pharmaphorum deepdive

Patients & Partnerships 2022

October 2022

Why real patient centricity is more essential than ever before

The power of patient perspectives in the publication of real-world studies

Realising the vision of value-based healthcare in cataract surgery

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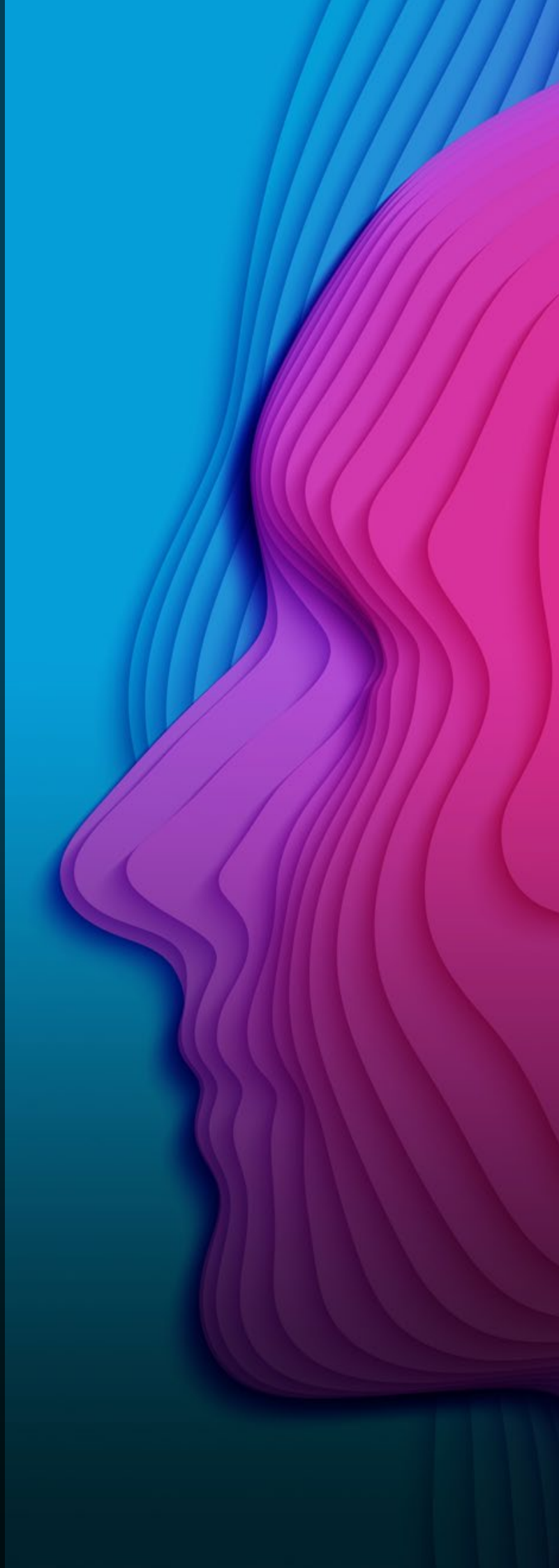

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Deep Dive: Patients & Partnerships 2022

The concept of patient partnerships has been extensively debated in recent years. While most would agree that patients can contribute valuable insights to the overall healthcare ecosystem, just how and where the pharma industry should champion the patient voice remains unclear.

Thanks to advancements in technology, there are more opportunities to engage patients in both clinical and home settings. As such, the potential avenues for patient partnerships and industry collaborations are increasing.

In this issue of Deep Dive, Sharon Suchotliff, associate principal at ZS Associates, details why patient centricity is more important than ever, Envision Pharma Group's Dr Dawn Lobban and Vitaccess' Dr Catherine Bottomley discuss ways that patients can contribute to the development and publication of real-world studies, and Erin McEachren and Syed Rashid of Johnson & Johnson explore how strategic partnerships can help to improve transparency and outcomes for vision patients.

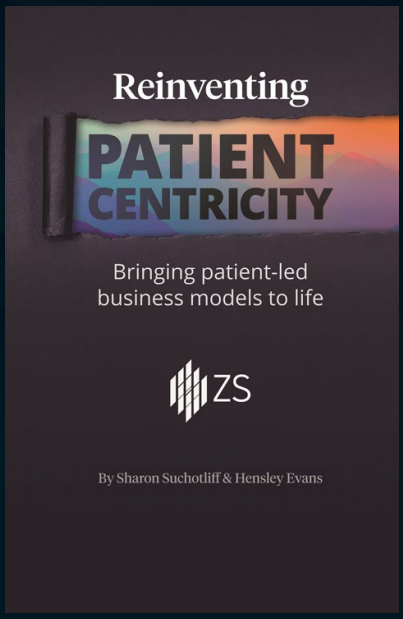
Plus, experts from Roche consider the importance of community voices in the future of neurology care, IPSEN's Dr Oleksandr Gorbenko and MediPaCe's Dr Sandeep Bagga discuss co-creating a patient engagement impact framework, Lumanity's Ann-Marie Chapman and Marieke Schurer explore how burden of illness studies can add valuable context in HTA submissions, and Research Partnership's Mariel Metcalfe and Nicole Syms share key insights into the patient experience of endometriosis.

For all this and more, read on.

I hope you are staying safe.

Eloise

Eloise McLennan – editor, Deep Dive



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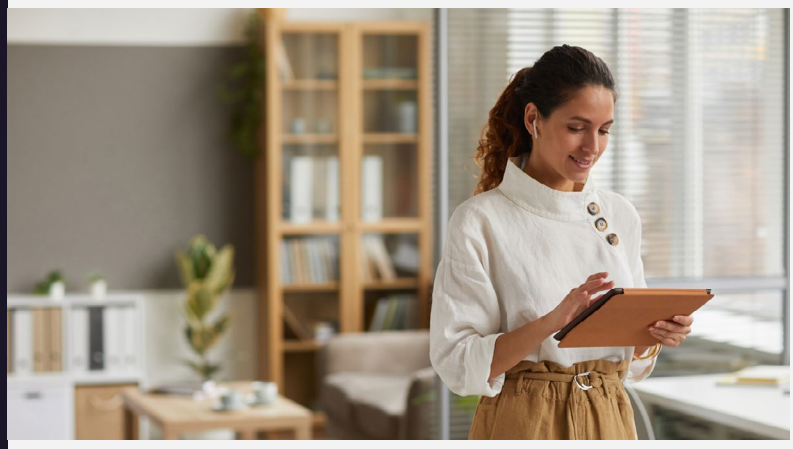
Why real patient centricity is more essential than ever before

Ask nearly any pharma company these days if they're patient-centric, and you'll probably get an answer in the affirmative. But dig deeper, and you might find that patient centricity means a lot of different things to a lot of different people. And that some of those who think they have it figured out might actually be the furthest from the mark of real, meaningful patient centricity.

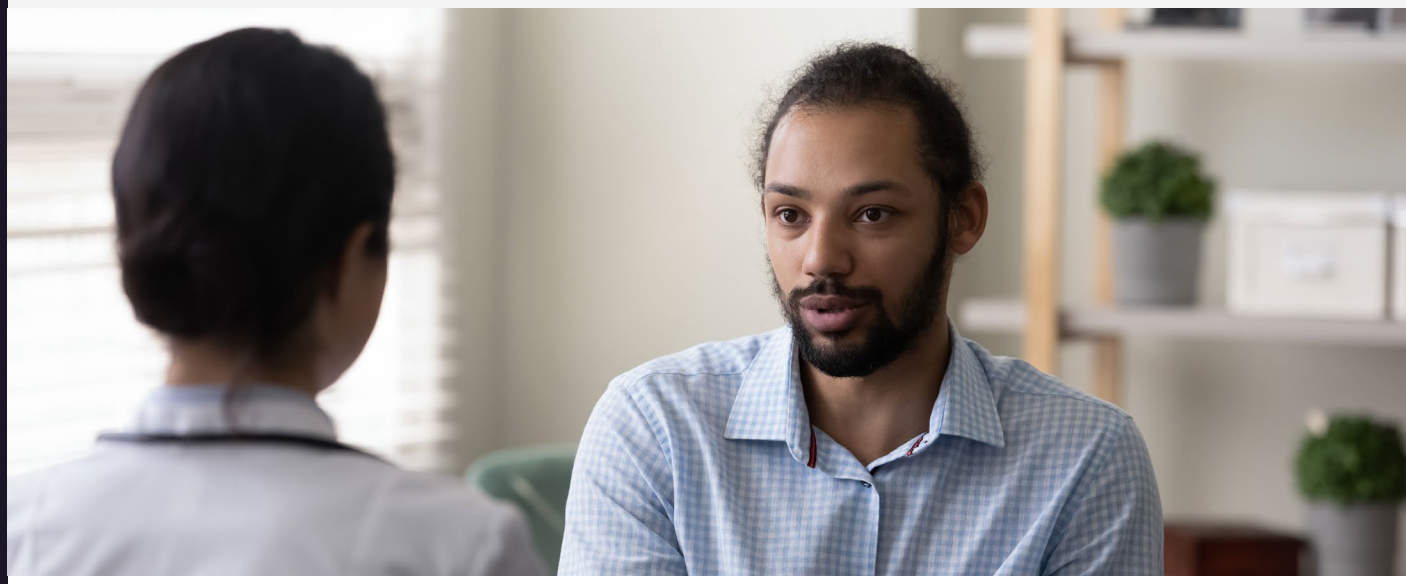
Sharon Suchotliff is an associate principal at ZS Associates and co-author with Hensley Evans, who leads the global patient and consumer health practice at ZS, of *Reinventing Patient Centricity: Bringing patient-led business models to life*. Suchotliff sat down with Deep Dive to discuss the current state of patient centricity in pharma, some of the common foibles and misconceptions, and how pharma companies can get themselves on the right track.

