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Longitudinal Qualitative Research: Methodological Approaches and Potential Applications to Patient-centered Outcomes Research

Session Date : Wednesday, 15 November 2023
Date/Time: Wednesday, 15 November 2023 | 8:00 - 9:00 CET
Session Location: Room C3, Bella Center Copenhagen

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Conflict of Interest Statement

- Carla Dias-Barbosa is an employee of and has stocks/shares in Evidera. Funding was not provided for this workshop.
- Vanessa Merker has received funding for longitudinal qualitative research studies from the Children's Tumor Foundation and Neurofibromatosis Northeast. Funding was not provided for this workshop.
- Eduard Sidelnikov is an employee of Amgen (Europe) GmbH and holds stock in Amgen Inc. Funding was not provided for this workshop.
- Kimmie McLaurin is an employee of and owns stock in AstraZeneca. Funding was not provided for this workshop.

Purpose

- Longitudinal qualitative research (LQR) is a relatively recent methodology in health research that can provide unique insights in clinical trial and real-world settings, such as:
  - Characterising a clinically meaningful change or treatment benefit for the population under study
  - Understanding disease trajectory to describe the course or progression of disease.
- However, LQR is underutilised and/or poorly reported in many domains of patient outcome research.
- Understanding the current state of LQR methods and potential applications can help guide the expansion of this methodological approach in the health outcomes research domain.

This workshop will provide insights on benefits and potential challenges of LQR and provide opportunities to learn about methodological approaches for collecting and analysing longitudinal qualitative data in clinical trials and real-world studies.

Key Learning Objectives

At the end of the workshop, participants will be able to:

- ✓ Understand the benefits of LQR data and its value in drug development and real-world settings.
- ✓ Understand methodological approaches to implement LQR and potential challenges.
- ✓ Discuss potential applications and outcomes of LQR in patient outcome research.

Agenda

- Introduction to LQR, potential applications, and value to stakeholders
- Designing LQR studies and practical challenges
- Case study on the use of longitudinal mixed methods approach to assess treatment benefit in migraine in the context of a clinical trial
- Real-world LQR opportunities using ovarian and breast cancer cases studies as examples
- Question and Answer

It’s Time for a Pool!

- Before starting, please participate in our polling session via ISPOR Europe 2023 mobile app.
- Navigate to the session and scroll down to Q&A/ Polling under “Resources”
- Select the current pool to respond.
- Results will show in real time.
- Results will be discussed at the end of the presentation.

Q1: Which Stakeholders in your opinion could use results of longitudinal qualitative studies?

- ❑ Regulators
- ❑ Payers
- ❑ Policy Makers
- ❑ Industry
- ❑ All of the above
- ❑ Other (please specify)

Q2: Have you ever conducted a longitudinal qualitative study?

- ❑ Yes
- ❑ No, but I am planning to/ would like to
- ❑ No

Q3: In what context have you conducted longitudinal qualitative research?

- ❑ Standalone longitudinal qualitative study
- ❑ Within a clinical trial
- ❑ Within a real-world evidence study
- ❑ Within a natural history study
- ❑ Other (please specify)

Q4: Main reasons for NOT conducting longitudinal qualitative research (LQR) studies

- ❑ Lack of awareness
- ❑ Lack of guidance for the design of LQR
- ❑ Lack of guidance on LQR data analysis
- ❑ Practical or operational challenges associated with LQR studies
- ❑ Other (please specify)

Introduction to LQR, potential applications, and value to stakeholders

Carla Dias-Barbosa, MSc, Evidera, London, UK

What is Longitudinal Qualitative Research?

- LQR, also called qualitative longitudinal research (QLR), defines qualitative studies with multiple data collection points that focus on temporality (e.g., time and change) of a phenomenon.
- LQR prioritises the study of change (or stability) using qualitative data material (e.g., interviews, observations and/or text documents) and explores the temporal dimension of experience, i.e., transitions, trajectories, and changes in people's health experiences.
- LQR also provides insights into the nature, causes, and consequences of change (or its absence).

Value of LQR

- While traditional qualitative research focuses on “how” and “why” healthcare is experienced, LQR focuses on “how” and “why” these experiences change (or stay the same) over time.
- There is an opportunity to use ***longitudinal mixed methods research design,*** combining the more standard longitudinal quantitative analysis approach, which can provide answers to *“what”* change question, with longitudinal qualitative analysis approach that can provide answers to *“how” and “why”* of the change.

Longitudinal mixed method design can be particularly helpful for assessing a health intervention across a trial as it can provide insight into what constitutes a meaningful change for study participants.

Quantitative data provides numerical change data (e.g., change in a clinical outcome assessment [COA] score based on statistical significance)

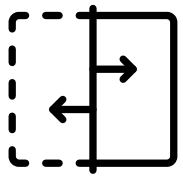
Qualitative data provides patients’ narratives explaining the context, the nature of the change, and if the change is meaningful to them

Traditional Qualitative Research vs. LQR

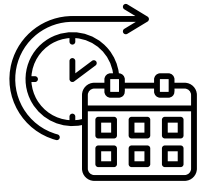
Considerations	Traditional Qualitative Research	Longitudinal Qualitative Research
Research focus	• Allows understanding of participants' experiences in **a specific time** and **context** or to understand prior experiences • Allows comparisons of the participants or groups of participants at a single point in time; "snapshot" of the population at a certain time	• Allows understanding of participants' or groups of participants' experiences **over time** • Allows qualitative comparisons of the same participants over a period of time • Detects changes in the target population at the group and individual levels • Establishes sequences of events and causes and consequences of changes
Study design	• Cross-sectional • Observational; one observation, e.g., one-time interview or focus group	• Longitudinal • Observational; at least two observations of the same participants over a period of time, e.g., serial or longitudinal interviews
Data collection	• Retrospective reports of previous experiences based on participant recall; **Can be influenced by recall bias**	• Implies returning to participants to explore changes as they occur in real time; **less recall bias**
Methodological approach	• Well-known • Analytic framework well-established	• Emerging methodology; lack of methodological clarity • Riche source of data but analysis is complex and multidimensional
Time	• Can be done quickly	• Longer process – follow-up assessment • Data analysis more complex and time-consuming

Analysis Approach of Longitudinal Qualitative Data

Two primary approaches to analyse longitudinal qualitative analysis



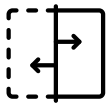
Recurrent cross-sectional analysis



Trajectory analysis

Grossoehme and Lipstein. (2016). Analyzing longitudinal qualitative data: the application of trajectory and recurrent cross-sectional approaches. BMC Res Notes.

