PATIENT-CENTERED RESEARCH DESIGN FOR HEALTHCARE INNOVATION

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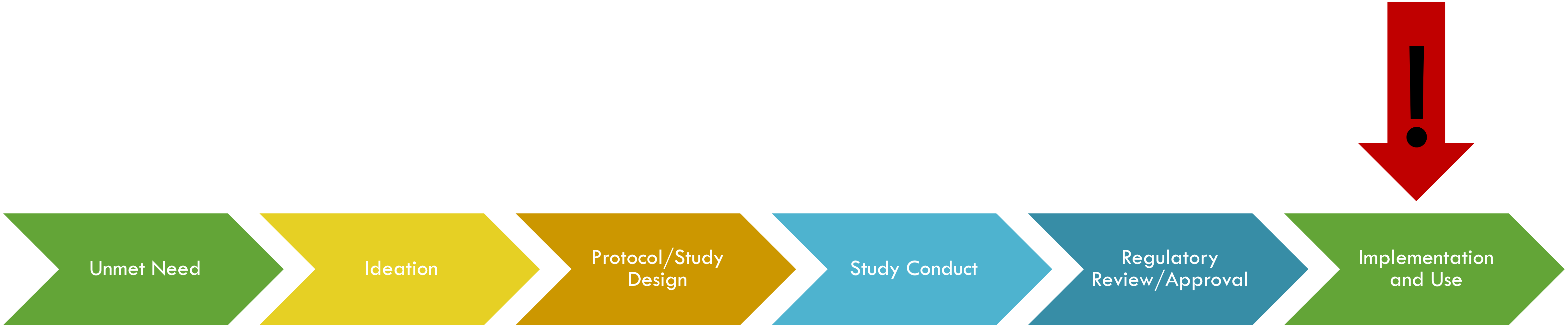
ExPPect

PATIENT-ENGAGED RESEARCH & DESIGN

- Terminology and Timing
- Proof
- Tools

TIMING

IS IMPLEMENTATION REALLY AT THE “END”?


