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ISPOR Special Interest Group

Health Preference Research Today: How Patient-Centered Is It and How Can It Be More Patient-Centric?

ISPOR Patient-Centered SIG &
ISPOR Health Preference Research SIG

Thursday, May 15 | 11:45 AM - 12:45 PM

Discussion Topics

	Topic	Presenter(s)
1	Patient-Centricity in Health Preference Research: Where Are We?	Jessica Roydhouse
2	Patterns in attribute selection and development reporting in patient preference studies	Siu Hing Lo
3	Patient Centered Benefit-Risk: Case Study in Duchenne Gene Therapy	Ryan Fischer
4	Patient-Centricity in Health Preference Research is Improving- shared learnings in musculoskeletal conditions	Angie Botto-van Bemden

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Author's Disclosure

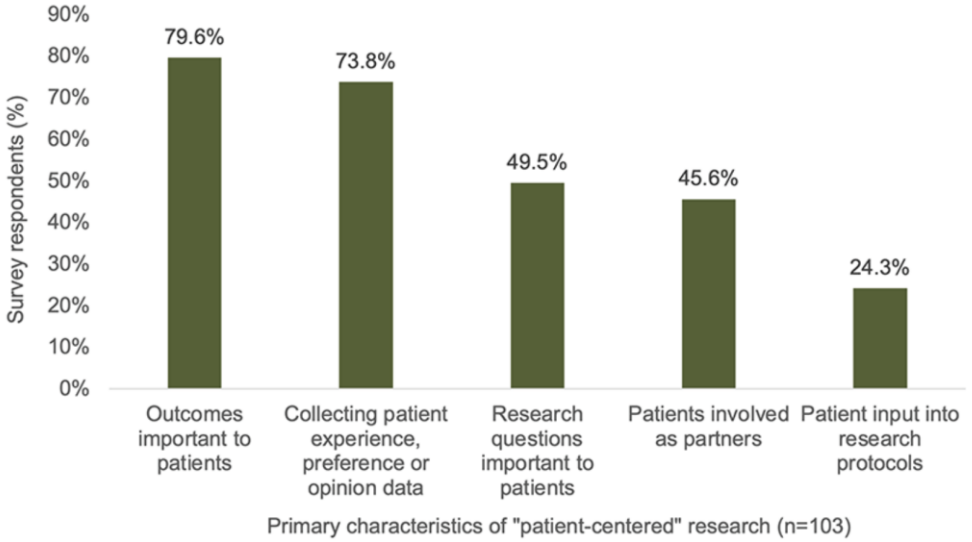
- Jessica Roydhouse has received funding from Pfizer, Inc., in the form of a contract with her institution, the University of Tasmania.
- Siu Hing Lo is an employee of Acaster Lloyd Consulting Ltd, United Kingdom.
- Ryan Fischer, Foundation for Angelman Syndrome Therapeutics.
  Mr. Fischer has no relevant financial or nonfinancial relationships to disclose.
- Angie Botto-van Bemden, Musculoskeletal Research International, has no relevant financial or nonfinancial relationships to disclose.

1

Patient-Centricity in Health Preference Research: Where Are We?

Jessica Roydhouse, PhD
Menzies Institute for Medical Research, University of Tasmania

Background to the session: What makes a study patient centric?



Note: The full wording of these response options in the survey were as follows: "Focused on outcomes important to patients," "Collecting patient experience, preference or opinion data through methods such as focus groups, interviews, or preference elicitation methods," "Focused on research questions important to patients," "Patients involved as partners (ie, coinvestigator, advisor, consultant)," and "Patient input into research protocols."

From Llewellyn et al, ISPOR Value and Outcomes Spotlight 2024; 10(6) https://www.ispor.org/publications/journals/value-outcomes-spotlight/vos-archives/issue/view/value-assessments/quality-of-patient-engagement-activities-in-health-economics-and-outcomes-research--insights-from-the-ispor-community

Background to the session

- Patient preference information: value patients place on aspects of therapy (FDA CDRH)
- Patient experience data: patient experiences, perspectives, and needs, such as signs and symptoms and the impact of treatment (FDA PFDD)
- PED or patient preference data: patients as participants, not (necessarily) partners
- Engaging patients in PED or preference studies can be complementary
- When and how should we engage patients for greatest benefit to patients and studies?

FDA PFDD: https://www.fda.gov/media/139088/download
FDA CDRH: https://www.fda.gov/about-fda/division-patient-centered-development/patient-preference-information-ppi-medical-device-decision-making

Introduction – Recent Review Example: Do Preference Studies Engage Patients?

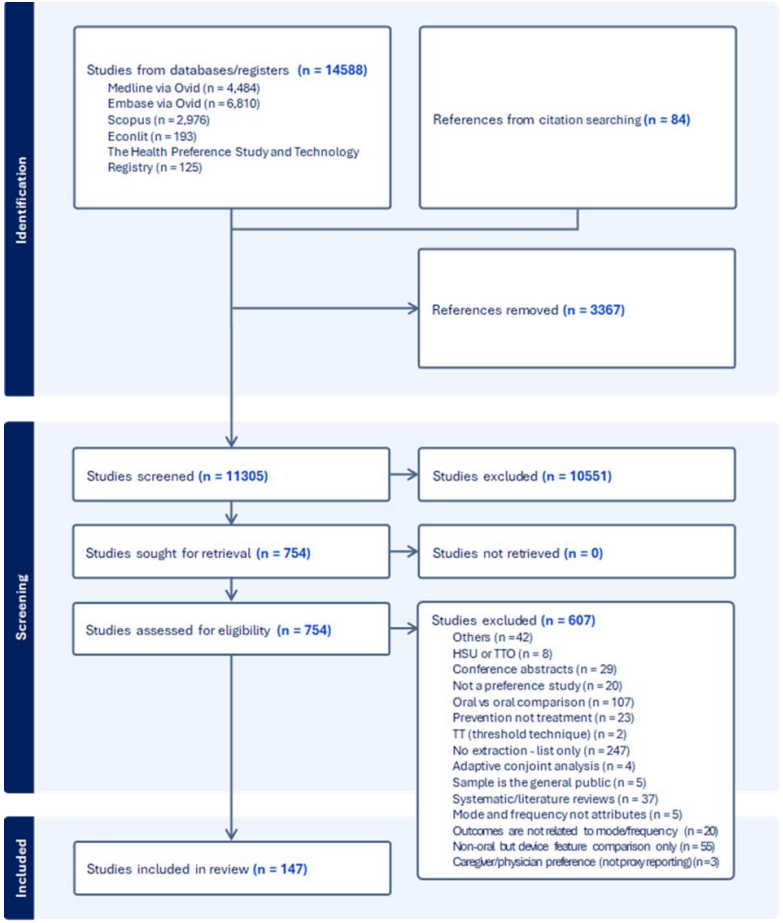
- Overview of findings from a recent large review of health preference studies
- This review was supported financially by a grant from Pfizer, Inc. to the University of Tasmania
- The review focused on understanding patient preferences regarding treatment processes (frequency of therapy; mode of delivery of therapy)
- We extracted information on patient engagement *as reported by authors*

What Do We Mean When We Say 'Patient Engagement'

"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 RL 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 2020; 23(6): 677-688.


Identification
Studies from databases/registers (n = 14588)
Medline via Ovid (n = 4,484)
Embase via Ovid (n = 6,810)
Scopus (n = 2,976)
Econlit (n = 193)
The Health Preference Study and Technology Registry (n = 125)
References from citation searching (n = 84)
References removed (n = 3367)
Screening
Studies screened (n = 11305)
Studies excluded (n = 10551)
Studies sought for retrieval (n = 754)
Studies not retrieved (n = 0)
Studies assessed for eligibility (n = 754)
Studies excluded (n = 607)
Others (n = 42)
HSU or TTO (n = 8)
Conference abstracts (n = 29)
Not a preference study (n = 20)
Oral vs oral comparison (n = 107)
Prevention not treatment (n = 23)
TT (threshold technique) (n = 2)
No extraction - list only (n = 247)
Adaptive conjoint analysis (n = 4)
Sample is the general public (n = 5)
Systematic/literature reviews (n = 37)
Mode and frequency not attributes (n = 5)
Outcomes are not related to mode/frequency (n = 20)
Non-oral but device feature comparison only (n = 55)
Caregiver/physician preference (not proxy reporting) (n = 3)
Included
Studies included in review (n = 147)

Did studies engage patients?

Patient engagement?	N (%) (denominator is 147)
Yes	18 (12.2%)
No	123 (83.7%)
Unclear	6 (4.1%)

How did studies engage patients?

Type of patient engagement	N (%) (denominator is 18)
Inform questionnaire design	9 (50%)
Study governance role	5 (28%)
Co-authorship	3 (17%)
Funding	1 (6%)

Patient centricity in health preference research: next steps

- Findings suggest ample room for improvement
- How are patients engaged in selecting attributes for preference studies, and study design more broadly? (Siu-Hing Lo)
- What is the patient partner experience of engagement?
  - Case Study 1 – Angie Botto-van Bemden
  - Case Study 2 – Ryan Fischer
- Discussion and Q&A (Moderator)

2

Patterns in attribute selection and development reporting in patient preference studies

Siu Hing Lo, PhD
Acaster Lloyd, London, United Kingdom

Acknowledgements

- The study was conducted in collaboration with Rebekah Hall, Gin Nie Chua and Joy Wong (*research*) and supported by Sebastian Snow (*medical writing*)

Background

Trend of increased publication of attribute selection and development papers

Attribute selection and development is foundational in DCE design impacting:

- Validity
- Reliability
- Applicability to real-world healthcare decisions **and patient-centricity**!

