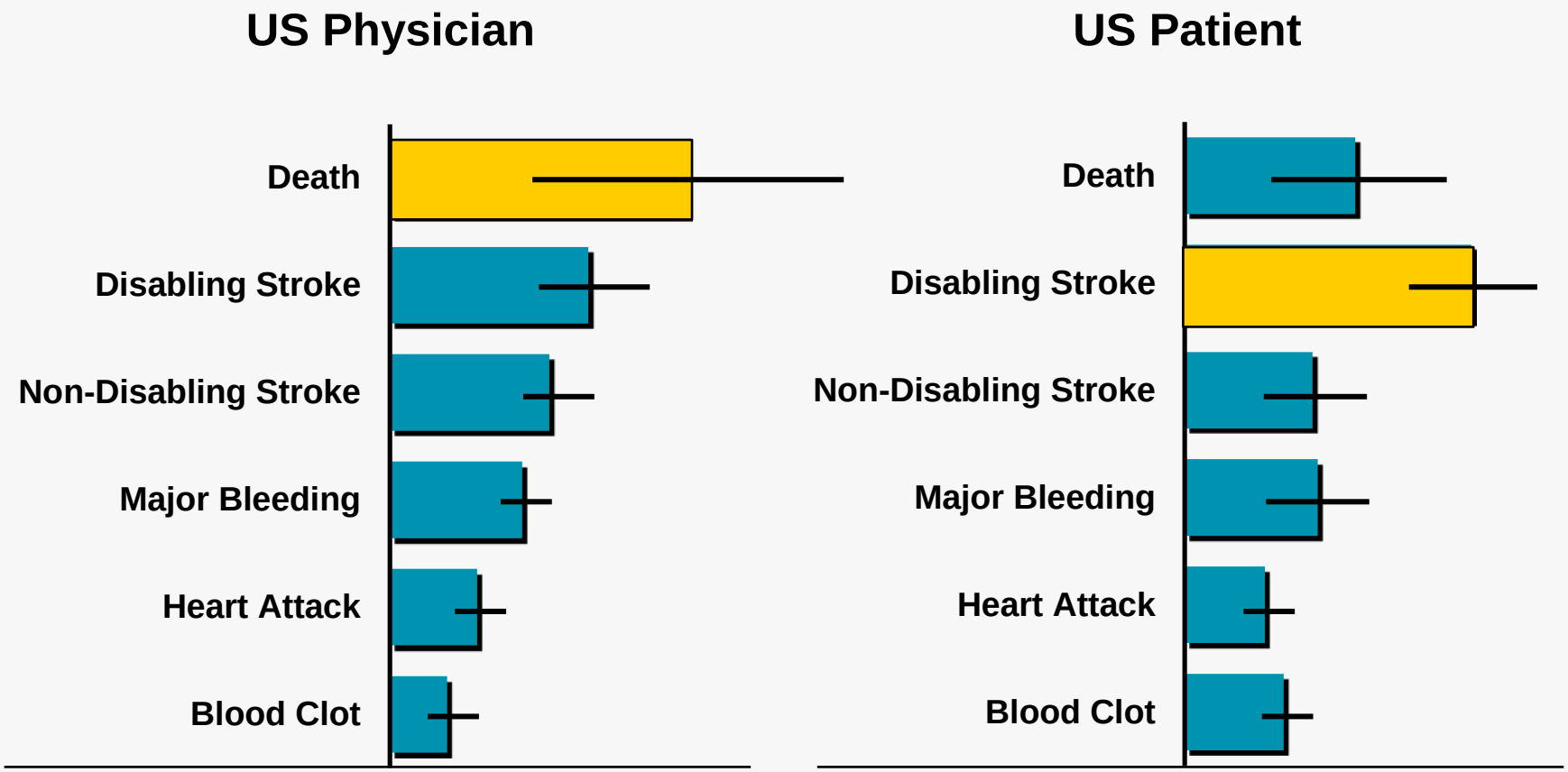
Endpoint	# of Events (/10,000 patient-years) Study Drug	Comparator
All-cause mortality	188	222
Disabling stroke	38	50
Non-CNS systemic embolism	4	19
Non-disabling stroke	79	77
Myocardial infarction	91	112
Major bleeding	360	345
Non-major clinically relevant bleeding	1180	1137



† Endpoints in order of health state utility, a value that reflects preference for health states relative to perfect health and death.

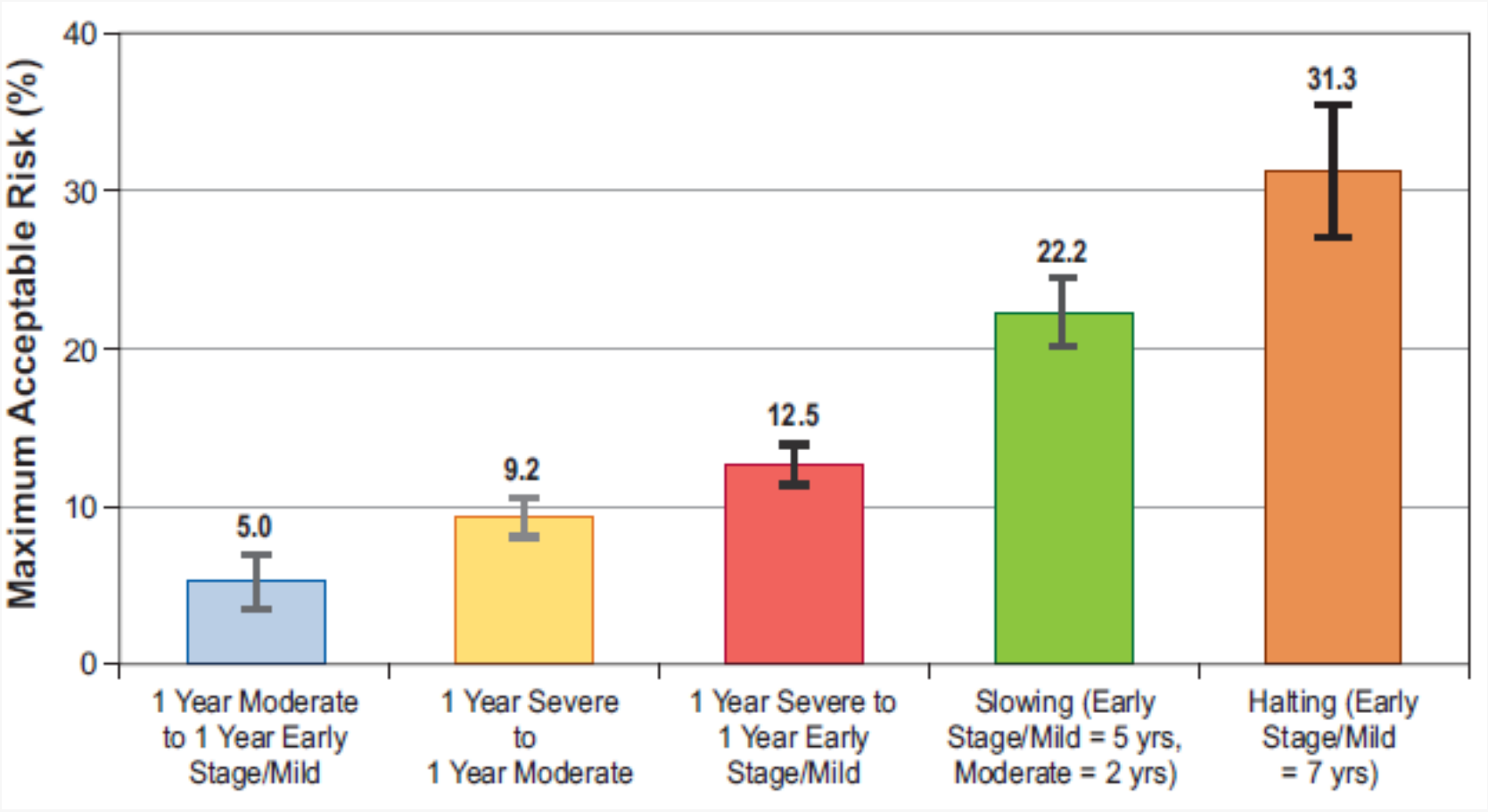
Identifying Differences Between Key Stakeholders