Unmet Need

Ideation

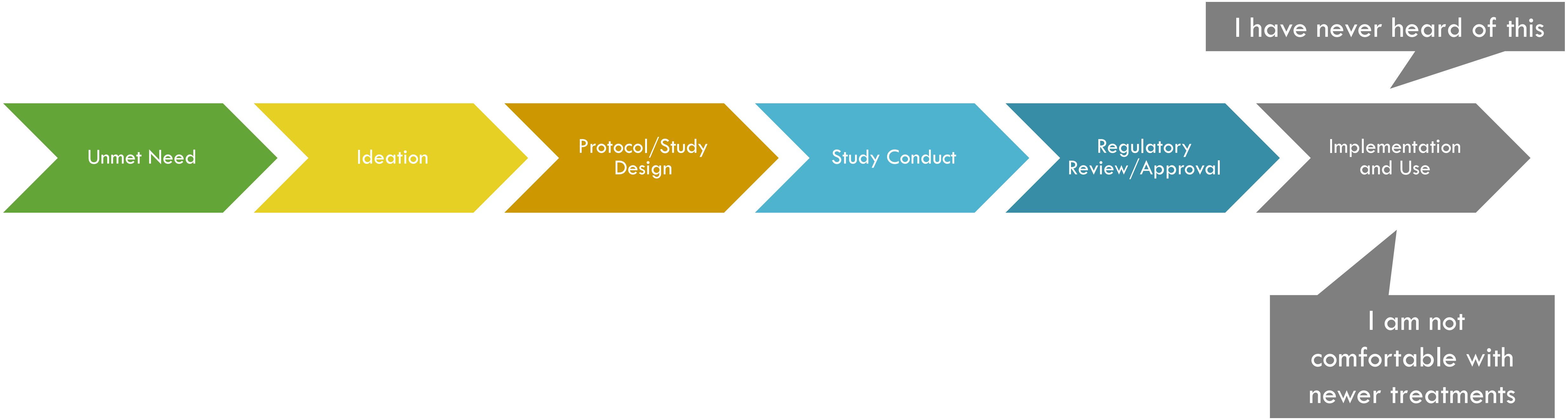
Protocol/Study Design

Study Conduct

Regulatory Review/Approval

Implementation and Use

DOWNSTREAM CHALLENGES



I have never heard of this

Unmet Need

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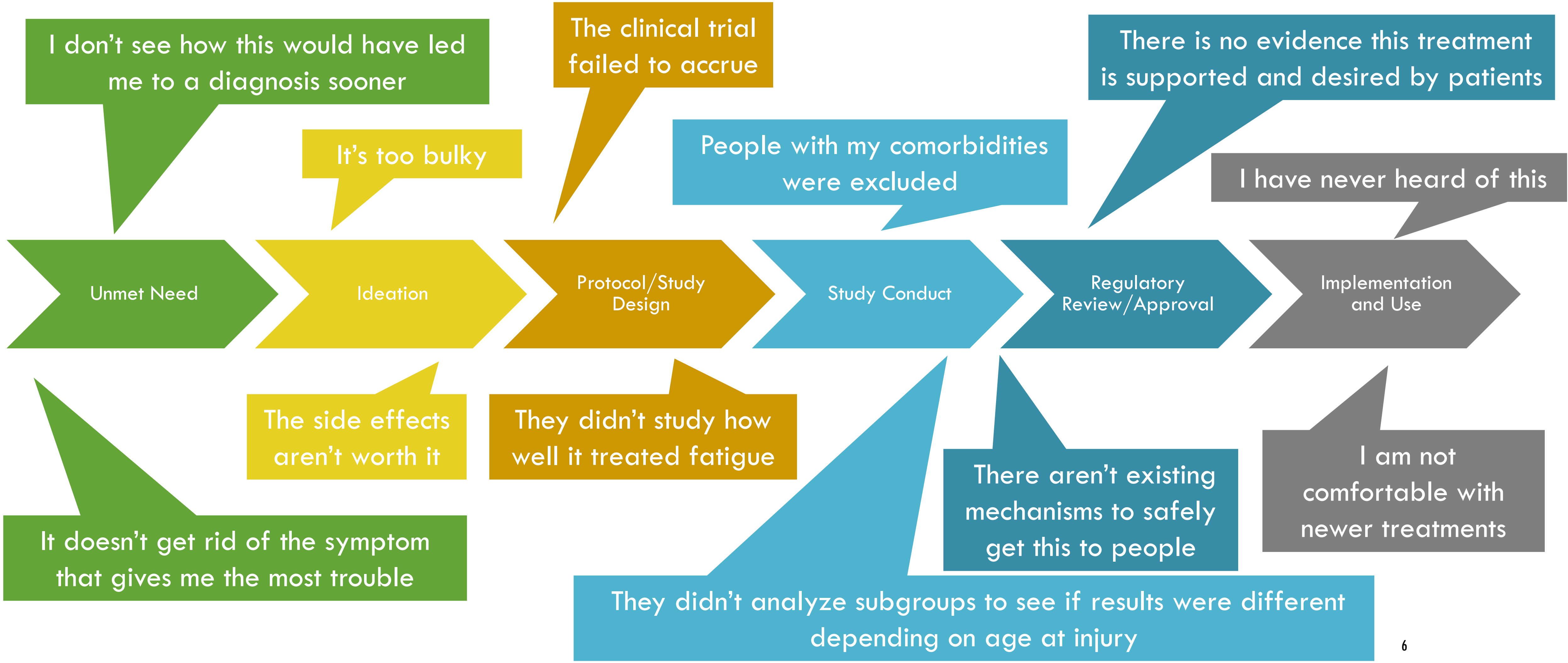
I am not comfortable with newer treatments

UPSTREAM SOLUTIONS



Unmet Need

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I don't see how this would have led me to a diagnosis sooner

It doesn't get rid of the symptom that gives me the most trouble

It's too bulky

The side effects aren't worth it

The clinical trial failed to accrue

They didn't study how well it treated fatigue

People with my comorbidities were excluded

They didn't analyze subgroups to see if results were different depending on age at injury

There is no evidence this treatment is supported and desired by patients

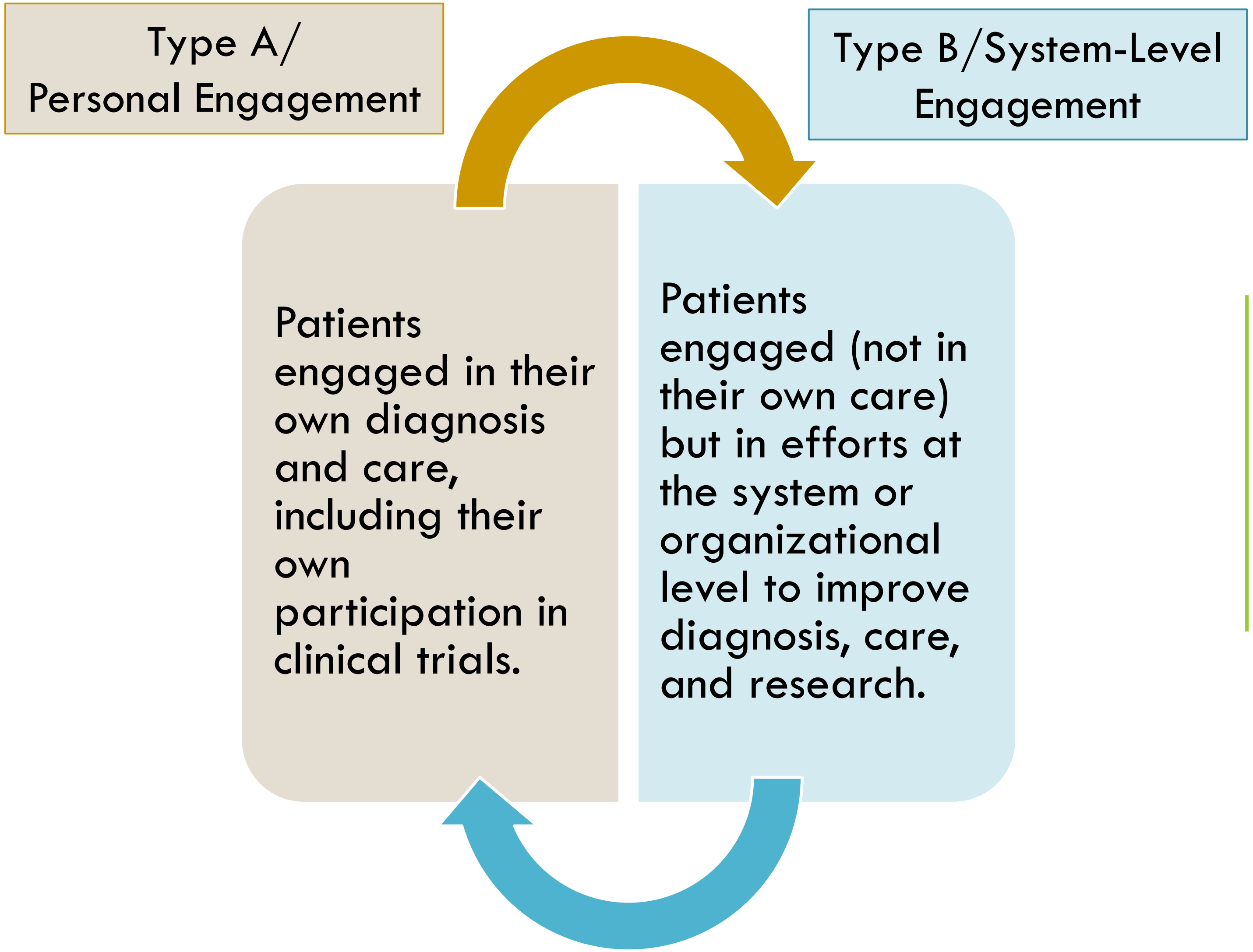
There aren't existing mechanisms to safely get this to people