"I started my career in fashion and luxury lifestyle and I cut my teeth on brands like W Hotels, Marriott, and Mercedes. I understood customer experience, and what a good experience needs to look like," she explains.



Suchotliff went back to school for an Executive Master's in Public Health and Healthcare Management and studied with some of the experts behind crafting the US's Affordable Care Act – the beginning of more than a decade of work on improving the patient experience.

"I started to think, what's pharma's role in all this? Why patient centricity in life sciences now?" she says. "Which led me to think about how we can help life sciences focus more meaningfully on people, on patients, and include them as part of thinking about, which products to develop, how to develop them, and what is needed, in a way that it actually has impact for people and the business."



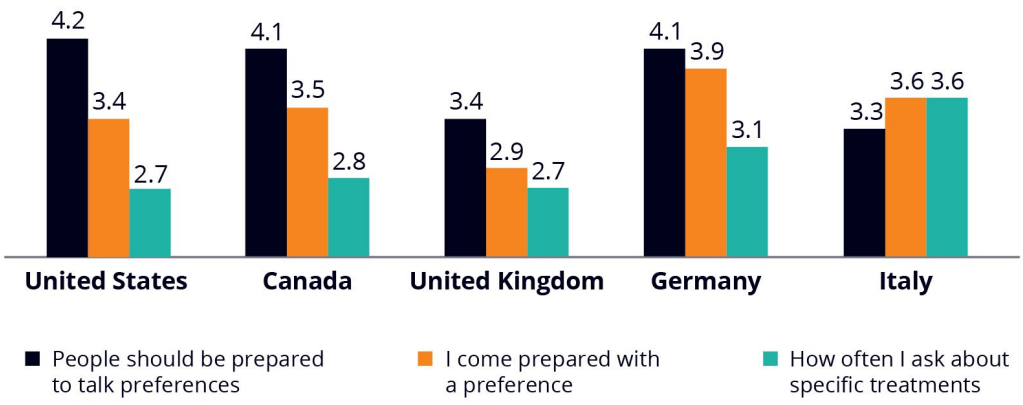
Why is patient centricity important?

Long a topic of discussion, many events have brought patient centricity to the forefront in recent years. Prior to now, Suchotliff says, pharma has had a run of profitable years and no "burning platform" pushing them to change.

"That's changing for many reasons," she explains. "One being consumer behaviour and expectations; people saying, 'I want what I want when I want it', and I'm starting to expect that also from my interactions with healthcare and pharma and my treatments."

And while people might assume that this phenomenon is more common in the US, where direct-to-patient pharma marketing is permitted, ZS has actually found that it's occurring everywhere.

Patient involvement in treatment



Numbers indicate "Level of agreement with the statement from 1-5"

John B. McKinlay et al., "Effects of Patient Medication Requests on Physician Prescribing Behavior: Results of a Factorial Experiment," in Medical Care 52, no. 4 (April 2014): 294-299, https://doi.org/10.1097/MLR.0000000000000096.

In addition to changing consumer expectations, regulatory requirements are also starting to reflect an emphasis on patient-focused drug development and collecting experience data. Increased competition makes it more important for pharma to optimise every edge, including patient experience. And, in general, the availability of new data streams creates new opportunities that pharma must explore or risk falling behind.

"Today, we have the ability to collect more data on people, their interactions, their preferences, and we have a technology to analyse it, understand, and even anticipate what people might need before they even need it," says Suchotliff. "We can anticipate when someone might drop off. We can anticipate when someone might be close to having a catastrophic event. We just need to focus on it."

Defining patient centricity

In her book, Suchotliff and her co-author, Hensley Evans, define patient centricity as "having an organisational culture, business practices and capabilities that put patients at the heart of decisions, meet patient needs as articulated by patients themselves, and drive business outcomes". But to help people incorporate patient centric approaches into ways of working, they use the idea of the four I's – insight, integrate, improve, and impact.

'Insight' refers to really understanding what patients need and want and how they live with their condition. Oftentimes what pharma assumes patients want turns out to be off base when they actually investigate by asking the patients.

"We did this really interesting study about a year or two ago where we evaluated patient support services in oncology," says Suchotliff. "What we heard from people, patients, and caregivers, is that there are lots of services, but what they really want from pharma is 'with the pill' offerings, not 'beyond the pill'. Yet here we are at pharma talking about 'beyond the pill' and investing a lot in that."

'Integrate' deals with company culture – making sure that when new insights are gathered, there's a process to act on them and change practices. And 'improve' is the corollary, ensuring that a change in company culture towards patient centricity also leads to improvements in patient experience.

"If you're asking someone to change their behaviour, you don't want them to take it as, 'Oh gosh, one more thing I have to do'," Suchotliff says. "They have to understand the value and want to change, and the only way that might happen is by a mindset shift. That's the culture. And in order to truly integrate the insights and improve the experience for patients, organisations also have to build new capabilities and enable their workforce to tap into these."

Finally, 'impact' recognises the reality that if patient centricity efforts improve patient experience but don't result in business benefits to the organisation, it will be hard to garner sustained investment from pharma companies. So patient centricity efforts must be created to be a win-win for the business and the patients.

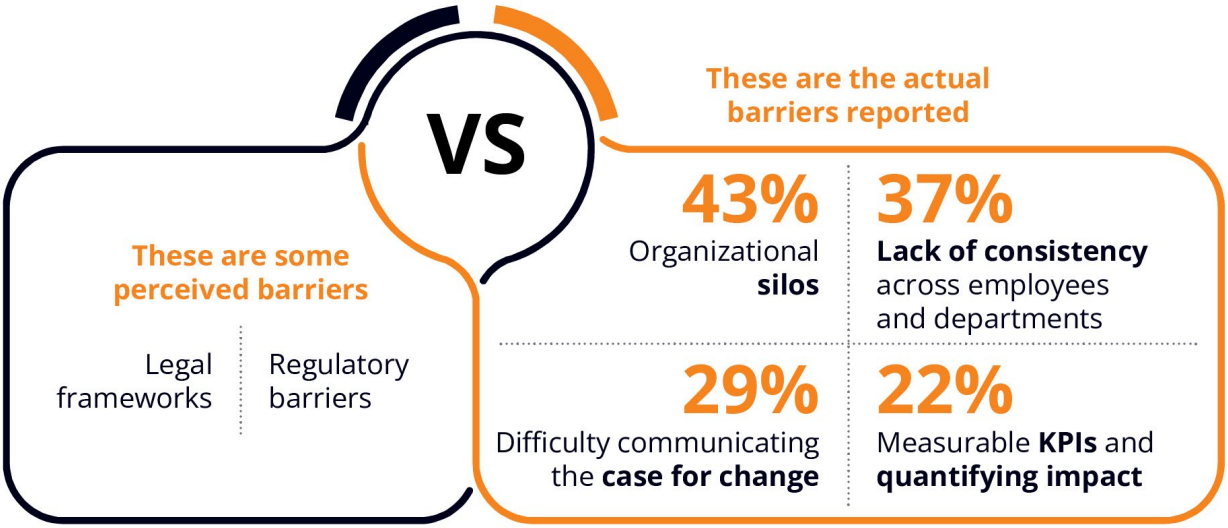
Implementing patient centricity

When it comes to putting patient centricity into practice, Suchotliff says, there are many obstacles pharma companies run up against. One, interestingly, is that people often think they're already doing enough in patient centricity.

