“Whole Health” Value Assessment: Universal Survey Framework for Integrating Patient Experience Data in Health Technology Assessment

Kimberly Westrich, Chief Strategy Officer, NPC

National Pharmaceutical Council®
70 YEARS

Patient Insights Should Guide Research and Decisions (IAD)

Definition:
Patient Engagement in Research

The active, meaningful, and collaborative **interaction**

+

between patients and researchers across **all stages** of the research process

+

where research **decision making is guided by patients' contributions** as partners

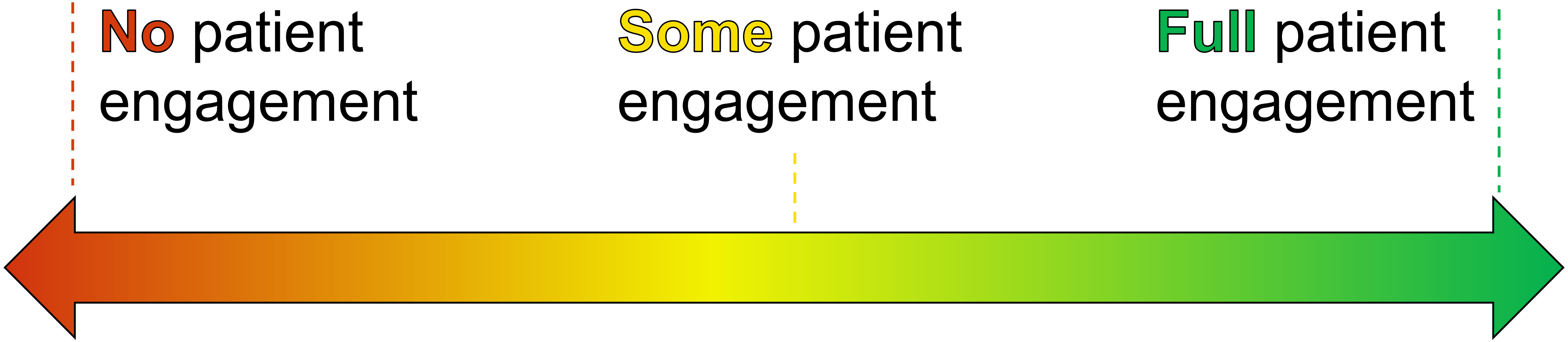
+

recognizing their specific **experiences, values, and expertise**.

Harrington, Rachel L. et al. Defining Patient Engagement in Research: Results of a Systematic Review and Analysis: Report of the ISPOR Patient-Centered Special Interest Group. *Value in Health*, Volume 23, Issue 6, 677 - 688

Integrating the Patient Experience Into Healthcare Decisions Occurs Along a Continuum

Don’t let perfect be the enemy of good; some engagement is better than no engagement



No patient engagement

Some patient engagement

Full patient engagement

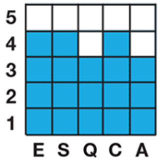
Patient Experience Can Inform a Range of Decision-Making Contexts

- Patient-Focused Drug Development
- Formulary
- Value Assessment
- Care Management
- Value-Based Contracting
- IRA Medicare Drug Price Negotiation

A Spectrum of US Value Assessment Approaches

Patient engagement intensity varies across US value assessment approaches

NCCN EVIDENCE BLOCKS CATEGORIES AND DEFINITIONS



E = Efficacy of Regimen/Agent
S = Safety of Regimen/Agent
Q = Quality of Evidence
C = Consistency of Evidence
A = Affordability of Regimen/Agent

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ICER
INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW

IVI
INNOVATION AND VALUE INITIATIVE

ICER's Approach to Patient Engagement Has Evolved Over Time

2014

No patient engagement program

2017

Informal Approach to Patient Engagement

- Following release of 2017 VAF, ICER revised its **Patient Engagement Guide**
- **Informal outreach** to patients and patient groups "as appropriate to learn about the patient perspective"
- **Informal policy** of including patient representatives in the public meeting and policy roundtable

2020

ICER Implements Formal Patient Engagement Program

- ICER engages select patient groups **prior to topic announcement**
- Potential to include patients in **key informant interviews**
- **Patient input questionnaire**
- **Dedicated report chapter** on patient experience
- Inclusion of a patient representative as an **expert reviewer**
- Inclusion of patient representatives in the **public meeting and policy roundtable**
- Potential to **debrief with key patient organizations** following the public meeting

2023

ICER Expands Patient Engagement Program

- Revised "**share your story**" form
- **Formalized small-group patient and caregiver discussions** after the scoping phase
- **Compensate patient representatives** for their time
- Creation of a **Patient Council**
- Publish "**ICER Snapshot for Patients**" resources

ICER's Patient Engagement Program: Opportunities For Improvement

Transparency

Meaningful integration of patient voice

Process improvements

Advancing “Whole Health” Value Assessment

Kayleigh Majercak, MS

Joe Vandigo, MBA, PhD

Brett McQueen, PhD

Mitigating Evidence Gaps: A Survey Template to Inform Patient-Centered Value Assessment

Kayleigh Majercak, PhD, MS
PhD Student
University of Maryland Baltimore, School of Pharmacy

ISPOR Annual
May 7, 2024
1:45 PM - 2:45 PM ET

PUTTING PATIENTS FIRST
NATIONAL HEALTH COUNCIL

UNIVERSITY of MARYLAND BALTIMORE

Acknowledgements

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Committee:

- C. Daniel Mullins, PhD (Chair)
- Roxanne Jensen, PhD
- Eleanor Perfetto, PhD, MS
- Julia Slejko, PhD
- Bari Talente, JD

Patients & Patient Organization Partners:

aafa Asthma and Allergy Foundation of America

F!GHT COLORECTAL CANCER

MS National Multiple Sclerosis Society

National Health Council (NHC):

- Randall Rutta, MA, CEO
- Patient-centered Value Assessment Advisory Committee
- Value Initiative Advisory Board
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- Omar Escontrías
- Silke Schoch

Other contributors:

- Laurie Burke
- Stacie Hudgens
- Maggie Jalowsky
- Joey Mattingly
- Elisabeth Oehrlein
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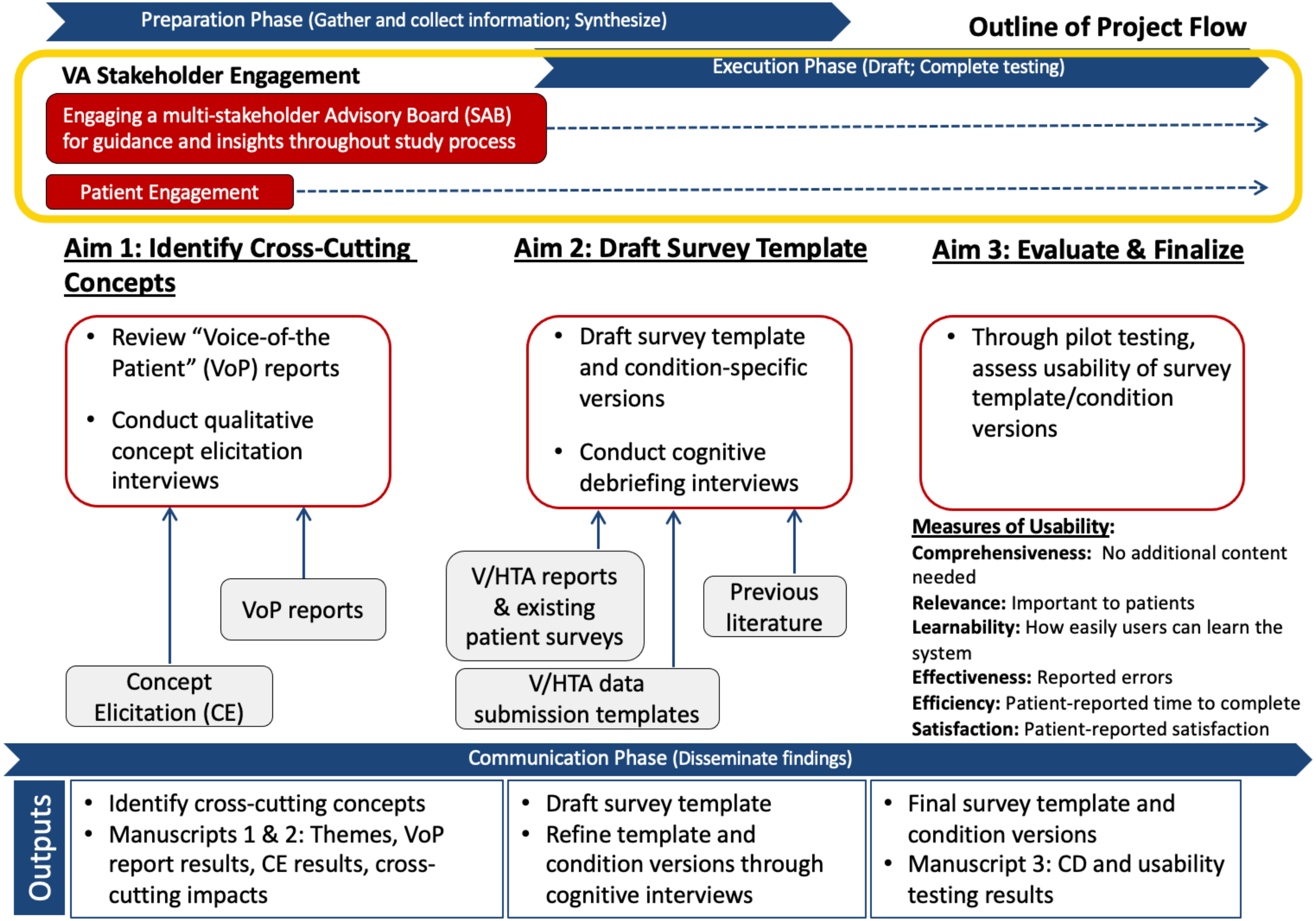
Study Objective

- Using a mixed-methods approach, identify patient experience data (PED) concepts common across conditions (i.e., cross-cutting PED concepts) to co-develop a disease-agnostic survey template that informs a standardized approach to fill patient-centered evidence gaps in value/health technology assessment.