Framework for attribute development (*Helter and Boehler, 2016*)

Recent review of high-quality qualitative research for DCE development concluded standardized reporting remains challenge *(Gonzalez Bohorques et al., 2024)*

Study Objectives

1

How is attribute selection and development in DCE studies reported?

- *Studies with in-depth description of attribute selection and/or development included*

2

To identify patterns and gaps in reporting on attribute selection and development.

Methods (1 of 2)

Search Strategy:

Concept 1: "patients"	Concept 2: "discrete choice experiments"	Concept 3: "attribute selection/development"

Eligibility Criteria:

- Peer-reviewed manuscripts in English eligible for inclusion if meeting all below:
  - Patient preference DCEs in any health condition requiring medical intervention.
  - Reporting on at least one of following relating to attribute selection/development:
    - ✓ Evidence sources consulted
    - ✓ Formative methods
    - ✓ Decision-making

Methods (2 of 2)

Title/Abstract and Full-Text Screening

Data Extraction and Coding

- Form developed in Microsoft Excel and piloted on subset of studies through independent extraction and coding by multiple reviewers for >20% of included studies, followed by discussion
- ***Data extraction:*** study characteristics (authors, publication year, objectives)
- ***Data coding (Y/N):*** reporting of attribute selection and/or development:
  - Aspects of methods
  - Results
  - Decision-making ('how' and 'why')
  - Patient and public involvement (PPI) / patient engagement

Patients as research participants

Patients as research partners

Data Synthesis

It's time for a poll!

Have a guess... what percentage of included papers have reported patient engagement, defined as involving patients as research partners in the study?

a) Less than 10%
b) Between 10 and <20%
c) Between 20 and <30%
d) Between 30 and <50%
e) Over 50%

Results

28 included studies

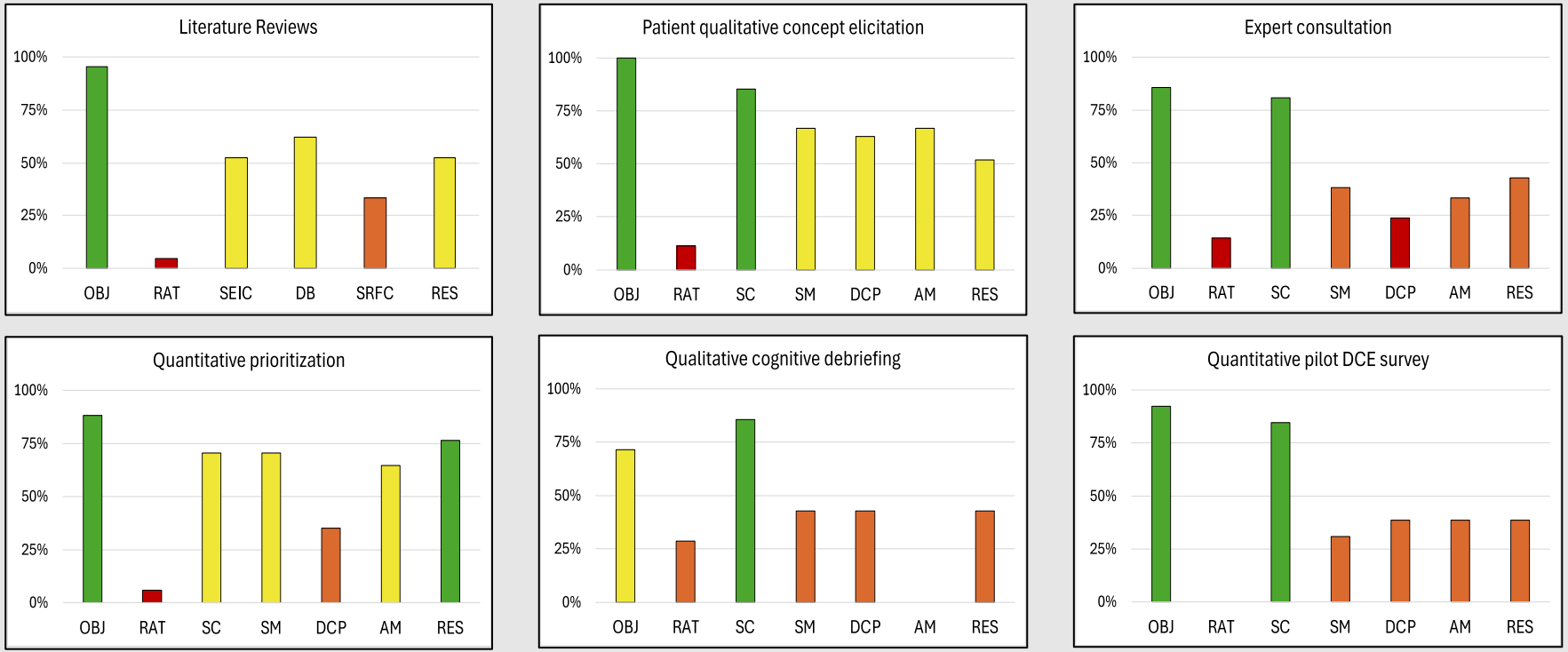
Study Characteristics

- **Publication Dates:** 2007 – 2024, median: 2020
- **Disease/health areas:** Oncology (19%), diabetes (11%), pregnancy (11%), arthritis (7%), osteoporosis (7%), hypodontia (7%), amongst others
- **PPI**: stated if any: 6 (21%), patient engagement: 2 (7%)

Methods for Attribute Selection / Development

- **Literature reviews**: 21 (75%)
- **Patient qualitative concept elicitation:** 27 (96%)
- **Expert consultation:** 21 (75%)
- **Quantitative prioritization:** 17 (61%)
- **Qualitative cognitive debriefing:** 7 (25%)
- **Quantitative pilot DCE survey:** 13 (46%)

Figure 1. Proportion of papers for each method reporting study elements



AM: Analysis Methods; DB: Database(s); DCP: Data Collection Procedures; OBJ: Objectives; RAT: Rationale; RES: Results; SC: Sample Characteristics; SEIC: Search terms and inclusion/exclusion criteria; SM: Sampling Methods; SRFC: Screening results/flow chart

Key: Green: >75% reported; **Yellow:** >50% to ≥ 75% reported; **Orange:** 25% to ≤50% reported; **Red:** ≤25% reported

Figure 2. Proportion of studies reporting list of attributes following completion of method