"We just ran a study on enabling patient centric transformation amongst marketers," says Suchotliff. "What we found is that the more patient-centric somebody believes they already are, the less likely they were to be excited about corporate changes to integrate patient centricity into business objectives."

In a way, patient centricity is a victim of its own success as a concept, because the hype around it has created a bit of a 'boy who cried wolf' effect.

"As an industry, we've been talking about patient centricity for a long time, and at some companies, it really has been more lip service as opposed to real action, and so it might feel like, 'Oh, not this again'," Suchotliff explains.



Barrier - % are based on top two boxes
Sources: Patient Centricity Survey 2019.

Another barrier is siloing: one part of the organisation might be doing well with patient centricity, but that culture shift hasn't spread to the wider company. A lack of clarity and understanding about legal and compliance is another thing that can keep people from moving proactively toward patient centricity, especially when it comes to things like using data to better understand patient needs.

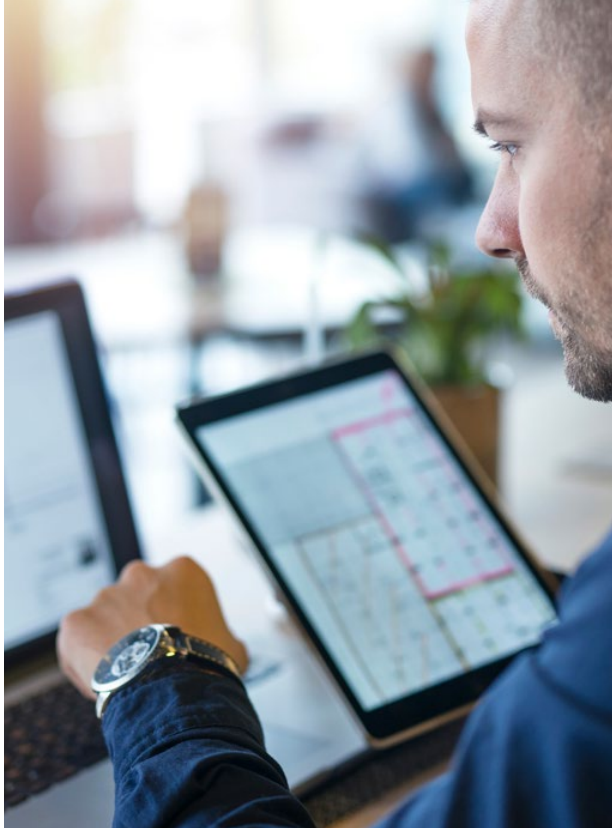
"When we ask, 'What gets in the way of being patient-centric?' We often hear, 'Oh, legal and compliance. I can't legally do this, or compliance won't let me do it', but the reality is it's really about lack of clarity," Suchotliff says. "Somebody else in your organisation may have done it, but we're not connecting the dots because there is not an overarching strategy, or an overarching mandate or mission, and so efforts are inconsistent and disjointed."

Finally, patient centricity initiatives often suffer when leadership changes and the patient centric culture isn't codified in KPIs or other concrete ways.

"We've seen instances where leaders have invested a great deal in enabling patient centricity at their organisations. Then as that leader moves on, because there haven't been KPIs or incentives put in place, those structures and practices go away. Some of these very same companies ride like a pendulum swing, and today, are now again, back at trying to figure out how to enable patient centricity at their organisation," explains Suchotliff.

The solutions to some of these problems are evident: organisations must increase internal communication and share best practices, codify their patient centricity culture with documented processes and KPIs, and be open-minded – at every level – about the possibility that their current patient-centricity efforts might not be enough.

Measuring patient centricity



Towards that end, it's important that organisations are able to measure and quantify the status of their patient centricity efforts. ZS has developed the Patient Centricity Index to help them assess companies' efforts on four axes: leadership and culture, structures and practices, data analytics and tech, and cross-industry collaboration.

"Within each one of these dimensions, there are various elements," Suchotliff explained. "There are different subcategories that we look at across the spectrum of one to five, one being not very patient-centric, and five being incredibly transformational, which we haven't seen any organisation quite get there yet."

ZS Patient Centricity Maturity Model

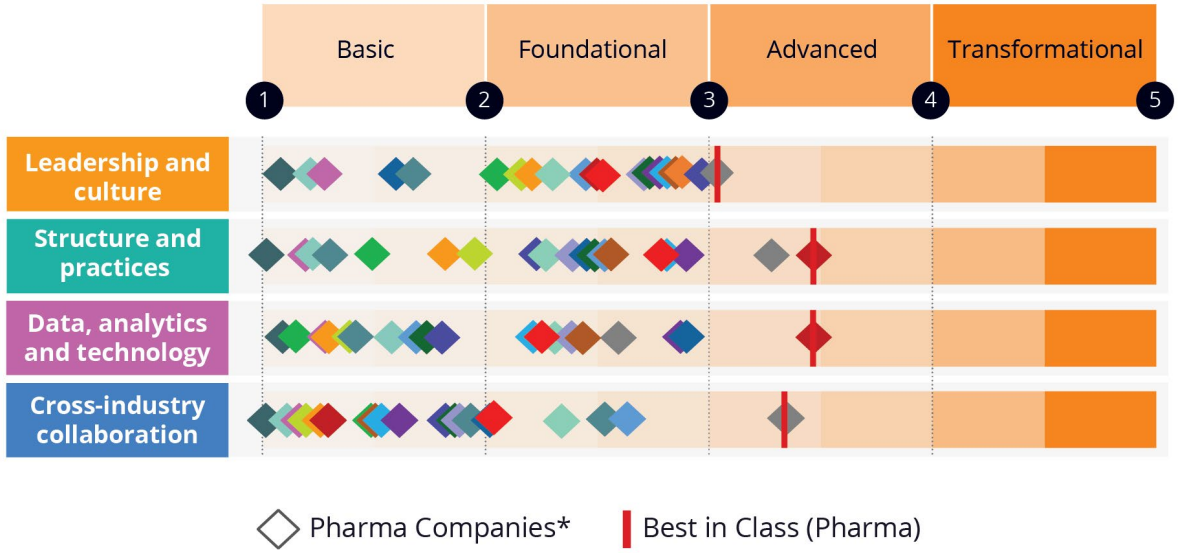
In doing assessments on this index, Suchotliff says her firm has found some interesting trends.

"What we typically find is that there seems to be this optimism gap, where folks in more senior positions believe the company is more advanced than people at middle management or doing the work on the ground, believe that the company is," she says.

While you might expect certain trends related to company size, location, or area of expertise, Suchotliff notes that, in reality, those correlations are few and far between.

"We often get the question of, 'Well, companies in rare disease are more patient-centric by design, right?'" she explains. "When you look at the capabilities and the behaviours, that's not always true."

Since they started doing assessments with the index, Suchotliff says the industry has been improving.



* Mid to large size, global companies with experience conducting patient centric initiatives
Source: Internal SME interviews, completed between April and June 2021 by ZS Associates; Patient Centricity Maturity Model by ZS Associates.

Internal SME interviews, completed between April and June 2021 by ZS Associates

"The area where we've seen the most progress between 2018 and 2021, is in leadership and culture, which makes a lot of sense, since that's where many might start," she says. "The area where we've seen the least amount of movement is in cross-industry collaboration, but my hypothesis is that will change because partnering is going to become increasingly more important as you move to an outcomes-based world."

Partnering for patient centricity success

That last point is an important one, Suchotliff says, because the nature of the patient experience is that it's not just an experience with one stakeholder. A recent global study by Suchotliff's team, of 100 individuals (patients, caregivers, and patient advocates) who have received a cell or gene therapy or might be eligible for one in the near future, gives an example.

"One of the biggest gaps that we found is that pharma really focuses on this one sliver of the experience, the actual delivery of the treatment itself," she says. "However, when people needed the most support was actually even earlier on. Understanding what is done in gene therapy – What is this thing about to do? What can I expect? The reason why that matters is because we saw that for about 30% of people, not having that understanding delayed treatment. If you think about certain conditions, in cancer or paediatric rare disease, that time is precious."

Pharma needs to partner with other healthcare stakeholders like providers and payors – as well as with their competitors – to make sure every part of the patient experience is looked after, in a seamless way. And when it comes to technology, that means interoperability.

"Think about an ATM," says Suchotliff. "Twenty years ago, in order to take money out of your account, you needed to walk into a bank with a slip of paper and say, 'Hey, can you please access money from my account?' Today, you can be in the middle of nowhere and find an ATM and within seconds, access funds from your account. How did this happen? By an agreement in the banking industry that banks will share certain data. Not all the data, but they'll share your name, your account number, and how much money is in your account, so we can make those transactions happen. How powerful would it be if we as a pharmaceutical industry could share certain data that would help us understand what's going on with someone's experience and therefore be able to improve that?"

