Lumanity

# The story behind the numbers: how burden of illness studies add value to HTA submissions

**In a time of increasing cost pressures, limited resource capacity, and heightened demand for new treatments, Health Technology Assessments (HTAs) play a vital role in evaluating whether new interventions will provide sufficient patient benefits to justify the cost to the health service.**

Traditionally, HTA submissions leverage significant amounts of data: data about cost, clinical efficacy, benefits, and risks. But when viewed in isolation, these robust data sets may only provide HTA bodies with a blinkered view of the broader impact of an intervention.

Despite the direct impact that new treatments have on the lives of patients, their stories and lived experiences have been largely absent in HTA submissions, with many agencies actively seeking to analyse the impact of an intervention on the patient through consideration of clinical outcomes assessments (COAs), usually patient-reported outcomes (PROs). But, while PROs consider the status of a patient's health condition and the potential impact of an intervention, the HTA/reimbursement decision-maker may have difficulty interpreting what a numerical improvement in quality of life actually means for the patient on a day-to-day basis.

Including burden of illness (BoI) – or burden of disease (BoD) – studies alongside PRO data in HTA submissions can help to capture the broader impact of a disease and intervention on patients, carers, family members, and the wider healthcare system. When used alongside PROs, this mixed-methods approach of marrying quantitative analysis with qualitative patient narratives can help decision-makers to make more informed decisions.

"There's a real shift towards more shared decision-making and patient-centric care, really incorporating the patient's voice," says Marieke Schurer, principal insight analyst within Lumanity's insight team. "This is reflected in the shift that we see in HTA decision-making, where it also becomes more patient-centred and, therefore, burden-of-illness becomes more important."


# What are qualitative BoI studies?

**As the name suggests, BoI studies aim to understand how a health condition affects patients, their families, and caregivers. Using a variety of data collection methods, including interviews, surveys, and, where appropriate, literature reviews, teams can generate a more holistic view of the overall burden associated with a disease or condition.**

Within the health economics and outcomes research (HEOR) setting, BoI data complements traditional clinical development information. Conventionally, health economists relied on data from patient-reported outcomes (PROs), patient-reported outcome measures (PROMs), and patient-reported experience measures (PREMs) to generate relevant data on quality of life, usually reflected in a numerical value with statistical significance.

"In the context of economic models, health-related quality of life is often expressed in the form of a utility value. That's a numerical value ranging from zero – representing death – to one – representing full health," explains Schurer. "But if you see a utility value of 0.7, what does that actually mean? Does that mean that a patient has feelings of depression and anxiety, or is their condition impacting their mobility?"

While the data generated by PROs, PROMs, and PREMs provides important information for HTA decision-makers, it can be challenging to relate the numbers to the reality of living with a particular health condition. This is where qualitative-BoI studies can add valuable context to an HTA submission, including the emotional impact a disease can have on both patients and their loved ones.

As Ann-Marie Chapman, head of insight, HEOR at Lumanity, notes, qualitative BoI studies offer a degree of flexibility that is not appropriate to introduce with quantitative PRO studies conducted within clinical trials.

"When you capture patient-reported outcomes using a generic or disease-specific instrument, it's a simplification of real life, it doesn't allow you to capture all the nuances and complexities that exist in real life," she explains. "Given the importance of PRO instruments, they need to be rigorously tested before they can be validated. Such instruments are tested for construct validity, content validity, convergent validity, etc., to ensure a standardised and widely accepted instrument that can be used repeatedly and with confidence. For a BoI study, we can introduce flexibility, such that, while we have agreed questions to ask, we can explore topics that emerge from discussions with the patient, we are listening and learning from the patient to better inform what is important to them."

