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4

Implementation/Process of Obtaining and Using PPI – the 'Beyond'

SESSION 4



Virtual ISPOR-FDA Summit 2020



BAROSTIM
THERAPY™

MDIC Patient Preference in Heart Failure

Interest Statement

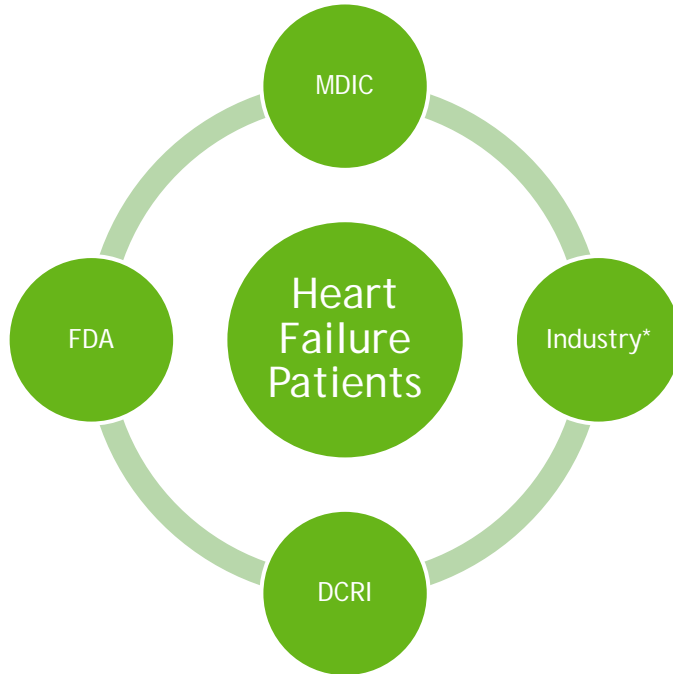
- Dean Bruhn-Ding, Vice President of Regulatory Affairs & Quality Assurance
 - Officer & Shareholder of CVRx, Inc.



MDIC's Heart Failure Patient Preference Project

- The objective of this project was to advance the science of regulatory patient preference assessment by giving medical device industry sponsors, regulatory agencies, and preference assessment experts another example of a disease-specific patient preference study.
- This study built on the MDIC Patient-Centered Clinical Trial Design project in Parkinson's Disease by building a coalition of medical device sponsors, heart failure patients, FDA regulators, and patient preference assessment experts to conduct a preference study with heart failure patients.

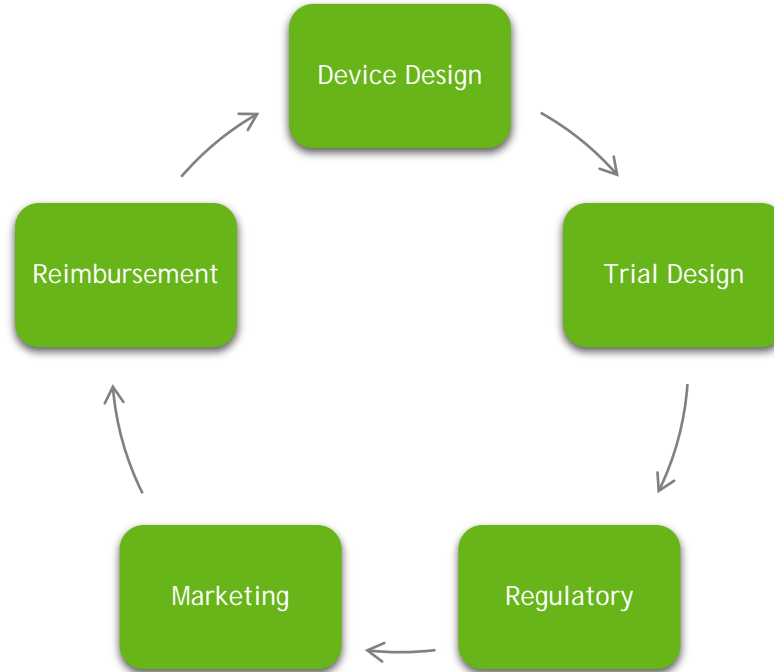
Who's involved in this project?