LQR Analysis: Recurrent Cross-sectional Analysis



- Explore themes and changes over time at the level of whole sample (or subsample)
- Describe the similarities and differences (themes/ concepts) between two timepoints
- Can start analysis as each timepoint is completed

Patient ID	Time 1	Time 2	Time 3
XX-XXX	✓	✓	✓
XX-XXX	✓	X	✓
XX-XXX	✓	✓	X
XX-XXX	✓	✓	✓
XX-XXX	✓	✓	✓
XX-XXX	✓	X	✓
XX-XXX	✓	✓	✓
XX-XXX	✓	✓	✓
XX-XXX	✓	✓	X
XX-XXX	✓	✓	✓
XX-XXX	✓	✓	✓
XX-XXX		✓	X
XX-XXX		✓	✓
	CSA 1	**CSA 2**	**CSA 3**

✓patient interviewed

X patient not interviewed *(e.g., patient may die or is too unwell to participate)*

Abbreviation: CSA = Cross-Sectional Analysis

LQR Analysis: Trajectory Analysis

- Focuses on changes over time for an individual or small groups of individuals (e.g., families)
- Allows to understand individuals' experiences over time
- Allows comparison within each individual and comparison between individuals.
- Can NOT start analysis until data are collected at all timepoints

Patient ID	Time 1	Time 2	Time 3
XX-XXX	✓	✓	✓
XX-XXX	✓	X	✓
XX-XXX	✓	✓	✓
XX-XXX	X	✓	✓
XX-XXX	✓	✓	✓
XX-XXX	✓	X	✓
XX-XXX	✓	✓	✓
XX-XXX	X	✓	✓
XX-XXX	✓	✓	X
XX-XXX	✓	✓	X
XX-XXX	✓	✓	✓
XX-XXX	✓	✓	X
XX-XXX	✓	X	✓
…			

✓patient interviewed

X patient not interviewed *(e.g., patient may die or is too unwell to participate)*

Potential Applications of LQR

NATURAL HISTORY STUDIES

Understanding the disease

- Understand the progression of a disease process over time, in the absence of treatment

✓ **Particularly useful in rare disease**

CLINICAL TRIALS (PHASE II, III)

Characterisation of treatment benefit and support Interpretation of trial outcomes

- Characterise the treatment benefit
  - Onset, duration, and magnitude of the effect
- Identify what constitutes a meaningful effect/change
- Understanding the process of change
- Mixed method analysis to help interpret a clinically meaningful change in trial outcomes
- Supportive data for risk-benefit assessment

✓ **Generate sound value messages for payers, physicians, and patients based on direct input from patients**

PHASE IV, POST-MARKETING

Product safety and market surveillance

- Detect long-term effects of treatment
- Explore long-term experience of living with new treatment
- Explore adherence and compliance to treatment

REAL-WORLD EVIDENCE STUDIES

Understanding the patient's journey

- Understand patient experiences with their disease and treatments over time
- Identify unmet medical needs

Longitudinal Qualitative Data is Relevant to Various Stakeholders

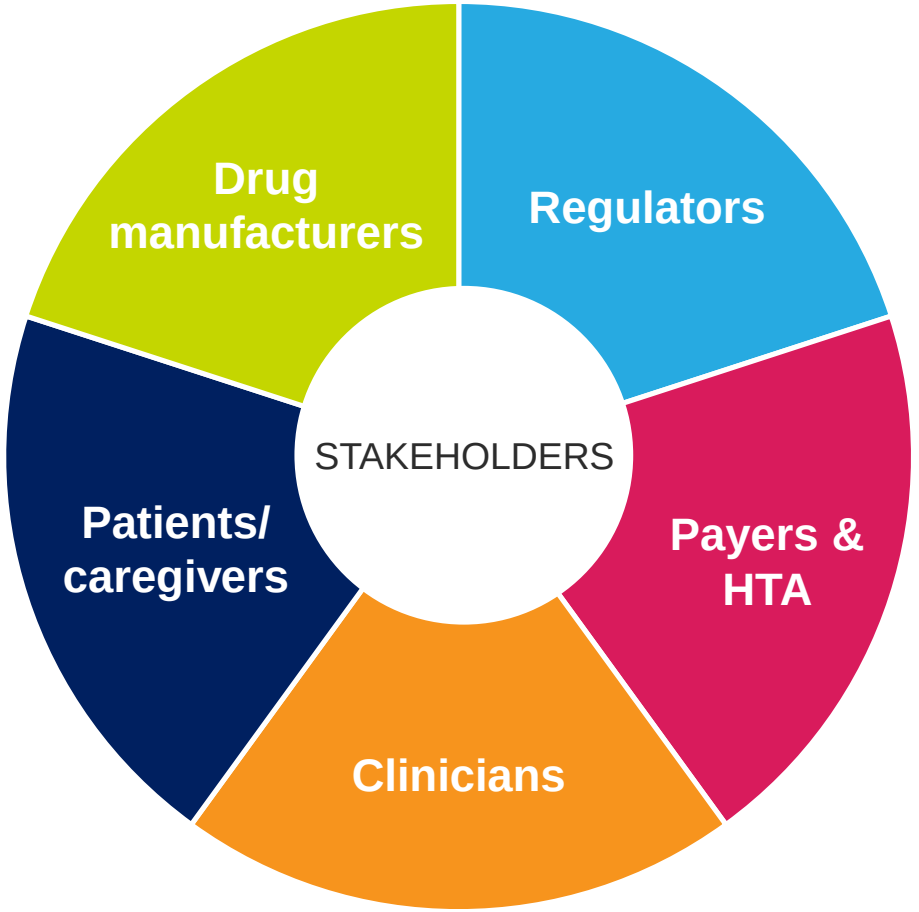
- Increase knowledge of the disease and understand patient unmet needs
- Identify health outcomes relevant to patients
- Address regulators' requests

- Support interpretation of clinical trial findings (e.g., a clinically meaningful change/benefit)
- Incorporate patient experience and needs into regulatory decisions

- Develop better value messages on treatment benefit, treatment satisfaction, and patient/caregiver burden to inform reimbursement decision-making

- Better understand patient experience over the course of the disease
- Improve patient-clinician communication
- Support individualised treatment decisions

- Ensure that patient experience and needs are meaningfully incorporated into decisions
- Improve quality of care



Abbreviations: HTA = health technology assessment

Designing LQR studies and practical challenges

Vanessa Merker, PhD, Massachusetts General Hospital and Harvard Medical School, MA, USA

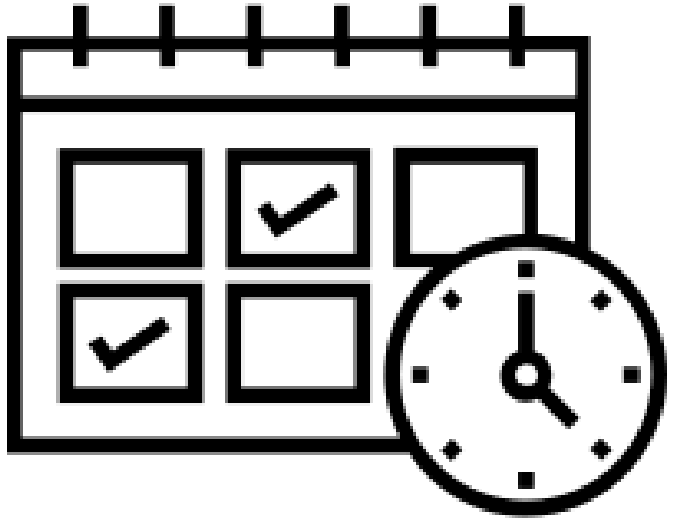
Selecting Data Collection Timepoints

Length of Data Collection (Timeframe)

- Fixed vs. flexible timeframes

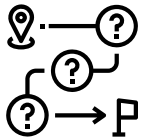
Number and Timing of Interviews (Tempo)

- Researcher-led events
- Participant-led events
- Regular time intervals



Wanat et al. (2021) Fam Med Com Health.