I have never heard of this

I am not comfortable with newer treatments

TERMINOLOGY

THERE ARE TWO DISTINCT TYPES OF PATIENT ENGAGEMENT



Type A/ Personal Engagement

Patients engaged in their own diagnosis and care, including their own participation in clinical trials.

Type B/System-Level Engagement

Patients engaged (not in their own care) but in efforts at the system or organizational level to improve diagnosis, care, and research.

APPLICATION IN MEDICAL PRODUCT R&D

Type A

- Shared decision-making with providers about diagnostic, treatment, and management options
- Well-supported participation in clinical trials, with feasible protocols and person-centered information and resources
- Programs to educate and empower patients to best navigate and manage their conditions

Type B

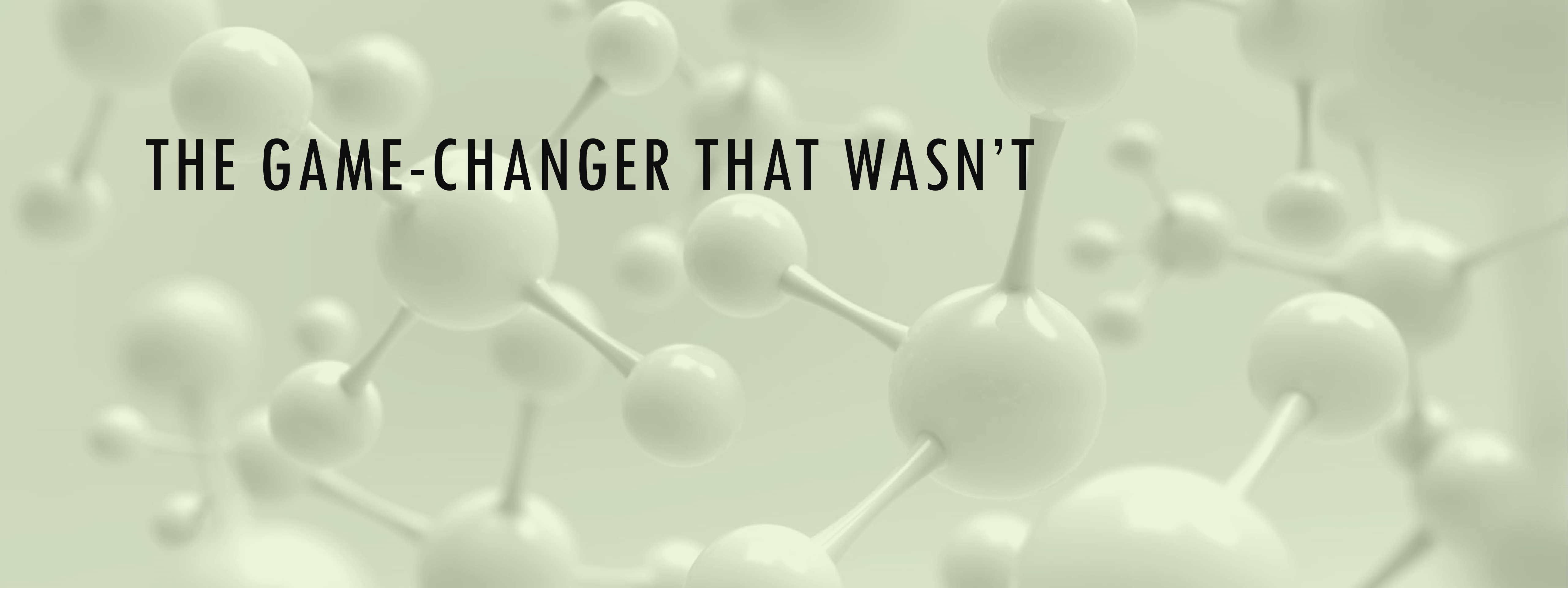
- Patient groups identifying unmet needs and diagnostic and treatment priorities
- Patient partners on a clinical trial protocol team, co-creating feasible and accessible study design and selecting endpoints and outcomes that matter to them
- Patient partners on a clinical trial team helping to interpret and contextualize incoming study data

PROOF

REMEMBER THE EDSEL?