*6 Companies:

- Abbott
- Abiomed
- Boston Scientific
- CVRx
- Edwards Lifesciences
- Medtronic

Challenge - Integrating Patient Preference Across the Lifecycle





Patient-Centered Preference (PreProCare) Assessment Intervention Trial-Helping prostate cancer patients choose the treatment that is right for them

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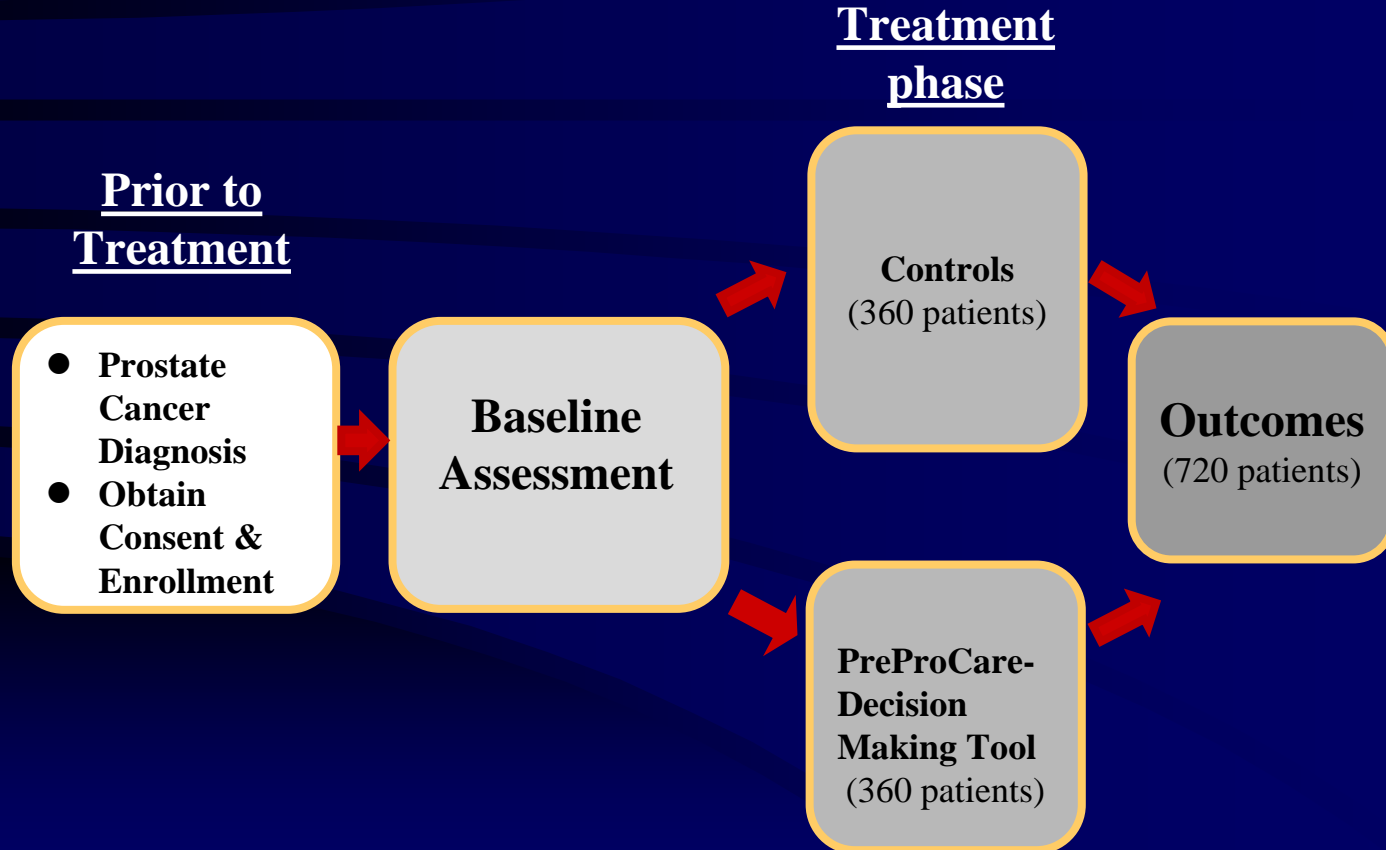
The authors have no conflict of interest to report

Objective

- Study the effectiveness of our Patient Preferences for Prostate Cancer Care (PreProCare) tool, a preference assessment intervention, compared to usual care.
- Identify preferred features of prostate cancer treatments (including active surveillance) that will aid in designing ways to help patients weigh treatment pros and cons.
- Will PreProCare improve patient satisfaction with care, satisfaction with their treatment decision, reduce decision regret, and align treatment choice with their prostate cancer risk?