Special Timing Considerations for LQR in Clinical Trials



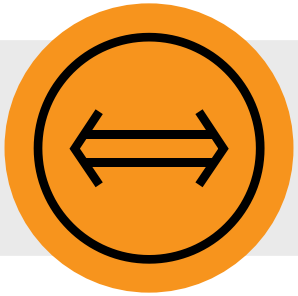
Potential Challenges

- Difficulty recruiting for baseline interviews due to participant overwhelm
- Tight time windows for baseline interviews
- Need to maintain trial integrity and reduce bias
- Difficulty retaining participants over time
- Missing data and/or higher attrition in subgroups with more aggressive disease or more side effects

Recommendations

- Be sensitive and clear in approach; consider whether interviews should be optional or required
- Good inter-site communication; be flexible in weekend/after-hours scheduling
- Complete interviews after patient-reported outcomes and other quantitative data; exit interviews should be prior to participant unblinding
- Use multiple contact methods; plan for participant engagement
- Allow participants who miss a timepoint to complete rest of study; plan for data collection upon early trial discontinuation

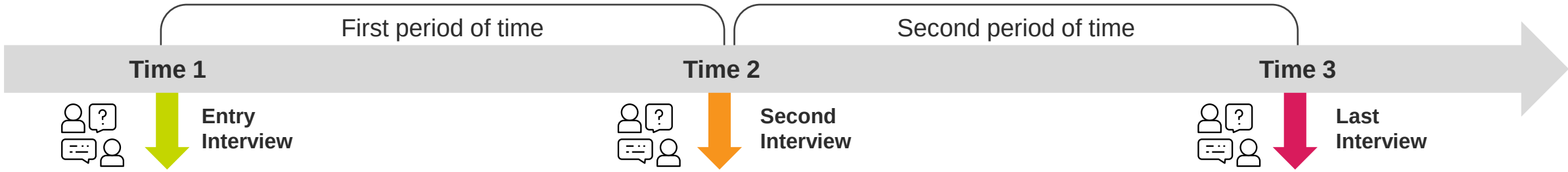
Developing Interview Guides



Same topics or **different topics** across timepoints?

Standard or **personalised** questions in follow-up interviews?

Examples of Longitudinal Interview Questions for Trajectory Analysis



At the beginning of the interview, the interviewer can summarise the symptoms and impacts reported during the previous interview to minimise recall bias.

What symptoms did you have before you started the current study?

What were the worst symptom/ the worst impact?

Before the start of the study, how did your symptom(s) impact your daily life/activities?

How often do you experience this *symptom*?

*How did your symptom(s)/impact(s) change during the **period 1** of the study? (e.g., worsen, improve, stay the same)*

*Did you experience any **new** symptom or impact?*

*Was the change **important or meaningful** to you?*

*Has any symptom or impact been **resolved**?*

*How did your symptom(s)/impact(s) change during the **period 2** of the study? (e.g., worsen, improve, stay the same)*

How satisfied are you with the treatment or intervention you received in this study?

Developing Follow-up Interview Guides

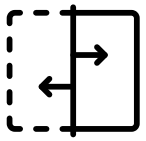
	Standardised Follow-up Questions	Personalised Follow-up Questions
Advantages	• Facilitates cross-sectional analysis • Often adequate for domain- or group-level analysis • Takes less time to prepare for interview and possibly to conduct analysis	• Facilitates trajectory analysis • Facilitates concept-level analysis • More precise and comprehensive description of change over time
Disadvantages	• May miss follow-up of symptoms/ impacts important to each individual • May be more difficult to describe precise changes over time within individuals	• Requires time for interviewer to review previous interviews to tailor questions • May require ethics amendments if substantial changes in topics covered

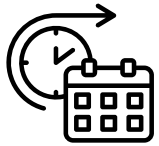
Example of Trajectory Data Analysis

- Typical interview questions seek to understand how data collected at each timepoint relates to data from the other timepoints (e.g., defining when exactly changes occurred and in what context)
- Each theme/concept identified during the first timepoint is probed during subsequent timepoints

	Time 1	Time 2	Time 3
01	• **A lot of itchiness**; this is very irritating, itch impacts sleep and causes redness	• **Still present**, but itch improves after four weeks of treatment. Able to sleep most of the night. The change was meaningful and improve the quality of sleep.	• **Still present, stable since Time 2**
02	• **Itch is extremely painful**; itch is present all the time and affects sleep a lot	• **Slightly better; but itch is still present** and cause sleep disturbance. The change was somewhat meaningful.	• **No change since Time 2.** Itch still present but not as painful as study entry
03	• The severity of **itch is moderate**	• **Resolved** after eight weeks of treatment	• **Stable since Time 2.** Complete resolution of itch.

Choosing Your Analysis Approach





	Recurrent cross-sectional analysis	Trajectory analysis
Research focus	• Describe the differences between timepoints	• Describe how processes or experiences change over time
Study sample	• The cohort at each timepoint may be the same or different	• Must maintain same cohort
Data collection	• More standardised topic guide	• More personalised topic guide
Level of analysis	• Whole sample (or subsamples)	• Individual people or individual groups (e.g., families)
Timing of analysis	• May analyse as each timepoint is completed	• Can't finalise data analysis until data is collected at all timepoints
Types of themes	• Themes tied to timepoints	• Themes spanning times

Grossoehme and Lipstein. (2016). BMC Res Notes

Dealing with Large, Complex LQR Data Sets

Importance of data management plans

Leaving enough time for analysis

Planning for staff transitions

Ethical Issues in LQR

Consent

Confidentiality

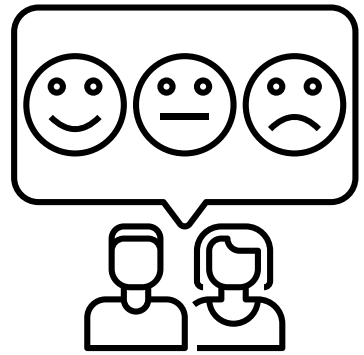
Long-term relationships

Calman et al. (2013). BMC Med Res Methodol.

Longitudinal Relationships between Participants and Researchers

- Interacting with participants over time can build trust and encourage disclosure.
- There can be blurring of boundaries as individuals develop stronger relationships.

- Have plans to manage participant-initiated contacts, especially about medical information/advice.
- Prepare interviewers to manage difficult topics and emotions, esp. if participants are expected to experience significant health decline or death
- Have plans for ending the relationship.



Current Research Initiatives

- Two research initiatives conducted as part of the Mixed Methods Research Special Interest Group of International Society for Quality of Life Research (ISOQOL)

1. **Scoping Review: Longitudinal qualitative methodological review**
    - Five databases (MEDLINE, CINAHL, PsycINFO, Academic Search Complete, EMBASE)
    - No time limit
    - Yielded 10,110 references; 93 studies selected for data extraction
    - Data analysis ongoing; manuscript coming soon

2. **Scoping Review : Longitudinal qualitative research methods in clinical trials**
    - Five databases (MEDLINE, CINAHL, PsycINFO, Academic Search Complete, EMBASE)
    - Published January 1, 2018 – May 12, 2023 [date of search]
    - Yielded 627 studies
    - Abstract screening complete; Full text review ongoing

Additional Resources and References

- Calman et al. (2013) Developing longitudinal qualitative designs: lessons learned and recommendations for health services research. BMC Med Res Methodol.

- Grossoehme and Lipstein. (2016). Analyzing longitudinal qualitative data: the application of trajectory and recurrent cross-sectional approaches. BMC Res Notes.

- Wanat et al. (2021) Value, challenges and practical considerations when designing, conducting and analysing a longitudinal qualitative study in family medicine. Fam Med Com Health.