Outline of Project Flow



Preparation Phase (Gather and collect information; Synthesize)

Execution Phase (Draft; Complete testing)

VA Stakeholder Engagement

Engaging a multi-stakeholder Advisory Board (SAB) for guidance and insights throughout study process

Patient Engagement

Aim 1: Identify Cross-Cutting Concepts

- Review "Voice-of-the Patient" (VoP) reports
- Conduct qualitative concept elicitation interviews

Concept Elicitation (CE)

VoP reports

Aim 2: Draft Survey Template

- Draft survey template and condition-specific versions
- Conduct cognitive debriefing interviews

V/HTA reports & existing patient surveys

V/HTA data submission templates

Previous literature

Aim 3: Evaluate & Finalize

- Through pilot testing, assess usability of survey template/condition versions

Measures of Usability:
Comprehensiveness: No additional content needed
Relevance: Important to patients
Learnability: How easily users can learn the system
Effectiveness: Reported errors
Efficiency: Patient-reported time to complete
Satisfaction: Patient-reported satisfaction

Communication Phase (Disseminate findings)

Outputs		
• Identify cross-cutting concepts • Manuscripts 1 & 2: Themes, VoP report results, CE results, cross-cutting impacts	• Draft survey template • Refine template and condition versions through cognitive interviews	• Final survey template and condition versions • Manuscript 3: CD and usability testing results

CD: Cognitive Debriefing, V/HTA: Value/Health Technology Assessment, VoP: Voice of the Patient

Three Conditions for Development and Testing

Asthma

Asthma and Allergy Foundation of America

Colorectal Cancer

F!GHT COLORECTAL CANCER

Multiple Sclerosis

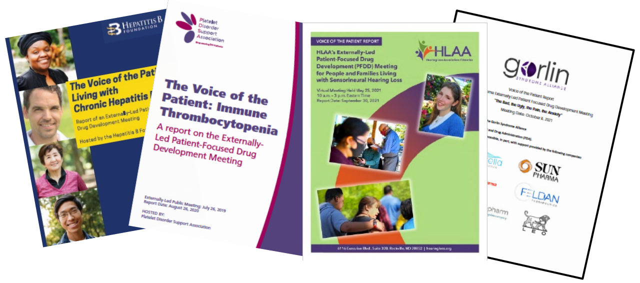
National Multiple Sclerosis Society

- Supported by patient group partners
  - AAFA
  - FightCRC
  - NMSS
- Recruitment targets for population characteristics
  - Concept elicitation
  - Cognitive interviewing
  - Usability testing

How Were the PED Cross-cutting Concepts Identified?

"Voice-of-the-Patient" Reports (n=15)

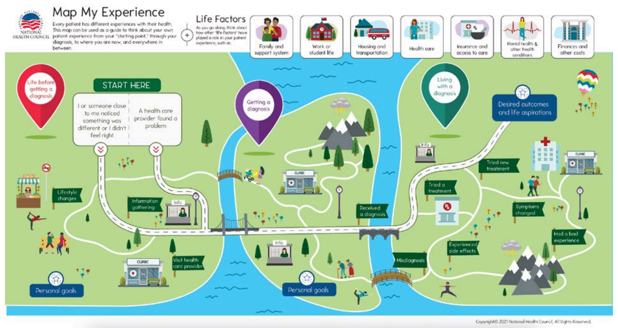
- Randomly selected reports to obtain a balance of rare (n=8) and prevalent (n=7) conditions based on a 200,000/population threshold



Concept elicitation interviews with patients

(n=24)

- Generate list of impacts reported across conditions while noting condition-specific impacts



List of commonly reported impacts across conditions

- List created using a 70% threshold or retained if reported as most bothersome for further evaluation

Follow-up meetings with subset of patients

(n=12)

- Narrowed down the list to the most important impacts & understanding the aspect(s) of the impact that makes it important (concepts)

PED: Patient Experience Data

For Patients, With Patients: Development and Testing

Drafting the survey template

- Adopted/adapted survey items from existing surveys
- Developed new items
- Revisions based on stakeholder advisory board, patient advisors, and expert input

Cognitive debriefing with patients

(n=20)

- Preference for format (matrix vs single-item)
- Phrasing (words used by patients)
- Appropriate response options (options resonate/missing options)

Usability testing with patients

(n=16)

- Additional revisions (examples/items/options, formatting)
- Time to complete
- Usability metrics

Finalized survey template and condition-specific version

- Final survey template: 31 items (+ sub-items)
- Three examples of how survey tailored for asthma, CRC, and MS

Survey Template Item Content

- Symptoms
- Impact of symptoms on daily functioning
- Treatment and treatment-related experiences
- Ability to work
- Wellness
- Financial impact
- Healthcare utilization
- Condition stability
- Treatment preferences
- Healthcare and provider experiences
- Access to healthcare services and treatment
- Experiences living with the condition
- Impact on life
- Support from others
- Impact on others
- Additional patient insights

Survey Template Item-Response Scales

- **Severity responses:**
  None / Mild / Moderate / Severe / Very Severe

- **Difficulty responses:**
  No difficulty / Mild difficulty / Moderate difficulty / Severe difficulty / Very severe difficulty / Unable to do*

- **Interference responses:**
  None / Less than a day / 1 – 2 days / 3 – 4 days / 5 – 6 days / 7 days

- **Level of agreement responses:**
  Does not apply / Strongly disagree / Disagree / Neutral / Agree / Strongly Agree

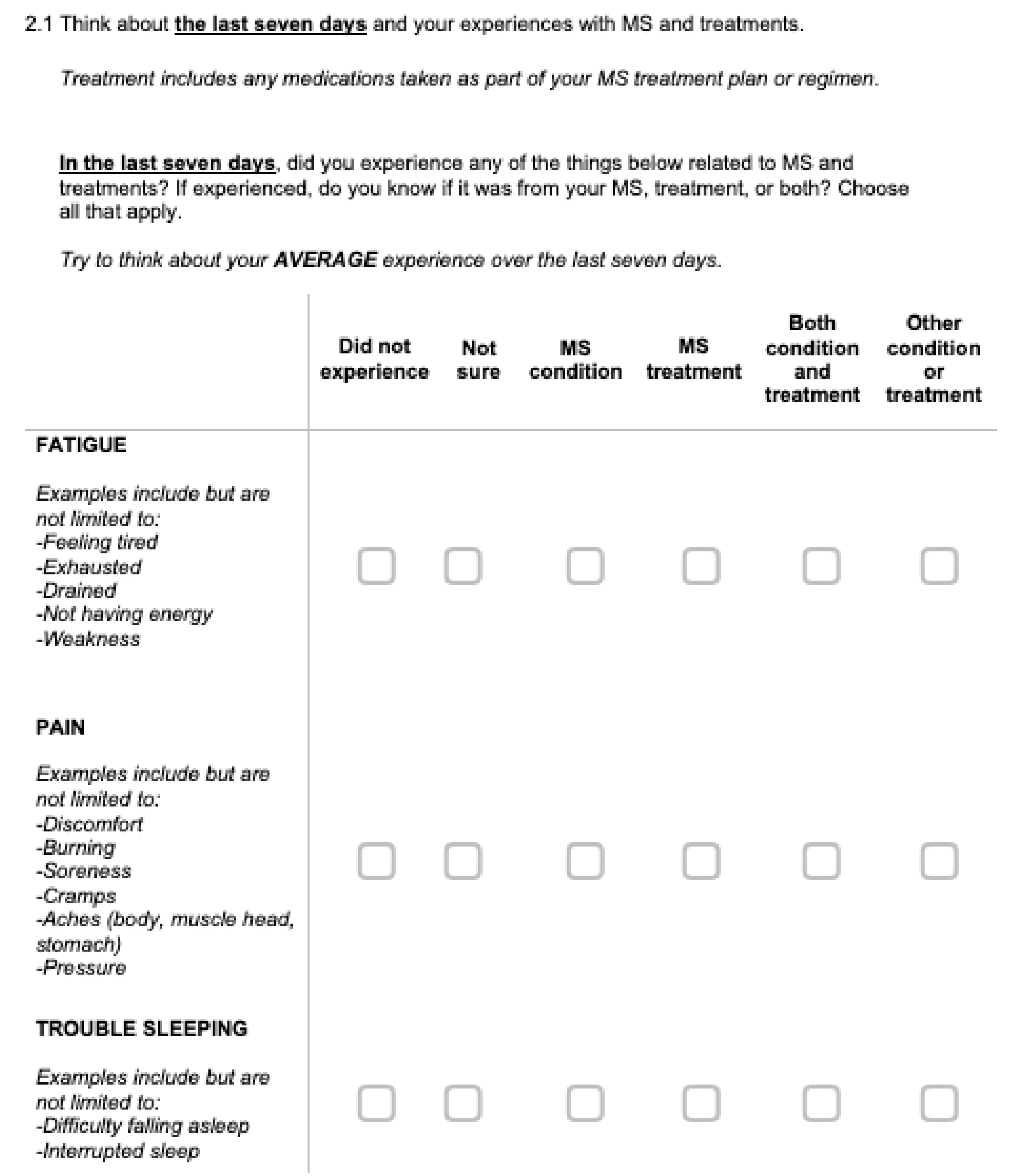
- **Other items response scales:** Based on specific patient experiences

*MS and CRC only

How Patient Engagement Shaped the Survey Template

CASE EXAMPLES

Case Example #1:
Matrix vs Single-item Formatting



2.1 Think about **the last seven days** and your experiences with MS and treatments.

Treatment includes any medications taken as part of your MS treatment plan or regimen.