“As for the design, it was arrived at without even a pretense of consulting the polls, and by the method that has been standard for years in the designing of automobiles — that of simply pooling the hunches of sundry company committees.“

--John Brooks, “Business Adventures”



THE GAME-CHANGER THAT WASN’T

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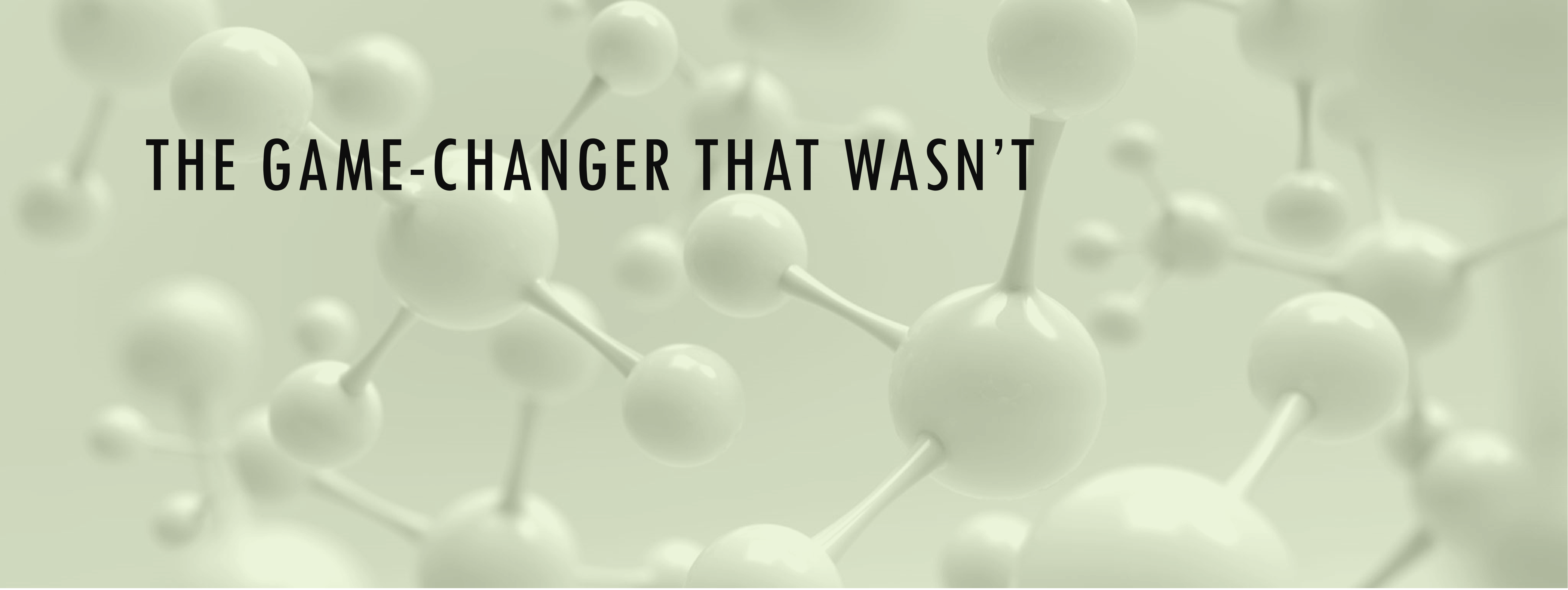


- Unmet Need?
- Ideation?
- Protocol/Study Design?
- Study Conduct?
- Regulatory Review/Approval?
- Implementation and Use?

CALCULATING THE "RETURN ON ENGAGEMENT"

Levitan B, Getz K, Eisenstein EL, et al. Assessing the Financial Value of Patient Engagement: A Quantitative Approach from CTTI's Patient Groups and Clinical Trials Project. *Therapeutic Innovation & Regulatory Science.* 2018;52(2):220-229. doi:10.1177/2168479017716715

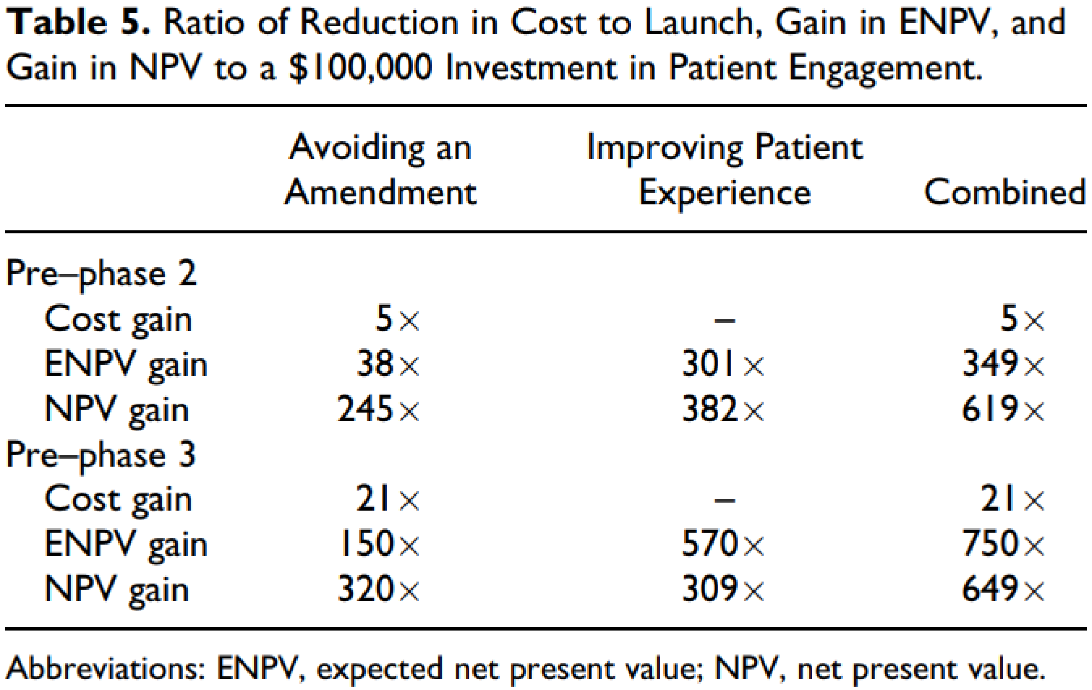


Table 5. Ratio of Reduction in Cost to Launch, Gain in ENPV, and Gain in NPV to a $100,000 Investment in Patient Engagement.