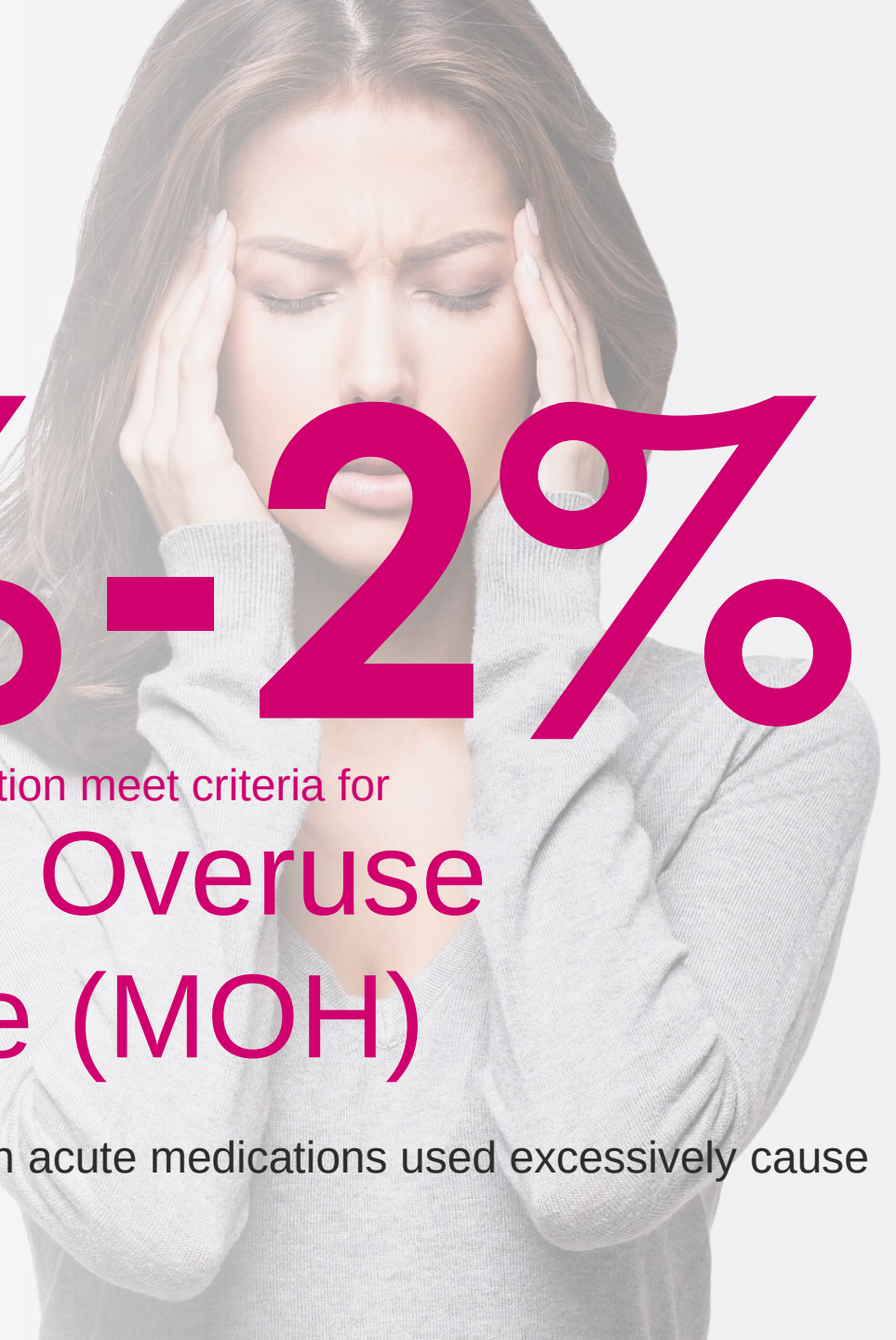
- Tuthill et al. (2020). Longitudinal Qualitative Methods in Health Behavior and Nursing Research: Assumptions, Design, Analysis and Lessons Learned. Int J Qual Methods.

- Johnny Saldaña. Longitudinal Qualitative Research: Analyzing Change Through Time. AltaMira Press, 2003.

Case study on the use of longitudinal mixed methods approach to assess treatment benefit in migraine in the context of a clinical trial

Eduard Sidelnikov, MD, PhD, Global Health Economics and Outcomes Research, Amgen (Europe) GmbH, Rotkreuz, Switzerland

MIGRAINE



1 Billion

Patients suffering worldwide

1%-2%

of adult Western population meet criteria for

Medication Overuse Headache (MOH)

MOH is a chronic daily headache and a secondary disorder in which acute medications used excessively cause headache in a headache-prone patient*.

* Kristoffersen ES, Lundqvist C. Medication overuse headache: a review J Pain Research 2014;7:367-378.

Benefits of Longitudinal Qualitative Interviews

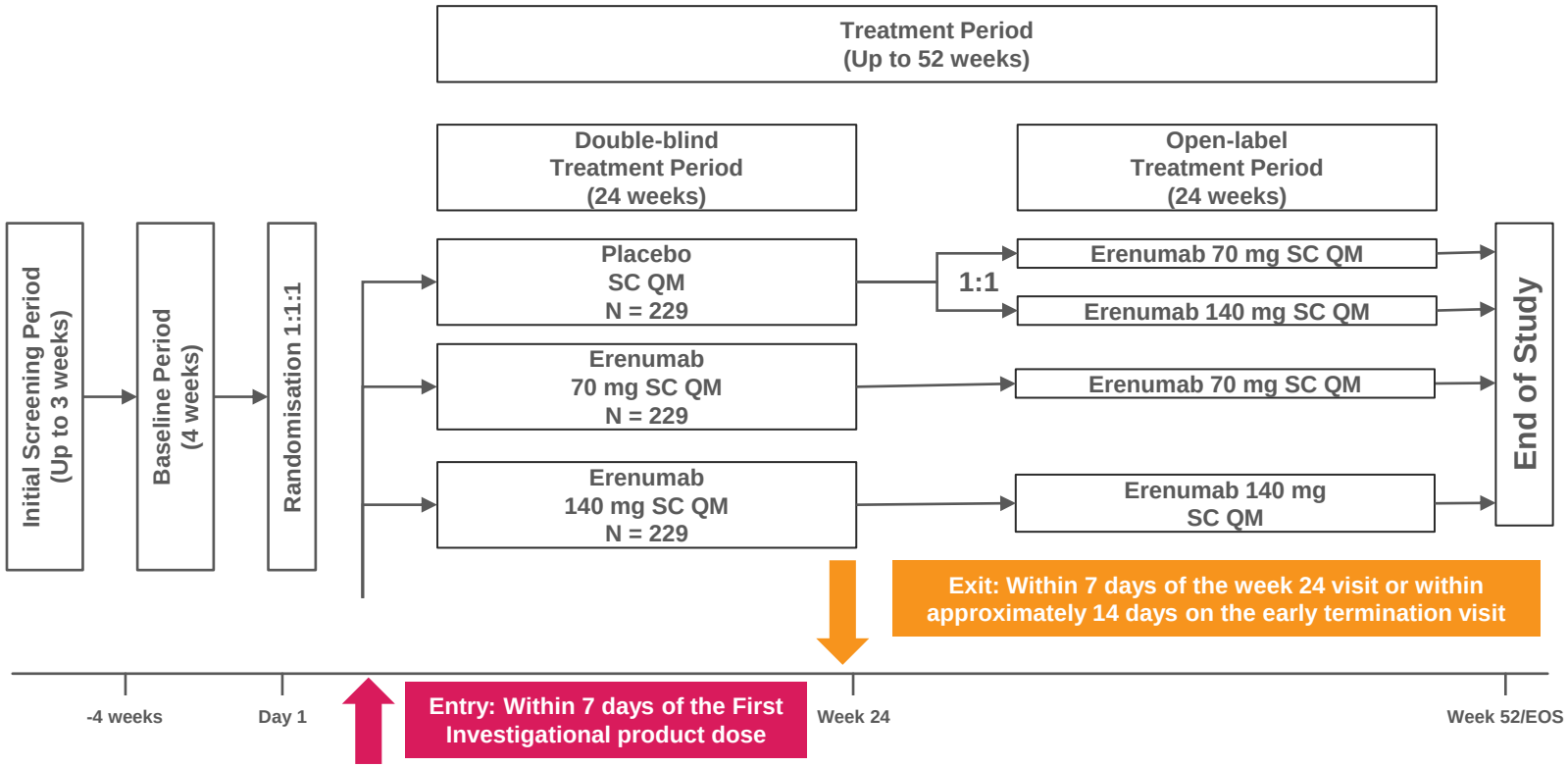
- Regulatory agencies have shown increased interest in understanding patients' experiences of the risk-benefit related to new interventions being tested in clinical trials (1).
- Qualitative interviews help understand a patient's experience of the treatment in the clinical trial setting and help identify benefits most relevant and meaningful to patients.
- Presence and severity of symptoms in some diseases (such as migraine) are difficult or impossible to measure by means other than interviews or questionnaires.
- Qualitative interviews can also be used to assess overall patient satisfaction with the treatment and factors contributing to the satisfaction.
- Qualitative interviews can help generate value messages for the product.