The final piece of the puzzle is social determinants of health because people's healthcare experience isn't limited to experiences with the healthcare system.

"Lifestyle, social factors, and social determinants of health account for about 70% of an individual's outcomes," Suchotliff says. "There are some pretty stark examples of the implications of things like household income on an individual's health. We share a story in the book about Alec, a 26-year-old restaurant manager with type 1 diabetes. He had just aged out of his mother's insurance and couldn't afford to pay for insurance on his own, which led him to start rationing his insulin. Alec died less than a month after going off of his mother's insurance plan. We need to be aware of the factors surrounding people's lives if we're going to improve health outcomes. Partnering with others in the ecosystem will allow us to do that."

Moving in the right direction

Pharma is talking about patient centricity a lot these days, and that's a great step forward for the industry. But one of the industry's challenges will be making sure that companies are walking the walk as much as they're talking the talk.

"Oftentimes we hear from clients that they get a bit uncomfortable when they think about business outcomes as they relate to patient centricity," Suchotliff explains. "I think the key thing to keep in mind is that we're not focusing on patient centricity in order to advance the business. However, there is an absolute connection between improving the experience for people, improving their health outcomes, and your business outcomes."

For all those reasons, Suchotliff thinks the industry is moving in the right direction. That point was driven home for Suchotliff by a conversation with one of ZS Associate's advisors, a former C-suite executive at a major global pharma company.

"We [asked him] the question of, 'How do we get CEOs to care about patient centricity?' He said that it's when the board of directors starts asking for it. It's when shareholders start to demand it," she says. "I think given the continued movement towards value-based care, we're not that far off. My hope is that we will, within the next ten years, get to a place where we look at the impact of our investments on health outcomes – what we call patient outcomes impact, or POI – just as much as we look at ROI."

About the authors

Sharon Suchotliff leads ZS's patient centricity work in the US. She brings more than 15 years of marketing communications experience in and outside of healthcare, fused with a background in public health. At ZS, Sharon helps companies develop strategies and build capabilities to further patient centricity and meaningfully incorporate the patient voice into business decisions. Sharon holds a BS in public relations from the SI Newhouse School of Public Communications at Syracuse University and an Executive MPH from the Mailman School of Public Health at Columbia University.

About ZS Associates

ZS is transforming global healthcare by driving toward a connected ecosystem. We leverage the power of data, science, and technology to make more intelligent healthcare decisions and deliver innovative solutions to improve health outcomes for all.

Inside the pharmaceutical greenhouse

Value-based healthcare (VBHC) is gaining traction across the industry for its potential to improve patient care by standardising clinical and patient outcomes. Erin McEachren and Syed Rashid of Johnson & Johnson Vision describe why cataract surgery is well-positioned for the VBHC model, and how strategic partnerships can help build datasets and practices that improve transparency and outcomes for vision patients.

Oddly enough, the majority of these plants are also highly toxic if incorrectly used, but within their leaves, flowers, and bark is a world of medical wonders that can be used to cure and treat a plethora of modern ailments.

While not all of these 'treatments' have the same level of scientific backing, today approximately 11% of drugs deemed essential by the World Health Organization have roots in botanicals. In fact, many of these plants get their names from the doctors that studied them, as botany and medicine were intrinsically linked up until the 17th century.

Here, we take a look at some of the most influential examples of plants in medicine.

Willow bark (Salix alba)

For centuries, willow bark has held a place of honour in traditional medicine as a source of pain relief. However, it is unlikely that the ancient Sumerians and Egyptians could have known that the bark they were advised to chew on, contained an ingredient that would become of one of the most used drugs in the world – aspirin.

It wasn't until 1828 that the active ingredient in willow bark was finally discovered, when German pharmacologist Johann Buchner refined the bark into yellow crystals, which he subsequently named Salicin, after the Salix genus of the plant. This process was taken a step further in 1838, when Italian chemist Raffaele Piria used these yellow crystals to develop salicylic acid.

Building on the work of Buchner and Piria, French chemist Charles Gerhardt became the first to modify salicylic acid with an acetyl group, resulting in an early claim to the discovery of aspirin. However, as the compound lacked stability, Gerhardt opted not to develop it further.

The breakthrough for aspirin eventually came in 1890, when the German dye manufacturer Bayer established a pharmaceutical division. While there is ongoing controversy over how each individual should be credited for the discovery of aspirin at Bayer, we do know that there were three key figures involved: Arthur Eichengrün, Felix Hoffmann, and Heinrich Dreser.

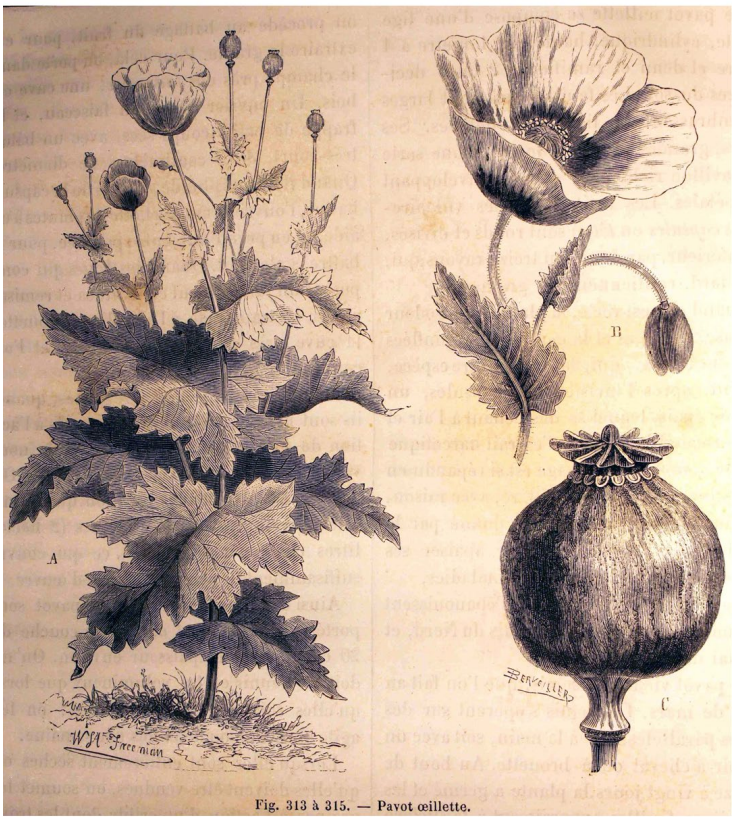


Fig. 313 à 315. — Pavot œillette.

Opium poppy

(Papaver somniferum)

Perhaps the most well-known plant in medicine, the opium poppy is also one of the oldest in recorded history. The specific date and location of discovery of the plant has been lost to history. However, the tale of the opium poppy can be found in prominent texts, including a believed reference to the plant on the Sumerian clay tablet. Dated around 2100 BCE, this is considered to be the world's oldest recorded list of medical prescriptions.

The milky latex sap found within the unripe poppy seed pod contains upwards of 80 alkaloids that can be used to target the opioid receptors that regulate pain and temperature control. German pharmacist and pioneer of alkaloid chemistry Friedrich Sertürner became the first to isolate morphine from opium (as well as the first person to isolate the active ingredient associated with a medicinal plant or herb).

Following this, in 1832, French chemist Pierre Jean Robiquet isolated codeine from opium's several active components while working to refine morphine extraction processes.

A similar compound, diamorphine, was synthesised by the chemist C.R Alder Wright in 1874. Better known by its other name – heroin – this compound remained relatively unchanged for the next 23 years, until a re-synthesised version developed by aspirin-creator Felix Hoffmann caught the attention of Bayer Pharmaceuticals and was transformed into a pain management drug.

Until the early 19th century, a mixture of raw opium and ethanol, known as laudanum, was used to treat a wide array of ailments. Given the dangers associated with misuse of opioids, heroin has since been banned in most countries, while other opium derived medications are now heavily regulated.

Snowdrop (Galanthus nivalis)

Despite its dainty stature, the snowdrop, Galanthus nivalis, has carved out a substantial place in pharmaceutical history. Early descriptions of the mind-altering properties of ingesting an extract from snowdrops can be found as far back as ancient Greece, with references to an herb with "a black root, but milklike flower" seen in Homer's Odyssey, widely attributed to the snowdrop.

Although the plant has been extensively used in traditional medicines, it wasn't until the 1950s that researchers began to explore its potential in pharmaceuticals.