	Avoiding an Amendment	Improving Patient Experience	Combined
Pre–phase 2			
Cost gain	5×	–	5×
ENPV gain	38×	301×	349×
NPV gain	245×	382×	619×
Pre–phase 3			
Cost gain	21×	–	21×
ENPV gain	150×	570×	750×
NPV gain	320×	309×	649×

Abbreviations: ENPV, expected net present value; NPV, net present value.

ENGAGEMENT WIN: THE NEED TO EXPLAIN “NORMAL”

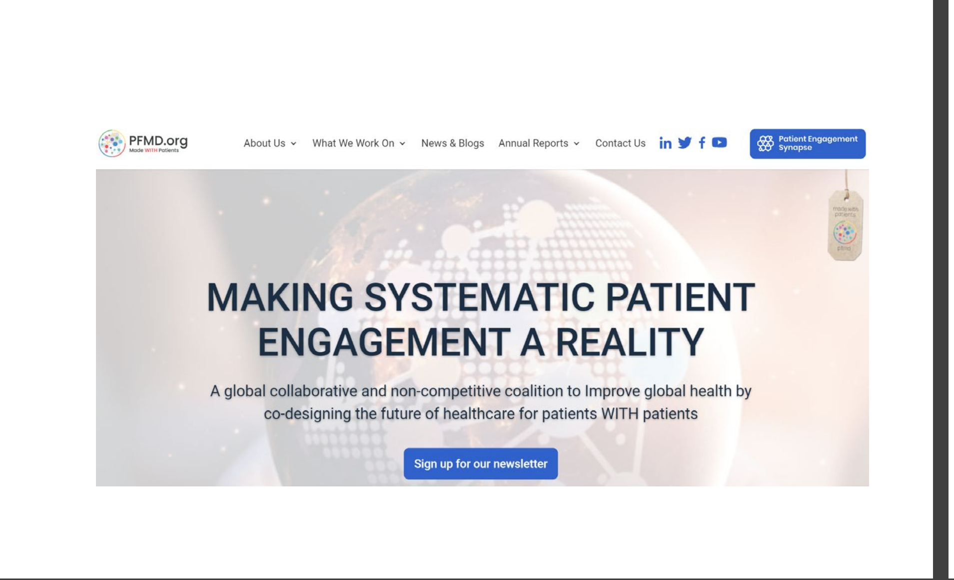
- I didn’t know my symptoms were abnormal so I didn’t report them
- This is how it has been since childhood so I assumed it was normal
- When they asked if there were any changes I said “no” because my symptoms were the same as always

ENGAGEMENT WIN: PATIENT-PROVIDED CONTEXT IN DATA ANALYSIS

Claims Data, RA Therapeutics

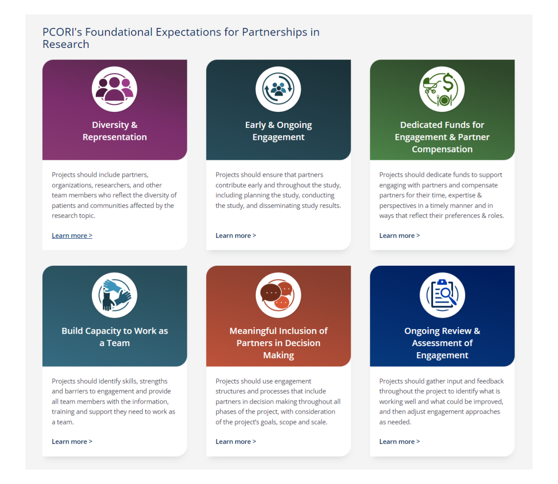
	Treatment History, Patient 1	**Treatment History, Patient 2**	**Treatment History, Patient 3**
Jan	Therapy A, B, and F	Therapy A	Therapy D
Feb	Therapy B and F, not A	No therapy	Therapy D
Mar	Therapy B and F, not A	No therapy	Therapy D and A
Apr	No therapy	Therapy A	Therapy D and A
May	Therapy A, B, not F	Therapy C, not A	Therapy A, not D
Jun	No therapy	Therapy A and C	Therapy D

(recreation of data, not actual representation)



MAKING SYSTEMATIC PATIENT ENGAGEMENT A REALITY

A global collaborative and non-competitive coalition to Improve global health by co-designing the future of healthcare for patients WITH patients



PCORI's Foundational Expectations for Partnerships in Research

Diversity & Representation

Projects should include partners, organizations, researchers, and other team members who reflect the diversity of patients and communities affected by the research topic.

Early & Ongoing Engagement

Projects should ensure that partners contribute early and throughout the study, including planning the study, conducting the study, and disseminating study results.

Dedicated Funds for Engagement & Partner Compensation

Projects should dedicate funds to support engaging with partners and compensate partners for their time, expertise & perspectives in a timely manner and in ways that reflect their preferences & roles.

Build Capacity to Work as a Team

Projects should identify skills, strengths and barriers to engagement and provide all team members with the information, training and support they need to work as a team.