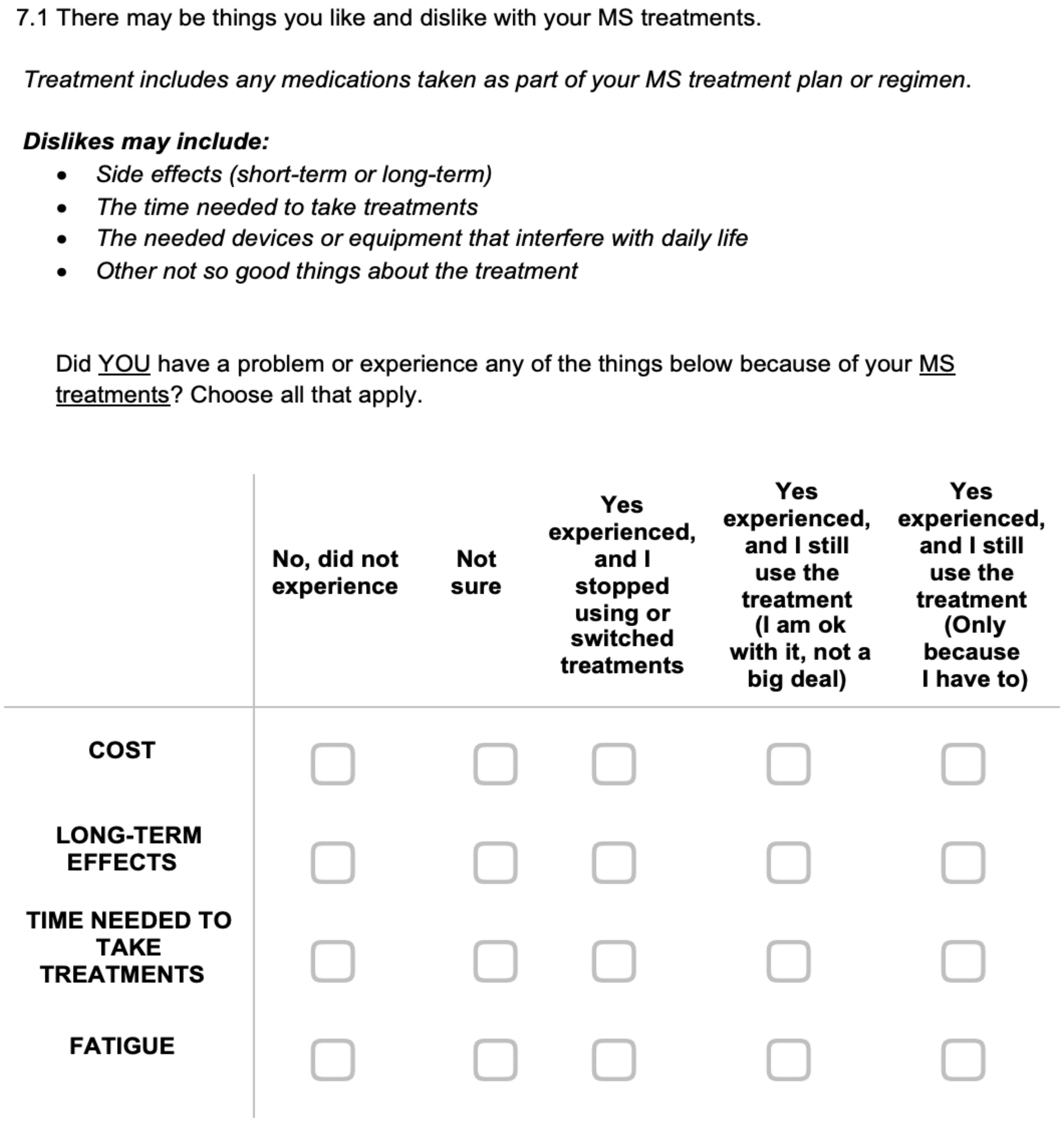
In the last seven days, did you experience any of the things below related to MS and treatments? If experienced, do you know if it was from your MS, treatment, or both? Choose all that apply.

*Try to think about your **AVERAGE** experience over the last seven days.*

	Did not experience	Not sure	MS condition	MS treatment	Both condition and treatment	Other condition or treatment
FATIGUE *Examples include but are not limited to:* *-Feeling tired* *-Exhausted* *-Drained* *-Not having energy* *-Weakness*	☐	☐	☐	☐	☐	☐
PAIN *Examples include but are not limited to:* *-Discomfort* *-Burning* *-Soreness* *-Cramps* *-Aches (body, muscle head, stomach)* *-Pressure*	☐	☐	☐	☐	☐	☐
TROUBLE SLEEPING *Examples include but are not limited to:* *-Difficulty falling asleep* *-Interrupted sleep*	☐	☐	☐	☐	☐	☐

- Preference for the matrix-style format
- Easier to respond
- Able to see the full list of items

Case Example #2: Phrasing and Response Options



7.1 There may be things you like and dislike with your MS treatments.

Treatment includes any medications taken as part of your MS treatment plan or regimen.

Dislikes may include:

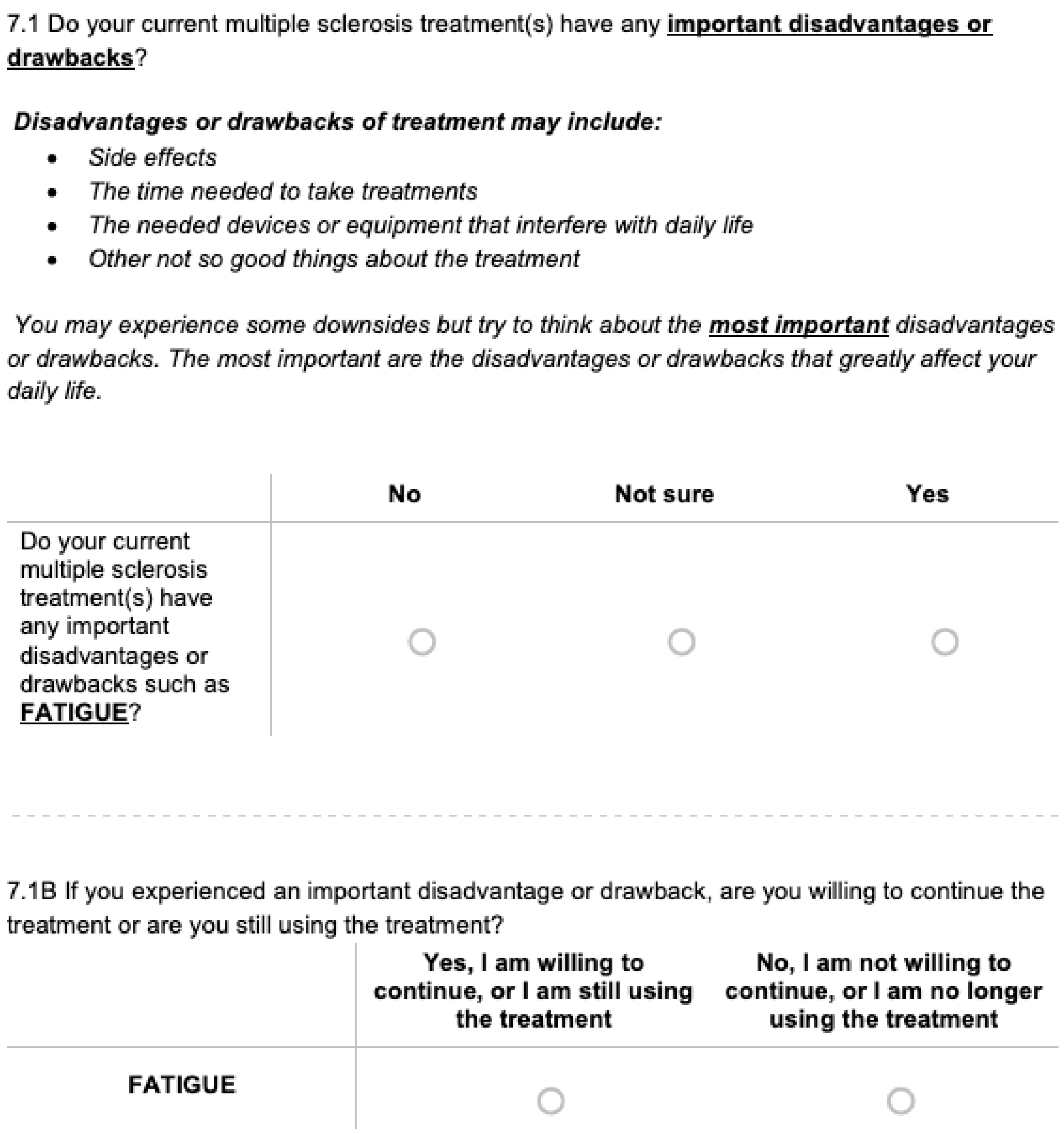
- *Side effects (short-term or long-term)*
- *The time needed to take treatments*
- *The needed devices or equipment that interfere with daily life*
- *Other not so good things about the treatment*

Did YOU have a problem or experience any of the things below because of your MS treatments? Choose all that apply.

	No, did not experience	Not sure	Yes experienced, and I stopped using or switched treatments	Yes experienced, and I still use the treatment (I am ok with it, not a big deal)	Yes experienced, and I still use the treatment (Only because I have to)
COST	☐	☐	☐	☐	☐
LONG-TERM EFFECTS	☐	☐	☐	☐	☐
TIME NEEDED TO TAKE TREATMENTS	☐	☐	☐	☐	☐
FATIGUE	☐	☐	☐	☐	☐

- Combined two questions
- Avoid formal language, just ask the question or state the response
  - Draft felt *mechanical*
- Distinguish between using the treatment
  - *Ok, not a big deal*
  - *Only because I have to*
- Rearranging order of items, i.e., cost
  - Confirmed spontaneously by participant after modification

Draft



7.1 Do your current multiple sclerosis treatment(s) have any **important disadvantages or drawbacks**?

Disadvantages or drawbacks of treatment may include:

- *Side effects*
- *The time needed to take treatments*
- *The needed devices or equipment that interfere with daily life*
- *Other not so good things about the treatment*