Research Design

Randomized Controlled Study



PreProCare Instrument



Introduction

Instructions

Attributes

Choices

Preferences

Preference Assessment Decision Aid

**Welcome to
My Prostate Cancer, My Choice**



the cure is with in
ABRAMSON CANCER CENTER

ATTRIBUTES

- Suppose you are given two treatment options for your prostate cancer. They are identical in every way, except for their rate of survival.
 - Treatment A may make many (85%) of patients survive 10 years
 - Treatment B may make almost all (98%) of patients survive 10 years
- How important would this difference in survival be to you?



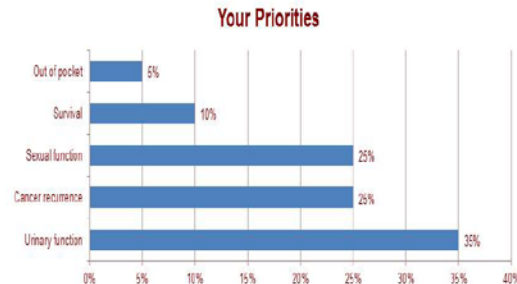
CHOICE SCENARIOS

- If these two prostate cancer treatments were identical in all other ways, which would you prefer?

Treatment A	Treatment B
<ul style="list-style-type: none"> More than half (60%) may experience urinary function problems in the short-term & Less than half (40%) may experience urinary function problems in the long-term 	<ul style="list-style-type: none"> Some (20%) may experience urinary function problems in the short-term & Very few (10%) may experience urinary function problems in the long-term
<ul style="list-style-type: none"> Some (20%) may experience psychological distress 	<ul style="list-style-type: none"> Very few (10%) may experience psychological distress