Meaningful Inclusion of Partners in Decision Making

Projects should use engagement structures and processes that include partners in decision making throughout all phases of the project, with consideration of the project's goals, scope and scale.

Ongoing Review & Assessment of Engagement

Projects should gather input and feedback throughout the project to identify what is working well and what could be improved, and then adjust engagement approaches as needed.

FDA Patient-Focused Drug Development Guidance Series for Enhancing the Incorporation of the Patient's Voice in Medical Product Development and Regulatory Decision Making

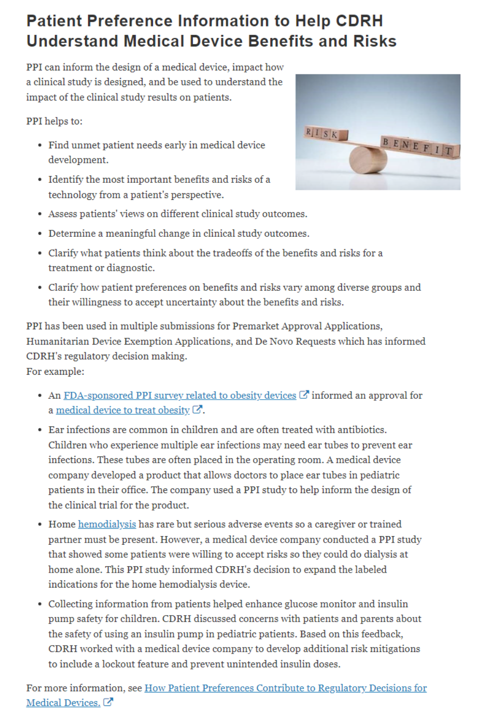
FDA is developing a series of four methodological patient-focused drug development (PFDD) guidance documents to address, in a stepwise manner, how stakeholders can collect and submit patient experience data and other relevant information from patients and caregivers for medical product development and regulatory decision making. This series of guidance documents is intended to facilitate the advancement and use of systematic approaches to collect and use robust and meaningful patient and caregiver input that can better inform medical product development and regulatory decision making.

These guidances are part of FDA's PFDD efforts in accordance with the 21st Century Cures Act and The Food and Drug Administration Reauthorization Act of 2017 Title I.

This webpage contains information and documents related to FDA's development of the methodological PFDD guidances, including public workshops, draft guidances, and hypothetical scenarios, which are all intended to serve as a basis for dialogue. To supplement the PFDD Guidance Series, FDA issued two technical specifications guidance documents, *Submitting Clinical Trial Datasets and Documentation for Clinical Outcome Assessments Using Item Response Theory* and *Submitting Patient-Reported Outcome Data in Cancer Clinical Trials*.

Content current as of:
02/14/2024

Regulated Product(s)
Drugs



Patient Preference Information to Help CDRH Understand Medical Device Benefits and Risks

PPI can inform the design of a medical device, impact how a clinical study is designed, and be used to understand the impact of the clinical study results on patients.

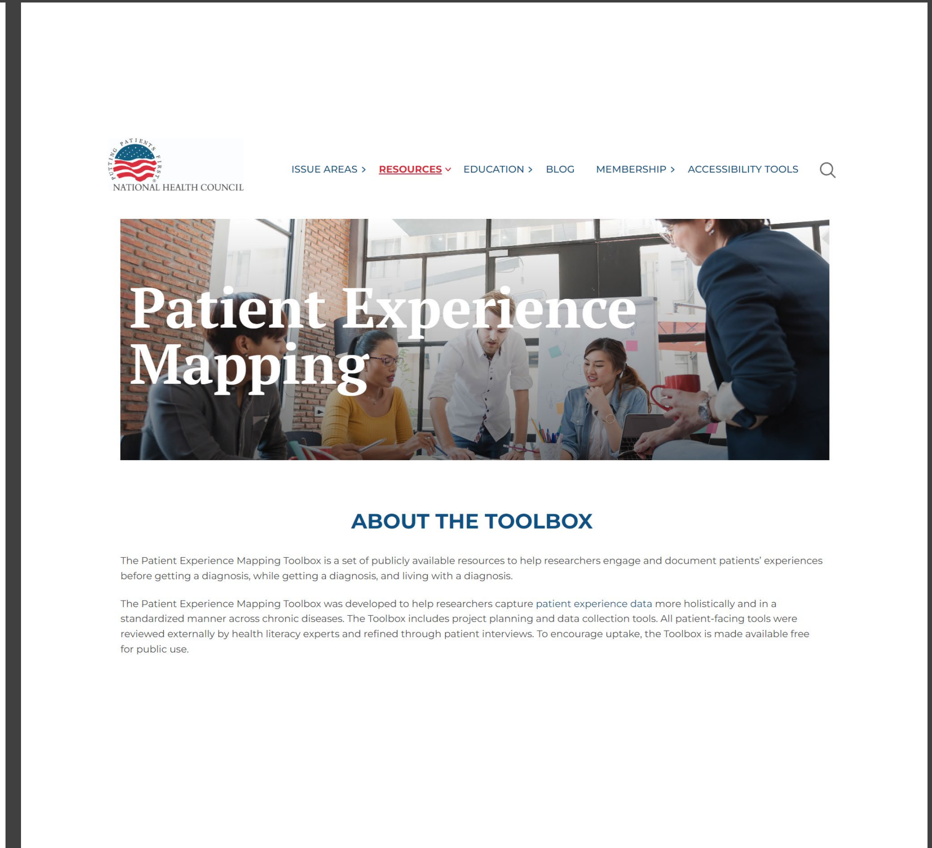
PPI helps to:

- Find unmet patient needs early in medical device development.
- Identify the most important benefits and risks of a technology from a patient's perspective.
- Assess patients' views on different clinical study outcomes.
- Determine a meaningful change in clinical study outcomes.
- Clarify what patients think about the tradeoffs of the benefits and risks for a treatment or diagnostic.
- Clarify how patient preferences on benefits and risks vary among diverse groups and their willingness to accept uncertainty about the benefits and risks.