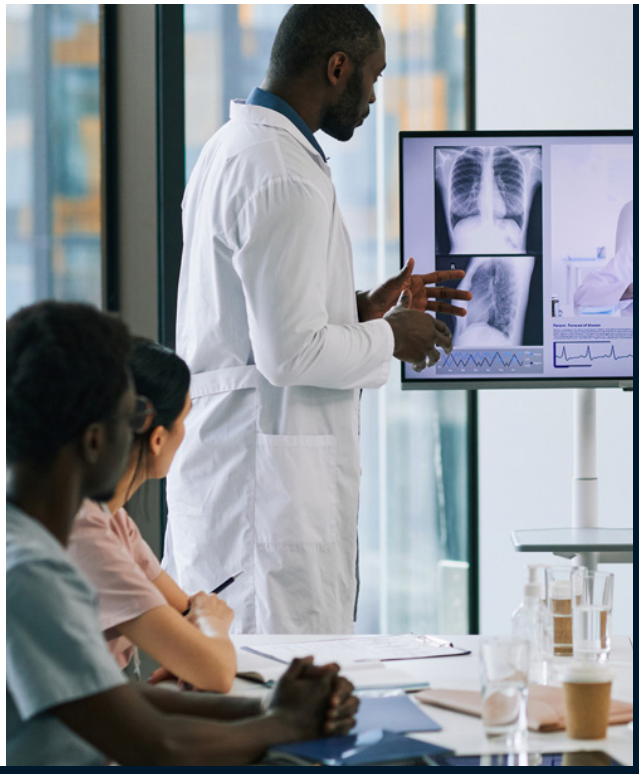
# Amplifying the patient narrative in HTA submissions


**The value of the information provided by BoI studies is not limited to the end stages of HTA submissions. In fact, patient insights can be incorporated into more technical projects and help to inform decision-making across the drug development lifecycle.**

Leveraging patient insights collected through qualitative BoI studies, researchers can identify areas of unmet need and ascertain which outcomes are most important for patients. As such, if a BoI study is conducted early in product development to help inform clinical trial design, researchers can confidently demonstrate that the clinical outcomes reported to HTA committees are relevant to patients and clinicians.

"Individual patient stories are always insightful, but conducting a well-designed BoI provides a level of confidence that we are not just selecting the most emotive patient, but that the issues, concerns, and challenges are seen across a wider patient population," explains Chapman. "This also helps to identify those areas where seemingly small changes can have a big impact."

Gathering insight into the patient experience can also be a valuable asset in areas such as rare diseases, where patient populations are limited or have high dropout or mortality rates. In these spaces, it may be challenging to collect enough data to assess whether PROs reflect changes in a patient's quality of life. For Chapman, this is a key area where BoI studies can add value to more technical projects.



"From a health technology assessment angle, the priority is getting the quality-of-life utility element that can feed into the cost-effectiveness model," explains Chapman. "But, in a rare disease space, there may not be a quality-of-life instrument to use, or the generic instruments that can be used aren't appropriate because they're not responsive enough to detect change with treatment or sensitive enough to detect a treatment difference compared to an alternative. You might never actually show a difference with the intervention using a generic instrument. Conducting a BoI in this scenario can provide the necessary information to design a vignette study; for example, to elicit an appropriate utility value for use in economic modelling."


## Building a broader understanding of the patient experience

**When examining the value of BoI studies, it is important to acknowledge that decisions made by HTA assessors and health economists directly impact real people with real conditions and experiences that develop over time. These individuals are far more multifaceted than the snapshot image captured by quantitative studies portrays, and often have different priorities and perspectives to research teams when it comes to determining the success of a new treatment. But to understand what really matters to the patient and maximise their input, researchers must be willing to actively listen to their stories.**

"Nowadays, it's quite rare for someone to carve out a full hour and just listen to someone's story," says Schurer. "Without judgment. Just listen and ask questions, even if it's about very difficult topics."

"We quite often get feedback that it feels meaningful to people participating in BOI studies that they can share their stories and contribute to the medical and scientific community in getting a better understanding of a disease. Particularly in rare diseases where literature is so scarce."

Listening to patients, family members, and caregivers, and asking them questions, gives researchers a unique opportunity to step into the shoes of those living with a condition and examine the impact of an intervention from their perspective.

"It's not only about survival and effectiveness of a treatment from a physical functioning perspective, but also about emotional wellbeing," says Chapman. "Sometimes things come out that we weren't expecting. For example, we might have spoken to a clinician, and he's told us X, Y, and Z, but when you speak to the patient, something new comes out that the clinician hasn't been privy to."