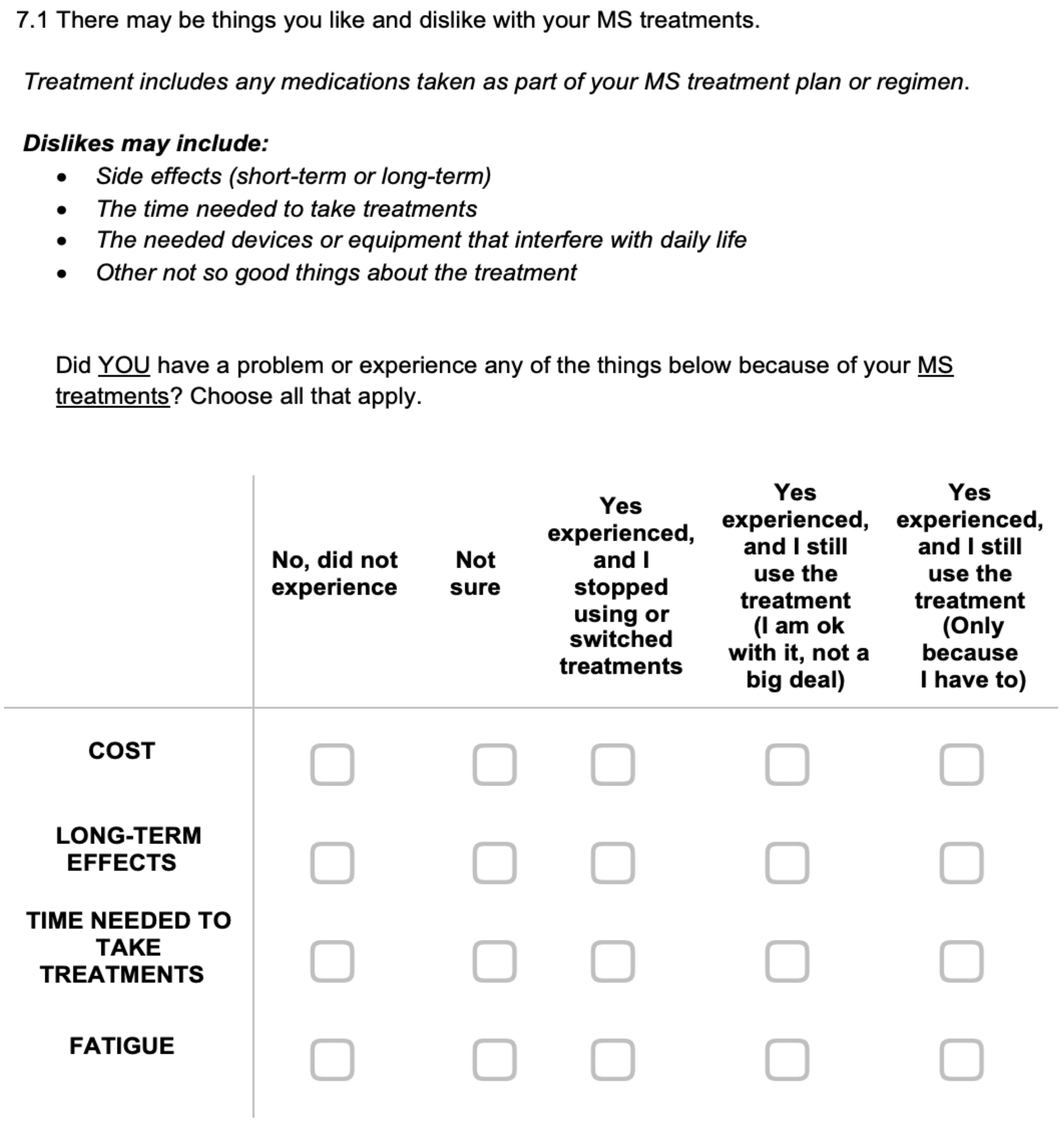
*You may experience some downsides but try to think about the **most important** disadvantages or drawbacks. The most important are the disadvantages or drawbacks that greatly affect your daily life.*

	No	**Not sure**	**Yes**
Do your current multiple sclerosis treatment(s) have any important disadvantages or drawbacks such as **FATIGUE**?	○	○	○

7.1B If you experienced an important disadvantage or drawback, are you willing to continue the treatment or are you still using the treatment?

	Yes, I am willing to continue, or I am still using the treatment	**No, I am not willing to continue, or I am no longer using the treatment**
FATIGUE	○	○

Final



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Dislikes may include:

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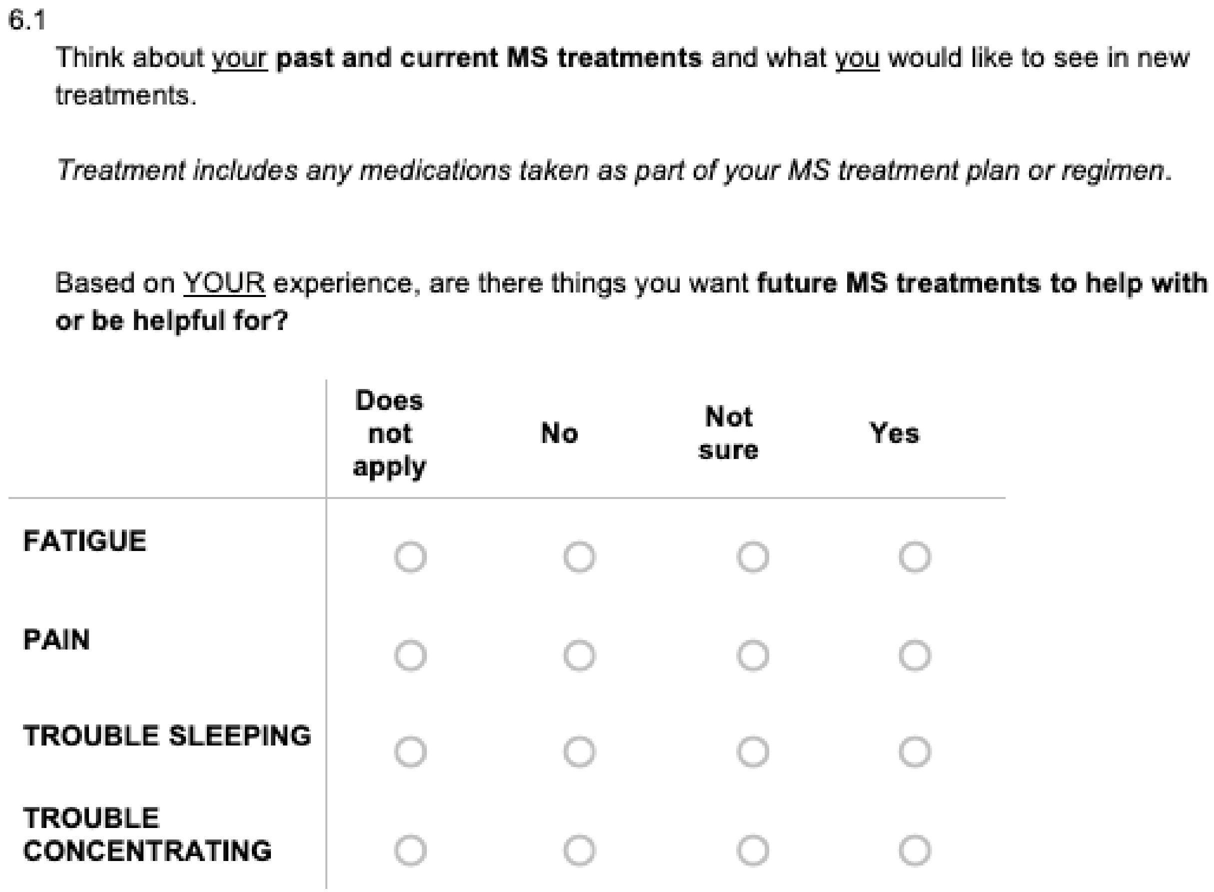
- *The time needed to take treatments*
- *The needed devices or equipment that interfere with daily life*
- *Other not so good things about the treatment*

Did YOU have a problem or experience any of the things below because of your MS treatments? Choose all that apply.

	No, did not experience	**Not sure**	**Yes experienced, and I stopped using or switched treatments**	**Yes experienced, and I still use the treatment (I am ok with it, not a big deal)**	**Yes experienced, and I still use the treatment (Only because I have to)**
COST	☐	☐	☐	☐	☐
LONG-TERM EFFECTS	☐	☐	☐	☐	☐
TIME NEEDED TO TAKE TREATMENTS	☐	☐	☐	☐	☐
FATIGUE	☐	☐	☐	☐	☐

Case Example #3: “Meaningful treatment benefit” does not resonate with patients



6.1

Think about your **past and current MS treatments** and what you would like to see in new treatments.

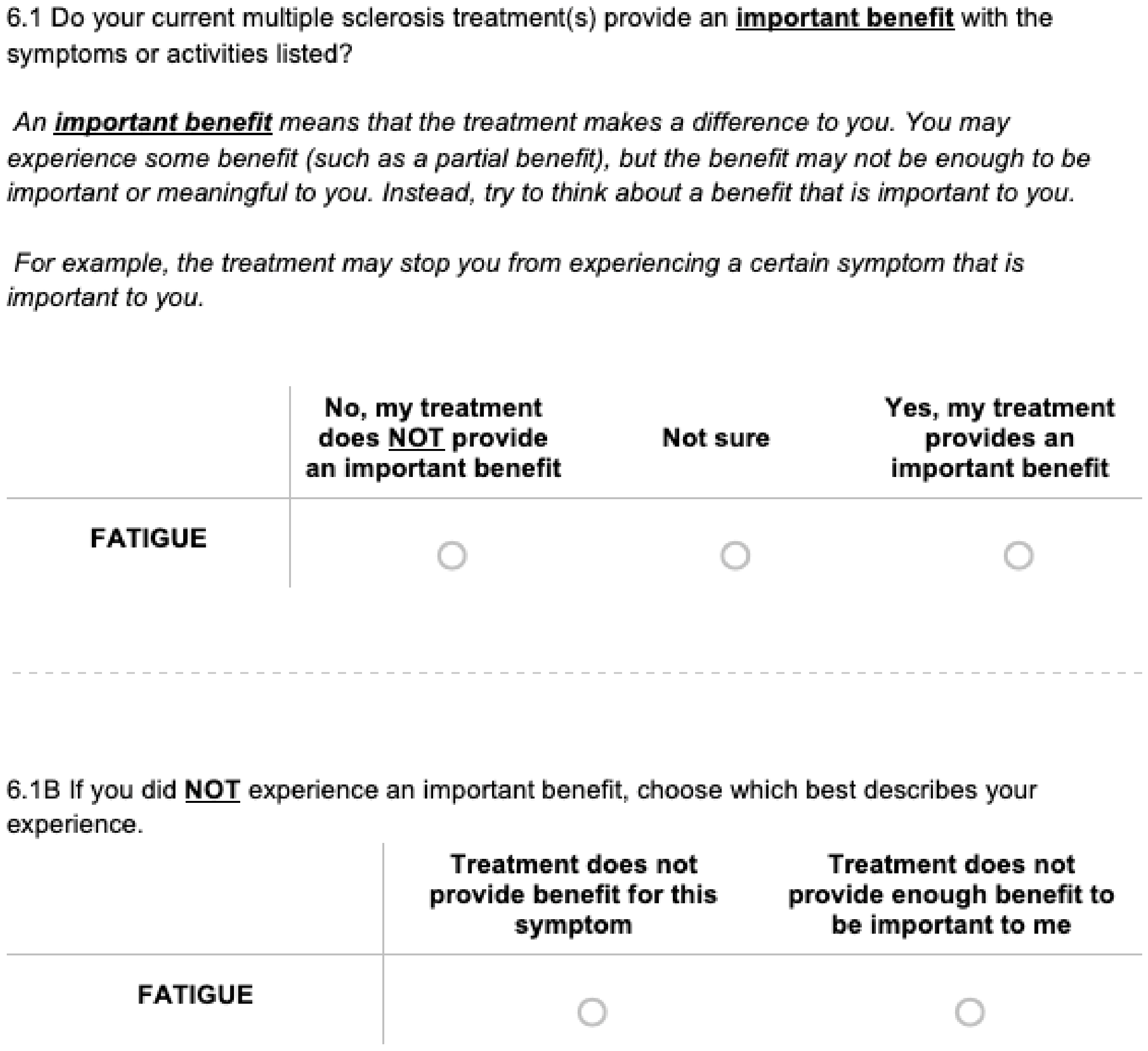
Treatment includes any medications taken as part of your MS treatment plan or regimen.