The official story behind the discovery of snowdrops' medical properties remains uncertain, but a leading theory links traditional uses with the development of the compound, galantamine. Having observed how villagers would rub the bulbs and leaves of snowdrops on their foreheads to ease nerve pain, Bulgarian chemist Dr Dimitar Paskov and his team isolated and extracted the alkaloid galantamine, a competitive, reversible, acetylcholinesterase inhibitor, as part of efforts to treat poliomyelitis.

Today, galantamine is commonly used to slow the progression of Alzheimer's disease and mitigate its symptoms, such as memory loss, although it cannot cure the disease.

While the alkaloid was initially isolated from the snowdrop (most notably G. woronowii), nowadays, galantamine is obtained from daffodils (Narcissus) and snowflakes (Leucojum), as well as synthetically.

Madagascar periwinkle

(Catharanthus roseus)

The aptly named Madagascar periwinkle is native to the island nation of Madagascar. It's delicate pink and white flowers have made it a popular decorative plant in gardens around the world. However, traditionally, infusions made from the leaves of the Madagascar periwinkle were used by the indigenous people of the island to treat a variety of conditions, most notably diabetes.

Frederick Banting and Charles Best's discovery of insulin in 1921, and its subsequent commercialisation by Eli Lilly, marked a significant turning point in the treatment of diabetes. However, as some receiving the treatment began to develop resistance, researchers set out to find alternative lead compounds.

In the 1950s, this search brought researchers to the Madagascar periwinkle. Two research teams from Eli Lilly and the University of Western Ontario independently began to investigate the potential of chemicals derived from the plant in the fight against diabetes.

While the plant extracts proved to be less effective in lowering blood sugar in mice subjects, researchers noticed a significant decrease in white blood cells. As such, the chemicals found within the Madagascar periwinkle could be highly effective in fighting some types of cancer, specifically those involving a proliferation of white blood cells.

The two research teams decided to join forces to investigate further. Through this collaboration, they identified two key alkaloids: vinblastine, a potent inhibitor of cell division, and vincristine, which inhibits leukocyte production and maturation.

These alkaloids work by preventing cells from dividing by blocking tubulin.

Following FDA approval of vincristine in 1963, the drug has since been successfully used for treatment of childhood acute lymphoblastic leukaemia and non-Hodgkin lymphomas.

Meanwhile, vinblastine is used in combination with other chemotherapy drugs to combat lymphomas, as well as testicular, ovarian, breast, bladder, and lung cancers.

"Vinca. Catharanthus roseus. Choix des plus belles fleurs -et des plus beaux fruits par P.J. Redouté. (1833)" by Swallowtail Garden Seeds is marked with Public Domain Mark 1.0 .

Sweet wormwood

(Artemisia annua)

Stories of using traditional medicine to combat malaria exist throughout history. But as is often the case, it was necessity that fuelled interest in exploring how traditional applications could inform pharmaceutical developments. In 1967, at the height of the cultural revolution, then-Chairman of the Communist Party of China, Mao Zedong launched a covert military operation, code named Project 532, to find antimalarial medications.

At the time, China's forces were heavily embroiled in the Vietnam war, where the emergence of drug resistant malaria created an urgent need for new treatments. Project 532 was split into three main branches, one for developing synthetic compounds, one for clinical studies, and another for investigating traditional Chinese medicine. It was headed by the famed Chinese pharmaceutical chemist and malariologist Tu Youyou.

Biomedical interest in Traditional Chinese Medicine was not a new concept, however, Tu's embrace of Chinese meteria medica, notably The Handbook of Prescriptions for Emergency Treatments, written in 340 by Ge Hong, is a more recent example of efforts to investigate the 'clinical' potential of substances. From an initial list of 2,000 traditional herbal preparations, Tu and her team of phytochemical researchers identified possible antimalarial activities in 640 candidates.

Of the more than 380 extracts obtained from Chinese herbs, the Artemisinin (or qinghaosu, as it is known in China) derived from extracts of sweet wormwood was demonstrated as a strong potential for treatment of intermittent fevers, a key symptom of malaria.

As head of the research group, Tu volunteered to be the first human subject. Once the product was deemed safe, clinical trials were then conducted in human subjects. Artemisinin-based combination therapies are now standard treatment worldwide for P. falciparum malaria, as well as malaria.

For her work in malaria research, Tu was awarded the Nobel Prize in Medicine in 2015.

Oceancetaceen – Alice Chodura, Public domain, via Wikimedia Commons

About the author

Eloise McLennan is the editor for pharmaphorum's Deep Dive magazine. She has been a journalist and editor in the healthcare field for more than five years and has worked at several leading publications in the UK.

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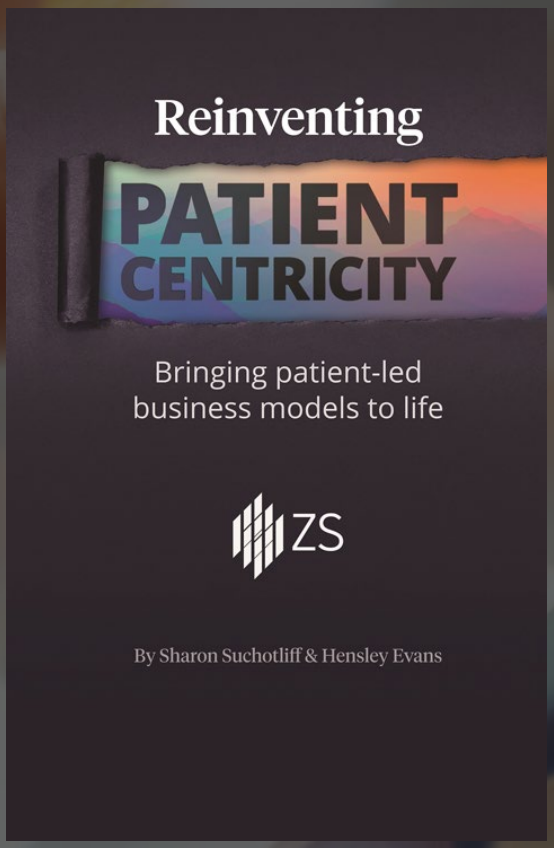

ZS is reinventing patient centricity

Patients belong at the centre of healthcare. ZS's new book shows why it's time to turn the talk about putting patients first into action.

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ENVISION PHARMA GROUP
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The power of patient perspectives in the publication of real-world studies

Patients have a unique first-hand insight into their condition, which can be a valuable asset in the design, delivery, and publication of real-world studies, as Envision Pharma Group's Dr Dawn Lobban and Vitaccess' Dr Catherine Bottomley discuss.

The value of championing the patient voice throughout the medicine development journey is evident. Patients have a unique understanding of their condition and can provide valuable insights into lived experience, unmet needs, and treatment impact, and yet, historically, detailed insights on the patient perspective have been largely lacking in data generated from clinical trials.

Amid growing interest in real-world evidence (RWE), generated outside of a clinical trial setting, acceptance and appreciation of the patient voice is shifting. Now, life science companies are actively exploring ways to work in partnership with patients to improve the design, delivery, and analysis of real-world studies. But whilst efforts to champion patient engagement in the early stages of study and protocol design have been widely demonstrated, few have explored the potential of extending the patient perspective to authorship and publication.

Patients have much to offer to the development and publication of real-world studies, yet, there is much to be done to improve awareness of the contributions these individuals can make to the peer-reviewed literature.

"The research that we're doing is either for patients, about them, or both," says Dr Catherine Bottomley, chief scientific officer at Vitaccess. "Ultimately, they're the people who know their condition best and can tell us what really matters to them."

Patient involvement in real-world data generation

To understand how patients can contribute to the publication of RWE, it is important to consider the broader role of patients in data generation and analysis. Existing guidance for where patients can (and should) be involved in randomised, controlled trials for drug development advocates for patient involvement in the initial stages of identifying research priorities and protocol design, through to conducting research and communicating results.

Many of these activities are also applicable to studies outside of the clinical trial setting. However, as Bottomley notes, RWE studies can offer more flexibility for patient involvement.

"Randomised controlled trials are governed by best practice guidance and standardised recommendations, particularly when it comes to the patient-reported components of them, and so you have potentially limited ability to be able to implement changes that patients might suggest during the study design process," she explains. "For example, they might identify that a range of particular outcomes are important to them when they start taking a treatment, but within the constraints of that clinical trial, there's no space to ask those questions.

"In the real-world setting, there's often more flexibility in terms of study design, scheduling data collection, and time points, which means that you can ask additional questions and collect additional information."