Preferences for Anticoagulants in Atrial Fibrillation



Levitan, Yuan, González, et al., ISPOR 18th Ann Int Mtg, 2013

Maximum Acceptable Risk of Treatment-Related Death or Permanent Severe Disability Due to Stroke



Hauber AB, Johnson FR, Fillit H, et al. Older Americans' risk-benefit preferences for modifying the course of Alzheimer disease. *Alzheimer Dis Assoc Disord.* Jan-Mar 2009;23(1):23-32

Key Idea

To use patient preference information in a regulatory context requires more than expressions of feelings or opinions – it needs defensible **data**

Preference studies have the potential to obtain these data reliably

A Goal

Endpoint	Study Drug/ 10,000 pt-yrs	Comparator Rate/ 10,000 pt-yrs	HR (95% CI)	Risk Difference / 10,000 pt-yrs
Primary efficacy (Stroke + embolism)	177	218	0.78 (0.65, 0.94)	-41 (-78, -5)
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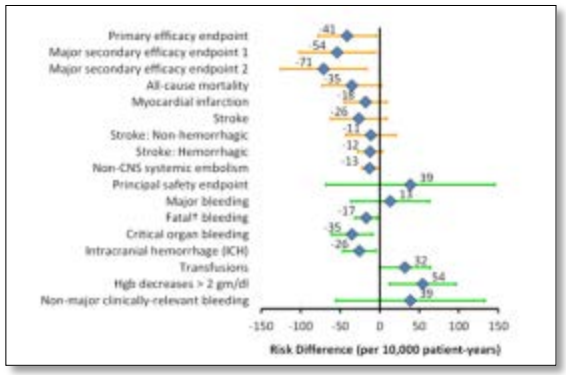
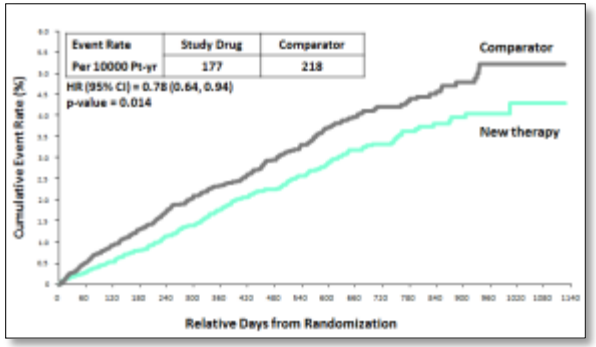
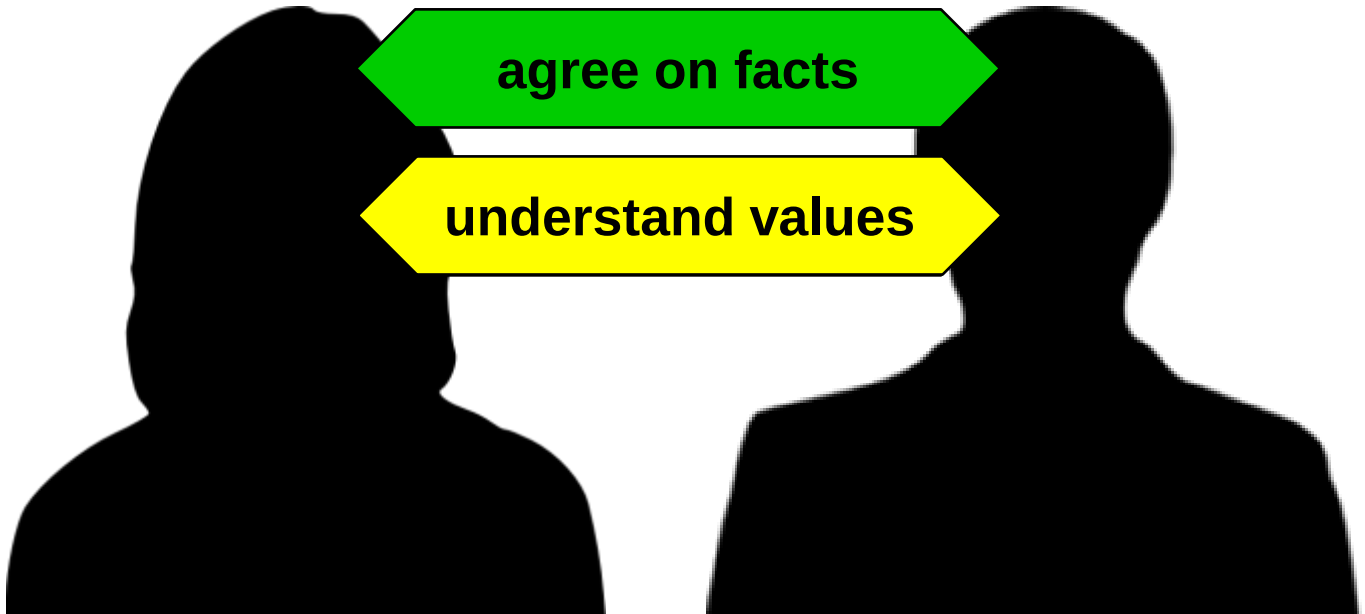
Secondary efficacy 2 (stroke, embolism + vascular death + MI)	396	467	0.84 (0.73, 0.95)	-71 (-125, -17)







Backups

Potential PCORI Applications

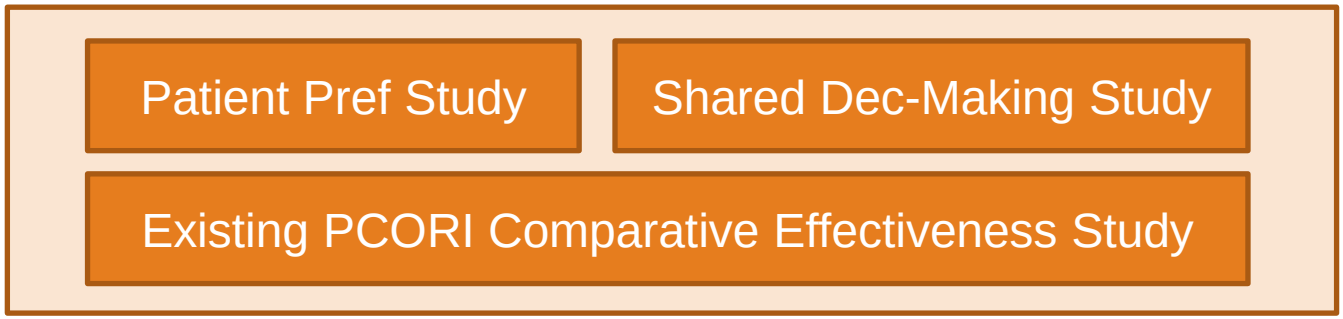
First 10 PCORI Pragmatic Clinical Studies

1. **Breast cancer screening tailored to individual risk and preferences vs. annual mammography** for detecting breast cancer and minimizing screening-related harms in women 40-80
2. **Annual vs. biennial surveillance CT scanning** in patients found to have small, r
3. **Stan facto** medi colore
4. **Com home** preventing hospital readmissions and mortality in stroke survivors?
5. **Primary care plus prompt referral to physical therapy and cognitive behavioral therapy vs. usual primary care** to prevent acute back pain from becoming chronic

- Natural benefit-risk problem:
  - Detect occult cancer with benefits dependent on stage
  - Consequences of false positives
- Apples to oranges comparison – preference studies appropriate
- Will be large heterogeneity in risk tolerance – preference studies assess
- Clear connection to shared decision making

One Potential Role for Patient Preference Studies in PCORI

"Meta project" on a key topic with a natural benefit-risk tradeoff and a clear need for the patient perspective to render a benefit-risk decision



- Component studies form a package that encompasses the entire domain from research to patient treatment

Developing PRO Scales with Patient Preference Studies

Common Concerns / Open Issues / Areas for Additional Future Research

- Can patients do these surveys reliably?
- Stated choice is not actual choice
- Choosing the right method
- Industry can bias these surveys
- Selecting the attributes
- Sample selection – whose preferences and when?
- Sample size
- Formal assessments of validity
- Regulatory requirements

Some MDIC Suggestions for Future Research

- Use multiple patient-preference methods to address the same research question
- Conduct the same study with different samples with different characteristics
- Conduct the same study with patients with and without prior experience
- Conduct a study before and after a medical technology is available
- Conduct a study in which patients are randomized to two different (but overlapping) sets of attributes
- Review validity standards in other types of studies (e.g., clinical or PRO)

Questions?