Storing medical equipment and treatments is a notable example of how patients' life experiences can illuminate potential issues for researchers; for example, bringing a therapy into a patient's home blurs the lines between the clinical and personal setting, a feature that may, on the surface, appear beneficial as it removes time and travel limitations. However, as Schurer explains, this can actually add to the burden experienced by some.

"Some patients still prefer to go to the treatment centres because they find aspects of home delivery challenging, such as limited timeslots for scheduling home care drug delivery," she says. "Also, patients may experience issues that researchers maybe wouldn't have thought about. For example, I remember one patient who was just really fed up with needing to use a separate freezer because he had to store the medication at a certain temperature."

# Putting patients at the heart of HTA decisions


**For Chapman and Schurer, the benefits of amplifying the patient narrative to support HTA submissions are clear. However, as with many areas of healthcare, while some are quick to accept qualitative BoI studies, others remain slow to realise the potential value of putting context around the technical numbers.**

"It's important to recognise that even though things have changed, not all HTA bodies will look at your burden of illness studies," explains Schurer. "Pharmaceutical companies need to make quite tough decisions when deciding how to use resources, so I think the first step is considering, 'what are my key countries, where am I going to submit an HTA to, and will they actually consider this data or not?'."

As HTA bodies become more accepting of BoI studies, companies have an opportunity to contribute to a foundation of clinical data and personal quality of life information that puts the patient at the centre of future healthcare development. In addition, by incorporating these studies early on, companies can foster meaningful partnerships with patients and patient groups, leveraging the information provided by individuals to address a wider array of concerns.

"On paper, your clinical trial data may look very positive, but in a real-life setting, when you are considering effectiveness, patient adherence to treatment can make a big difference. If a patient has difficulty taking their medication, whether that be the route of administration or perhaps the time intervals required, there will be consequences on effectiveness and potential side effects. The more a company understands about the patient, their daily life, things that are important to them, the better they can consider these elements into their product design," notes Chapman.

While the potential uses for BoI studies are extensive, telling the patient's story remains paramount. With their input and insights, companies can work to broaden the collective understanding of disease and ensure that the health system invests in treatments that provide tangible, real-world effects that help to alleviate the burden of disease for patients.

"Every story matters because it contributes to understanding that range of experiences that we want to communicate in a balanced way to decision-makers," explains Schurer. "With the context provided through BoI studies, HTA bodies and payers can make more informed decisions that will ultimately lead to better outcomes for patients, getting the right, most effective care for them."

## About the interviewees

**Marieke Schurer,** principal insight analyst, HEOR at Lumanity

Marieke completed a master's degree in Health Care Management at the Erasmus University Rotterdam in the Netherlands and has a background in biomedical sciences.

She is a passionate qualitative researcher who joined Lumanity (legacy BresMed) in 2014. Marieke has experience in a range of different research methods that she employs in patient-focused research, to ensure the patient voice is heard in HTA and the wider HEOR setting.

---

**Ann-Marie Chapman,** head of insight, HEOR at Lumanity

Ann-Marie joined the company in 2014, when the company was known as BresMed, and has over 18 years of experience in health economics and outcomes research across the consultancy, pharmaceutical, and medical technology industries.

Ann-Marie has wide-ranging experience in supporting health technology assessment submissions, new product implementation, and stakeholder engagement with appropriate value messages.

## About Lumanity

Lumanity

Lumanity applies incisive thinking and decisive action to cut through complex situations and deliver transformative outcomes to accelerate and optimise access to medical advances. By transforming data and information into real world insights and evidence, Lumanity powers successful commercialisation and empowers patients, providers, payers, and regulators to take timely and decisive action.

With offices in North America, the United Kingdom, the European Union, and Asia, and work conducted in over 50 countries, its 1,200+ experts work with nearly all the top pharmaceutical companies and more than 100 biotech companies around the world. Turning aspiration into reality, Lumanity supports over 50 payer submissions across 20+ countries, launch readiness and commercialisation of 80 brands and new indications, and numerous award-winning product campaigns every year.

For more information on Lumanity, please visit lumanity.com and connect with them on Twitter and LinkedIn.