Based on YOUR experience, are there things you want **future MS treatments to help with or be helpful for?**

	Does not apply	No	Not sure	Yes
FATIGUE	○	○	○	○
PAIN	○	○	○	○
TROUBLE SLEEPING	○	○	○	○
TROUBLE CONCENTRATING	○	○	○	○

- One of the more challenging items to find the right phrasing
    - *Terms*
        - Meaningful benefit/improvement
        - Important benefit/improvement
        - Significant improvement
        - Improvement that makes a difference
        - Noticeable improvement
    - *Answering question as intended*
- Reverse engineered to go about asking in a different way
    - *Combined two questions*

Draft



6.1 Do your current multiple sclerosis treatment(s) provide an **important benefit** with the symptoms or activities listed?

An ***important benefit*** *means that the treatment makes a difference to you. You may experience some benefit (such as a partial benefit), but the benefit may not be enough to be important or meaningful to you. Instead, try to think about a benefit that is important to you.*

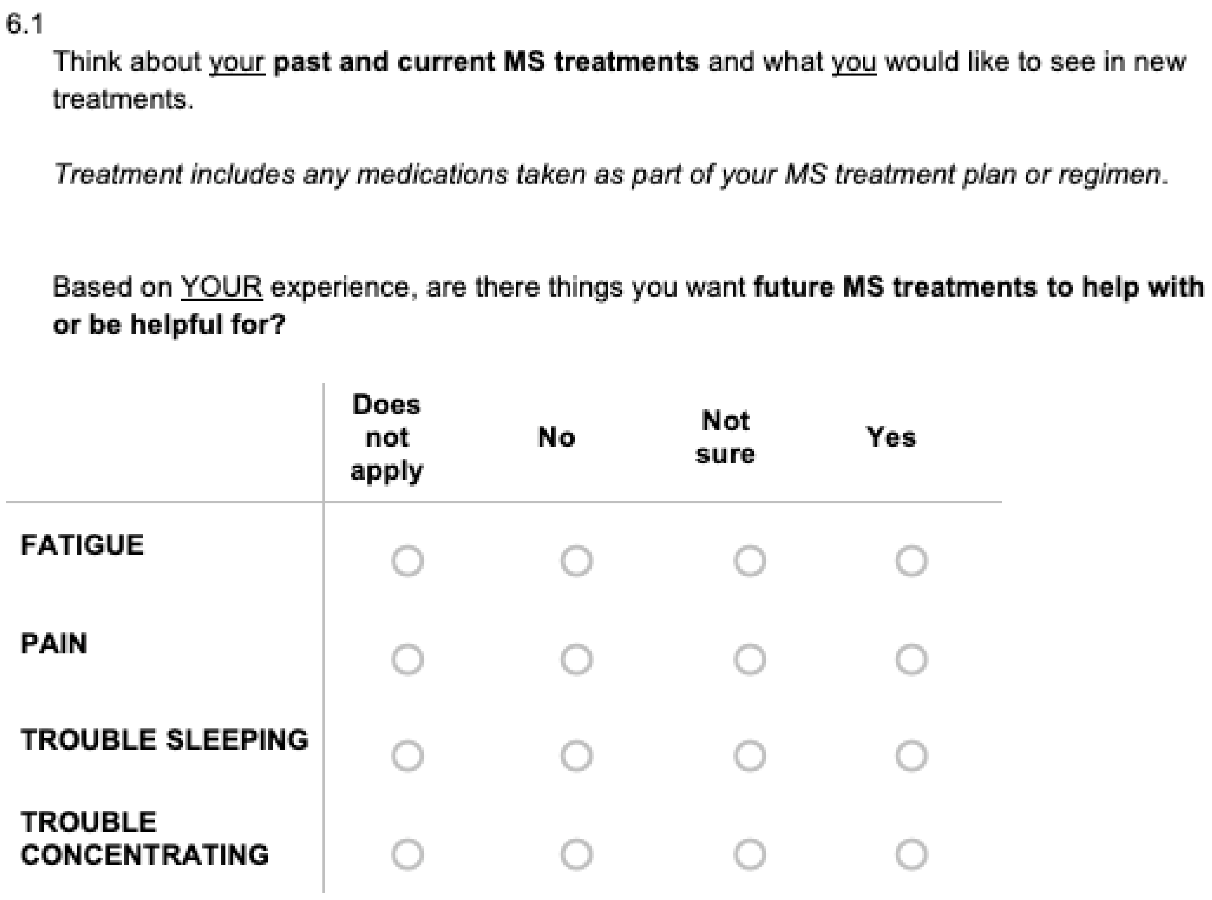
For example, the treatment may stop you from experiencing a certain symptom that is important to you.

	No, my treatment does NOT provide an important benefit	**Not sure**	**Yes, my treatment provides an important benefit**
FATIGUE	○	○	○

6.1B If you did **NOT** experience an important benefit, choose which best describes your experience.

	Treatment does not provide benefit for this symptom	**Treatment does not provide enough benefit to be important to me**
FATIGUE	○	○

Final



6.1

Think about your **past and current MS treatments** and what you would like to see in new treatments.

Treatment includes any medications taken as part of your MS treatment plan or regimen.

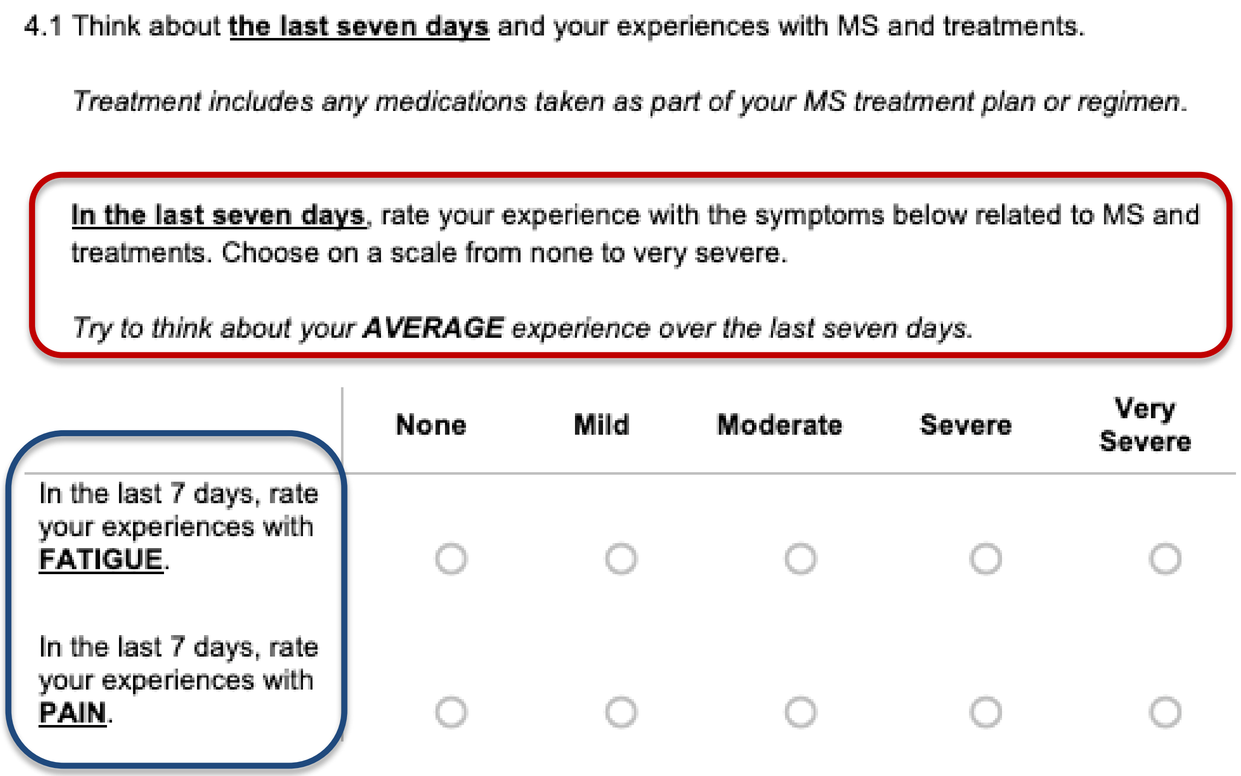
Based on YOUR experience, are there things you want **future MS treatments to help with or be helpful for?**

	Does not apply	**No**	**Not sure**	**Yes**
FATIGUE	○	○	○	○
PAIN	○	○	○	○
TROUBLE SLEEPING	○	○	○	○
TROUBLE CONCENTRATING	○	○	○	○

Differences in Condition-versions of the Survey

- Symptoms and physical activity examples
- Response option anchor of “unable to do” for difficulty item for MS and CRC versions
- Formatting
  - Asthma: Do not repeat question with item, i.e., list only symptom or activity
  - MS: Repeat the question for each item to help remember and focus
- Response options
  - Number of days hospitalized, missed days of work for CRC
- Condition stability item
  - MS: Flares/relapse
  - Asthma: Flares/attacks
  - CRC: remission/NED