Training Follow-Up: FY 2016 Initiative in Team Science

Erica Sarnes, MA, Training Manager

Presentation to the Advisory Panel on Patient Engagement

October 27, 2015

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Feedback from Diverse Research Teams

Research team members (patients, stakeholders, researchers) should be trained together

Team Science Training Initiative

Traditional Definition of Team Science – a collaborative effort to address a scientific challenge that leverages the strengths and expertise of professionals trained in different fields

PCORI's Opportunity

- Traditional focus of team science – research teams comprised of interdisciplinary scientists and multi-disciplinary professionals
- Innovative application of team science – inclusion of patients and a variety of other stakeholders in the composition of research teams

PCORI's Team Science Training Goals

- To enable teams to learn about their prospective roles in performing team science
- To enable diverse team members (stakeholders, patients, researchers) to obtain the necessary skills to work interdependently in a team environment

Team Science Training Initiative (cont.)

Engagement/Science Work Group

- Purpose: To provide insights to inform the development of a Request for Proposal (RFP) that will meet the goals of the team science training initiative
  - Items that are in and out of scope for the RFP
  - PCORI staff responsibilities vs. vendor responsibilities
  - Evaluation criteria for vendor proposals

Questions?

Thank You

15-Minute Break

Tools for Partnership

Lisa Stewart, MA
Engagement Officer

Suzanne Schrandt, JD
Deputy Director of Patient Engagement

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Setting the Stage

- Transition from training to tools
- Evidence from the Evaluation and Analysis Team
- Review of current and proposed tools
- Discuss toolkit subcommittee

Current Tools

Tool/Topic	Status
Engagement Rubric	(On our website)
Compensation Framework	(On our website)
Sample Engagement Plans	(On our website)

Proposed Tools

Tool/Topic	Purpose	Comments/Discussion

Toolkit Subcommittee
Proposed Draft Charter/Tasks

- The toolkit subcommittee would serve in an advisory capacity to the Patient Engagement Advisory Panel and in concert with PCORI staff, would bring forward ideas and work product to the full panel for review and discussion.

Toolkit Subcommittee Proposed Draft Charter/Tasks

- The toolkit subcommittee would assist PCORI with:
  - Identifying resources not yet identified by PCORI as important components of the engagement toolkit
  - Collecting and collating existing resources for use in the engagement toolkit
  - Providing input on the design and modality of resources in the engagement toolkit
  - Assisting with prioritization of resources to create/refine/add
  - Helping PCORI to create resources not yet available

Pipeline to Proposals Update

Courtney Clyatt, MA

Senior Program Associate

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Pipeline to Proposal Awards (P2P)

- Mission: P2P aims to build a national community of patient, stakeholder, and researcher partnerships that have the expertise and passion to participate in patient-centered outcomes research within their community that leads to high-quality research. Additionally, the P2P is a tool to develop and strengthen the engagement in proposals submitted for funding

- Purpose:
  - Build community
  - Form or strengthen reciprocal relationships between researchers and non-research communities
  - Support capacity building, co-learning, and the development of a sustainable infrastructure to facilitate “research done differently”
  - Accelerate proposal submission (or resubmission)
  - Speed Dissemination and Implementation
  - Develop and strengthen engagement in funding proposals

The Pipeline to Proposal Initiative Is a Three-Tiered Award System

Researchers who unsuccessfully submitted a PFA and need to improve proposal

Or submissions to other PCOR/CER funders

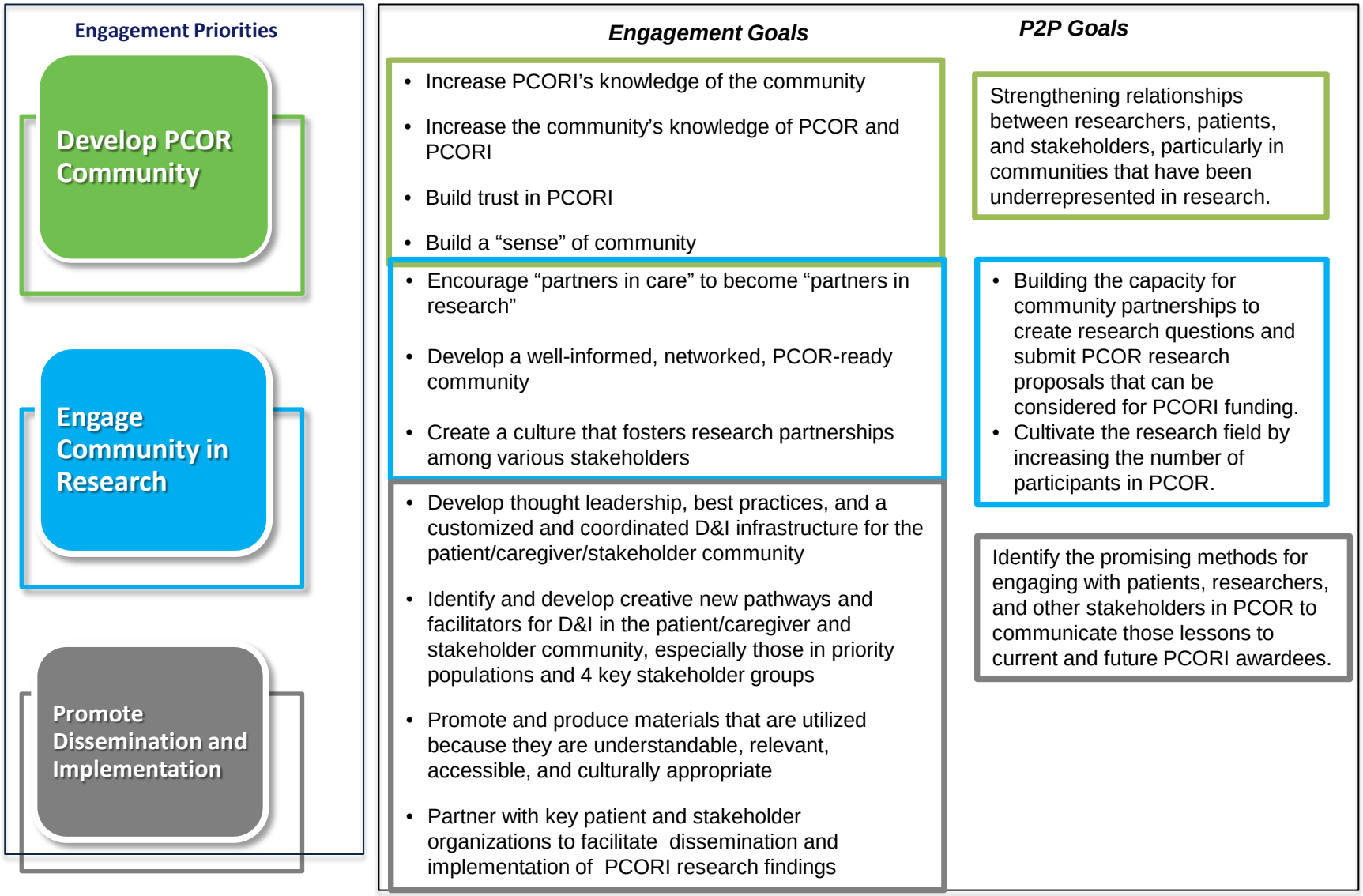
Opening a Pipeline to Patient-Centered Research Proposals

P2P Awards Strengthen the PCORI Research Enterprise

1) P2P helps foster capacity building for PCOR in the community before a study plan is even developed. This enables underserved/minority and otherwise "missing" communities to actively engage in the research process

2) It has been shown that when patient partners are engaged early on and throughout the research process, they are more likely to help in the implementation and dissemination of study results in their communities