My Prostate Cancer Treatment Features

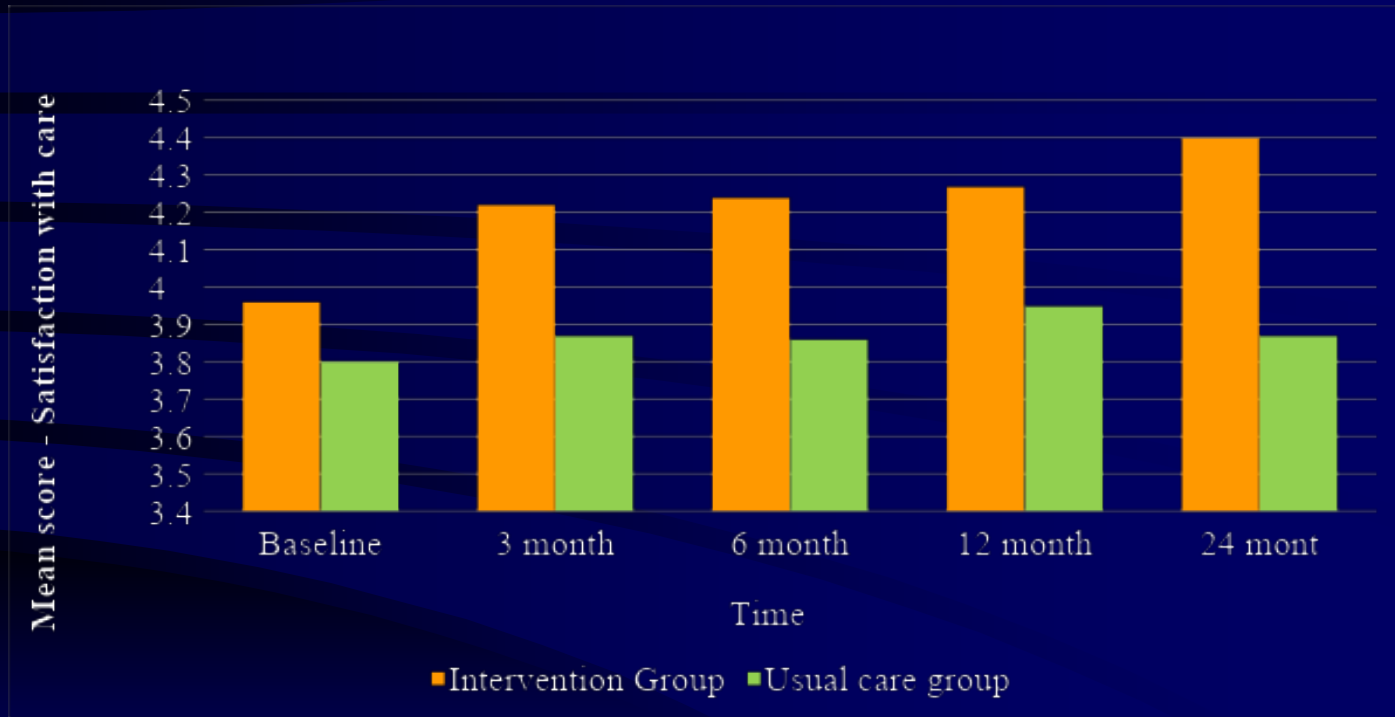


Based on your responses, following features of prostate cancer treatment are most important to you. You may want to talk with your physician about:

- Urinary function (such as leaked urine, blood in urine, pain/burning with urination, straining to urinate, a need for pads, or catheter).
- Cancer recurrence
- Sexual function (such as low sexual desire/libido, impotence or erectile dysfunction, change in penis length, loss of fertility, need to use condom regularly)
- Survival
- Out-of-pocket expenses (such as co-pays, transportation, travel, parking, and meals)

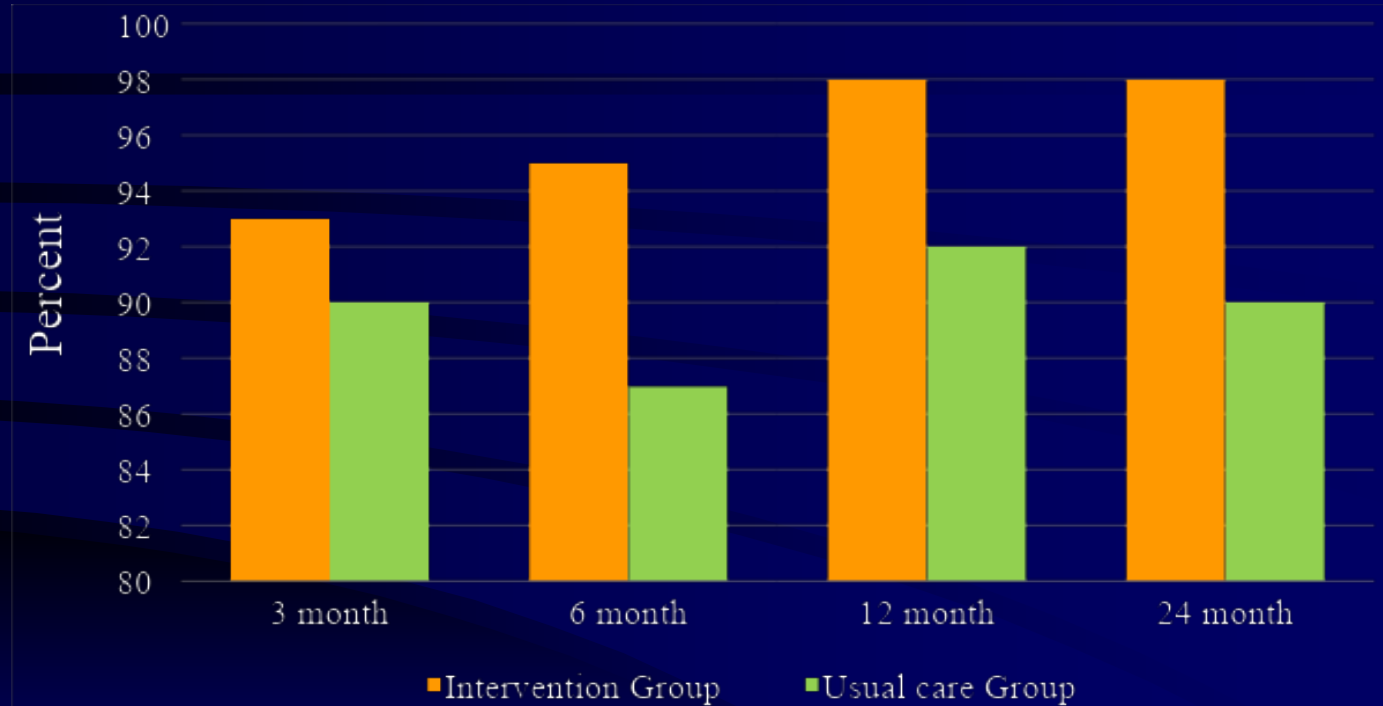
If you have questions about this decision aid, please call: 215-898-3798 or 215-573-2049 (Monday to Friday between 9 am to 4 pm), or 610-772-4070 at other times; email: kimberly.colegrove@uphs.upenn.edu