PPI has been used in multiple submissions for Premarket Approval Applications, Humanitarian Device Exemption Applications, and De Novo Requests which has informed CDRH's regulatory decision making.
For example:

- An FDA-sponsored PPI survey related to obesity devices informed an approval for a medical device to treat obesity.
- Ear infections are common in children and are often treated with antibiotics. Children who experience multiple ear infections may need ear tubes to prevent ear infections. These tubes are often placed in the operating room. A medical device company developed a product that allows doctors to place ear tubes in pediatric patients in their office. The company used a PPI study to help inform the design of the clinical trial for the product.
- Home hemodialysis has rare but serious adverse events so a caregiver or trained partner must be present. However, a medical device company conducted a PPI study that showed some patients were willing to accept risks so they could do dialysis at home alone. This PPI study informed CDRH's decision to expand the labeled indications for the home hemodialysis device.
- Collecting information from patients helped enhance glucose monitor and insulin pump safety for children. CDRH discussed concerns with patients and parents about the safety of using an insulin pump in pediatric patients. Based on this feedback, CDRH worked with a medical device company to develop additional risk mitigations to include a lockout feature and prevent unintended insulin doses.

For more information, see How Patient Preferences Contribute to Regulatory Decisions for Medical Devices.

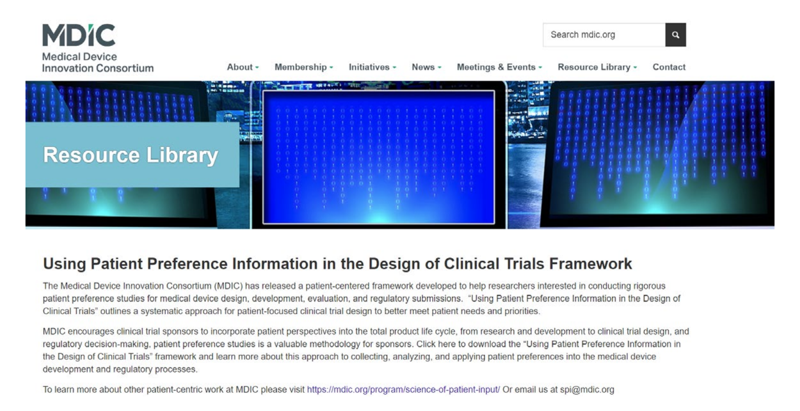


Patient Experience Mapping

ABOUT THE TOOLBOX

The Patient Experience Mapping Toolbox is a set of publicly available resources to help researchers engage and document patients' experiences before getting a diagnosis, while getting a diagnosis, and living with a diagnosis.

The Patient Experience Mapping Toolbox was developed to help researchers capture patient experience data more holistically and in a standardized manner across chronic diseases. The Toolbox includes project planning and data collection tools. All patient-facing tools were reviewed externally by health literacy experts and refined through patient interviews. To encourage uptake, the Toolbox is made available free for public use.

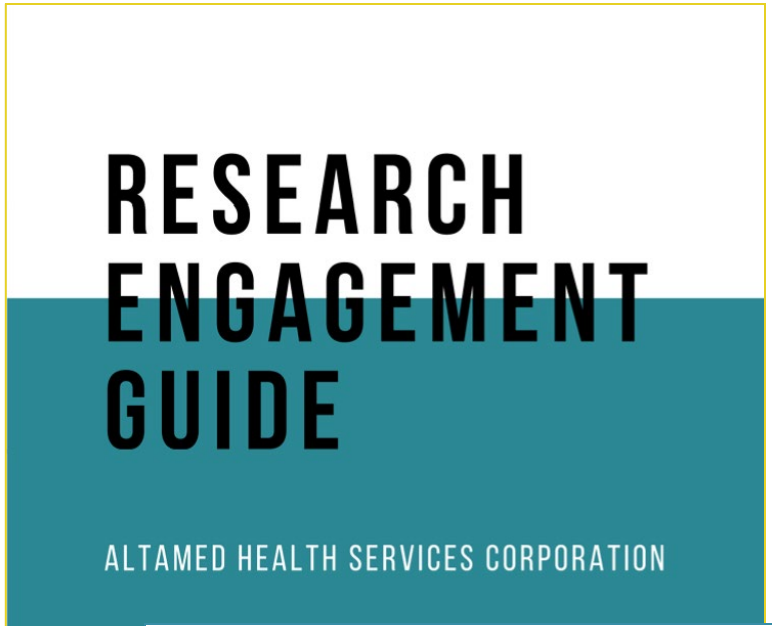


Using Patient Preference Information in the Design of Clinical Trials Framework

The Medical Device Innovation Consortium (MDIC) has released a patient-centered framework developed to help researchers interested in conducting rigorous patient preference studies for medical device design, development, evaluation, and regulatory submissions. "Using Patient Preference Information in the Design of Clinical Trials" outlines a systematic approach for patient-focused clinical trial design to better meet patient needs and priorities.