Engagement and P2P Goals

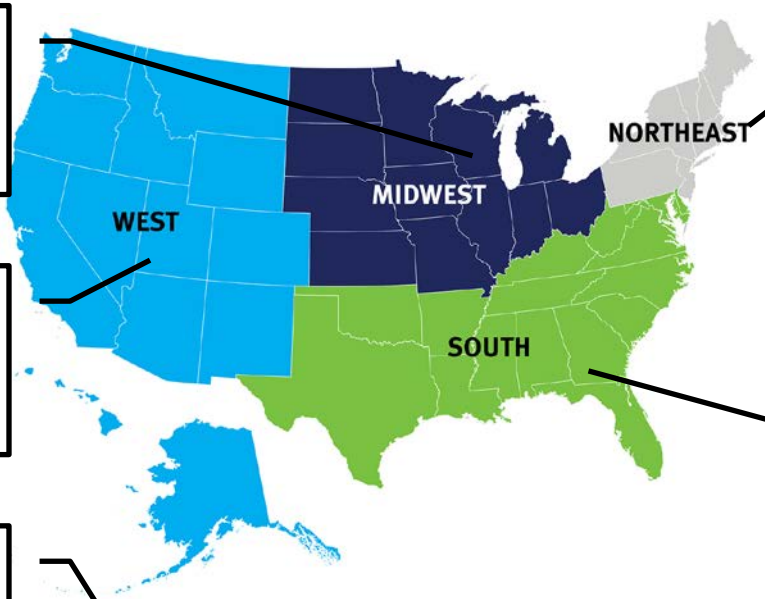


Regional Program Offices for Pipeline Awards

Michigan Public Health Institute

Colorado Foundation for Public Health and the Environment

National Network of Public Health Institutes



Health Resources in Action

Georgia Health Policy Center

Projects that cover more than one region are housed under the National Program Office

Strengthening PCOR Nationwide

Our *Pipeline to Proposal Awards* encourage PCOR in comparative clinical effectiveness research.

Number of projects:

Tier I – 77

Tier II – 27

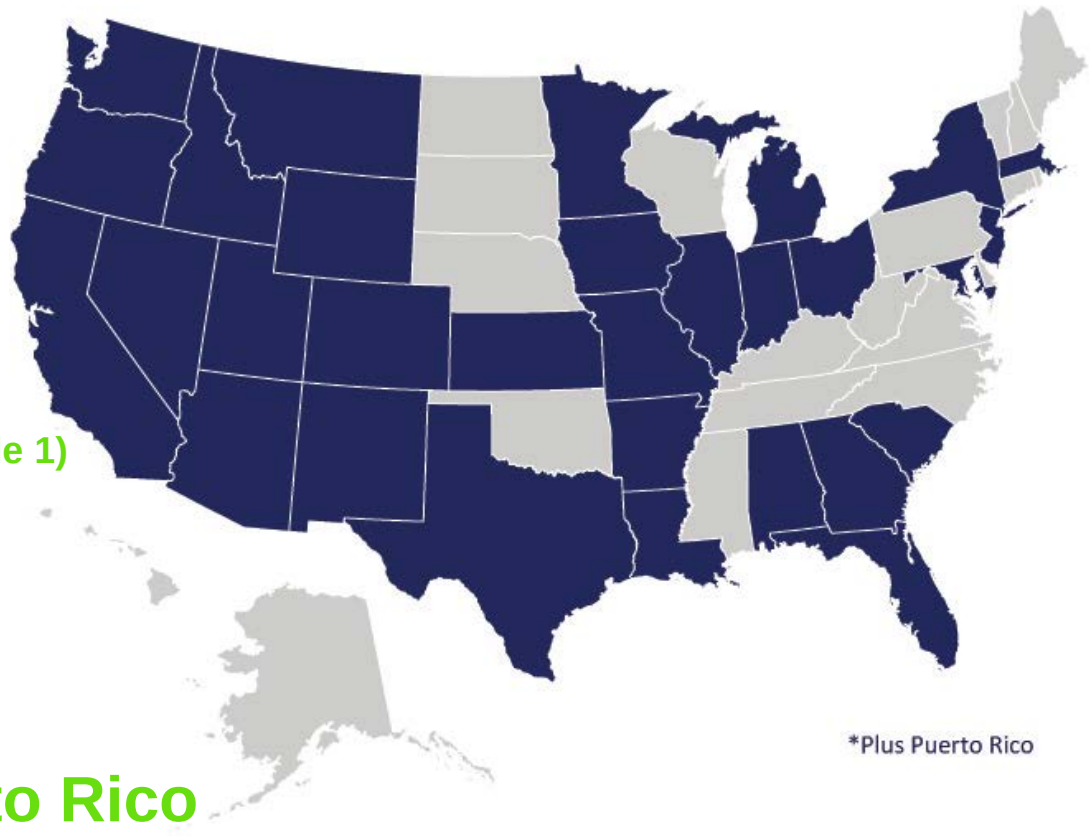
Amount awarded:

$1,813,999

(Tier I Cycles 1 & 2 and Tier II Cycle 1)

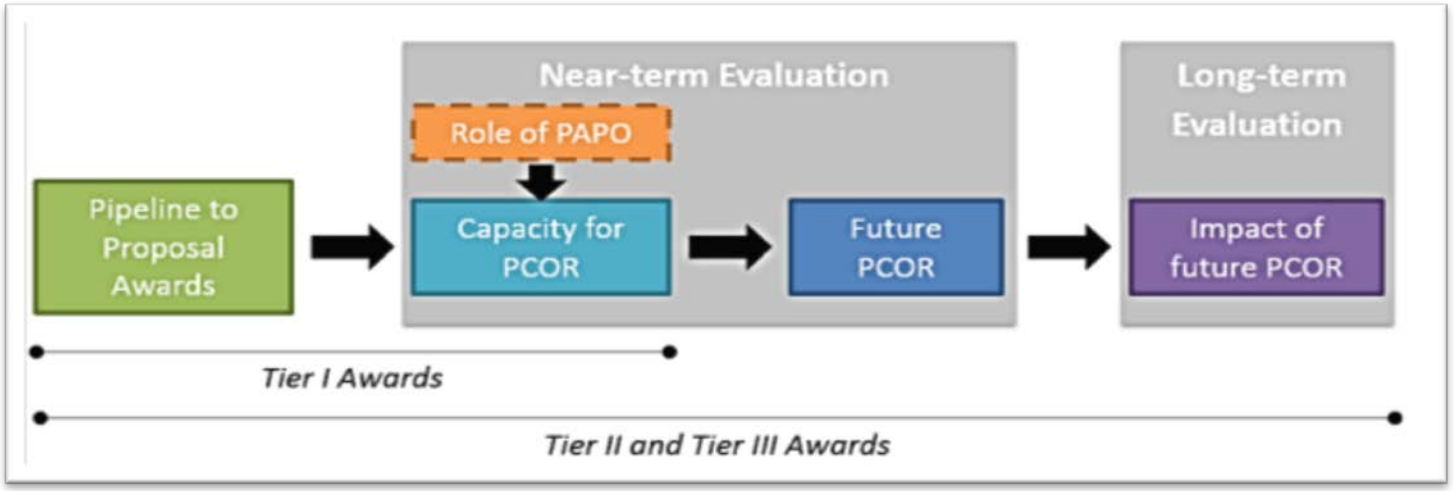
Number of states where we are funding projects:

30 states, District of Columbia and Puerto Rico



As of May 1, 2015

Evaluating the Pipeline to Proposal Awards



*We hypothesize that Pipeline to Proposal Awards will **promote capacity for PCOR** (e.g., organizational structures, resources, collaborative relationships, policies, procedural protocols, and commitment to patient-centeredness needed to conduct PCOR). Moreover, we expect that this capacity will **lead to future PCOR** that will ultimately have a scientific and clinical impact.*

Monitoring & Evaluation Tools

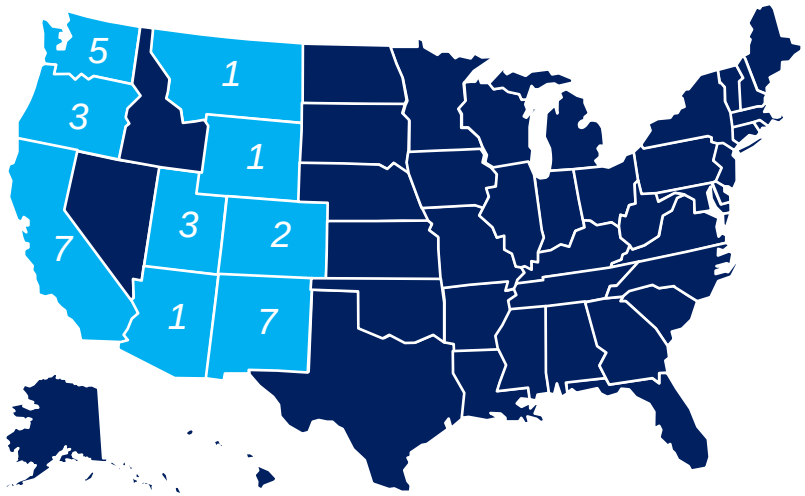
Data Collection Tool	Method	Completed by	Reported to
Award tracking	Internal Tracking	PCORI	PCORI
Monthly/Quarterly monitoring reports	Online Report	Awardee	PAPO
Midpoint reports	Online Report	Awardee	PAPO
End of Project report	Online Report	Awardee	PAPO
Learning About Partnerships (LEAP) Survey of awardees and partners	Online Survey	Awardee/ Partners	PCORI
Feedback from the Pipeline Awards Program Office (PAPO) (monthly reports from PAPO to PCORI)	Online Report	PAPO	PCORI
12-month, 24-month, and 48-month follow-up with Pipeline to Proposal awardees (including the awardee and patient/stakeholder partners when applicable) *TENTATIVE*	*Survey/ Phone Interview*	*Awardee/ Partners*	*PCORI (tentatively)*

Whenever possible and appropriate, collected information is reported to respondents either in aggregate or by project.