General satisfaction with care over time For Shared Decision Making & Usual Care



Satisfaction with care scores range from 1 to 5. Higher score indicates greater satisfaction with care. Positive change indicates higher satisfaction with care compared to baseline values.

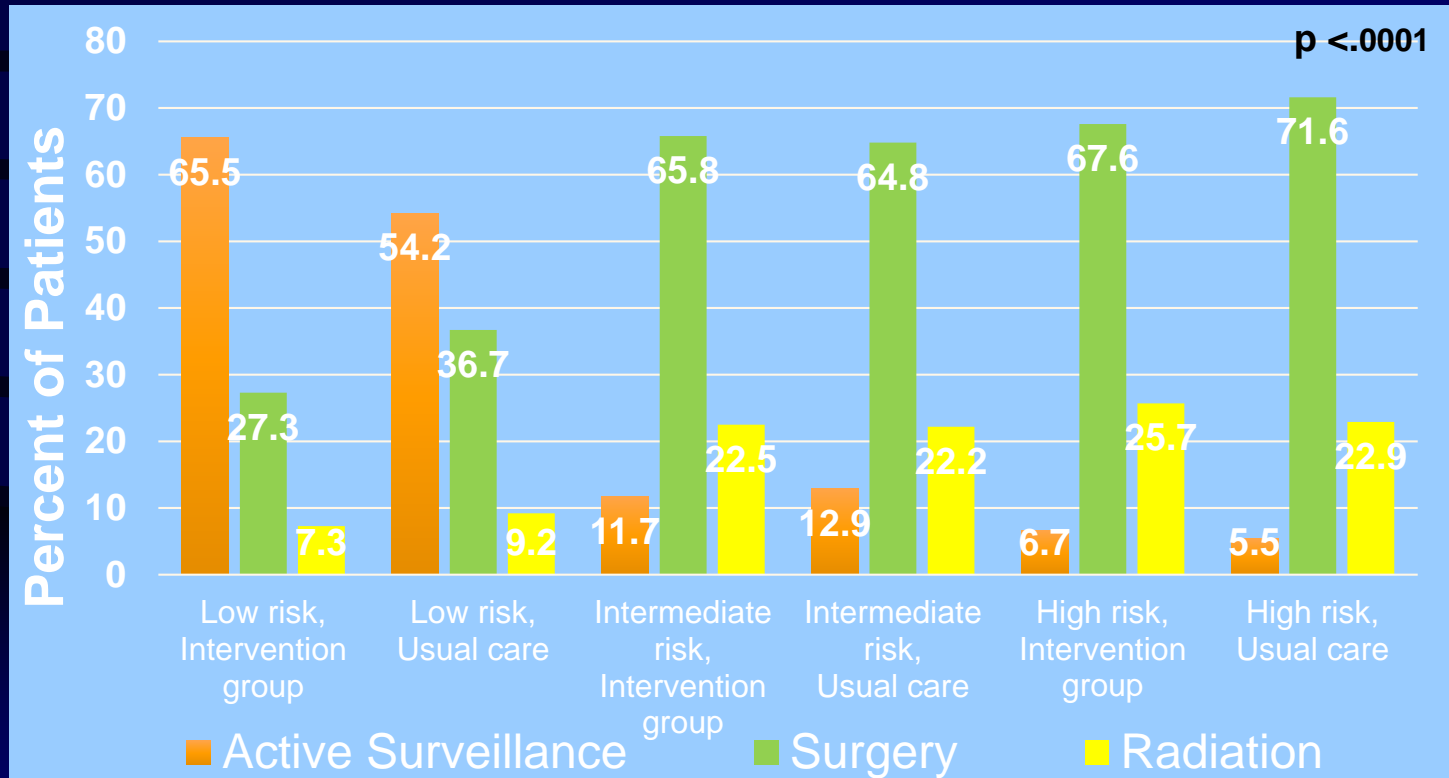
Proportion agreeing 'I am satisfied with my decision' , by intervention status



Source: Jayadevappa et al., JCO 2019

Each item of the Satisfaction with Decision instrument is scored on a Likert scale (ranging from 1=strongly agree to 5=strongly disagree). Strongly agree and agree are combined and reported as 'satisfied'.