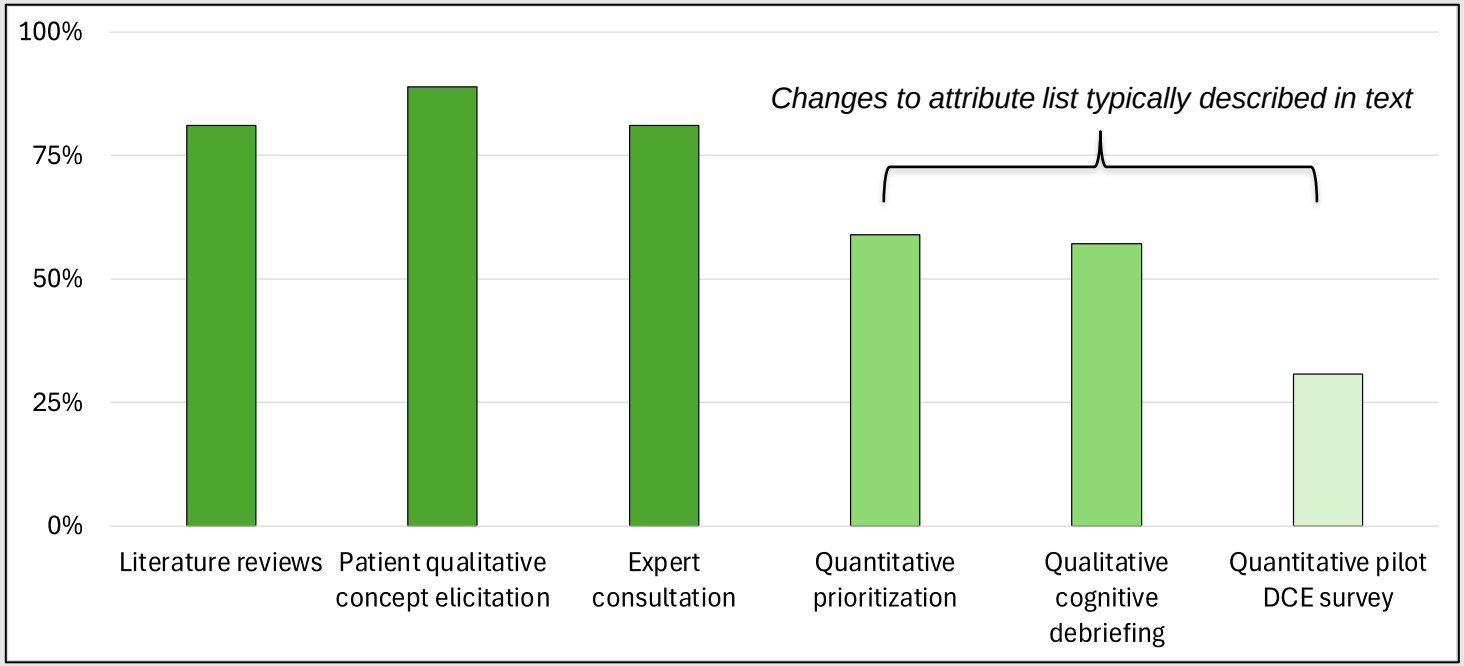
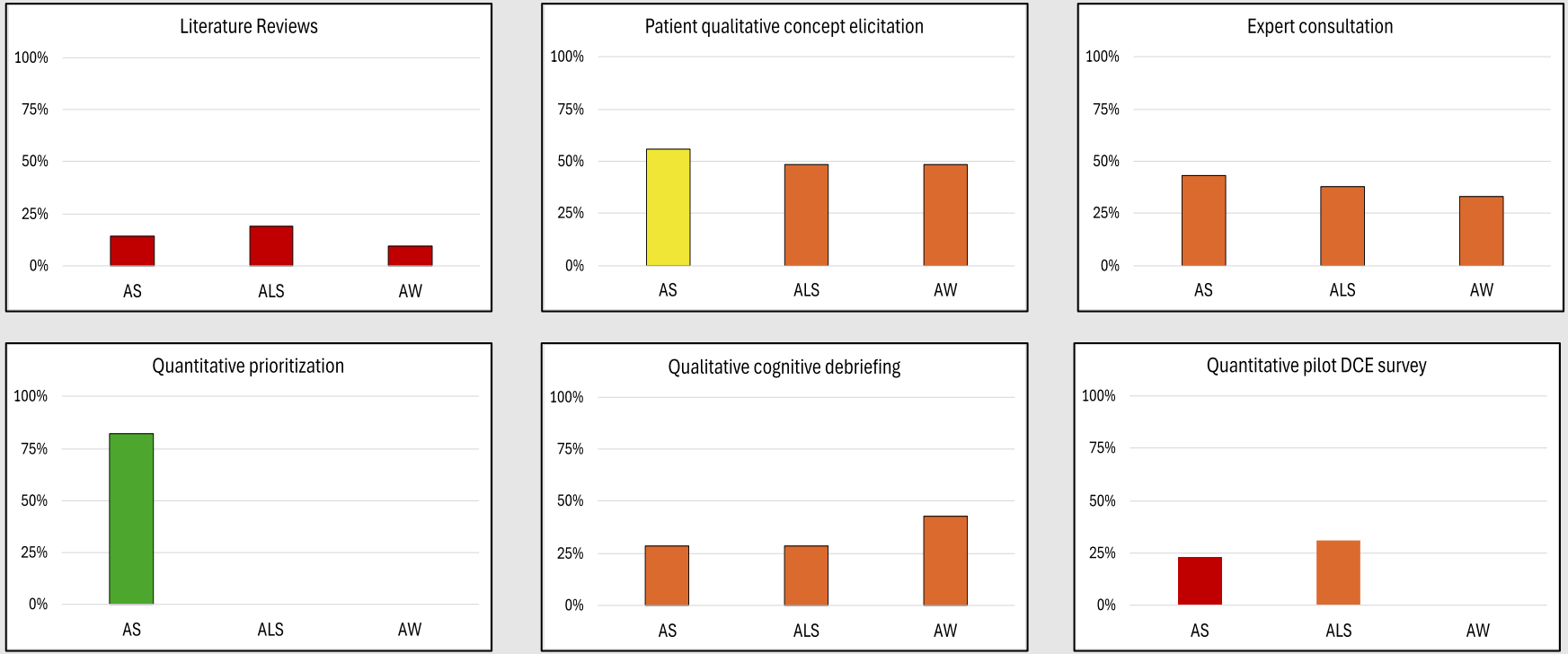


Figure 3. Proportion of studies reporting how each method informed attribute and level selection and wording



AS: Attribute Selection; ALS: Attribute Level Selection; AW: Attribute Wording

Key: Green: >75% reported; **Yellow:** >50% to ≥ 75% reported; **Orange:** 25% to ≤50% reported; **Red:** ≤25% reported. **Note:** Percentages represent the proportion of papers that reported on attribute/attribute level selection (for 'Attribute Selection' and 'Attribute Level Selection'), and refinement of attribute wording (for 'Attribute Wording').

Discussion

Mixed level of reporting across primary formative research methods

Contrast in reporting of research results: high reporting for quantitative prioritisation exercises but low for other formative methods

The 'how'? *DIRECT Checklist: describe **how** attributes and levels are derived, provide final **list of attributes** and levels (Ride et al., 2024)*

Higher levels of reporting of attribute lists, but partial reporting of formative method details and results, and low levels of reporting of how formative methods informed attribute and level selection and refinement

Patient engagement in patient preference DCE studies in its infancy

Conclusion

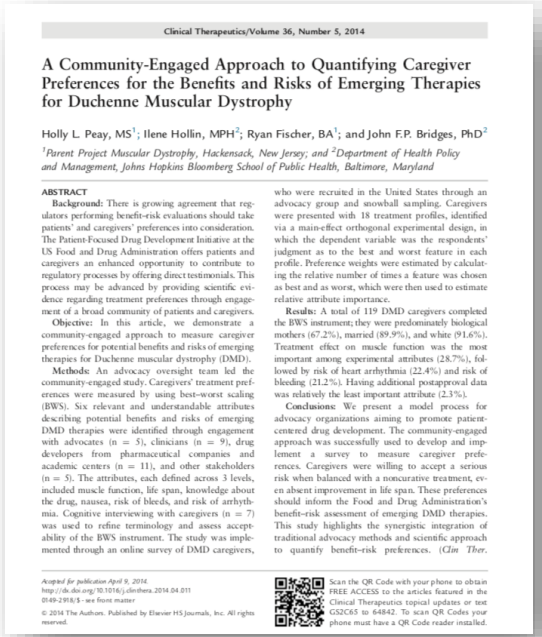
1	Incomprehensive reporting hinders evaluation of formative research, and ultimately the patient-centricity of the research
2	Need for more detailed, practical guidelines to describe attribute selection and development

3

Patient Centered Benefit-Risk: Case Study in Duchenne Gene Therapy

Ryan Fischer
COO, Foundation for Angelman Syndrome Therapeutics (FAST)

Community-Engaged Approach – *Key Principles*



Clinical Therapeutics/Volume 36, Number 5, 2014

A Community-Engaged Approach to Quantifying Caregiver Preferences for the Benefits and Risks of Emerging Therapies for Duchenne Muscular Dystrophy

Holly L. Peay, MS[1]; Ilene Hollin, MPH[2]; Ryan Fischer, BA[1]; and John F.P. Bridges, PhD[2]

[1]*Parent Project Muscular Dystrophy, Hackensack, New Jersey; and* [2]*Department of Health Policy and Management, Johns Hopkins Bloomberg School of Public Health, Baltimore, Maryland*

ABSTRACT

Background: There is growing agreement that regulators performing benefit-risk evaluations should take patients' and caregivers' preferences into consideration. The Patient-Focused Drug Development Initiative at the US Food and Drug Administration offers patients and caregivers an enhanced opportunity to contribute to regulatory processes by offering direct testimonials. This process may be advanced by providing scientific evidence regarding treatment preferences through engagement of a broad community of patients and caregivers.

Objective: In this article, we demonstrate a community-engaged approach to measure caregiver preferences for potential benefits and risks of emerging therapies for Duchenne muscular dystrophy (DMD).

Methods: An advocacy oversight team led the community-engaged study. Caregivers' treatment preferences were measured by using best–worst scaling (BWS). Six relevant and understandable attributes describing potential benefits and risks of emerging DMD therapies were identified through engagement with advocates (n = 5), clinicians (n = 9), drug developers from pharmaceutical companies and academic centers (n = 11), and other stakeholders (n = 5). The attributes, each defined across 3 levels, included muscle function, life span, knowledge about the drug, nausea, risk of bleeds, and risk of arrhythmia. Cognitive interviewing with caregivers (n = 7) was used to refine terminology and assess acceptability of the BWS instrument. The study was implemented through an online survey of DMD caregivers, who were recruited in the United States through an advocacy group and snowball sampling. Caregivers were presented with 18 treatment profiles, identified via a main-effect orthogonal experimental design, in which the dependent variable was the respondents' judgment as to the best and worst feature in each profile. Preference weights were estimated by calculating the relative number of times a feature was chosen as best and as worst, which were then used to estimate relative attribute importance.

Results: A total of 119 DMD caregivers completed the BWS instrument; they were predominately biological mothers (67.2%), married (89.9%), and white (91.6%). Treatment effect on muscle function was the most important among experimental attributes (28.7%), followed by risk of heart arrhythmia (22.4%) and risk of bleeding (21.2%). Having additional postapproval data was relatively the least important attribute (2.3%).