1. Food and Drug Administration. Patient-Focused Drug Development: Methods to Identify What Is Important to Patients Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders. 2022; https://www.fda.gov/media/131230/download; Accessed on April 24, 2023

Phase IV Clinical Trial to Evaluate Safety and Efficacy of Erenumab

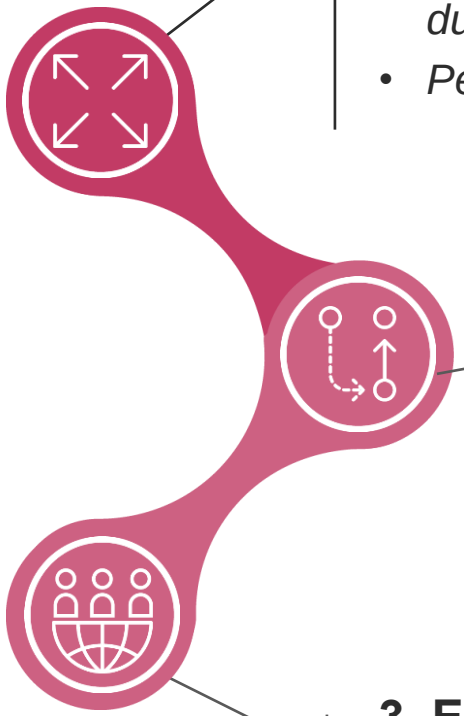
Phase IV, randomised, double-blind, double-dummy, parallel-group, placebo-controlled study to evaluate the safety and efficacy of erenumab against placebo in a chronic migraine population with MOH and prior history of treatment failure

Study Schema



Abbreviations: EOS = End of study; QM = monthly; SC = subcutaneous

Qualitative Longitudinal Interview Sub-study: Objectives



1. Evaluate individual subjects' experience related to migraine

- *Impact of migraine on individual functioning at the start of the study, e.g., migraine experience during and between migraine attacks*
- *Perceived changes in the individual experience of migraine during the study*

2. Evaluate individual subjects' experience with the study treatment

3. Explore subjects' experience with the clinical trial

- *Hopes and experiences of participating in the clinical trial*

Qualitative Longitudinal Interview Sub-study: Methods

Study population: convenience sample of clinical trial participants who opted to participate in the qualitative longitudinal interview sub-study

Inclusion criteria:

- Randomised in “parent” clinical trial and have successfully received a first dose of investigational product (IP)
- Have provided supplementary informed consent to participate in the sub-study
- Be willing and able to participate in two telephone interviews lasting approximately one hour in duration each

Exclusion criteria:

- Unable to complete entry interview within seven days from first IP dose
- Unable to complete exit interview within seven days prior to week 24 visit or within approximately 14 days post early termination visit

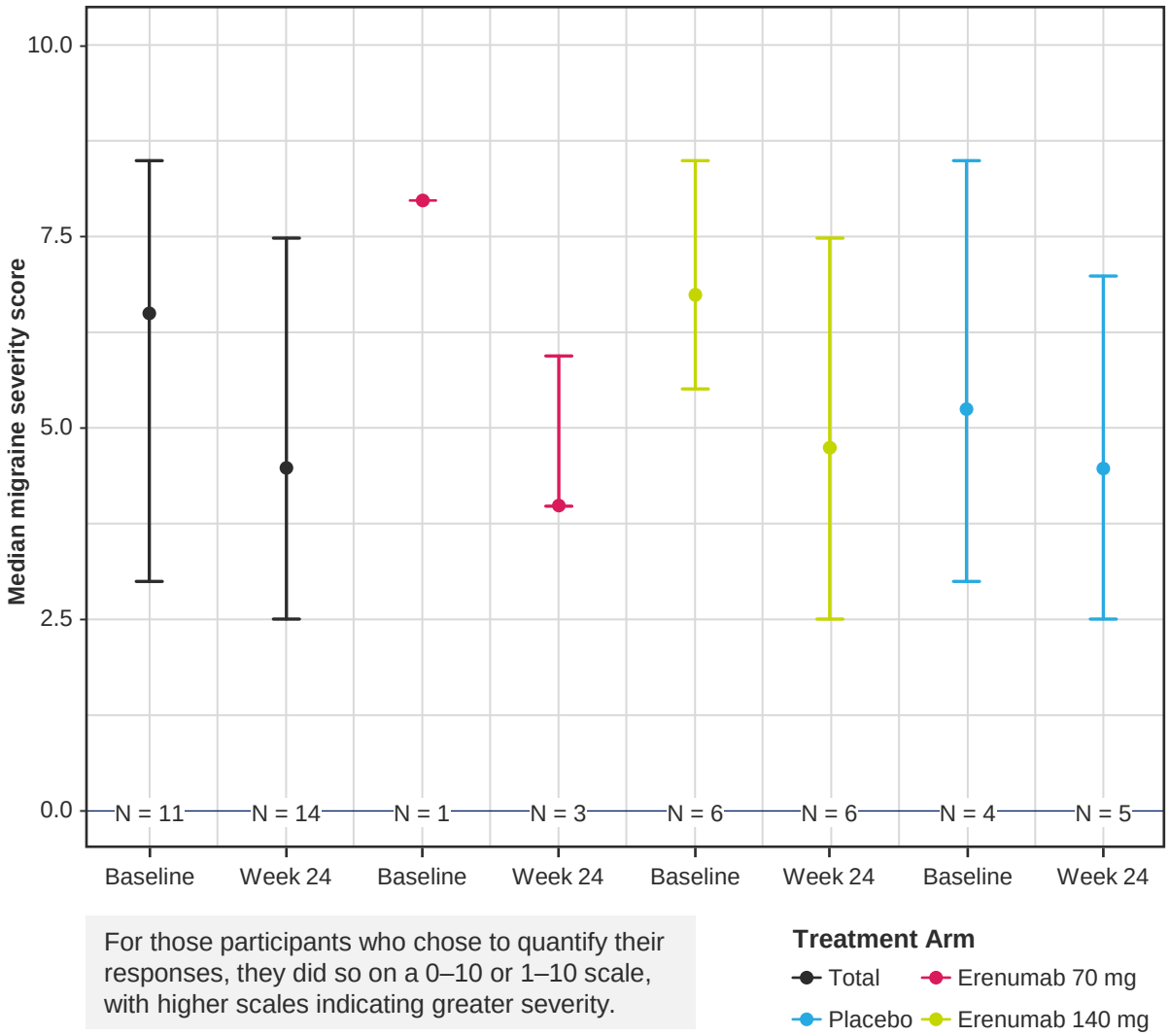
Example Interview Guide

While the semi-structured interview guide was developed comprehensively with both interview timepoints included, exit interviews were specifically tailored to reference the individual subject's responses to similar questions presented during the entry interview to allow for detailed comparisons and discussion on subject-by-subject basis.