Tier I Cycle 1 Awardees

Tier I: West Awardees by Stakeholder Community (N=30)

Tier I: West Awardees by State (N=30)



What We Hope to Learn From the Pipeline to Proposal

Are these investments successful in fostering partnerships?

What are some elements of successful partnership structures?

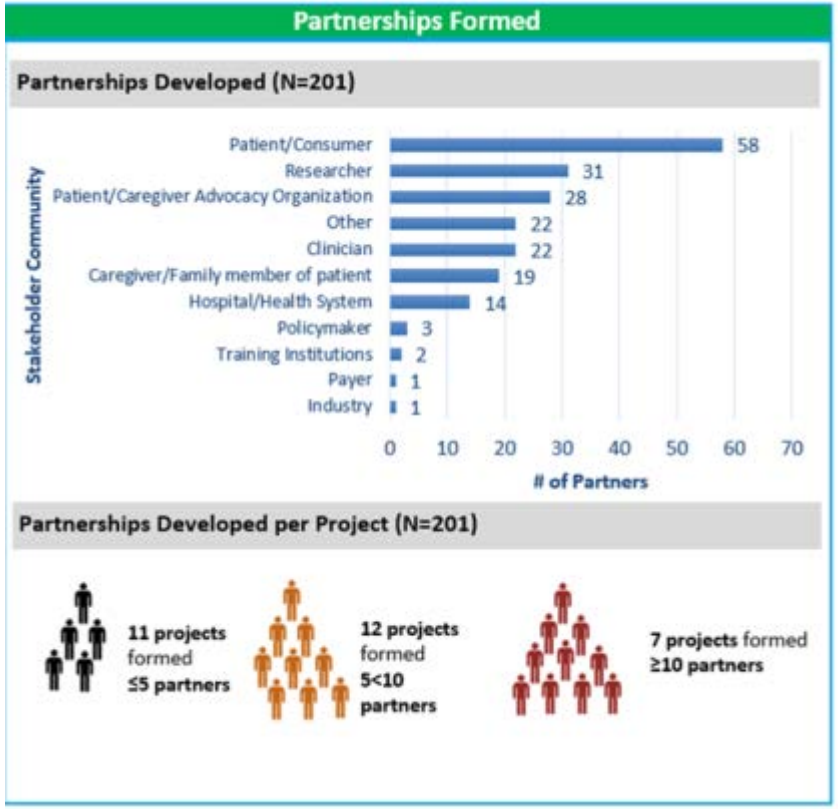
To what extent did this project prepare awardees to pursue research funding from PCORI or another funder?

Did these partnerships embody the PCORI Engagement Principles?

Initial Findings from Tier I Cycle 1

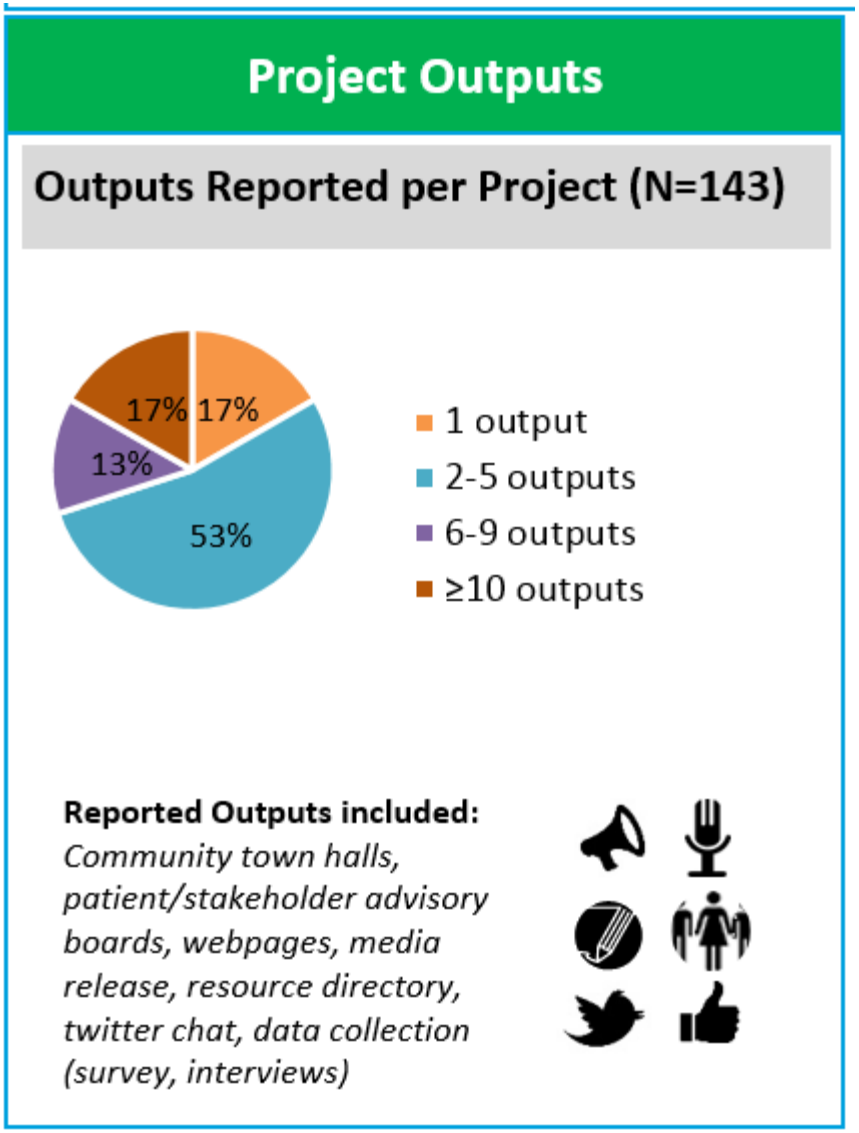
Are these investments successful in fostering partnerships?