Conclusions: We present a model process for advocacy organizations aiming to promote patient-centered drug development. The community-engaged approach was successfully used to develop and implement a survey to measure caregiver preferences. Caregivers were willing to accept a serious risk when balanced with a noncurative treatment, even absent improvement in life span. These preferences should inform the Food and Drug Administration's benefit-risk assessment of emerging DMD therapies. This study highlights the synergistic integration of traditional advocacy methods and scientific approach to quantify benefit-risk preferences. (*Clin Ther.*

Accepted for publication April 9, 2014.
http://dx.doi.org/10.1016/j.clinthera.2014.04.011
0149-2918/$ - see front matter

Scan the QR Code with your phone to obtain FREE ACCESS to the articles featured in the Clinical Therapeutics topical updates or text GSZC65 to 64842. To scan QR Codes your phone must have a QR Code reader installed.

Peay H., Fischer R. et al
2014

- Advocacy-led initiative
- Stakeholder-engaged process
- Data owned by the advocacy community
- Dissemination through patient group

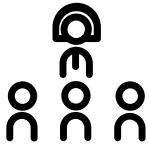
Patient-Centered Benefit-Risk:
Gene Therapy Preference Studies in Duchenne

- Opportunity to meaningfully engage and include families throughout the study

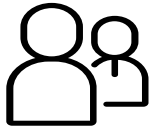
Stakeholder Advisory Board

- Development
- Review
- Publication

Focus Groups



Qualitative Interviews



Social media engagement

Pilot testing survey

Recruitment using family networks

Presentation of results via conferences and webinars

Educational material development

Duchenne Gene Therapy Preference Study 1 (2017-2018)

Pre-competitive collaboration

Pfizer

SOLID BIOSCIENCES

Parent Project Muscular Dystrophy

RTI INTERNATIONAL

Study Objectives

Qualitative Study (Interviews)

Quantitative Study (Survey)

Educational Initiative

Led by Stakeholder Advisory Board

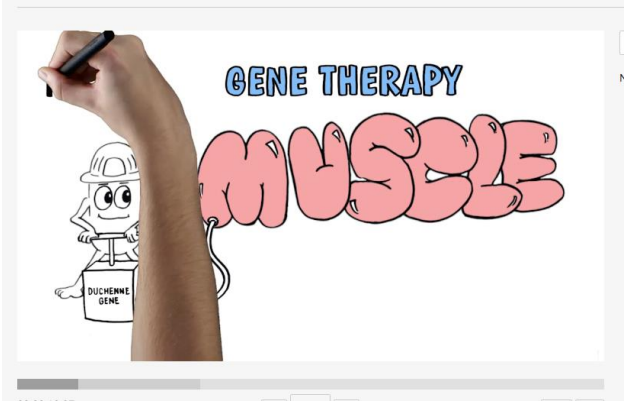
Research Objective:
Explore preferences and risk tolerance about emerging gene therapy technologies.

Mixed Methods:
Focus Groups (Qualitative)
Semi-structured interviews (Qualitative)
Threshold Technique and BW Scaling (Quantitative)

Study 1 survey set up: Gene Therapy Video

Key Messages:

- *We expect gene therapy to help people's muscles, lungs, and hearts work better for a longer amount of time.*
- *Gene therapy is not a cure for Duchenne.*
- *Very young children will probably have the most benefit, but gene therapy should be able to help almost everyone with Duchenne.*
- *Gene therapy may only be able to be used once in a person's entire life. This could change in the future with new research, but no one knows yet.*
- *Based on animal studies benefits could last for at least 10 years, but no one knows yet how long the benefits will last and who will benefit most.*



Aim: How much of a *risk of death* will Duchenne parents accept?

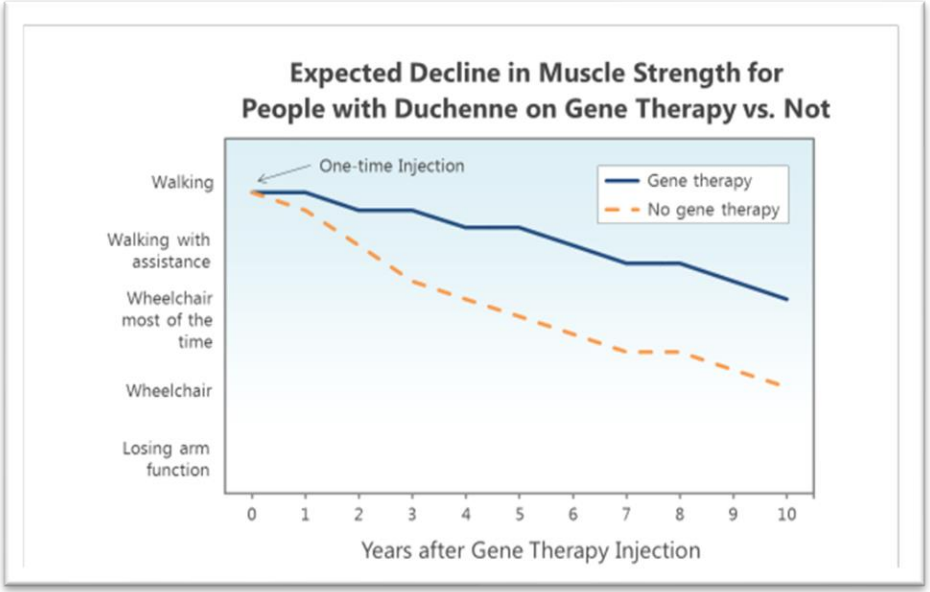
- We used **threshold technique** to measure how much risk of death participants would accept.
- We asked about the **risk of death** at the following stages:
  - Now (the present time)
  - Last year of walking well
  - Last year able to bring arms to mouth
  - In the newborn period

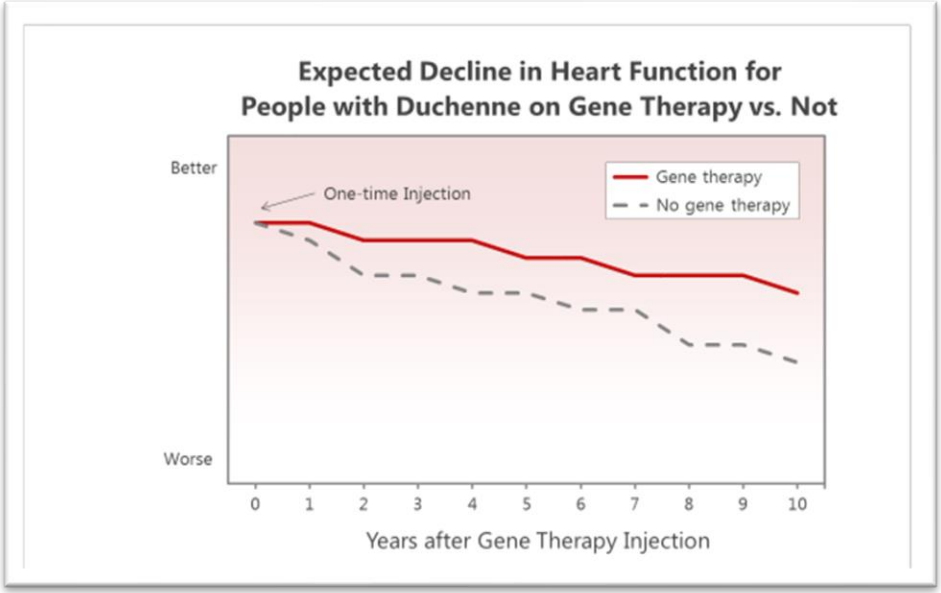
Threshold technique set up

Imagine that your child's doctor offers your child gene therapy for Duchenne.
The doctor shows you these two graphs.

They show the average benefit experienced by 2,000 people who used gene therapy.

The solid lines show how using gene therapy has helped their muscle strength and heart function compared to people who don't use gene therapy. The doctor cannot tell you how long the benefit will last. But it should last for 10 years.