Impact Description	Subject Example or Terminology	Endorsed at Entry Interview[1]	Selected as top 3 at Entry?[2]	Change in impact of migraine on functioning after starting the study?	Endorsed at Exit Interview[1]	Selected as top 3 at Exit?
Ability to move head		☐ S ☐ P ☐ N/A	☐	N/A	☐ S ☐ P ☒ N/A	☐
Ability to move body (for example, standing up, walking, bending)		☐ S ☒ P ☐ N/A	☐	☒	☐ S ☐ P ☒ N/A	☐
Ability to get in and out of bed	Doesn't want to move	☒ S ☐ P ☐ N/A	☒	☒	☐ S ☐ P ☒ N/A	☐
Ability to stand up	gets dizzy	☒ S ☐ P ☐ N/A	☐	☒	☐ S ☐ P ☒ N/A	☐
Ability to bend over	feels nauseous	☒ S ☐ P ☐ N/A	☐	☒	☐ S ☒ P ☐ N/A	☐
The 3 most significant impacts of migraines on <u>physical functioning</u> that the subject would like to change:		1. Wants to feel well enough to get out of bed and do activities 2. Wants to feel 'normal'; walk around without dizziness or nausea 3. Wants to be able to go through the day without having to rest or lie down because of headache				

These are example responses not based on any individual participant's responses; this not a sample from an actual participant.

Change in Overall Migraine Severity from Entry to Exit by Treatment Group



For those participants who chose to quantify their responses, they did so on a 0–10 or 1–10 scale, with higher scales indicating greater severity.

Entry: On average, how severe would you say your migraines are?

Exit: How severe were the migraines that you're having?

Erenumab 70 mg group: illustrative patient's quote

Entry: I had 2–3 severe migraines in the same week where normally I would have, I would say, two migraines a week and **the severity would be bedridden symptoms**.

Exit: I would say that they were on a scale of 1–10, 10 being the worst, I would say that one of them was a 5, and then I did actually have one last night, early this morning, and that one was probably closer to about a 7. But I went to bed and I woke up, and I didn't have one. So that was good.

Erenumab 140 mg group: illustrative patient's quote

Entry: I would say on scale of 1–10, they would be 7–8…And when I say 7–8, maybe one time it could be like a 9–10, and another time maybe it would be like a 4–5. But you have a headache that's a 4–5 level and if that lasts all day, it's trying on you when it just doesn't give up.

Exit: Not severe, so if my other migraines were a nine, these were like a two or three.

Placebo group: Illustrative patient quotes

Entry: Like on a scale of 1–10 they're probably a 6 or 7.

Exit: Probably like a 7 out of 10.

Change in Emotional Functioning Concepts and MFIQ Emotional Functioning Domain at Patient Level

Patient ID	Perceived changes from study entry to study exit on emotional functioning domains (from qualitative interviews)					From qualitative interviews	From clinical trials
	Feeling worried	Feeling like a burden	Feeling lack of control of life	Feeling frustrated	Disappointed	Overall perception of meaning change	MFIQ emotional function score change from baseline*
703-66032-003						Perceived meaningful improvement	-45
703-66031-004						Not asked	+20
703-66032-004	Not experienced	Not experienced	Not experienced		Not experienced	Perceived meaningful improvement	0

* MFIQ physical function score includes 5 items including Q1: frequency of limitation to movement of head, Q2: frequency of limitation to movement of body; Q3: frequency of limitation to usual activities requiring physical efforts, Q4: frequency of needing to rest or lie down, Q5: frequency of feeling tired to do things. Domain score is transformed to a 0-100 scale, where a higher score value indicated greater impact of migraine. Negative change indicated an improvement.
Abbreviation: MFIQ = Migraine Functional Impact Questionnaire

Change in Emotional Functioning Concepts and MFIQ Emotional Functioning Domain at Patient Level

Patient ID	Perceived changes from study entry to study exit on emotional functioning domains (from qualitative interviews)					From qualitative interviews	From clinical trials
	Feeling worried	Feeling like a burden	Feeling lack of control of life	Feeling frustrated	Disappointed	Overall perception of meaning change	MFIQ emotional function score change from baseline*
703-66031-015	I think it has gotten better. Like I worry less about them because they are happening less often, so, you know, I worried a lot about how it was impacting my kids more so than anything, and, you know, we've, we've had a lot more time together, and my headaches haven't taken up so much of our time. So yeah, I'm worrying less.	Interviewer: So this was what came up at the entry interview was feeling like a burden on others because of migraine, which you said you had occasionally… Have you noticed any changes in those? 703-66017-015: No. I think it's kind of like misconception on my husband's behalf. I feel like I'm in this trial, so I shouldn't be getting headaches anymore.	Yeah, I think it has gotten better, because they are happening less frequently, so -- Interviewer: Okay. And again, that's an area where at entry you didn't indicate an impact in that area, but now it sounds like at exit you do feel like you are seeing some improvement there because you are having less frequency. Am I understanding correctly? 703-66017-15: Yes.	I'm getting definitely less frustrated, because of fewer migraines.	But I also as far as disappointment goes, that actually has increased... Because when I do, when I go like a week without a migraine, it's like, okay, well maybe I am getting medication, maybe it's working, and it will just come back. And so that is like exceptionally disappointing.	Not asked	+20

* MFIQ physical function score includes 5 items including Q1: frequency of limitation to movement of head, Q2: frequency of limitation to movement of body; Q3: frequency of limitation to usual activities requiring physical efforts, Q4: frequency of needing to rest or lie down, Q5: frequency of feeling tired to do things. Domain score is transformed to a 0-100 scale, where a higher score value indicated greater impact of migraine. Negative change indicated an improvement.
Abbreviation: MFIQ = Migraine Functional Impact Questionnaire

Conclusions

- Longitudinal qualitative interviews are an important and useful tool to support clinical trial results, especially for conditions like migraine where subjective symptoms and associated impact are the most important components of the disease.
- The method allows for complementary evaluation of patients' experiences and describes the journey from patient's perspective.
- Data from qualitative research can be used to support health technology assessment submissions and interactions with payers especially for diseases where debilitating symptoms and associated impact are an important part of clinical presentation and ongoing assessment of care.

Real-world LQR opportunities using ovarian and breast cancer case studies as examples

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Opportunities for Longitudinal Qualitative Research

- LQR has an important role in understanding how and why health issues change over time.
- LQR is less common than one-time qualitative studies, potentially due to increased methodological complexity, concerns about recruiting/retaining people over time and higher budget requirements (Carduff et al. 2015).
- Real-world longitudinal observational studies provide an opportunity to conduct mixed-methods research, collecting quantitative and qualitative patient data.
- Two recent examples of ovarian and breast cancer longitudinal studies will be presented.

Carduff E, et al. BMC Res Notes. 2015 Apr 11;8:142. doi: 10.1186/s13104-015-1107-y.

Longitudinal Study of Women Newly Diagnosed with Ovarian Cancer

- Mixed-methods, longitudinal observational study to assess PROs, physical activity, and sleep patterns of women with newly diagnosed epithelial ovarian cancer (N=225).
  - A subset of patients included in qualitative interviews (N=40).
- Site-based study (two cancer centres)
- Patients followed until disease recurrence or up to 24 months.

