Housekeeping Rules

Attendees will be entered into the webcast on mute and will remain muted at all times.

Use the Chat Box on the webcast or the live Q/A feature on the conference app to share a question or comment and to communicate with presenters and moderators during the Summit.

If you have technical issues, please seek assistance from your local technical support.
Implementation/Process of Obtaining and Using PPI – the ‘Beyond’
SESSION 4

Virtual ISPOR-FDA Summit 2020
MDIC Patient Preference in Heart Failure
Interest Statement

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

Device Design

Reimbursement

Trial Design

Marketing

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

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Acknowledgement: This work was supported by the Patient Centered Outcomes Research Institute-CE-12-11-4973
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?
Randomized Controlled Study

Research Design

Prostate Cancer Diagnosis
Obtain Consent & Enrollment

Prior to Treatment

Baseline Assessment

Treatment phase

Controls (360 patients)

PreProCare-Decision Making Tool (360 patients)

Outcomes (720 patients)
PreProCare Instrument

Preference Assessment Decision Aid

Welcome to
My Prostate Cancer, My Choice
Part I: Attributes selection

- Suppose your 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?

<table>
<thead>
<tr>
<th>Not Important</th>
<th>Somewhat Important</th>
<th>Very Important</th>
<th>Extremely Important</th>
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Part II – Choice Scenarios

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

<table>
<thead>
<tr>
<th>Treatment A</th>
<th>Treatment B</th>
</tr>
</thead>
<tbody>
<tr>
<td>- More than half (60%) may experience urinary function problems in the short-term &amp; Less than half (45%) may experience urinary function problems in the long-term</td>
<td></td>
</tr>
<tr>
<td>- Some (20%) may experience urinary function problems in the short-term &amp; Very few (10%) may experience urinary function problems in the long-term</td>
<td></td>
</tr>
<tr>
<td>- Some (20%) may experience psychological distress</td>
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<tr>
<td>- Very few (10%) may experience psychological distress</td>
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</tbody>
</table>

Part III-Final Five Attributes

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

If you have questions about this decision aid, please call: 215-698-3798 or 215-573-3040 (Monday to Friday between 9 am to 4 pm), or 610-772-4070 at other times. email: kimberly.coeproe@upenn.edu

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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.

Source: Jayadevappa et al., JCO 2019
Proportion agreeing 'I am satisfied with my decision', by intervention status

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’.

Source: Jayadevappa et al., JCO 2019
Overall comparison of proportion with different treatment choice across PreProCare intervention status, stratified by prostate cancer risk group. N=674

Source: Jayadevappa et al., JCO 2019
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.
_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.
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.
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.
Louis B. Jacques, MD
SVP & Chief Clinical Officer, ADVI

Louis.Jacques@ADVI.COM
Speaker Roundtable Discussion

Q&A

To ask a question, either:
1. Use the live Q&A feature in the app
2. Click on the thought bubble icon in the webcast window