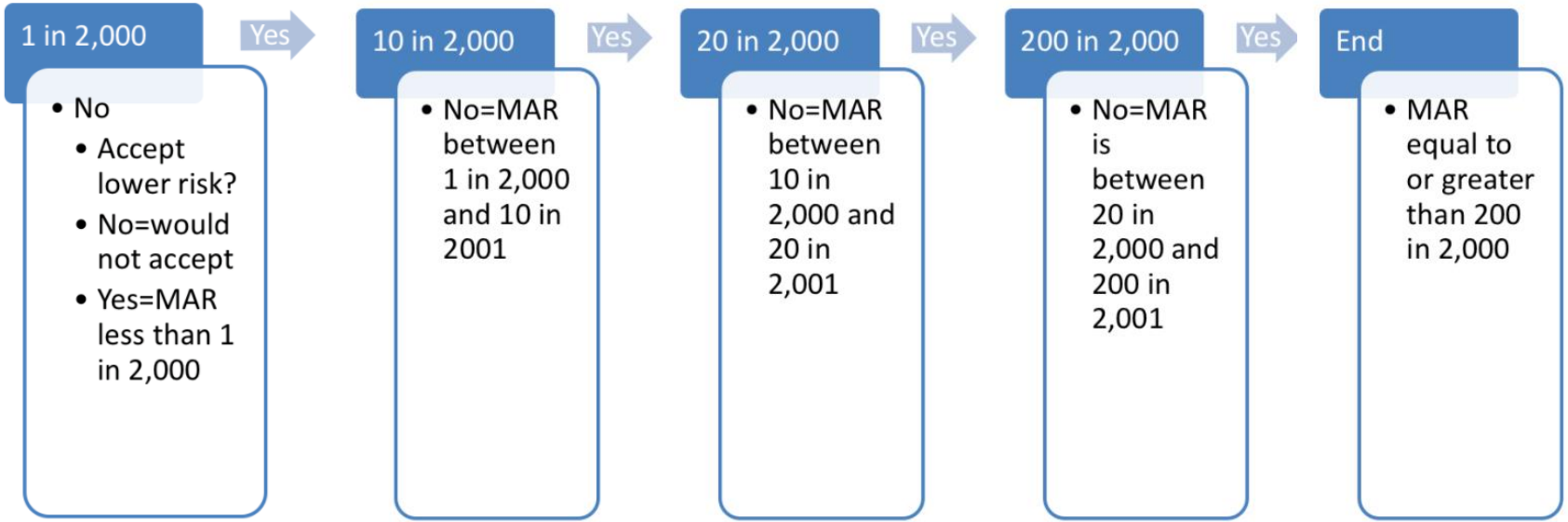
Survey design – what participants read leading up to threshold

Your doctor tells you about the **risk** of gene therapy. **1 out of 2,000** people with Duchenne will die from using gene therapy. The person will die within a week after using it. The other **1,999** people will not die from gene therapy.

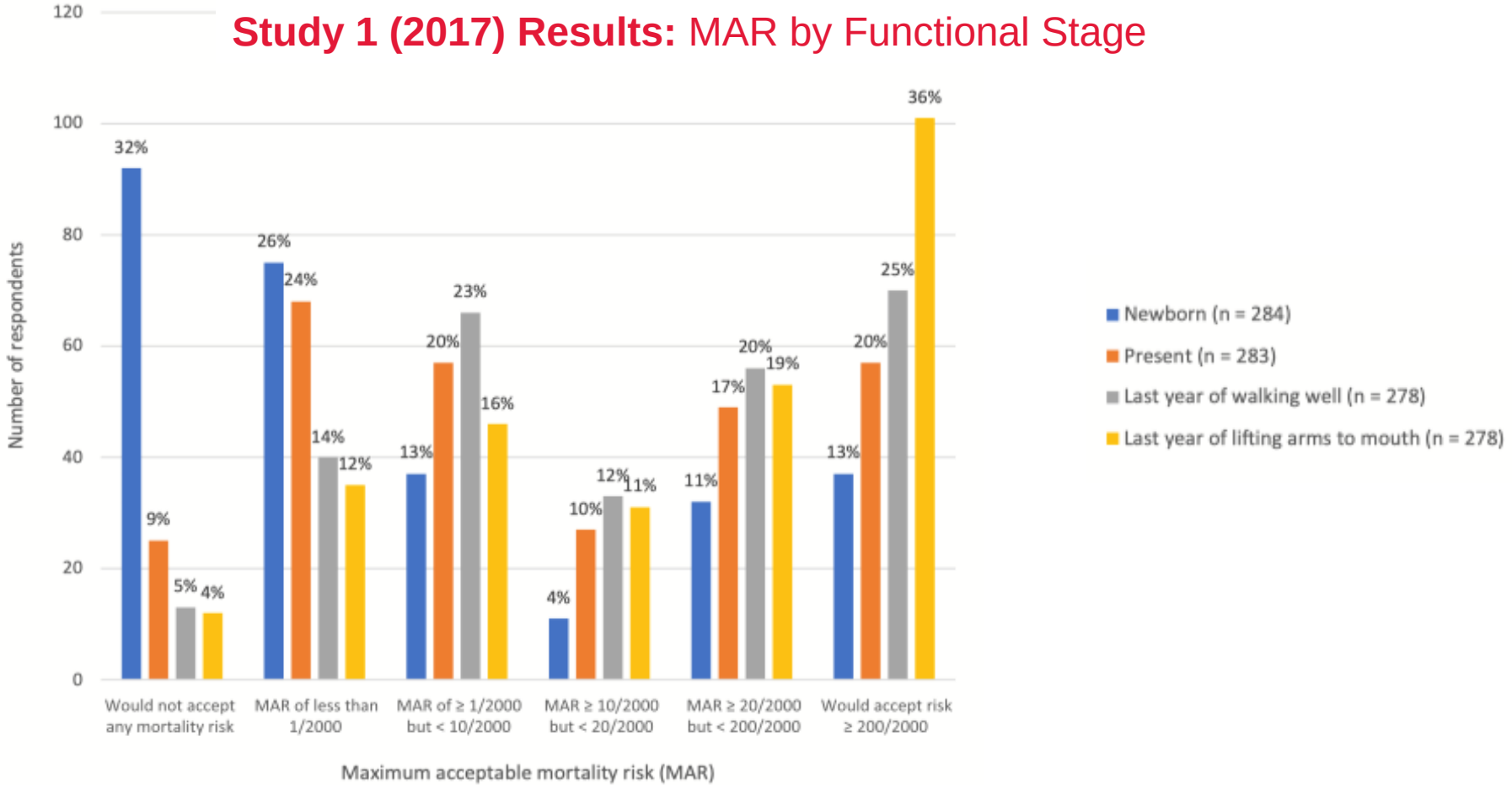
Would you choose gene therapy? (parent version)

	Yes	No
I would choose therapy for my child **now**	○	○
I would choose /have chosen gene therapy for my child when he was a **newborn**	○	○
I would choose /have chosen gene therapy for my child in **last year of walking well**	○	○
I would choose /have chosen gene therapy for my child in **last year he could lift arms to mouth**	○	○

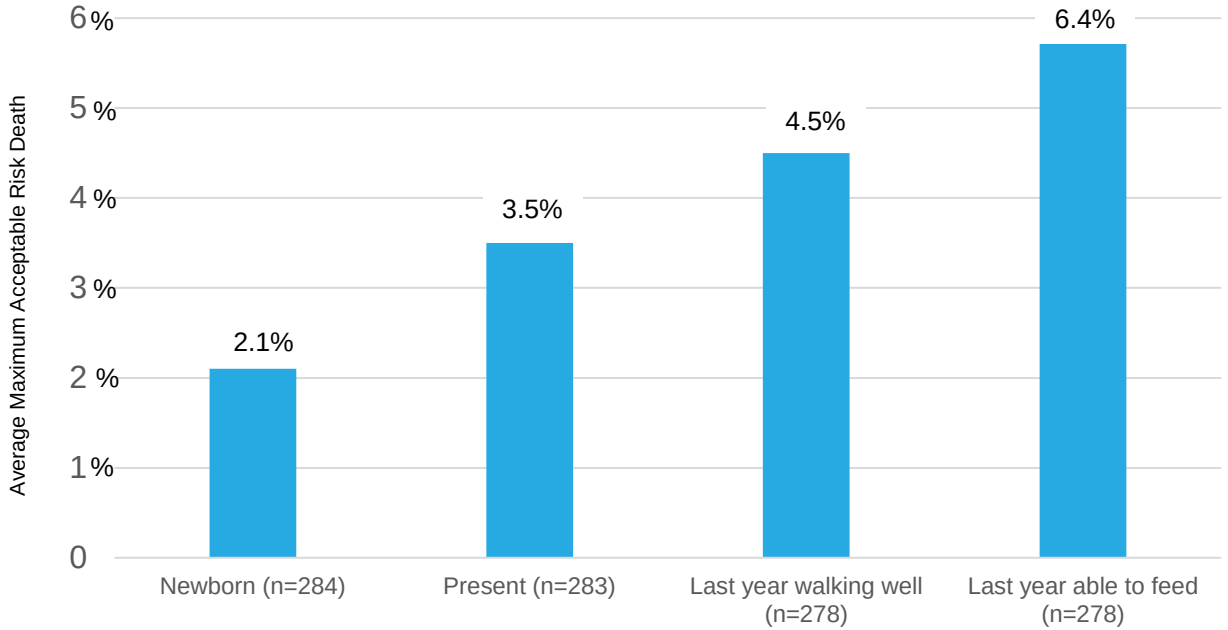
Aim: How much of a risk of death will participants accept?