Involving patients right at the beginning of the study planning process can significantly improve study design and delivery. The perspective offered by patients can help investigators to broaden their understanding of patient needs and consider the real-life implications of taking part in the study for the individuals involved, allowing researchers to resolve issues and improve study designs before the study opens for recruitment.

"By involving patients right at the start, you have the potential to develop better study designs and protocols that consider patient needs," says Bottomley. "And consider those protocols in real-life scenarios, asking, 'would you actually want to wear a tracker whilst you sleep? Are you willing to answer three questionnaires in a row that ask you about the impact of treatment on different areas of your life?'"

"Often when we're designing protocols, we make assumptions about what participants are or are not willing to do. Unless we actually involve patients in those discussions, we might be making some really broad assumptions that are then going to result in people not wanting to take part."

For Bottomley, it is important that project teams approach patient collaborations as an opportunity to foster meaningful partnerships that benefit both studies and patients. Just as researchers provide an expert scientific understanding of the study topic, patients are experts in understanding the reality of managing a condition. As such, their input should be respected, even if their perceptions do not align with those of investigators.

"It's really important to spend time developing relationships with the key patient stakeholders," she says. "We always suggest making a plan or an agenda for the patient involvement throughout the study, with all the details of activities, time commitments, and related reference material."

Encouraging and facilitating patient authorship

Whilst the value of patient contributions throughout the design and delivery of real-world studies has been increasingly recognised in recent years, when it comes to authorship, patient perspectives have been rare. This does appear to be changing, as pharma companies acknowledge the unique insight that patients can bring to the publication of RWE.

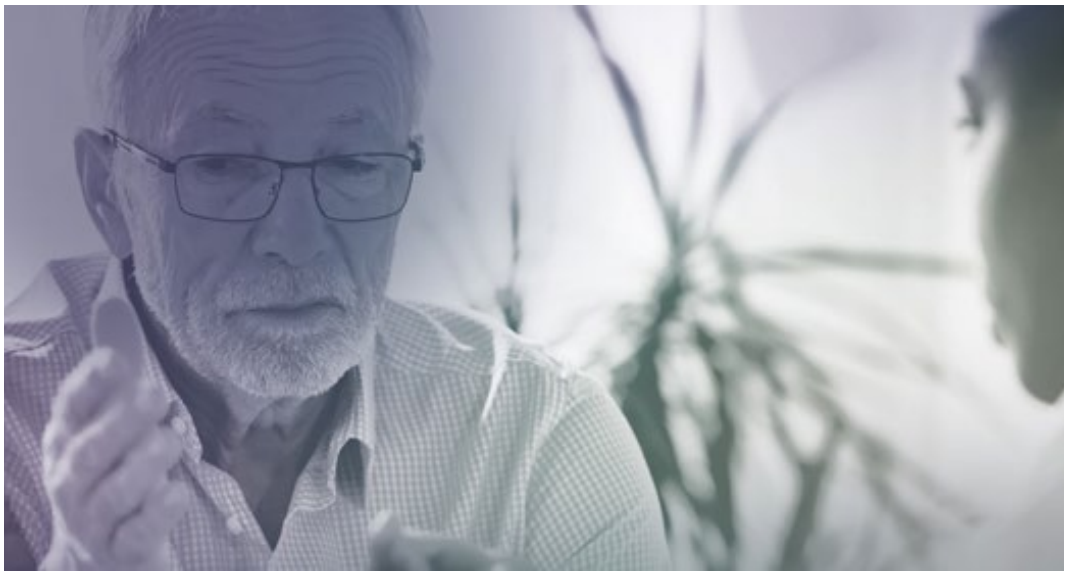
According to the International Committee of Medical Journal Editors, to be eligible for authorship, patient partners must meet all four of the following criteria:

- Substantial contributions to the conception or design of the work; or the acquisition, analysis, or interpretation of data for the work; AND
- Drafting the work or revising it critically for important intellectual content; AND
- Final approval of the version to be published; AND
- Agreement to be accountable for all aspects of the work in ensuring that questions related to the accuracy or integrity of any part of the work are appropriately investigated and resolved.

"When patients are involved in designing questions to really get to the heart of the issue being explored, or in analysing the patient insights generated during a study, they can easily fulfil that first criterion," explains Dr Dawn Lobban, global lead, patient partnerships at Envision the Patient. "Then, if given the opportunity to be involved from the outset as a co-author, they can actively participate in drafting, critically reviewing, and approving the final version to fulfil the second and third criteria."

She continues, “Building on this foundation, patients can proceed to the fourth requirement, becoming accountable for all aspects of the work and appropriately investigating and resolving any challenges to the publication, along with their co-authors.”

There are many benefits to be found in partnering with patient authors. Notably, as patient representatives, patient authors can ensure that the needs of their community are considered, keeping the focus of a study publication relevant and applicable. Patient authors can also ensure that the language and format used can be understood by both experts and laypeople.



“When we write medical publications, we write them for a scientific audience, but we also want them to be understood by a wide range of people,” says Lobban. “Patients often ask very insightful questions that can highlight areas of a publication that are perhaps not obvious to non-expert healthcare professionals. Patient co-authors may also consider publication enhancements, such as plain language summaries, which are designed specifically to broaden the reach to a wider non-specialist audience, including patients.”

Despite the benefits, there are barriers that can affect patient partners and their ability to collaborate as co-authors. Whilst scientists working in the industry are likely well-versed in the publication process and specific scientific terminology, patient partners have differing levels of understanding, which can be daunting for potential co-authors. Moreover, patient authors have to balance their participation in the publication with their own personal commitments, including managing their condition.

For Lobban, it is important to provide patients with the tools they need to optimise their potential as authors. Increasingly, the publication process and authorship are being included in training services designed to support patients looking to get involved in medicine development, such as the course developed through a collaboration between Envision Pharma Group and the European cancer patient advocacy network, WECAN.

Supporting patient engagement and authorship through partnerships

Whilst the recently updated Good Publication Practice guidelines (GPP 2022) broadly support patients as authors of company-sponsored biomedical research, further work is needed to optimise patient engagement in the generation and publication of RWE.

"There are still barriers to be broken down," explains Bottomley. "It's our responsibility as researchers to bridge the gap between somebody saying, 'I want to take part', and them actually being able to sit down with the research team with the knowledge and understanding of the study to contribute in a way that's going to be beneficial."

Understanding the needs, knowledge, and abilities of patients as both stakeholders in real-world studies and as publication authors is a central step towards developing respectful and symbiotic partnerships between patients and investigators. It is important to remember that engaging in real-world study and authorship activities usually occurs within the broader context of a patient's daily life. As such, the industry should prioritise supporting these patients throughout the study and publication process, to ensure that incorporating research responsibilities around work, friends, family, and managing their own health is as straightforward as possible.

Fostering a foundation of respect and support throughout the study and publication process is particularly important as regulators call for the broader inclusion of real-world patient perspectives in research submissions and evidence packages.

As Bottomley notes, "Organisations are understanding and seeing the benefits of involving the person who's actually suffering from a condition and appreciating and respecting the fact that they have something of vital importance to contribute."

"What we're advocating for is that patients can, and often do, qualify to be authors in the same way that other researchers and healthcare professionals qualify to be authors. With support and evidence-based resources, we can ensure that the patient voice is heard in the medical literature," concludes Lobban.

About the authors

Dr Dawn Lobban, global lead, patient partnerships at Envision the Patient.

As global lead, patient partnerships, Dawn Lobban heads up the Envision the Patient team within Envision Pharma Group. With a strong background in medical affairs, Dawn is particularly interested in optimal patient involvement in medicine development. Through active participation in key working groups, including ISMPP (International Society for Medical Publication Professionals) and PFMD (Patients Focused Medicine development), Dawn works hard to explore effective ways to ensure effective patient engagement. Her research and opinions are published in a number of abstracts and articles.

Dr Catherine Bottomley, chief scientific officer at Vitaccess

Catherine Bottomley is a PhD pharmacist with expertise in Patient Centred Outcome (PCO) research. She is currently chief scientific officer at Vitaccess, a patient-focused consultancy organisation offering innovative solutions to patient and carer reported data collection in real world settings. Catherine has overall responsibility for the pharma sponsored research studies conducted by Vitaccess including patient-reported tech-based registries, qualitative research, Patient Reported Outcome Measure (PROM) development and implementation, and observational studies. Catherine has over a decade of experience working in PCO and RWE roles across a variety of pharmaceutical consultancy settings, spanning associate researcher through to director.