100% of Tier I: West awardees developed partnerships with ≥1 partners



Initial Findings from Tier I Cycle 1

- Project Outputs



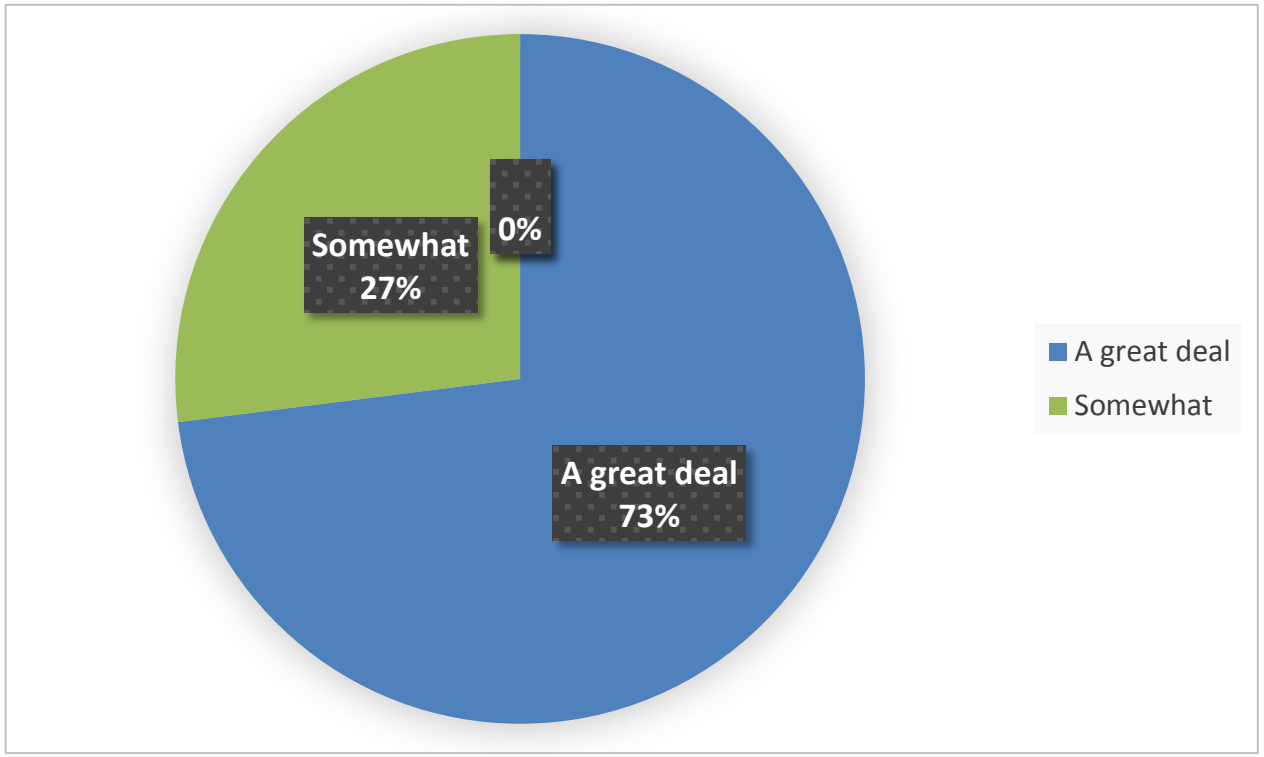
Initial Findings from Tier I Cycle 1

What are some elements of successful partnership structures?

Community stakeholders partnering with technical groups create synergy	•Academic institution partnering with a promotoras (community health worker) work group •Patient leaders partnering with physician groups •Labor union leader partnering with occupational health researchers
Partnership structures function to break down cultural, disciplinary, and/or professional barriers	•These relationships appear to foster further awareness, learning, and the development of innovative research projects led and engaged through underserved communities •Researchers building partnerships enabled them to connect with key community stakeholders, thereby gaining the trust of the communities •These projects serve as catalysts to bring diverse points of view and new perspectives to the problems of interest •Respondents mention that through partnership participation, community members learned about patient engagement principles and felt they had a "voice" in the issues discussed
Willingness to tailor to the needs of partners	•Creating a variety of ways for partners to remain actively engaged was essential and varied from project to project. •Project leaders reported a need to understand the resources for partners to engage effectively. •Adaptability is key - certain strategies, such as in-person meetings, may be more important at the beginning of a project when partnerships are still new and developing.
Establishing expectations and pace of partnership	•It was commonly framed as an investment in the long-term partnership that would prove worthwhile over time.

Initial Findings from Tier I Cycle 1

To what extent did this project develop your partnership to prepare you to pursue research funding for PCORI or another funder?



Next Steps

Fall 2015	• Release a funding call for up to 50 new Tier I Awards
Winter 2016	• Release a funding call for up to 50 independent Tier III Awards – this call would be for the purpose of boosting engagement for research proposals that are almost there
Winter 2016	• Transition up to 47 Tier I Awards to Tier II
Spring 2016	• Release funding call for up to 30 successive Tier III Awards

Ambassadors Program Update

Suzanne Schrandt, JD

Deputy Director of Patient Engagement

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Program Highlights: Training and Resources

- Training
  - Introduction to PCORI (Length: 20 minutes)
  - The Role of the Ambassador (Length: 40 minutes)
  - Basics of PCOR for Ambassadors (Length: 20 minutes)
  - Meaningful Patient and Stakeholder Engagement and the Research Team (Length: 15 minutes)
  - How PCORI-Funded Research Teams Work Together (Length: 35 minutes)
    - 147 individuals trained
    - 85 enrolled in the training
- Resources
  - Toolkit including FAQs, one-pagers, PowerPoint presentation, op-ed sample, talking points
  - Ambassador brochure

Program Highlights: Research Partnerships

- Ambassador names and profiles are posted on PCORI website for potential research partnership opportunities

- Matchmaking: Ambassadors are connected with various PCORI activities as well as with other healthcare agencies seeking skilled patient-centered representatives

Examples of Matchmaking

Joined PCORI's Advisory Panels, Twitter chats, roundtables and other gatherings

Joined a Training Advisory Group member for an initiative to develop a training for medical students focused on patient engagement

Reviewed abstract for advancing partnerships and to promote meaningful roles for patients and families in all stages of research studies

Provided patient partner input on a global perceptions on engagement and empowerment project

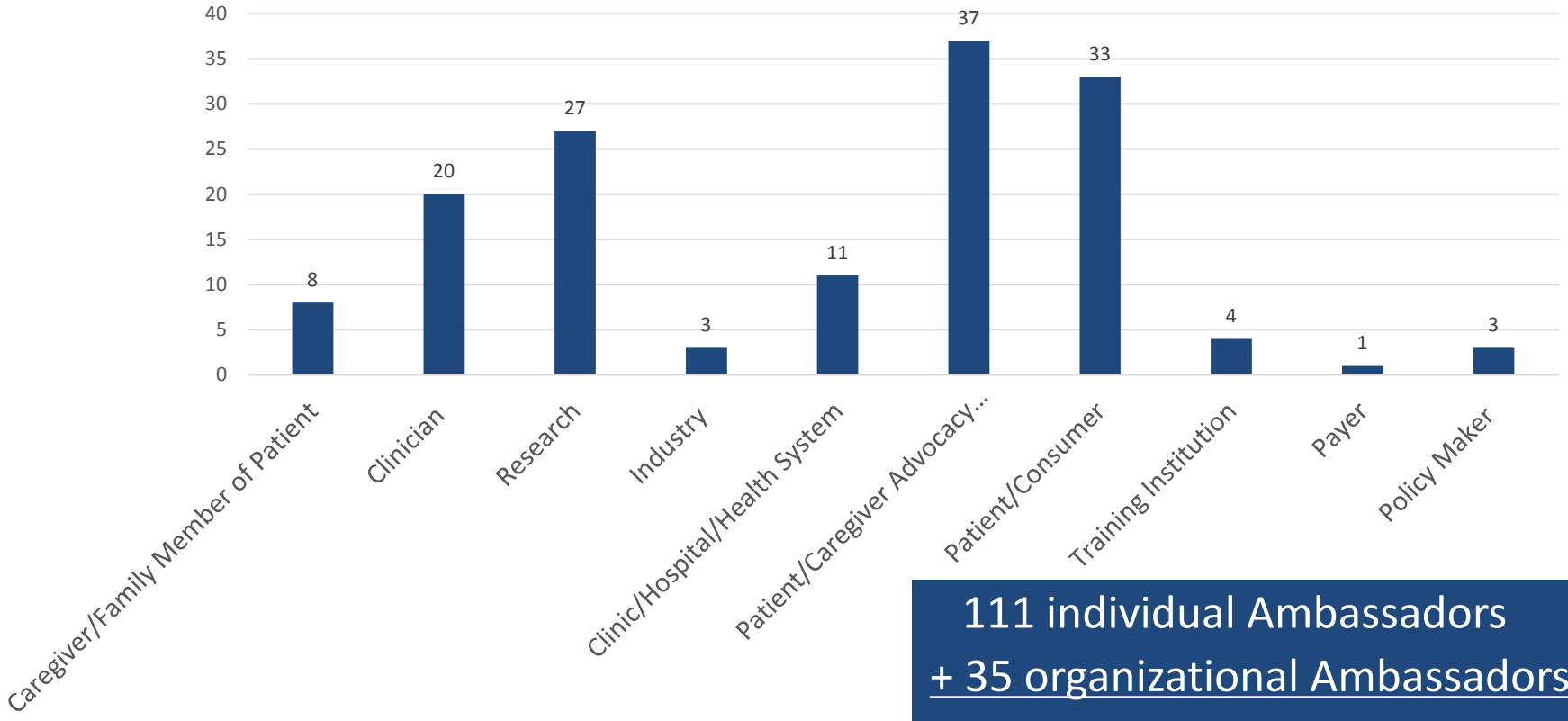
Provided feedback on the development of a system to assist consumers and patients in matching their unscheduled healthcare needs with local hospitals' critical care capabilities.