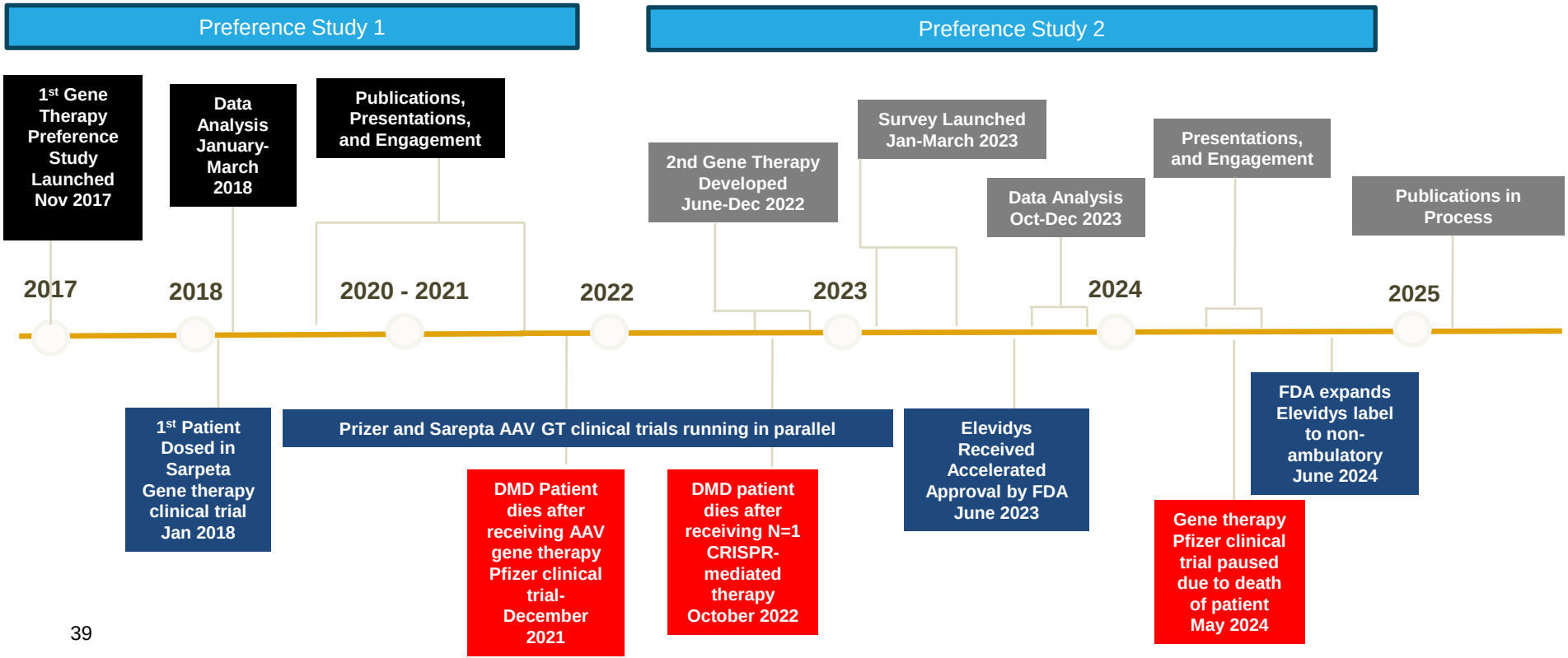
Study 1 (2017) Results: MAR by Functional Stage



Study 1 (2017) Results: Caregiver Average MAR of Gene Therapy-Related Death



Patient and Caregiver Engagement: Focus Groups, Interviews, Preference Surveys, & Presentations



Study 2 (2022)
Methods and Respondents

- Survey-based study again using the threshold technique to determine the maximum acceptable risk (MAR) of death from using gene therapy
  - Updated from prior survey
  - MAR “now”, in the last year of walking well, in the last year able to lift hands to mouth, in newborn period
- Survey only provided in English
- Eligible participants were adults (aged ≥ 18 years) with DMD or caregivers of children (of any age) with DMD.
- Convenience recruitment targeted to U.S. and U.K.
- Updated video
- **Duchenne UK and PPMD collaboration**
- **Supported by 6 Pharma companies in pre-competitive consortium**

Gene Therapy Perception (prior to teaching video)

Question	Total (N=263) n	%
Which best understands your understanding of gene therapy?		
Never heard the term	1	0.4%
Have heard it, but don't understand	20	7.6%
Have some understanding	114	43.3%
Understand quite well	71	27.0%
Understand and could explain to others	57	21.7%
For most people with Duchenne, how much benefit do you think would come from using gene therapy?		
No benefit	1	0.4%
A small amount of benefit	31	11.8%
A medium amount of benefit	55	20.9%
A large benefit but not a cure	127	48.3%
A cure	25	9.5%
I don't know	24	9.1%
For most people with Duchenne, how much risk do you think would come from using gene therapy?		
No risk for a serious side effect from gene therapy	5	1.9%
A small amount of risk for a serious side effect	80	30.4%
A medium amount of risk for a serious side effect	109	41.4%
A large risk for a serious side effect from gene therapy	27	10.3%
I don't know	42	16.0%

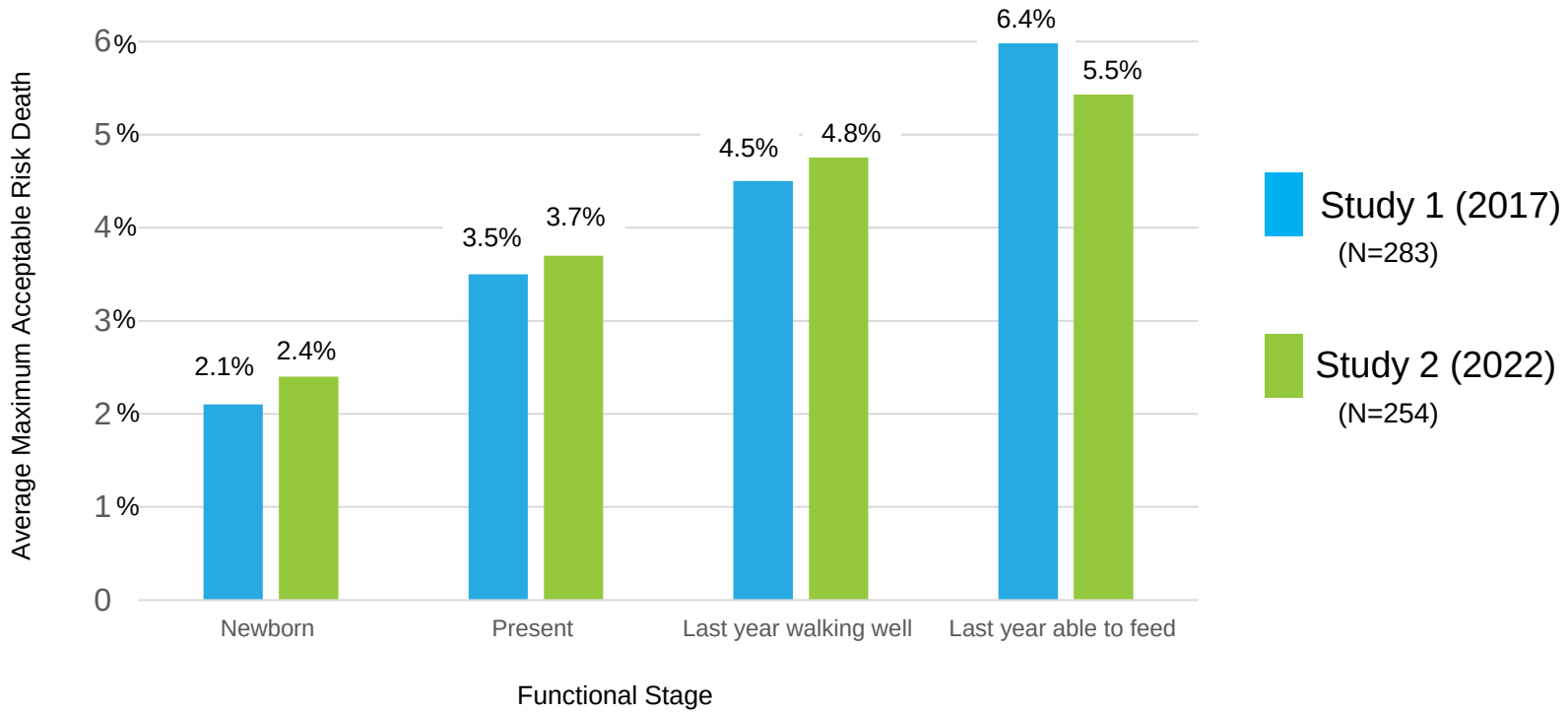
43% Some

49% Understand well

80% believe some level of benefit but not curative

70% believe small to medium amount of risk

Caregiver Average MAR of Gene Therapy-Related Death – **Study 1 vs. Study 2**



Post-Threshold Questions

Based on your own opinion, which is the worse risk of gene therapy {for you/your child}, the risk of death or the risk of lifelong kidney failure? If you used gene therapy right now:	**Caregivers (N=241)**	
	Mean (SD)	Median
	n	**%**
Death is the worse risk	103	42.7% ★
Needing lifelong dialysis because of kidney failure is the worse risk	24	10.0%
They are the same	103	42.7% ★
Missing	11	4.6%

Nearly Half: Death is worse
Nearly Half: They are the same

Real World Benefit-Risk Considerations

Reuters World Business Markets Sustainability Legal Breakingviews Technology Investigations