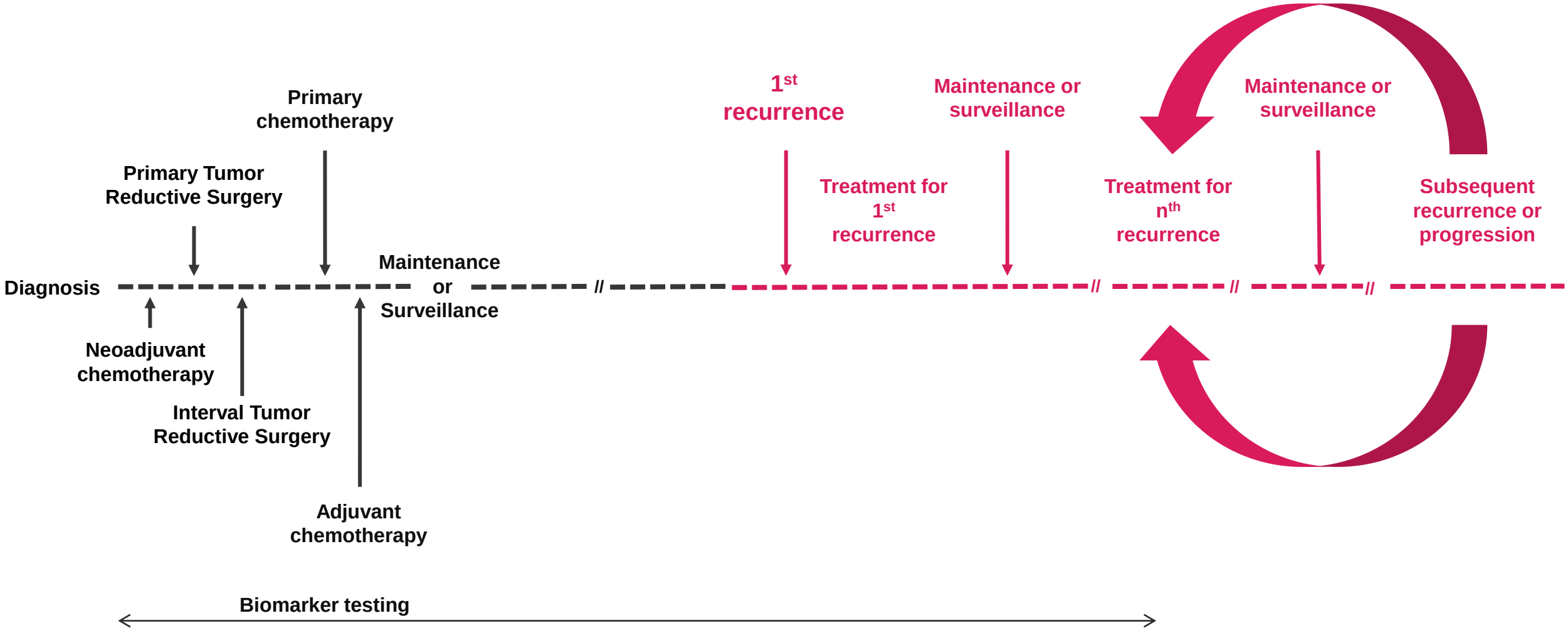
Study Objectives

Longitudinal assessment of symptom burden by treatment pathway

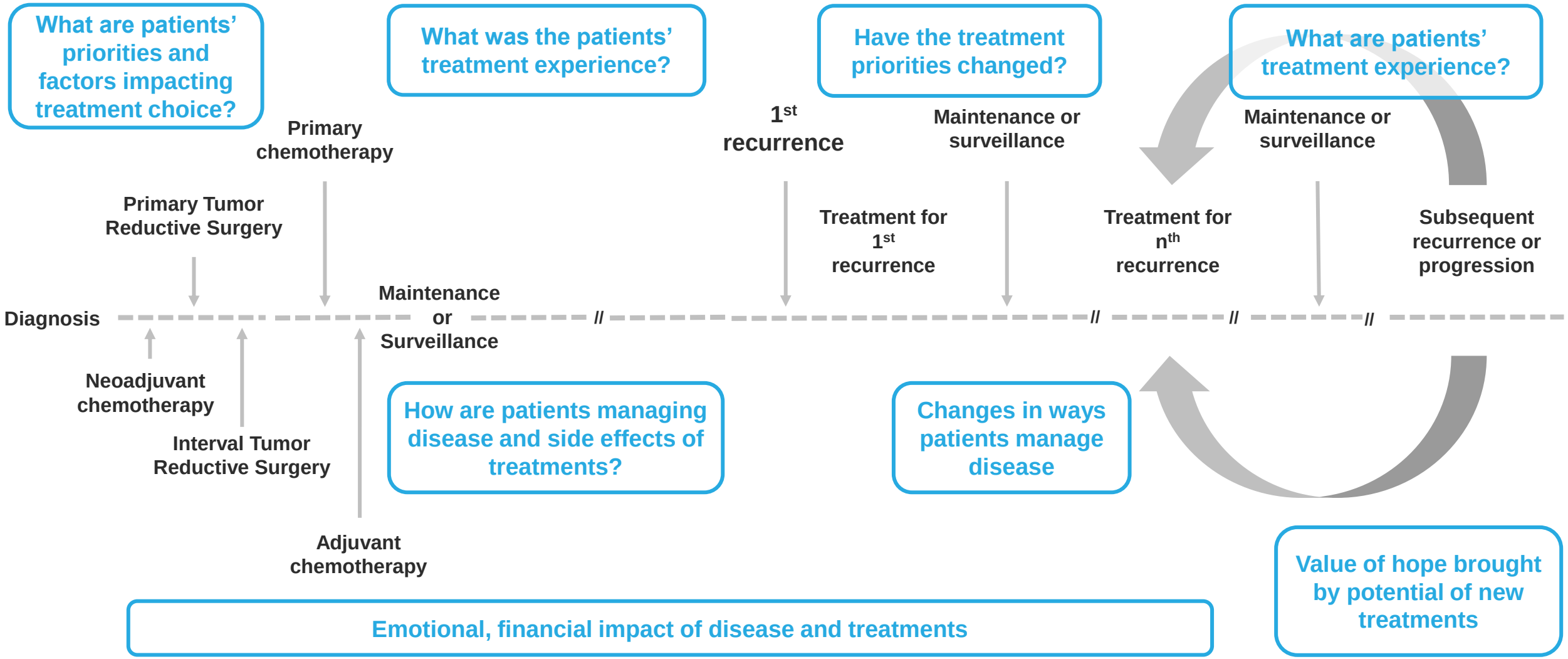
Assess daily physical activity and sleep patterns and their associations with the symptom burden profiles

Understand financial toxicity, factors impacting treatment decisions and disease- and treatment-related impact on daily life

Ovarian Cancer Patient Journey



Qualitative Research Questions of Interest



Registry for Women Diagnosed with Early Breast Cancer (stage I-III)

- Registry of patients with early breast cancer to evaluate impact of emerging therapies and novel biomarkers on patient and physician perceptions and clinical outcomes (N=3,000).
  - A subset of patients included in qualitative interviews (N=tbd).
- Patient-based registry identified through advocacy organisations, physician referral, and digital marketing. Initial study planned for eight years.