Overall comparison of proportion with different treatment choice across PreProCare intervention status, stratified by prostate cancer risk group. N=674



Conclusions

- In this first-of-its kind, large multicenter randomized controlled trial, our PreProCare tool improved 24-months satisfaction with care, satisfaction with decision, and reduced regrets.
- Preference assessment is a key component of patient-centered care and is feasible among localized prostate cancer patients.
- This PreProCare-Shared Decision Making Tool helped patients make treatment choices align with their values.

Policy and Practice Implications

- Preference assessment can help patients reveal their preferences, leading them to feel better about their treatment decision and medical care.
- Future research should identify strategies to ensure diagnosis and treatment options are communicated to patients accurately.

Thank You!



A Public Private Partnership between the ASN and the FDA

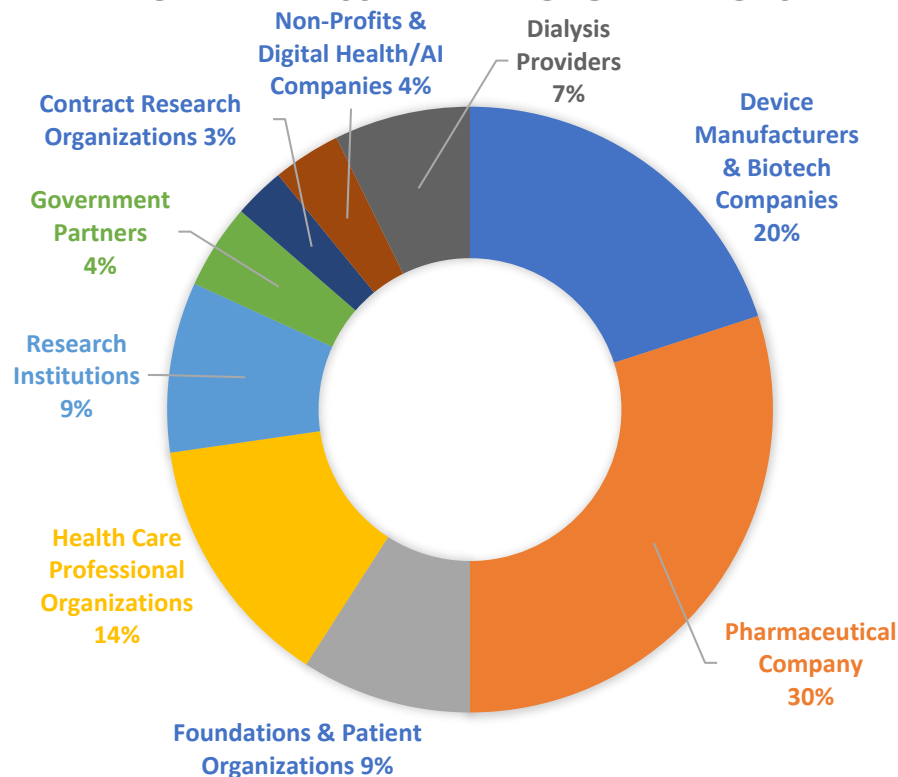
Established September 2012

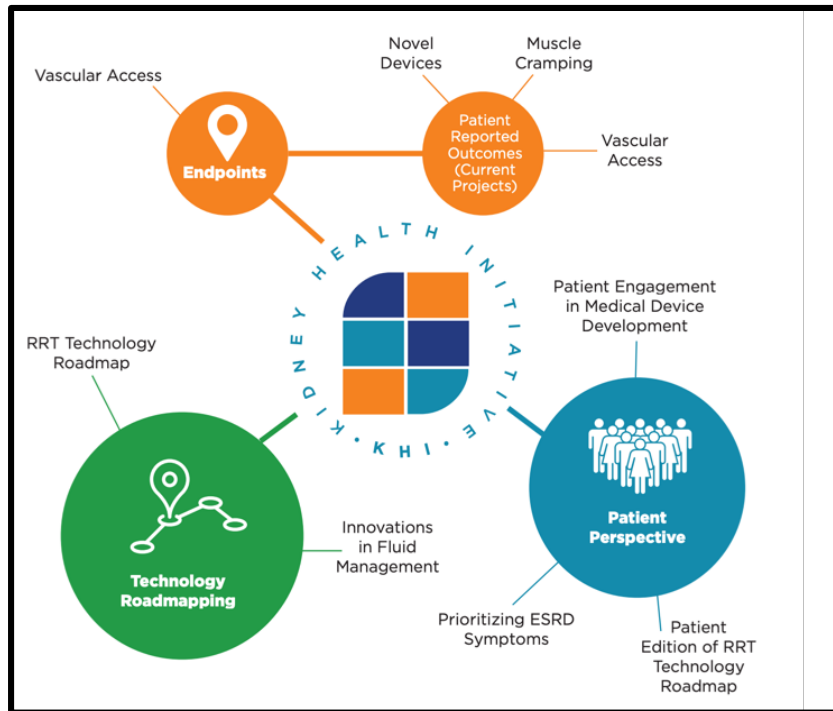
Mission

To catalyze innovation and the development of safe and effective patient-centered therapies for people living with kidney diseases.

KIDNEY HEALTH INITIATIVE

MORE THAN 100 MEMBER ORGANIZATIONS





Building Capacity to Incorporate Patient Preferences into the Development of Innovative Alternatives to Renal Replacement Therapy (RRT)

Overall objective: Develop a sustainable strategy for collecting patient preference information from a representative sample of dialysis patients to drive patient-centered innovation in kidney replacement therapy (KRT) devices

Louis Jacques MD - Disclosures

As an ADVI partner I have consulting relationships with a large number of medical device, biopharmaceutical, diagnostic, and other healthcare related companies. I have no conflicts of interest related to the material to be discussed in this presentation.

Do payers care about patient preference?

- It depends
 - Can it be determined adequately for policymaking purposes?
 - How can we account for preference heterogeneity?
 - How well can it be integrated into clinical trial designs to inform primary prespecified outcomes?
 - How is preference determined and assessed for infants, young children, and cognitively challenged persons?
 - What are the cross-cultural challenges to implementing patient preference?
 - Are PROs inherently necessary to measure patient preference outcomes?