Sarepta says teen died after its gene therapy treatment

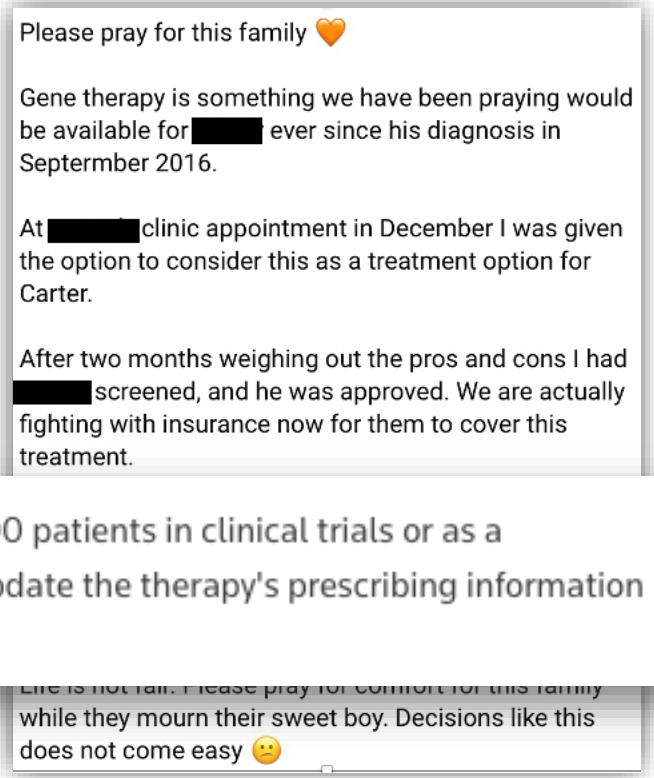
By **Sriparna Roy** and **Bhanvi Satija**

March 18, 2025 4:07 PM EDT · Updated a month ago

Summary Companies

- Patient was a 16-year-old teen, who underwent treatment in Dec
- First death reported after Elevidys treatment
- Liver injury a known risk with gene therapies

March 18 (Reuters) - Sarepta Therapeutics (SRPT.O) said on Tuesday that a 16-year-old boy died from acute liver failure months after receiving the company's U.S-approved gene therapy for a rare muscular dystrophy.



Please pray for this family 🧡

Gene therapy is something we have been praying would be available for [redacted] ever since his diagnosis in September 2016.

At [redacted] clinic appointment in December I was given the option to consider this as a treatment option for Carter.

After two months weighing out the pros and cons I had [redacted] screened, and he was approved. We are actually fighting with insurance now for them to cover this treatment.

while they mourn their sweet boy. Decisions like this does not come easy 😕

To date, Sarepta's therapy has been used to treat more than 800 patients in clinical trials or as a prescribed therapy, the company said adding that it plans to update the therapy's prescribing information to represent the death.

Considerations, implications, opportunities

- PCBR studies offer an opportunity for meaningful patient engagement throughout the process continuum (continuous learning)
  - Mixed methods allow for additional context
  - Supplement quantitative detail with qualitative context
- Competing influences include clinicians, presentations, and PPI (therapeutic misconception)
- Advocacy groups need to avoid raising unrealistic expectations with BR
- We need to be careful about how we construct vignettes
- Risk of changing behavior: Are these surveys interventions?
- Preferences are dynamic
- Potential opportunity to collect data from those who decided to take gene therapy – Full circle PCBR

4

Patient-Centricity in Health Preference Research is Improving- shared learnings in musculoskeletal conditions

Angie Botto-van Bemden, Patient Partner, Musculoskeletal Research International

- Notes to self: PPS QI c increased use of PPS guidance/checklists
- Additional room for PPS improvement adherence/affordability/appropriateness/impact/relevance/use in decision-making c PE/PRP/GRIPP guidance/checklists, collaboration/partnership throughout PFMD
- scorecards; PFDD, OA injection, implant approved/not, OP interventional threshold/adherence/relative imp., obesity, sarcopenia, lp(a), gene tx (ask others if want care/tx guidance exs or focus solely on PFMD)

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SYSTEMATIC REVIEW

"Improving," yet…

Stated Preferences of At-Risk Populations for the Treatment of Osteoporosis: A Systematic Review

Eva-Lotta Hinzpeter[1] · Lakshmi Nagendra[1,2] · Nadja Kairies-Schwarz[3,4] · Charlotte Beaudart[5] · Mickaël Hiligsmann[1]

Table 3 Quality assessment according to PREFS and ISPOR

Checklist	Item	Fraenkel, 2005 [25]	Fraenkel, 2006[a] [27]	Fraenkel, 2006[b] [26]	Fraenkel, 2007 [28]	de Bekker-Grob, 2008 [29]	Darbà, 2011 [30]	Silverman, 2013 [31]	Hiligsmann, 2014 [6]	Hiligsmann, 2017 [32]	Si, 2019 [33]	Cornelissen, 2020 [35]	Graham-Clarke, 2020 [34]	Beaudart, 2022 [36]	Liu, 2024 [37]
PREFS	Purpose	1	1	1	1	1	1	1	1	1	1	1	1	1	1
	Respondents	0	0	0	0	0	0	0	0	0	0	0	0	0	1
	Explanation	1	0	0	1	1	1	1	1	1	1	1	1	1	1
	Findings	1	1	1	1	1	1	1	1	1	1	0	1	0	1
	Significance	0	0	1	1	1	1	1	1	1	1	1	1	1	0
Total PREFS		3	2	3	4	4	4	4	4	4	4	3	4	3	4
ISPOR	1: Research question	3	2	2	3	3	3	3	3	3	2	2	3	2	3
	2: Attributes and levels	0	0	0	0	3	3	1	3	3	3	3	2	3	3
	3: Construction of tasks	1	2	2	1	3	3	2	3	3	3	3	3	2	3
	4: Experimental design	3	1	0	1	2	1	1	3	3	3	3	3	3	2
	5: Preference elicitation	1	1	1	2	3	2	2	3	3	3	3	1	3	1
	6: Instrument design	1	1	1	1	3	3	2	3	3	3	3	2	3	2
	7: Data collection	1	1	2	2	2	1	2	3	3	3	3	2	3	3
	8: Statistical analyses	1	1	1	2	3	1	2	3	3	2	3	2	3	2
	9: Results and conclusions	1	1	2	2	3	3	2	3	3	3	3	3	3	3
	10: Study presentation	2	2	2	2	3	3	2	3	3	3	3	3	3	3
Total ISPOR		14	12	13	16	28	23	19	30	30	28	29	24	28	25

Slide courtesy of Mickael Hiligsmann

Quality assessment tools used to evaluate preference-based studies.

The **PREFS** framework consists of five key criteria:

1. **Purpose** – Clarity of the study's objective.
2. **Respondents** – Appropriateness of the sample selection.
3. **Explanation** – Transparency in describing preference elicitation methods.
4. **Findings** – Reporting of results for the total sample.
5. **Significance** – Statistical testing and relevance of findings.

Each study is scored on a **5-point scale**, with **higher scores indicating better methodological quality**.

- **Score of 4 or above** is generally considered **high-quality**,
- while **below 4** may have methodological limitations.

Quality assessment tools used to evaluate preference-based studies.

The **2011 ISPOR checklist** on **Conjoint Analysis Applications in Health—A Checklist** provides **guidance on conducting and reporting conjoint analysis studies** in healthcare.

The checklist consists of **10 essential items** to ensure methodological rigor in **conjoint analysis**:

1. **Research Question** – Clearly define the study objective.
2. **Attributes and Levels** – Select relevant attributes and levels for preference elicitation.
3. **Construction of Tasks** – Design choice tasks that reflect real-world decision-making.
4. **Experimental Design** – Ensure a robust study design for valid preference estimation.
5. **Preference Elicitation** – Use appropriate methods (e.g., discrete choice experiments).
6. **Instrument Design** – Develop user-friendly and understandable survey instruments.
7. **Data Collection Plan** – Establish a structured approach for gathering patient preferences.
8. **Statistical Analyses** – Apply rigorous analytical methods to interpret results.
9. **Results and Conclusions** – Clearly report findings and their implications.
10. **Study Presentation** – Ensure transparent and accessible reporting.

50 *Bridges JFP, Hauber AB, Marshall D, et al. Conjoint analysis applications in health—a checklist: a Report of the ISPOR Good Research Practices for Conjoint Analysis Task Force. Value Health. 2011;14(4):403-413.*

Tool considerations for improving patient-centricity in patient preference studies.

ISPOR has developed a **Roadmap for Patient Preferences in Decision Making**, which includes **key questions** to guide researchers in assessing the **quality and impact** of patient preference studies. While ISPOR does not have a single standardized **quality assessment tool**, their **Good Practices Report** outlines five essential areas for evaluating patient preference research:

1. **Context** – Ensuring the study aligns with healthcare decision-making needs.
2. **Purpose** – Clearly defining the study's objectives and relevance.
3. **Population** – Selecting appropriate patient groups for meaningful insights.
4. **Method** – Using robust, fit-for-purpose preference elicitation techniques.
5. **Impact** – Assessing how findings influence healthcare decisions.

***These elements help researchers **critically appraise** patient preference studies and improve their **usefulness for decision-makers**.

51 Bridges JFP, de Bekker-Grob EW, Hauber AB, et al. A roadmap for increasing the usefulness and impact of patient-preference studies in decision making in health: a good practices report of an ISPOR task force. *Value Health*.2023;26(2):153–162.