MS



4.1 Think about **the last seven days** and your experiences with MS and treatments.

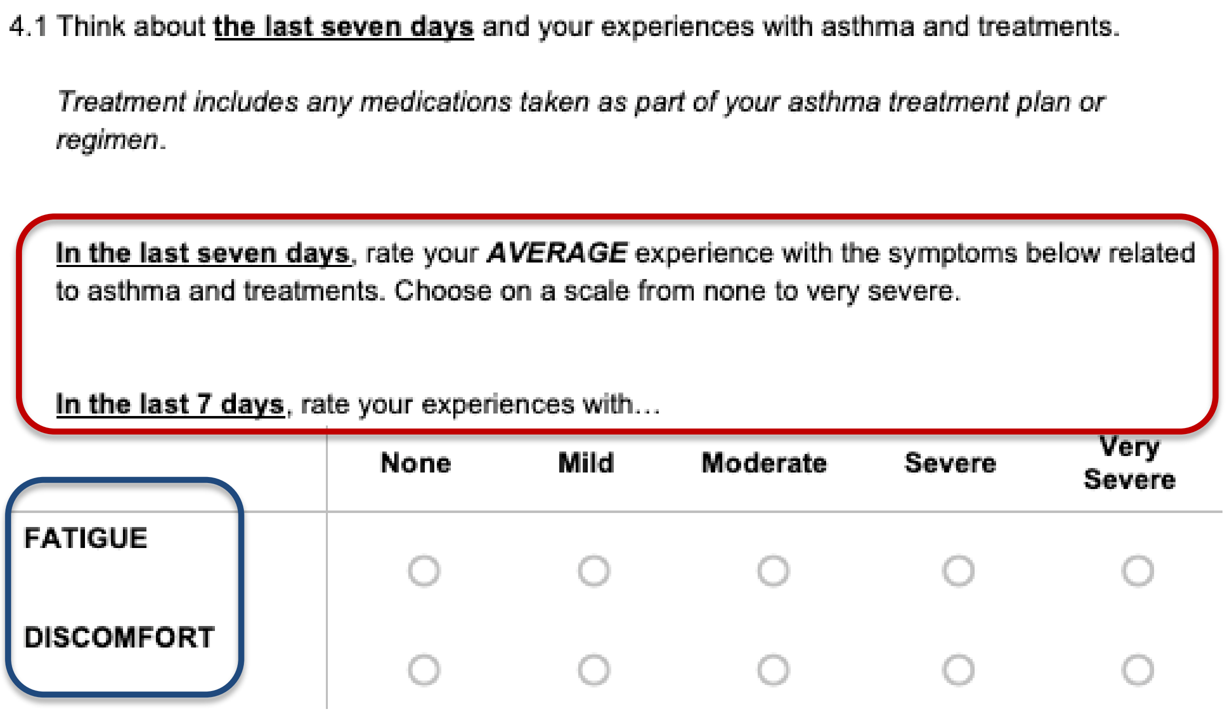
Treatment includes any medications taken as part of your MS treatment plan or regimen.

In the last seven days, rate your experience with the symptoms below related to MS and treatments. Choose on a scale from none to very severe.

Try to think about your ***AVERAGE*** *experience over the last seven days.*

	None	Mild	Moderate	Severe	Very Severe
In the last 7 days, rate your experiences with **FATIGUE**.	○	○	○	○	○
In the last 7 days, rate your experiences with **PAIN**.	○	○	○	○	○

Asthma



4.1 Think about **the last seven days** and your experiences with asthma and treatments.

Treatment includes any medications taken as part of your asthma treatment plan or regimen.

In the last seven days, rate your ***AVERAGE*** experience with the symptoms below related to asthma and treatments. Choose on a scale from none to very severe.

In the last 7 days, rate your experiences with…

	None	Mild	Moderate	Severe	Very Severe
FATIGUE	○	○	○	○	○
DISCOMFORT	○	○	○	○	○

TEMPLATE FOR SUCCESS

HOW A UNIVERSAL SURVEY TEMPLATE CAN CONTRIBUTE TO V/HTA

JOE VANDIGO
PRINCIPAL SCIENTIST & DIRECTOR OF VALUE
APPLIED PATIENT EXPERIENCE

apx

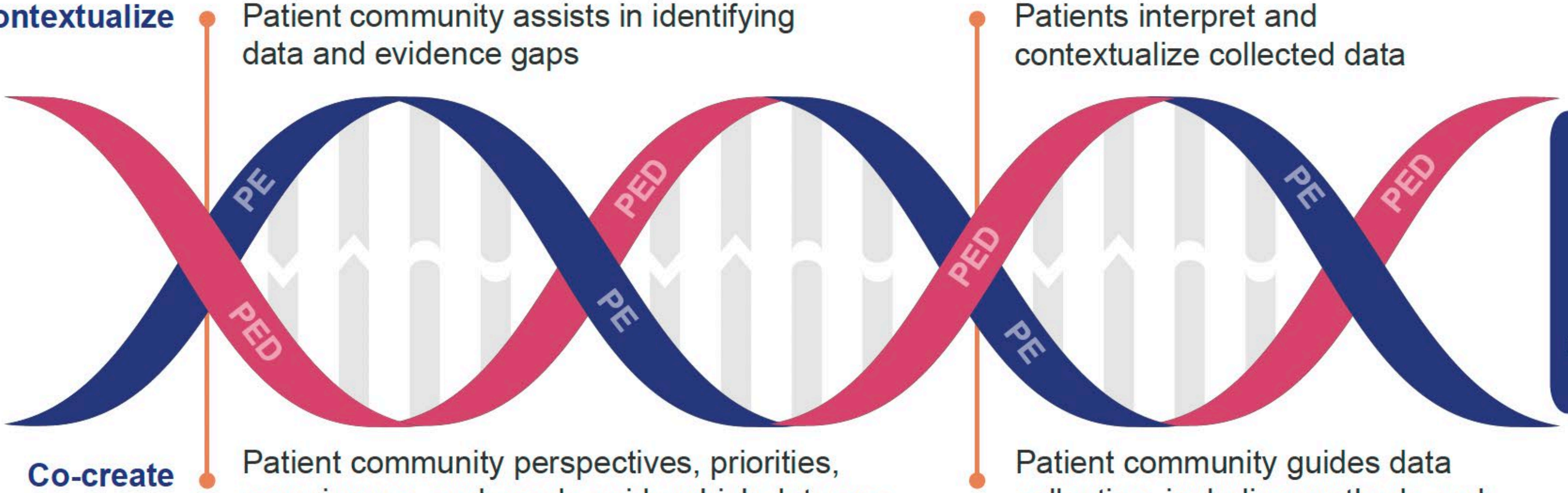
Value (Health Technology) Assessment

Value assessment advises whether a health service (e.g., drug, device, surgery) should be used, and if so, how it is best used in the health care system, and which patients are most likely to benefit from it.

Assessments vary, but most look at the health benefits and risks of using the treatment or technology. They can also look at costs and any other wider impacts it may have on a population or on a society.

Source: National Health Council. The Patient's View of Health Care Value. [Link]

Patient input guides data collection and communication



Contextualize

Patient community assists in identifying data and evidence gaps

Patients interpret and contextualize collected data

Patient Experience Data

Patient Engagement

Co-create

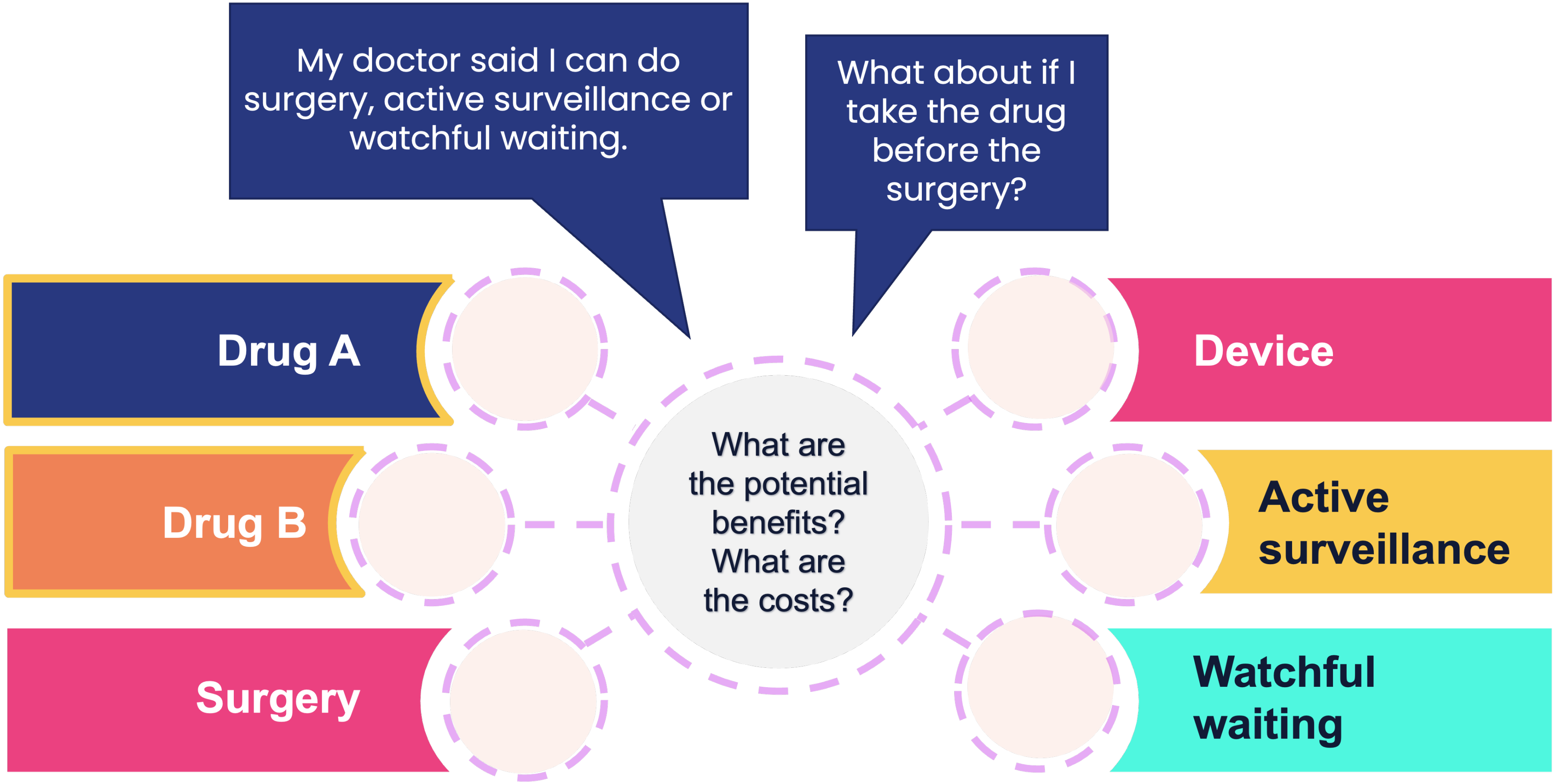
Patient community perspectives, priorities, experiences, and needs guide which data are collected

Patient community guides data collection, including methods and setting

Patient experience data and evidence are communicated to key audiences using language that is understandable, relevant, and timely.