About Envision Pharma Group

ENVISION PHARMA GROUP

Envision Pharma Group is a leading provider of evidence-based communication services and industry-leading technology solutions (iEnvision) that have applicability across many areas of medical affairs and related functional responsibility. Envision provides services and technology solutions to more than 90 companies, including all of the top 20 pharmaceutical companies.

About Vitaccess

Innovators in real-world research, we connect biopharma to patients and caregivers on any device, worldwide – harnessing our scientific expertise and cutting-edge digital technology to generate real-time, real-world insights through the powerful combination of our Vitaccess Real™ digital platform and expert consultative acumen. Born in Oxford, we have presence across Europe and the US.

We are health economists, researchers, eClinical health experts, data scientists, software developers, linguists, and medical writers, all committed to insightful digital research. We are passionate about inclusivity and open ways of working. We believe the more voices and views we listen to, the better we can make real progress, together.

Johnson & Johnson VISION

Realising the vision of value-based healthcare in cataract surgery

Value-based healthcare (VBHC) is gaining traction across the industry for its potential to improve patient care by standardising clinical and patient outcomes. Erin McEachren and Syed Rashid of Johnson & Johnson Vision describe why cataract surgery is well-positioned for the VBHC model, and how strategic partnerships can help build datasets and practices that improve transparency and outcomes for vision patients.

Cataract operations are one of the most common surgeries, with about ten million performed around the world each year. The procedure, in which a surgeon replaces a cloudy lens in the eye with an artificial one to improve a person's sight, is widely considered safe and effective. But there's a high degree of variability in patients' outcomes following the procedure, and some patients may benefit from it more than others.

In the United Kingdom, the National Health Service (NHS) found that cataract surgery may not improve vision for a particular subgroup of patients. In addition, many people go without the cataract care they need; the World Health Organization estimates that the cost of addressing current global gaps in necessary cataract procedures sits at around $8.8 billion USD.

VBHC, a method of improving patient care by reducing variability in outcomes, presents a unique opportunity in cataract care.

"Among ophthalmic surgical procedures, cataract is the number one procedure in the world – and it's one of the safest," Syed Rashid, director of market access and sales training for Europe, the Middle East, and Africa (EMEA) at Johnson & Johnson Vision, says. "Because of its minimal complications and relative high cost to the healthcare system, it has become a prime target to consider for VBHC."

VBHC involves transparently reporting data on a set of defined measurements, including both clinical and patient-reported outcomes. This information is organised into datasets which can be analysed to inform decisions. "Long-term, patients benefit from this transparency," says Erin McEachren, regional vice president for surgical vision EMEA at Johnson & Johnson Vision. In addition, by standardising outcomes, VBHC reduces the probability of unsuccessful surgeries, and costs that would have been spent on the procedures can be allocated where they're needed most.

As today's global community faces an economic downturn, ageing populations, and continued post-pandemic recovery efforts, the market is ripe for new ways to derive value through the healthcare system. Now, as stakeholders explore value-based arrangements, it's important to form cross-industry collaborations to ensure high-quality care for all patients.

"We need to remove the barriers between different members of the healthcare system and focus, as a collective, on the patient," Rashid says. "We're all treating the same people, and patients and hospitals don't differentiate outcomes based on which company supplied a product."

Building value-based datasets to power better outcomes in cataract care

In ophthalmic care, HCPs and patients use several metrics to determine the value of cataract surgery. On the clinical side, for example, physicians need to understand visual acuity, a clinical metric to assess clarity of vision.

Patient-reported outcomes reflect patients' quality of life before and after a procedure. These may include insights on complications due to surgery or changes to their intermediate vision, which includes seeing anything within an arm's length. Intermediate vision is an important data point, as patients with poor sight at this distance are more susceptible to trips, falls, and other accidents.

To collect these outcomes, Rashid explains that patients may fill out a survey to evaluate their sight. They repeat the same questionnaire about three months after their cataract surgery to assess how their vision has changed.

Using registries built from the real-world outcomes data, stakeholders across healthcare can run analyses to guide their next steps. McEachren explains that patients can use data to choose between doctors and hospitals, or to learn what post-op recovery may look like. For manufacturers, the data can help companies communicate the value of new products to providers, payers, regulators, and patients.

"When a manufacturer introduces a new innovation, we need to be able to demonstrate the value our product brings," Rashid says. "Having both clinical data and patient-reported outcomes offers a total package of evidence that differentiates our offering and enables us to approach customers with confidence."

Opportunities and challenges for VBHC adoption

While clinical measurement is key to understanding the value of a procedure, implementing measurement practices can be a challenge. "It's a question of creating a mindset and behaviour change. People are open to being measured if they know it's being done transparently and ethically, and that their data is being used and compared in a fair way that will potentially drive positive benefits for other patients," Rashid says. McEachren explains that IT infrastructure and data regulations can pose challenges as well.

But once the initial adjustments are overcome, the benefits of VBHC are plenty. For HCPs, Rashid explains, outcomes tracking allows them to show the quality of their work over time to differentiate themselves with payers, patients, and hospitals. Data can also be a powerful tool in managing expectations with patients, McEachren says, and care teams can use data to personalise patients' care.

"VBHC is about making sure we reach the best results for patients," McEachren explains. "It's not always easy, because we're asking clinicians to be very transparent. This can be especially challenging in vision, but it's key to normalising patient outcomes."

Early results from VBHC programmes are promising:

"We've seen evidence already in NHS Wales and other parts of Europe that there's a 20% reduction in variation of outcomes by using this way of working," Rashid says.

Maximising VBHC's impact through pilots and partnerships

Rashid and McEachren both emphasise the importance of cross-functional partnerships in driving VBHC success.

"No individual company can do it alone," McEachren says. "We need to work together to show the value of VBHC and ensure it gets adopted across the board."

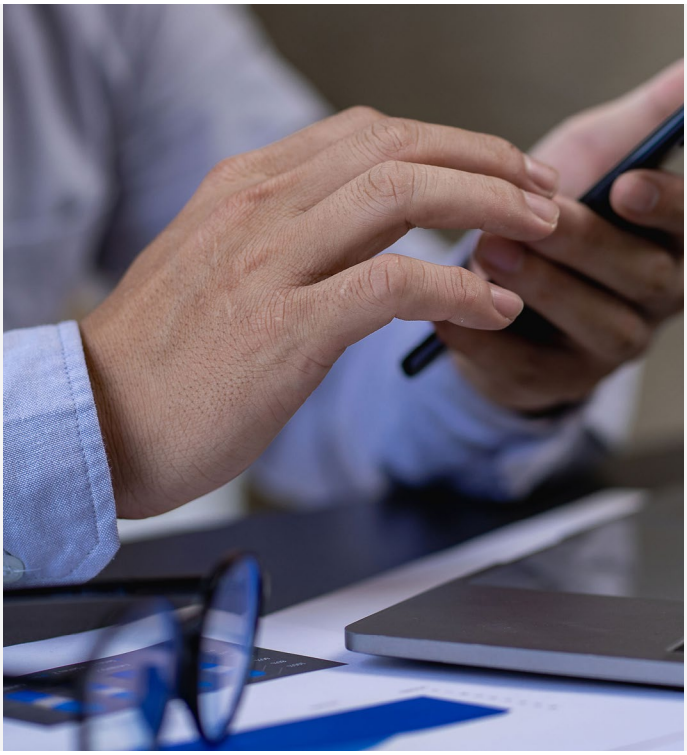
There are many programmes aimed at building datasets and outcomes-based arrangements in the ophthalmic space. Rashid tells us that the European Registry of Quality Outcomes for Cataract and Refractive Surgery, for example, has established a registry with transparent data to improve outcomes for patients, as has the UK, with a National Ophthalmology Database.

Among other pilots, Johnson & Johnson is engaged in a project with French non-governmental organisation (NGO) PromTime to test a new incentive scheme that prioritises transparent data collection, Rashid explains. McEachren discussed how PromTime developed a calibrated questionnaire for patients to record their functional vision, and then implement the system through hospital IT infrastructures. "This helps us identify trends among patients," she says.

Over time, additional partnerships and cross-functional projects will be key to advancing VBHC in cataract care and other sectors of healthcare.

Continuing the momentum for VBHC

As VBHC manifests across healthcare, ophthalmic stakeholders can learn from examples of partnerships and initiatives in other disease areas. Rashid shares that pharmaceutical companies developing diabetes treatments are considering partnerships with diagnostic or medical device manufacturers to share resources and generate real-world evidence. Manufacturers are also increasingly considering real-world outcomes in their evidence generation strategies to inform innovative payment models and run more effective clinical studies.