Patient-Led Research Scorecards

Integration Into Research Process

-2 Non-collaboration	-1 Minimal collaboration	0 Acceptable collaboration	1 Great collaboration	2 Ideal collaboration
Hypothesis Generation				
Research goals are siloed from patients' priorities. Patients' questions and experiences are not included and/or are dismissed when generating research hypotheses.	Research goals attempt to involve patients' priorities, but limited by communication or collaboration. Patients' inquiries and lived experiences are rarely included when generating research hypotheses. Patients may have suggested the research question with no further involvement.	Research goals take into account patients' priorities. Patients' inquiries and lived experiences are included when generating research hypotheses.	Research goals proactively address patients' priorities with sufficient ongoing collaboration. Patient organization's inquiries and lived experiences are included when generating research hypotheses. Patient organizations work with patients to co-design research hypothesis.	Research goals are based on patients' priorities and co-written by patient organization or patient-researchers. Patient's inquiries and lived experiences share an equal weight with research organization's interests when generating research hypotheses.
Study Design				
Research organization does not include patients in the study design process. Patients do not have the opportunity to provide input on study design. Patient groups are utilized for recruitment purposes only, if at all.	Research organization does not include patients in the study design process. Patients may be invited to review study design but feedback is rarely incorporated and no functioning accountability system is in place.	Select patient voices are approached to inform the study design. Patients are invited to review study design and have an impact on the study design.	Patient organization and their community's input are proactively invited to help inform the study design. Patient organizations are invited to co-design and review study design and patient feedback changes the study design.	Study design is co-written and reviewed by a diverse array of patient-researchers representative of the study's sub-populations. If applicable, protocol testing is done by the patient community.
Analysis				
Patients do not have input in what data to prioritize for analysis and methods of analysis.	Patients are asked to review manuscript drafts but have little say in what data to prioritize for analysis and methods of analysis.	Patients are involved in interpreting data and carrying out analysis in some capacity.	Patients or patient organizations are invited and involved in interpreting data and carrying out analysis anywhere in the study.	Patient-researchers co-lead on the interpretation and analysis and/or work concurrently with partner organization's research team to carry out analysis.
Publication				
Study results are inaccessible to patients and/or behind an academic paywall. Findings are not communicated in lay terms.	Research organization summarizes findings in lay terms, but study results are inaccessible to patients and/or are behind an academic paywall.	Study results are freely accessible to patients and the public. Findings are summarized in lay terms in ways that are informative to the patient population.	Study results are freely accessible to patients and the public. Findings are summarized in lay terms and are actively disseminated to patient population. Patient-researchers co-write the interpretation and analysis.	Study results are freely accessible to patients and the public. Findings are summarized in lay terms and are actively disseminated to patient population. Patient organizations invite patients to co-write findings and reports. A channel of communication is available for patients to ask questions of the research organization.
Attribution				
Patients' work is attributed to others and/or patients are not attributed at all.	Patients are listed as being involved without a description of how they were involved. Patients were not consulted on how they prefer to be attributed.	Patients are acknowledged/ credited in major public facing communication (press, announcements, papers), to the extent that patients wish to be named. Patients were consulted on how they prefer to be attributed.	Patient group is credited in all public-facing communication and included as authors on papers, to the extent that the patient group wishes to be named. Patient group was consulted on how they prefer to be attributed.	Patients are acknowledged specifically for what they did throughout the engagement process, are credited in all public-facing communication, and included as authors on papers, to the extent that the patient group wishes to be named. Patient group was consulted on how they prefer to be attributed.

OA Patient Advisory Panel

-2 Non-collaboration	-1 Minimal collaboration	0 Acceptable collaboration	1 Great collaboration	2 Ideal collaboration

1. Osteoarthritis

ISPOR Mapping	MSK Example	Patient Engagement Example	PLRC Patient-Centric Example (Score of 2)
Context	Chronic joint condition affecting mobility and quality of life.	Patients provide insights on mobility challenges through surveys or focus groups.	Patients co-develop research priorities, ensuring mobility challenges are central to study design.
Purpose	Identify patient preferences for treatment options (e.g., pain management, surgery, lifestyle changes).	Patients rank treatment priorities via structured decision-making workshops.	Research integrates real-world patient narratives to tailor treatment recommendations.
Population	Patients with varying severity, age groups, and comorbidities.	Engaging diverse patient groups to ensure representation in research.	Study includes a diverse, representative patient cohort selected with patient input.
Method	Discrete choice experiments (DCEs) or qualitative interviews to assess preferences for medication, physical therapy, or surgical interventions.	Patients participate in interactive preference-elicitation exercises.	Patients contribute directly to the development of preference-elicitation tools and research protocols.
Impact	Inform shared decision-making tools for personalized treatment plans.	Co-development of decision aids for patient-provider discussions.	Final research outcomes drive changes in clinical guidelines reflecting patient priorities.

2. Osteoporosis

ISPOR Mapping	MSK Example	Patient Engagement Example	PLRC Patient-Centric Example (Score of 2)
Context	Bone fragility leading to fractures, particularly in older adults.	Patients share experiences of fractures and daily challenges via patient advisory panels.	Patients lead discussions on long-term quality of life and fracture prevention strategies.
Purpose	Understand patient preferences for prevention and treatment (e.g., medication, lifestyle modifications).	Patients provide feedback on treatment burden in patient forums.	Research outcomes prioritize patient concerns over medication side effects and adherence challenges.
Population	Postmenopausal women, older adults, and individuals with secondary osteoporosis.	Inclusion of patient advocacy groups to reflect diverse preferences.	Patient-reported outcomes shape inclusion criteria and study objectives.
Method	Preference elicitation through surveys or conjoint analysis to evaluate trade-offs between medication efficacy, side effects, and adherence.	Patients participate in structured exercises ranking preferences for treatment options.	Patients test and refine survey tools to ensure accessible and meaningful preference elicitation.
Impact	Improve adherence to osteoporosis treatment guidelines and enhance patient-provider discussions.	Development of patient-centered educational materials to improve adherence.	Patients co-design adherence interventions that improve health outcomes.

3. Sarcopenia

ISPOR Mapping	MSK Example	Patient Engagement Example	PLRC Patient-Centric Example (Score of 2)
Context	Age-related muscle loss affecting mobility and independence.	Patients discuss functional limitations and intervention preferences in focus groups.	Patients lead research efforts to define meaningful functional outcomes beyond traditional clinical markers.
Purpose	Assess patient priorities for interventions (e.g., resistance training, nutrition, pharmacological options).	Patients contribute to designing user-friendly exercise and nutrition programs.	Study prioritizes interventions based on patient-defined effectiveness and feasibility.
Population	Older adults at risk of frailty.	Patient representatives provide input on intervention acceptability and feasibility.	Patients shape study recruitment, ensuring diverse representation across risk groups.
Method	Mixed-method studies combining qualitative insights with quantitative preference assessments.	Patients participate in interviews to refine study methodologies.	Patients co-author research publications to ensure findings are accessible and actionable.
Impact	Guide personalized exercise and nutrition programs to maintain muscle function.	Patients help shape community-based sarcopenia prevention programs.	Study results directly shape clinical care practices for muscle health interventions.

Obesity

ISPOR Mapping	MSK Example	Patient Engagement Example	PLRC Patient-Centric Example (Score of 2)
Context	Chronic condition with metabolic and cardiovascular implications.	Patients share perspectives on barriers to weight management.	Patients define what constitutes success in obesity treatment beyond weight loss metrics.
Purpose	Explore patient preferences for weight management strategies (e.g., lifestyle changes, pharmacotherapy, bariatric surgery).	Patients co-design motivational interventions with healthcare providers.	Study incorporates lived experiences, prioritizing mental health impacts alongside physical outcomes.
Population	Individuals with obesity and related comorbidities.	Patient advocates ensure diverse perspectives in research.	Patients provide input on study recruitment strategies to ensure inclusive representation.
Method	Patient preference studies using best-worst scaling to rank treatment options based on effectiveness, safety, and accessibility.	Patients engage in ranking exercises to assess the relative importance of treatment options.	Patient-led evaluation ensures research recommendations align with real-life accessibility concerns.
Impact	Support individualized obesity management plans and improve patient engagement.	Creation of personalized digital health tools incorporating patient preferences.	Findings directly inform healthcare policies promoting patient-centered obesity care.

5. Atherosclerosis

ISPOR Mapping	MSK Example	Patient Engagement Example	PLRC Patient-Centric Example (Score of 2)
Context	Progressive arterial disease leading to cardiovascular events.	Patients provide insights on challenges related to disease prevention.	Patients define what “quality of life” means in prevention and post-event care.
Purpose	Understand patient preferences for prevention and treatment (e.g., statins, lifestyle modifications, surgical interventions).	Patients share experiences of medication side effects and adherence barriers.	Study focuses on patient-preferred treatment balancing long-term risk vs. immediate quality of life.
Population	Individuals with high cardiovascular risk profiles.	Patients help refine risk assessment tools for better engagement.	Patients co-develop risk communication tools to improve shared decision-making.
Method	Preference elicitation through decision aids and shared decision-making models.	Patients participate in interactive workshops to shape decision-making frameworks.	Patients actively shape how risk and treatment trade-offs are communicated in research.
Impact	Enhance adherence to cardiovascular risk reduction strategies and improve patient-provider communication.	Co-development of personalized risk reduction plans with patient involvement.	Study outcomes influence personalized care models based on patient-defined priorities.

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Q & A

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