MDIC encourages clinical trial sponsors to incorporate patient perspectives into the total product life cycle, from research and development to clinical trial design, and regulatory decision-making, patient preference studies is a valuable methodology for sponsors. Click here to download the "Using Patient Preference Information in the Design of Clinical Trials" framework and learn more about this approach to collecting, analyzing, and applying patient preferences into the medical device development and regulatory processes.

To learn more about other patient-centric work at MDIC please visit https://mdic.org/program/science-of-patient-input/ Or email us at spi@mdic.org



RESEARCH ENGAGEMENT GUIDE

ALTAMED HEALTH SERVICES CORPORATION

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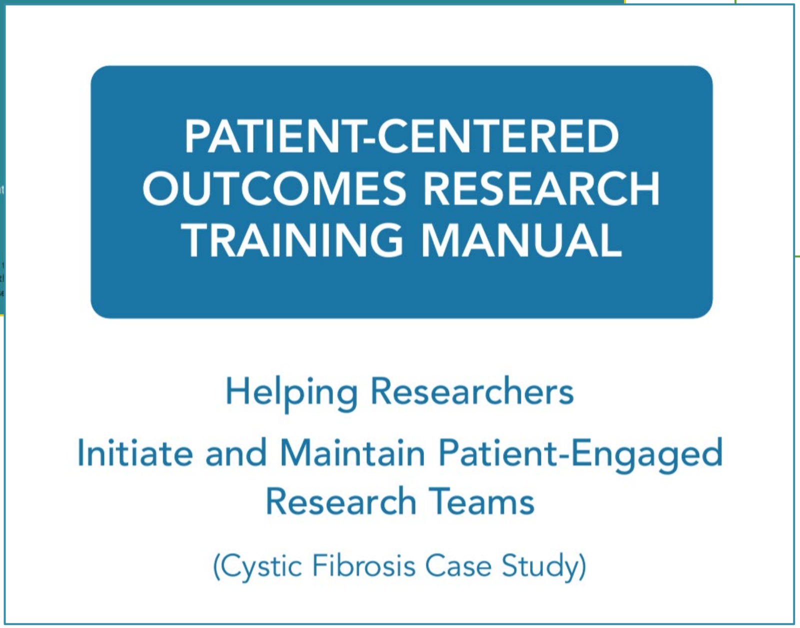
This project was funded
The views presented in t
Centered Outcomes Rese

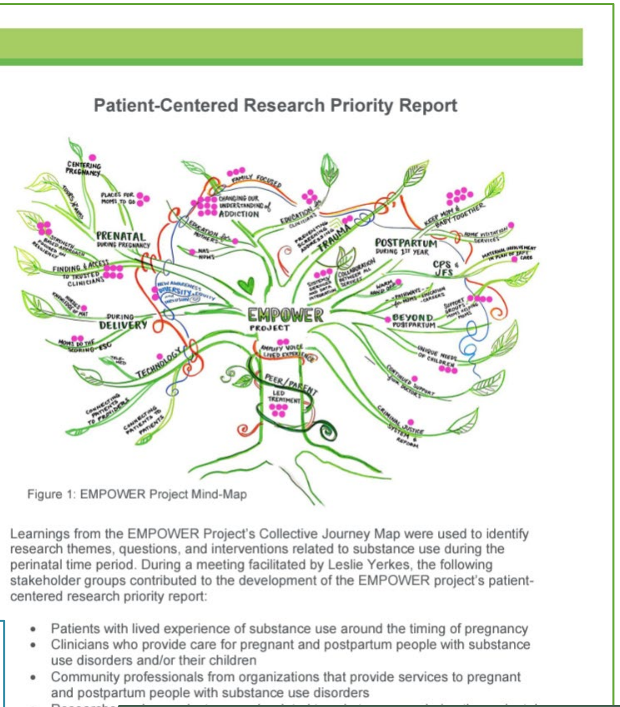


PATIENT-CENTERED OUTCOMES RESEARCH TRAINING MANUAL

Helping Researchers

Initiate and Maintain Patient-Engaged Research Teams

(Cystic Fibrosis Case Study)



Patient-Centered Research Priority Report

Figure 1: EMPOWER Project Mind-Map

Learnings from the EMPOWER Project's Collective Journey Map were used to identify research themes, questions, and interventions related to substance use during the perinatal time period. During a meeting facilitated by Leslie Yerkes, the following stakeholder groups contributed to the development of the EMPOWER project's patient-centered research priority report:

- Patients with lived experience of substance use around the timing of pregnancy
- Clinicians who provide care for pregnant and postpartum people with substance use disorders and/or their children
- Community professionals from organizations that provide services to pregnant and postpartum people with substance use disorders
- Researche
time period

EMPOWER Proje

2020 PFA Summit on Measure Development

April 6, 2020 Bringing Patients and Families to the Table

PFCCpartners
Patient & Family Centered Care

DISCERN
HEALTH

NATIONAL CENTER
for START SERVICES®
Institute on Disability
University of New Hampshire

Reconciling the Past and Changing the Future

What is this project about?

- How we developed the Truth & Reconciliation forums
- Lessons learned

Know about and work to prevent harms in IDD-MH

RECONCILING THE PAST
AND **CHANGING THE FUTURE**

Engaging Young Adults with IDD-MH and Researchers in Comparative Effectiveness Research

www.pcori.org/engagement/engagement-resources/Engagement-Tool-Resource-Repository

IN SUMMARY

- “Dabbling” in engagement often yields unsatisfactory results; comprehensiveness is key
- There is no step of medical product development that cannot benefit from engagement
- There are myriad resources available and patients and patient groups are eager to partner
- Engagement is not done for the sake of engagement, it’s done in pursuit of better science

THANK YOU!