Joined Technical Expert Panel for assessment of CMS Quality and Efficiency Measures

Program Highlights: Knowledge Sharing

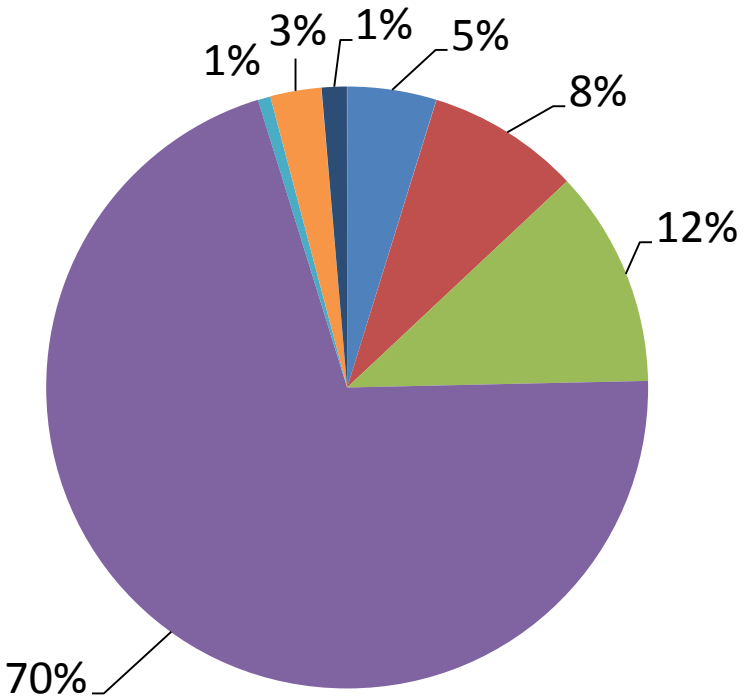
- Yammer
- Monthly Webinars
  - Ambassador Spotlight Topics
    - A Patient Stakeholder's Journey to Pipeline to Proposal
    - Examples of Ambassador Community Presentations
    - The CaReAlign Project
  - PCORI Lead Topics
    - Speakers Bureau
    - Social Media: The Michael J. Fox Foundation
- Annual Meeting
    - 91 Ambassadors attended
    - 5 Ambassador presentations

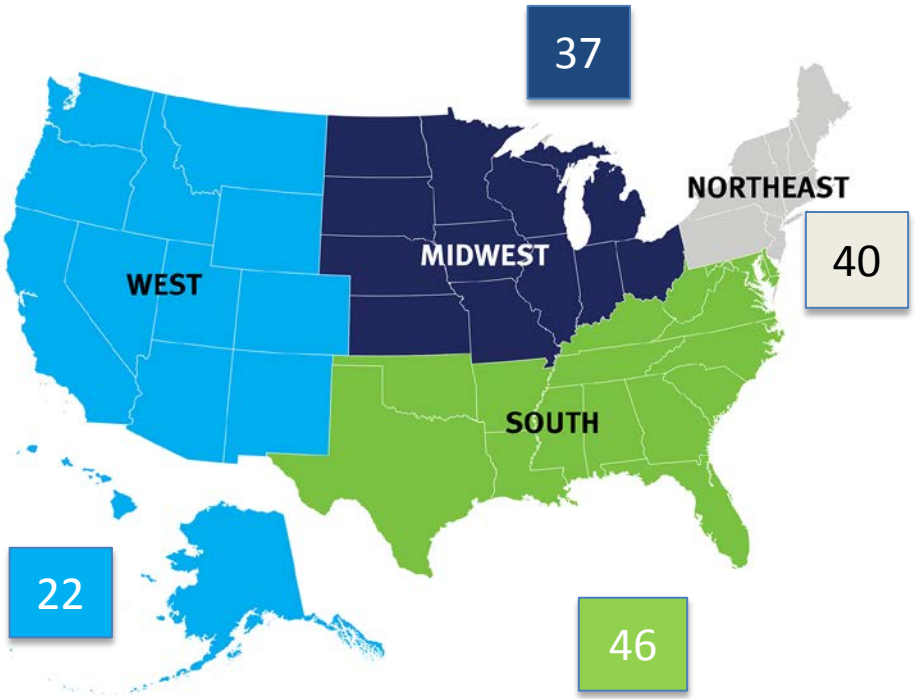
Ambassadors by Stakeholder Group



111 individual Ambassadors
+ 35 organizational Ambassadors
146 total Ambassadors to date

Ambassadors by Ethnicity and Region





- Asian (Not Hispanic or Latino)
- Black or African American (Not Hispanic or Latino)
- Hispanic or Latino American
- White (Not Hispanic or Latino)
- Native Hawaiian
- Prefer Not to Answer

Eugene Washington PCORI Engagement Award Program Update: Involvement of Patient Organizations

Lia Hotchkiss, MPH

Director of the Eugene Washington PCORI Engagement Awards Program

October 27, 2015

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Objectives

- Provide an update on the Engagement Award Program
- Share data on applications from and awards to patient organizations
- Discuss reasons for declined applications
- Provide examples of researcher-led projects with significant community-based organization involvement
- Share the Engagement Award Program focus for 2016

Engagement Awards Program Overview

- Support projects to bring more patients, caregivers, clinicians, and other healthcare stakeholders into the research process by:
  - expanding their **knowledge** and skills to participate in CER and PCOR
  - implementing **training** or skill **development** initiatives to build capacity for engaging in PCOR
  - building networks for **disseminating** research findings

- Meetings/conferences to exchange information or explore issues or areas of knowledge as they relate to PCOR and CER

Stakeholders Submitting Applications for Engagement Awards

Patients	Other Stakeholders
Caregiver/Family Member of Patient Patient/Caregiver Advocacy Organization Patient/Consumer	Researchers Clinicians Clinic/Hospital/Health System Industry Purchasers Payer Policy Maker Training Institution

- Stakeholder category is selected by the applicant at the time they submit an application
- Stakeholder category of the applicant does not necessarily represent all the stakeholder groups that will be co-leading the project/meeting, or that will be engaged as part of the project/meeting
- Engagement Awards are made to organizations, not individuals

Engagement Awards to Patient Organizations by 2015 Review cycle

	Engagement Award Projects				Engagement Award Meeting/Conference Support	
	LOIs Submitted	LOIs Invited to Submit Full Applications	Full EA Applications Submitted	EA Proposals Awarded	EAINs Submitted	EAINs Awarded
January Cycle, 2015	19	4	4	4	8	3
April Cycle, 2015	13	5	5	4	9	2
July Cycle, 2015	23	3	3	1*	15	5*
October Cycle, 2015	20	TBD	TBD	TBD	9	TBD
TOTAL	**75**	**12**	**12**	**9**	**41**	**10**

*still in the process of making awards

In 2015, 25% of all LOIs and applications submitted for Engagement Awards have been from patients/patient organizations and 26% of all Engagement Awards made have been to patient organizations.

Applications and Awards – January 2015 Review Cycle

January Cycle, 2015	Engagement Award Projects (Knowledge, Training & Development, Dissemination)				Engagement Award Meeting/Conference Support	
	LOIs Submitted # (%)	LOIs Invited to Submit Full Applications # (%)	Full EA Applications Submitted # (%)	EA Proposals Awarded # (%)	EAINs Submitted # (%)	EAINs Awarded # (%)
Patient Applicants	19 (25%)	4 (17%)	4 (17%)	4 (20%)	8 (31%)	3 (20%)
Other Stakeholder Applicants	56 (75%)	19 (83%)	19 (83%)	15 (80%)	16 (69%)	12 (80%)
TOTAL	**75 (100%)**	**23 (100%)**	**23 (100%)**	**19 (100%)**	**26 (100%)**	**15 (100%)**

January Review Cycle Highlighted Project

Project	Pastors 4 PCOR: Engaging Faith-Based Communities
Organization	Total Resource Community Development Organization
Project Lead	Paris Davis
Project Summary	This project aims to increase the participation of underserved communities of color in comparative effectiveness research and patient-centered outcomes research. We propose a program of training for faith-based community facilitators about the core values and practices of patient-centered outcomes research, and a network of research ministries in faith-based settings to enable engagement. These research ministries will interact with existing ministries in caregiving, health, and cluster engagement discussion groups on specific diseases. Research ministry facilitators will be equipped with tools and activities for research ministry, which can be delivered as a stand-alone program or embedded in existing groups. A flexible program of activities will introduce facilitators to patient-centered outcomes research, increase community capacity to identify faith-based community based health assets, increase likelihood of engaging with health research, and engage community members.