 - How much will it cost to do it?
 - How will it impact enrollment and premiums in a commercial health plan?

CMS cares about patient preference

- CMS has repeatedly endorsed shared decision making (SDM) in National Coverage Determinations for Medicare.
- Different than traditional informed consent – start with the patient's goals and values.
- The application process for Medicare coverage of IDE trials is an opportunity to discuss how you might incorporate patient preference data into your choice of prespecified outcomes.

<https://www.cms.gov/Medicare/Coverage/IDE>

SDM Discussions in Medicare Coverage Memoranda

- Acupuncture
- Cardiac pacemakers
- CAR-T
- Gender reassignment surgery
- ICDs
- Intensive behavioral tx for CVD
- Leadless pacemakers
- NGS testing in cancer
- Percutaneous LAAC
- CRC screening
- LDCT screening for lung CA
- TAVR
- TMVR (TEER)
- VADs

Assessment Challenges

- If patients prioritize feeling energized, is hemoglobin (Hb) an adequate (and complete) surrogate for energy level? [No]
- In health care, metrics that are easily measured tend to get measured, whether or not their manipulation improves patient outcomes. They may even incent inappropriate care.
- Lab results are easy to collect and summarize statistically, asking patients open ended questions is much harder.
- How can we balance counter-preferences in an imperfect world? I don't want to have a stroke from my AF but I don't want to bleed out from my anticoagulant either.

Summary

- Rigorous assessment of PP is challenging.
- Translating a preference into a robustly measurable outcome is vulnerable to bias.
- Implementing PP based health plan policies is probably more challenging, given respect for heterogeneity.
- Medicare likes outcome data that reflect the beneficiary's reasonable priorities and experience of illness and response to therapy. These might include QOL, measures of independent function, specific AE risks etc.
- PP based PROs are often more useful and persuasive than traditional regulatory trial outcomes, e.g. procedural success at 30 days.
- PROs are an obvious way to collect these data, but there are challenges to the rigorous collection and interpretation of PROs.

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Speaker Roundtable Discussion

Q&A

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- 2. Click on the thought bubble icon in the webcast window**