Study Objectives

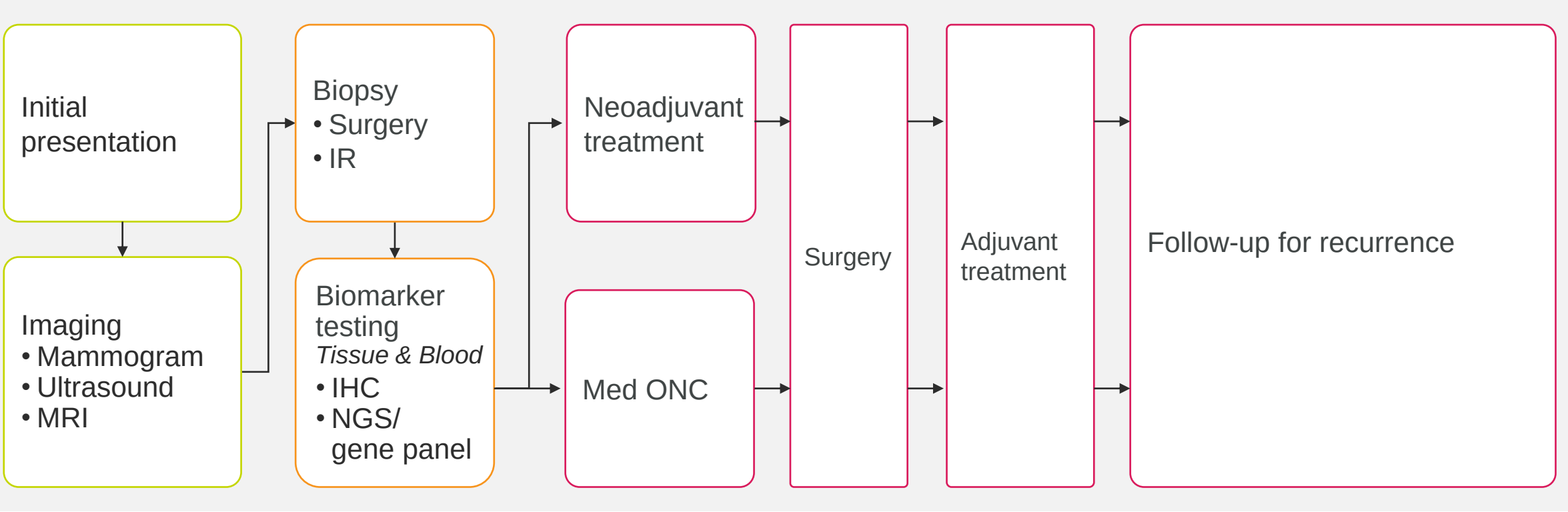
Describe demographic and clinical characteristics of patients with early breast cancer

Describe biomarker testing, treatment patterns, and clinical outcomes during the early breast cancer setting and in the metastatic breast cancer setting, for patients who progress

Assess patient perception of their adjuvant treatment experience overtime

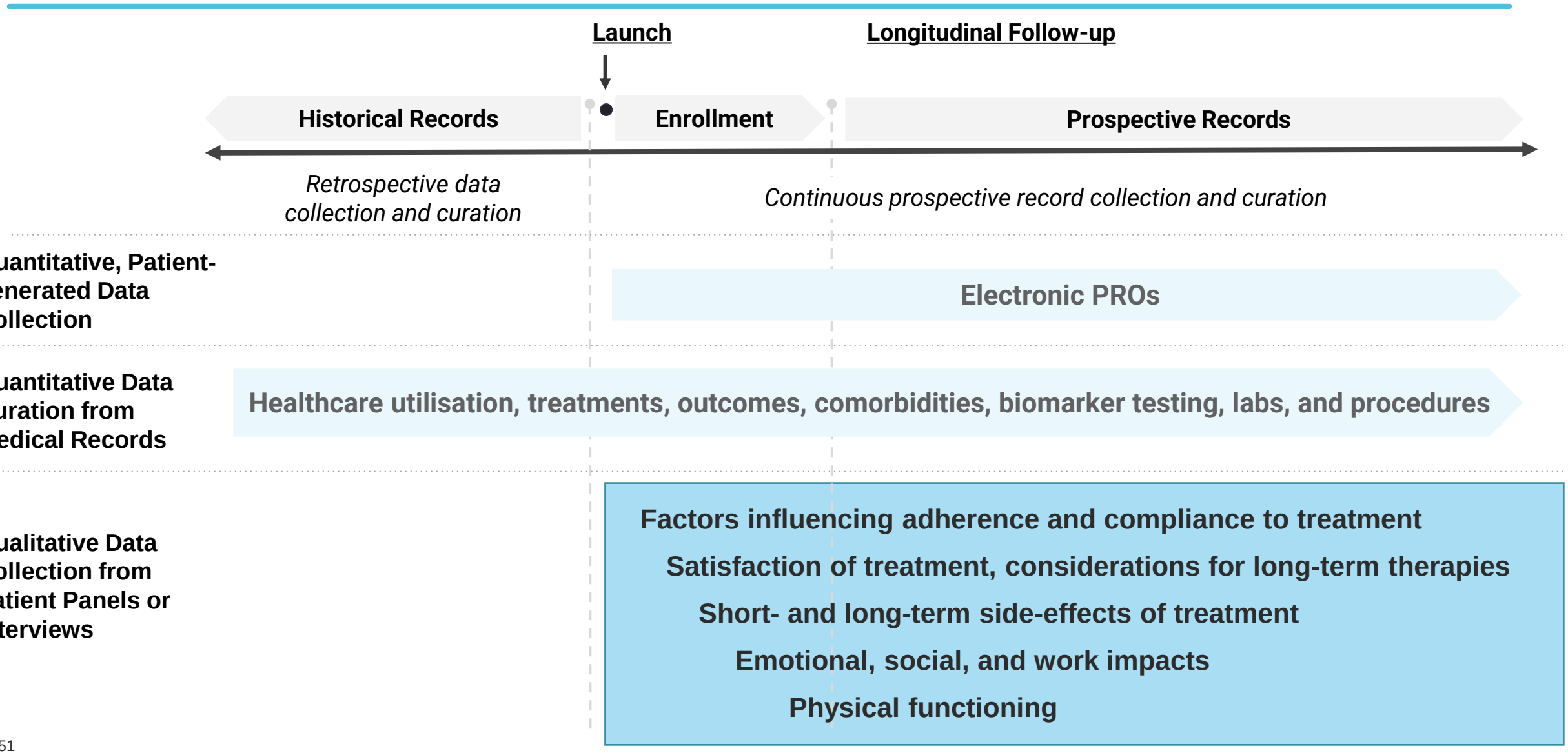
Early Breast Cancer Patient Journey

Initial Presentation & Workup → **Diagnosis & Molecular Testing** → **Early Breast Cancer Treatment & Management**



Abbreviations: IHC = immunohistochemistry; IR = Interventional Radiology; Med ONC = medical oncology; MRI = magnetic resonance imaging; NGS = next-generation sequencing

Study Design and Longitudinal Research Questions of Interest



Impact of Longitudinal Qualitative Research

- Understanding the patient experience and decision-making considerations throughout and across multiple phases of their care journey provides a unique opportunity ensure stakeholders make decisions that are in the best interest of patients.
  - Physicians are better informed on patient priorities, which helps them improve discussions around treatment selection and side-effect management over time.
  - Payers provide access to therapies that are not only based on clinical evidence and recommendations but are also aligned with patient priorities.
  - Biopharmaceutical manufacturers better understand the risk-benefit profile of their products and can leverage patient insights to inform future clinical development priorities.
  - All stakeholders can work together to provide the best possible support for patients and their families.

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Q & A

Wrap-up

Thank you for attending our workshop!!

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