Applications and Awards – April 2015 Review Cycle

April Cycle, 2015	Engagement Award Projects				Engagement Award Meeting/Conference Support	
	LOIs Submitted # (%)	LOIs Invited to Submit Full Applications # (%)	Full EA Applications Submitted # (%)	EA Proposals Awarded # (%)	EAINs Submitted # (%)	EAINs Awarded # (%)
Patient Applicants	13 (19%)	5 (33.5%)	5 (33.5%)	4 (44%)	9 (32%)	2 (25%)
Other Stakeholder Applicants	55 (81%)	10 (66.5%)	10 (66.5%)	5 (56%)	19 (68%)	6 (75%)
TOTAL	**68 (100%)**	**15 (100%)**	**15 (100%)**	**9 (100%)**	**28 (100%)**	**8 (100%)**

April Cycle Highlighted Project

Project	Patient Advisory Committee for Clinical Trials (PACCT+)
Organization	Aplastic Anemia & MDS International Foundation
Project Lead	Ellen Salkeld
Project Summary	The Aplastic Anemia and MDS International Foundation (AA&MDSIF) has initiated a Patient Advisory Committee for Clinical Trials (PACCT+) to bring the patient voice and experience to industry and academic researchers developing clinical and health services research for rare bone marrow failure disorders. Patients with these diseases are eager to propel research towards mitigation of disease, yet patient input in design of rare disease research is minimal. AA&MDSIF will identify, select, and provide basic research training to a representative cross-section of patients with bone marrow failure disease, parents, and caregivers. PACCT+ will be available for researchers to solicit nonbinding recommendations, demonstrating inclusion of meaningful patient input in design and implementation of research. PACCT+ will address health-based studies within bone marrow failure disease that would benefit from front-end patient input, clinical trials, health services research, and quality-of-life studies.

Applications and Awards – July 2015 Review Cycle

July Cycle, 2015	Engagement Award Projects				Engagement Award Meeting/Conference Support	
	LOIs Submitted # (%)	LOIs Invited to Submit Full Applications # (%)	Full EA Applications Submitted # (%)	EA Proposals Awarded* # (%)	EAINs Submitted # (%)	EAINs Awarded* # (%)
Patient Applicants	23 (22%)	3 (13%)	3 (14%)	1 (33.5%)	15 (41%)	5 (36%)
Other Stakeholder Applicants	81 (78%)	19 (87%)	18 (86%)	2 (66.5%)	22 (59%)	9 (64%)
TOTAL	**104 (100%)**	**22 (100%)**	**21 (100%)**	**3 (100%)**	**37 (100%)**	**14 (100%)**

*still in the process of making awards from the July cycle

July Cycle Highlighted Project

Project	Phelan-McDermid Syndrome Patient-Centered Outcomes Workshop
Organization	Phelan-McDermid Syndrome Foundation
Project Lead	Geraldine Bliss
Project Summary	Phelan-McDermid Syndrome (PMS) is a rare genetic condition that causes developmental disability, autism, hypotonia, and, often, complex medical and psychiatric conditions, which are not yet well understood. Our workshop will bring together researchers and families of people with PMS to discuss several topics of high interest. Each topic will include an introductory talk by an expert in the field, followed by a parent roundtable discussion and real-time polling paired with webcasting, and a panel discussion about future projects. Workshop findings will be summarized in a white paper, which will be made available through www.pmsf.org. Our goals are (1) to communicate family concerns and priorities to the medical and scientific community to inform the design and conduct of future research and (2) to improve the flow of information to help families make the appropriate medical, behavioral, and educational decisions together with their children's medical professionals.

Applications and Awards – October 2015 Review Cycle

October Cycle, 2015	Engagement Award Projects				Engagement Award Meeting/Conference Support	
	LOIs Submitted # (%)	LOIs Invited to Submit Full Applications # (%)	Full EA Applications Submitted # (%)	EA Proposals Awarded # (%)	EAINs Submitted # (%)	EAINs Awarded # (%)
Patient Applicants	20 (20%)	TBD	TBD	TBD	9 (38%)	TBD
Other Stakeholder Applicants	81 (80%)	TBD	TBD	TBD	15 (62%)	TBD
TOTAL	**101 (100%)**	**TBD**	**TBD**	**TBD**	**24 (100%)**	**TBD**

Trends in Declined LOIs/Proposals

Focused on patients and stakeholders as study subjects in clinical trials vs. integrated in research process
Proposed to use funds to create patient registries
Proposed fundraising or advocacy activities
Insufficient tie to PCOR or CER in project and/or proposal
Proposed project budget disproportionate to organizational budget
Misdirected application
Proposed development of treatment or care models or decision aids
Proposed to conduct human subjects research or pilot study
Proposed to develop and test web applications
Insufficient details in proposal regarding activities, project scope, budget, and/or key personnel

Examples of Researcher-Led Projects with Significant Community-Based Organization Involvement

Project	**Building On a Culturally Sensitive Network for PCOR/CER Dissemination**
Lead	Carol Connell - The University of Southern Mississippi
Co-Lead [Project Manager]	Freddie White-Johnson - Mississippi Network for Cancer Control, USM • ***Background:*** Founder of The Fannie Lou Hamer Cancer Foundation and Teens Against Premature Pregnancy Outreach – both thriving nonprofit community-based organizations. Has 15 years of experience establishing networks and coalitions between agencies, universities, organizations, and the community
Project Summary	The rural Mississippi Delta region has unique assets and disadvantages related to its culture and heritage. Favorable health outcomes are compromised by limitations in healthcare access, and economic and social well-being, among others. Delta citizens can contribute as PCORI stakeholders to developing approaches for PCOR/CER participation and dissemination that build on cultural strengths. Among those strengths is a grassroots network that aims to reduce disparities in cancer mortality through awareness, education, and advocacy. This engagement project builds on work of the Mississippi Network for Cancer Control and Prevention to discover how PCOR is received by stakeholders through the network and other means, how network community health advisors disseminate PCOR, and how research capacity-building activities with Delta stakeholder groups strengthen their capacity for engagement in PCOR.

Project	Partnership for Training Community Health Workers in PCOR
Lead	Olveen Carrasquillo - University of Miami
Key Personnel [Stakeholder Partners]	Marisel Losa - Health Council of South Florida, Inc. Marion Banzhaf - Bureau of Chronic Disease Prevention, Florida Department of Health • ***Background:*** Community healthcare leaders with decades of on-the-ground experience in South Florida and elsewhere working to enhance the development and professionalism of community health workers. Their experience and background support the implementation and development of structured PCOR training programs in partnership with researchers through this project.
Project Summary	Community Health Workers (CHWs) are an important stakeholder group in Patient-Centered Outcomes Research (PCOR). In this application to the Eugene Washington Engagement Awards Training and Development Awards program, we propose to develop and implement a structured research training program in PCOR for CHWs. Over the last four years, the University of Miami along with the Health Council of South Florida and the Florida Community Health Worker Coalition have led a multi-stakeholder group in developing a structured training and state certification program for CHWs. We now propose to build on this experience to develop a PCOR elective module that can be used by CHWs toward their state certification. We will use this training program to train 100 CHWs in Florida in PCOR. We will then develop a toolkit that can be used by groups in other states to develop a similarly structured program for training CHWs in PCOR.

Engagement Award Program Focus in 2016

- Provide greater transparency into projects and project teams
- Develop and implement strategy for reviewing and sharing EA work products
- Raise awareness about the EA program and encourage applications from patient organizations and community-based organizations
- Continue to create a robust portfolio of awards inclusive of projects spanning PCORI stakeholder groups and populations of interest
- Focus on laying the groundwork for disseminating research findings
- Coordinate with PCORI training initiatives
- Continue to communicate the value and impact of the EA program

Reviews in February, June, and October 2016

Questions?

Thank You

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Wrap-Up, Next Steps, and Reflections

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Safe Travels!

Thank you