Source: Adapted from PFMD PED Navigator [Link]; figure from AppliedPX/NHC/PFMD Infographic

Learn which comparators are relevant by speaking with patients



My doctor said I can do surgery, active surveillance or watchful waiting.

What about if I take the drug before the surgery?

What are the potential benefits? What are the costs?

Learn which outcomes to measure by speaking with patients

PATIENT-CENTERED OUTCOMES:

"Outcomes that people who represent the population of interest notice and care about"

Disease	Patient Input	Patient-Centered Outcome
Lung cancer	When asked to discuss the tradeoffs, a **majority appeared to value prolonging life above all other factors**.	Survival
Hemophilia A, Hemophilia B, von Willebrand Disease and Other Heritable Bleeding Disorders	**Joint damage and/or pain** as having the most significant impact on their or their loved one's daily life. One participant told of having "six joint replacements – both knees, both elbows, one ankle, and one hip."	Joint damage Pain
Hereditary Angioedema	54% of the participants present in the room and 40% of the webcast participants reported that it **took 10 years or greater for them to receive the correct diagnosis**.	Time to Diagnosis

Source: Adapted from PCORI; FDA Voice of the Patient Reports

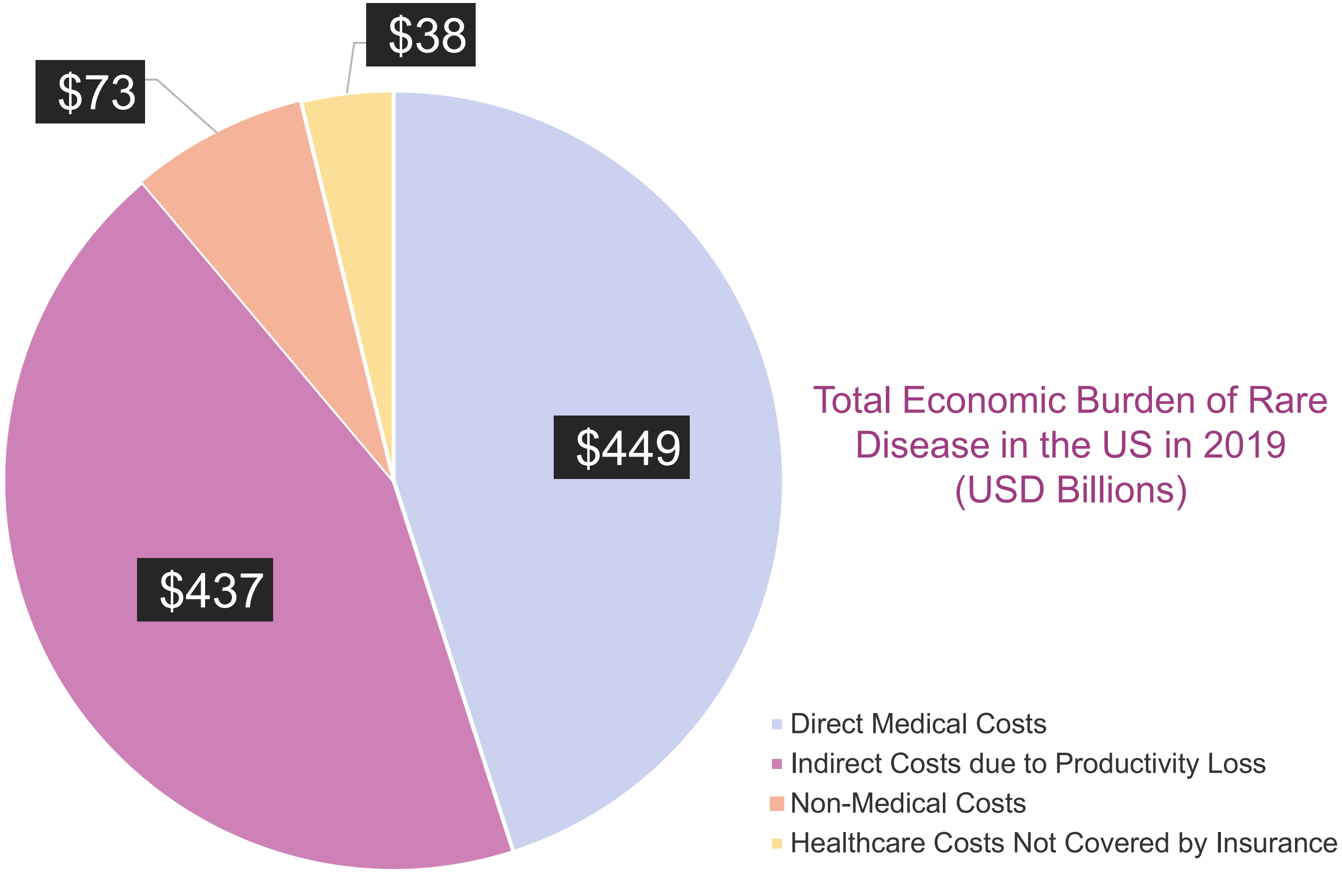
Learn which costs to measure by speaking with patients

OUT-OF-POCKET COSTS

"The portion of payments for ***covered*** health services required to be paid by the enrollee, including co-payments, coinsurance, and deductibles."

- Academy of Managed Care Pharmacy Managed Care Glossary

Is this consistent with how insured members or patients would define **"out-of-pocket costs"**?



Source: Yang G, Cintina I, Pariser A, Oehrlein E, Sullivan J, Kennedy A. The national economic burden of rare disease in the United States in 2019. [Link]

Examples of costs that are important to patients but not covered by insurance

- Experimental, alternative, or non-traditional treatments
- Over-the-counter drug therapies
- Dental surgeries
- Medical foods
- Paid daily care
- Necessary home modification
- Necessary special equipment at home or on a personal or family vehicle
- Transportation costs
- Special education

Source: Yang G, Cintina I, Pariser A, Oehrlein E, Sullivan J, Kennedy A. The national economic burden of rare disease in the United States in 2019. [Link]

Getting the Context Right

Factors important to patients when considering treatment options

- Insurance coverage and costs
- Side effects and risks
- Impact on symptoms, QoL, and functioning
- Geographic location
- Mobility to access care
- Quality of care
- Trust in health care provider
- Convenience, lifestyle, and preferences
- ”Track record” or years of use
- Time until treatment is effective
- How invasive is a treatment or surgery
- Whether a treatment is addictive
- Interactions with other treatments or comorbidities
- Personal responsibilities, family support, and mental health

Source: Project sponsored by the National Health Council: “*Identifying essential questions for patients to ask health care providers*.”

Example: Global Heart Hub IPEC

Insights from People living with Elevated Cholesterol

- Insurance coverage and costs
- Side effects and risks
- Impact on symptoms, QoL, and functioning
- Geographic location
- Mobility to access care
- Quality of care
- Trust in health care provider
- Convenience, lifestyle, and preferences
- ”Track record” or years of use
- Time until treatment is effective
- How invasive is a treatment or surgery
- Whether a treatment is addictive
- Interactions with other treatments or comorbidities
- Personal responsibilities, family support, and mental health

Source: Global Heart Hub IPEC Study

When should PED data be collected during the product lifecycle?

Health Authority Review and Marketing Authorization

Postmarketing

Source: BIO. Framework for the Use of Patient Experience Data Throughout the Product Lifecycle [Link]

When should PED data be collected during the product lifecycle?

Research and Discovery

Preclinical Development

Clinical Development

Health Authority Review and Marketing Authorization

Postmarketing

Source: BIO. Framework for the Use of Patient Experience Data Throughout the Product Lifecycle [Link]

Emerging approach to consolidate the patient perspective: The PED Dossier Template

Template for patient groups to consolidate patient experience data from a variety of sources

Consistent format across patient groups, but patient experience data is disease-specific

Living document that is updated over time

POTENTIAL TOPICS

- Symptoms and natural history of the disease
- Patient-centered outcomes or impacts
- Prevalence and important subgroups
- Impact on functioning and daily life
- Caregiver burden
- Experiences with available treatments
- Costs to patients and caregivers

POTENTIAL SOURCES

- Patient-Focused Drug Development Meetings
- Patient registries or surveys
- Patient-centered core impact sets
- Patient experience mapping
- Qualitative studies
- Patient preference studies
- Economic burden studies

Source: National Health Council. Exploring the Patient Experience Dossier [Link]

Skaggs School of Pharmacy
and Pharmaceutical Sciences
UNIVERSITY OF COLORADO
ANSCHUTZ MEDICAL CAMPUS

Whole Health Value Assessment: Universal Survey Framework for Integrating Patient Experience Data in Health Technology Assessment

R. Brett McQueen, PhD
Associate Professor
Director, Pharmaceutical Value (pValue)

pValue
Evidence for Decisions

Role and purpose of modeling in value assessment

- "…analytic methodology that accounts for events over time and across populations, … to estimate the effects of an intervention on valued health consequences and costs."*
- Aid to a specific decision context
- Links intermediate outcomes to long-run outcomes**

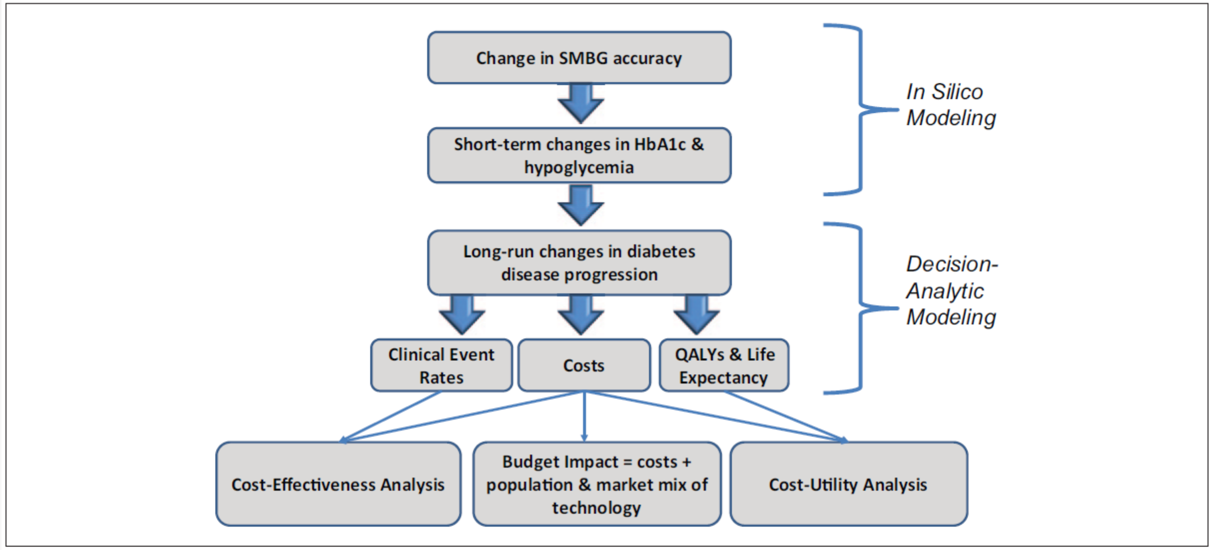


Figure 1. Evidence flow chart. Differences in the accuracy of self-monitoring blood glucose devices (SMBG) are associated with changes in HbA1c and hypoglycemia.[3] The analysis links these changes in hemoglobin A1c (HbA1c) and hypoglycemia to long-run diabetes disease progression. Disease progression in turn impacts costs, clinical event rates, and quality-adjusted life years (QALYs). Cost-effectiveness, budget impact, and cost-utility analyses are performed to investigate the long-run impact of accuracy differences in blood glucose monitoring devices.

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*Weinstein MC, O'Brien B, Hornberger J, Jackson J, Johannesson M, McCabe C, Luce BR; ISPOR Task Force on Good Research Practices--Modeling Studies. Principles of good practice for decision analytic modeling in health-care evaluation: report of the ISPOR Task Force on Good Research Practices--Modeling Studies. Value Health. 2003 Jan-Feb;6(1):9-17

**McQueen RB et al. Economic value of improved accuracy for self-monitoring of blood glucose devices for type 1 diabetes in Canada. JDST 2015.

Do we need to model everything?

- Quantifiable information from primary/secondary sources
  - Cost and cost offsets (numerator)
  - Validated preference-based instruments for long-term composite QoL outcomes (denominator)
    - Potential extension to non preference-based instruments in cost-efficiency frontier
- Qualitative information in a deliberative setting is impactful*
  - Additional qualitative information from patient or caregivers
  - Societal contextual considerations

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*Trenaman L, Pearson SD, Hoch JS. How Are Incremental Cost-Effectiveness, Contextual Considerations, and Other Benefits Viewed in Health Technology Assessment Recommendations in the United States? Value Health. 2020 May;23(5):576-584

Importance of treatment effects in modeling

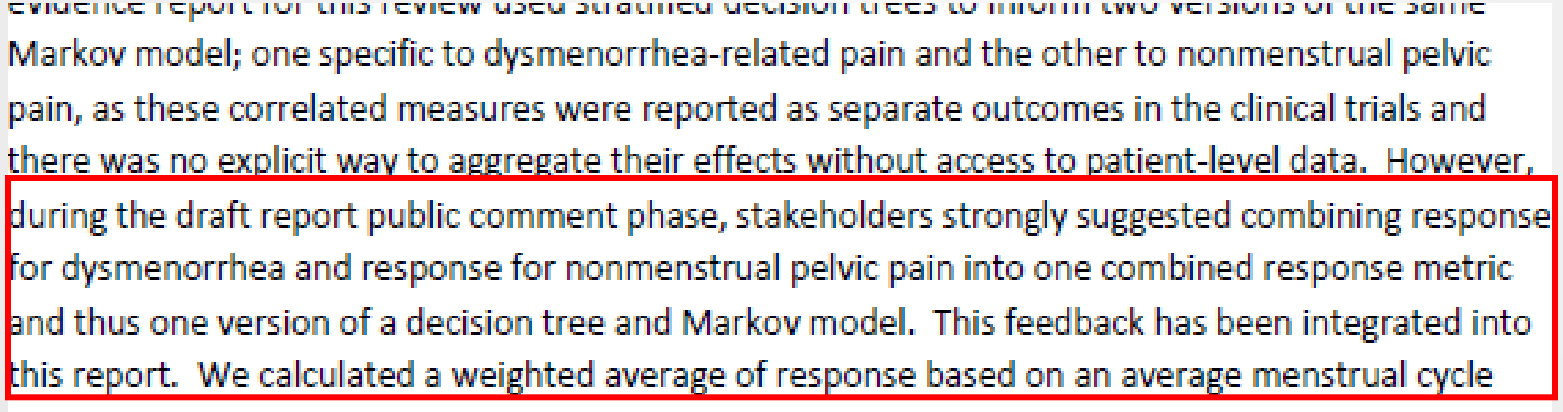
- Crucial to isolate the effect of the treatment on the numerator and denominator (e.g., randomized environment)
- Introducing a new instrument requires validation, consideration of scaling, and weighting for importance
- Without validation, avoid “scoring” therapies

Where can a universal survey help modelers?

- Validate the model structure/conceptual framework
- Contribute to selection of appropriate inputs or instruments that highlight aspects of disease most important for a treatment
- Out of pocket expenses when a “treatment effect” is measured

Conceptual framing for modeling endometriosis

- Trial effects were not based on the patient experience with endometriosis
- We used patient experience input to calculate a combined metric for response to pain

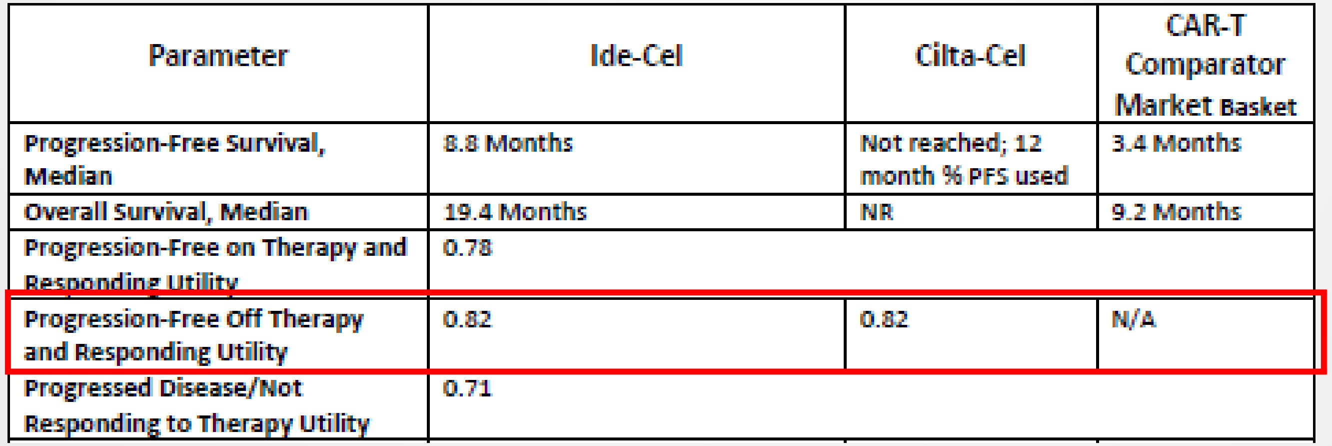


evidence report for this review used stratified decision trees to inform two versions of the same Markov model; one specific to dysmenorrhea-related pain and the other to nonmenstrual pelvic pain, as these correlated measures were reported as separate outcomes in the clinical trials and there was no explicit way to aggregate their effects without access to patient-level data. However, during the draft report public comment phase, stakeholders strongly suggested combining response for dysmenorrhea and response for nonmenstrual pelvic pain into one combined response metric and thus one version of a decision tree and Markov model. This feedback has been integrated into this report. We calculated a weighted average of response based on an average menstrual cycle

Atlas S, McQueen RB, Campbell JD, Whittington MD, Ollendorf DA. Institute for Clinical and Economic Review (ICER). Evidence Report: Elagolix for Treating Endometriosis. August 3, 2018. Accessed at: https://icer.org/wp-content/uploads/2020/10/ICER_Elagolix_Final_Evidence_Report_080318.pdf.

Health-related quality of life inputs in multiple myeloma

- Patient input was crucial in understanding the benefits of staying “off treatment”
- Utility inputs were chosen based on the biggest spread in values



Parameter	Ide-Cel	Cilta-Cel	CAR-T Comparator Market Basket
Progression-Free Survival, Median	8.8 Months	Not reached; 12 month % PFS used	3.4 Months
Overall Survival, Median	19.4 Months	NR	9.2 Months
Progression-Free on Therapy and Responding Utility	0.78		
Progression-Free Off Therapy and Responding Utility	0.82	0.82	N/A
Progressed Disease/Not Responding to Therapy Utility	0.71		

Modeling team lead: Lee SJ, McQueen RB, Beinfeld M, Fluetsch N, Whittington MD, Pearson SD, Ollendorf DA. Anti B-Cell Maturation Antigen CAR T-cell and Antibody Drug Conjugate Therapy for Heavily Pre-Treated Relapsed and Refractory Multiple Myeloma; Final Evidence Report. Institute for Clinical and Economic Review, May 11, 2021. https://icer.org/wp-content/uploads/2020/10/ICER_Multiple-Myeloma_Final-Report_Update_09102021.pdf.

Health-related quality of life for women experience vasomotor symptoms from menopause

- Severity vs. frequency of vasomotor symptoms from menopause
- Not only were treatment effects consistent, but patient input was crucial in this assumption

Assumption	Rationale
Relative treatment effects are consistent across baseline VMS severity and frequency.	There is limited evidence on relative treatment effects across starting levels of VMS severity and frequency, therefore we will not adjust treatment effects in subgroup analyses that may change the baseline severity or frequency of VMS.

Beaudoin FL, McQueen RB, Wright A, Yeung K, Moradi A, Herron-Smith S, Gutierrez E, Rind DM, Pearson SD, Lin GA. Fezolinetant for Moderate to Severe Vasomotor Symptoms Associated with Menopause: Effectiveness and Value; Final Evidence Report. Institute for Clinical and Economic Review, January 23, 2023. https://icer.org/wp-content/uploads/2022/06/ICER_Menopause_FinalReport_01232023.pdf.

How can uptake of universal survey instruments increase in value assessment?

- Modelers are already using patient input for assumptions and input selection
- Quantification of instruments into models requires validation and treatment effects
- Potential mapping (i.e., participants fill out both on same follow-up visits) with EQ-5D may increase uptake in short-run

Questions?

Kayleigh Majercak

kmajercak@
umaryland.edu

Joe Vandigo

jvandigo@
appliedpx.com

Brett McQueen

robert.mcqueen@
cuanschutz.edu

Kimberly Westrich

kwestrich@
npcnow.org