Continuing to power innovative work requires more high-quality data, especially that which comes directly from patients. "We would like to see more patient diaries," Rashid says. "Similar to how diabetes patients record their food intake, vision diaries can allow cataract patients to track the ways they use their vision in daily life. We can use this information to become more bespoke in how we partner with HCPs and decide on the best vision option for patients."

In addition, by fostering collaborative relationships between the often-siloed members of the healthcare system, we can bring together diverse perspectives to work toward a world in which patients truly benefit from medical interventions.

For Johnson & Johnson, the opportunity to contribute to strategic partnerships is a privilege the organisation looks forward to exploring through further collaborations. "We're delighted to see how patient outcomes influence ecosystems," Rashid says. "In vision care, we should be more open to having more strategic partnerships that could be of value to all."

"We have an opportunity to support providers and surgeons with their own, validated, real-world evidence that they can learn from to help their patients," McEachren says. With more data, more stakeholders on board, and a greater understanding of the value new methods can bring to healthcare, Rashid and McEachren's VBHC vision can become a reality.

About the interviewees

Erin McEachren is regional vice president for Johnson & Johnson Surgical Vision, EMEA, leading the team to address the unmet needs of patients and eye care professionals around the world.

Erin was previously a top-level skier and after suffering injury, has a deep connection to medical devices, in which she now excels. She is an exceptional leader, with a track record and breadth of experience in commercial leadership, franchise development, and sales execution across medical devices and in growth areas, such as robotics.

Syed Rashid is director of market access and sales training at Johnson & Johnson Vision, EMEA. He is a highly experienced professional with a demonstrated history of working in the medical device and pharmaceutical industry. His passion is for value-based healthcare and ophthalmology.

About Johnson & Johnson Surgical Vision

Johnson & Johnson VISION

At Johnson & Johnson Surgical Vision we are passionate about changing the trajectory of eye health and dedicated to partnering with our customers to help more people around the world preserve and restore sight. For over 130 years we have been guided by our CREDO, focusing on improving the lives of the patients and the communities we serve.

Our mission is to bring science driven innovation to life through our products and our customer partnerships to elevate eye health for the benefit of all patients. Our commitment to innovation sees us offer a broad range of eye health solutions in ophthalmic care. Our TECNIS® IOL portfolio, for example, delivers unsurpassed visual outcomes using a combination of proprietary and industry leading optics technologies. We also offer advanced astigmatism management solutions and are a global leader in refractive laser surgery, with an innovative suite of technologies to deliver best in class outcomes.

Company profile: ZS Associates

ZS Associates is transforming global healthcare by driving toward a connected ecosystem. We leverage the power of data, science, and technology to make more intelligent healthcare decisions and deliver innovative solutions to improve health outcomes for all. We serve all top 50 pharmaceutical companies and 21 of the top 25 medical technology firms, accomplishing great things at every turn.

Creating real-world impact with our patient-centric solutions

ZS provides patient-centric solutions to 48 of the top 50 pharma companies. Here are a few examples of our impact:

- **30%-40%** increase in patient duration of therapy through tailored patient support programmes
- **15%** increase in patient engagement across channels
- **20x** increased clinical trial enrolment by changing the patient experience

ZS provides an analytically driven understanding of patient behaviour to help your organisation become more patient-centric and reduce disparities in care:

Enterprise readiness: Is your organisation prepared for patient centricity? We'll assess your readiness and search for the overlaps between patient needs and business objectives to find the best opportunities for delivering next-level patient initiatives. We then work with your team to build out the capabilities and processes to help you succeed in delivering exceptional experiences and value to patients and your business.

Health equity: We take a multifaceted approach to tackling health equity, leveraging our research capabilities, healthcare expertise, and problem-solving approaches to uncover fresh insights and incubate innovation at the leading edge of the fight against health inequities. We drive change by embedding health equity across our work, from strategy and insights to execution and operations. We support our clients in maximising their own impact and driving meaningful change progress in the populations they serve while also partnering directly with organisations across healthcare, such as the Healthcare Leadership Council, to understand and drive meaningful and measurable progress.

Insights and research: Precise insights into patient decision-making and behaviours provide the baseline for improving patient engagement. We offer a broad range of collaborative market research, data analysis, behavioural and health decision science research, social listening, patient barrier analysis, and more.

Patient strategy: The right message at the right time to the right audience is the key to successful communications. We assess your value proposition, map the patient journey, segment your market, and craft your messaging to help you interact with patients in a meaningful way.

Patient support and programme measurement: We can help you plan your investments, promotions, and channel mix, and measure the impact through tracking studies, closed-loop measurement approach to DTC impact measurement, marketing mix models, KPI tracking and benchmarking, and predictive modelling.

About "Reinventing Patient Centricity: Bringing patient-led business models to life"

Sharon Suchotliff and Hensley Evans wanted to capture their years of experience working directly with biopharma companies and patients to share how critical it is to put the patient at the centre of the healthcare ecosystem. The result is a book that offers a provocative perspective of not only why patient centricity is important, but how companies can change their mindsets and practices to achieve it.

Experts across ZS contributed to the book's chapters, including Michael Thomas, Fiona Taylor, Albert Whangbo, Lisa Bance, Nikita Reznik, Emily Mandell, Torsten Bernewitz, Mary Ann Godwin, Victoria Summers, Tanya Shepley, Sophie Kondor, and Greg Fry.

Who we are

ZS is a management consulting and technology firm focused on transforming global healthcare and beyond. We leverage our leading-edge analytics, plus the power of data, science, and products to help our clients make more intelligent decisions, deliver innovative solutions, and improve outcomes for all.

Year founded

1983

Headquarters location

Evanston, Illinois

Number of locations

35

Number of employees

12,000+

Our areas of expertise

We leverage our deep industry expertise, leading-edge analytics, technology, and strategy to create actionable solutions for your most complex challenges. Our solutions include:

- Strategy & Advisory
- AI & Analytics
- Digital & Technology
- Life Sciences R&D & Medical
- Portfolio & Pipeline
- Value & Access
- Marketing & Sales

Industries we work in

- Pharmaceuticals & Biotech
- Health Plans
- Medical Technology
- Consumer Goods
- Financial Services
- High-tech & Telecommunications
- Industrials & Business Services
- Private Equity
- Retail
- Travel & Hospitality

Why real patient centricity is more essential than ever before

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Co-creating a patient engagement impact framework

Recognising patients as key stakeholders and embedding the patient voice, in a meaningful way, into every stage of the medicine's development process is gradually being recognised as a means to deliver value to both life science companies and to patients and their care partners.

To identify best practices, the difference made – or the 'impact' of engaging with patients – must be measured. Ipsen and MediPaCe explored patient experts' and patient organisations' (PO) perceptions and experiences with impact measurement and worked with them to co-create an impact framework for internal company utilisation.

Depth interviews were conducted with 13 patient organisation representatives and patient experts, exploring their experiences of engagement and thoughts on 23 candidate impact measures categorised into five groups:

- Medicines R&D priorities
- Clinical trial design
- Regulatory and market access submissions
- Product support and information
- Disease support and information

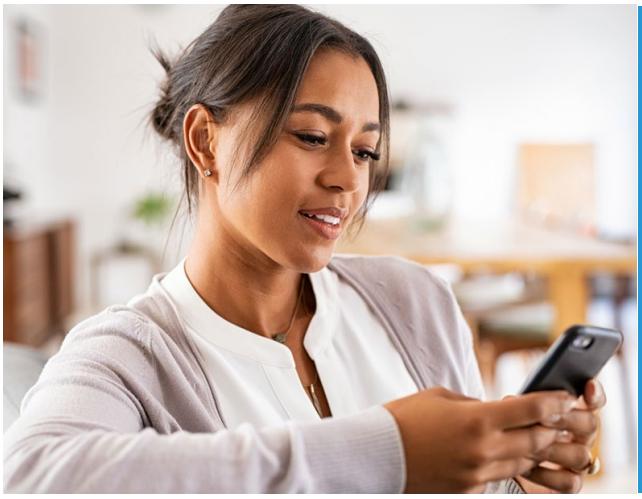
Findings were discussed and validated with a patient-led working group. The full results outlining how the impact measures were discussed and revised have been published. Here, we examine how patients view the impact from projects and their experiences of discussing impact measurement with life science companies.

Impact conceptualised as useful outcomes

Generally speaking, participants described impact from patient engagement projects in terms of positive and favourable outcomes; in other words, 'value'. It can be measured numerically or more subjectively through interpretations. For example, one participant in Argentina spoke about an increase in the number of patients correctly receiving a diagnosis following a physician awareness campaign.

Others wanted to be able to look beyond numbers and say, for example, "So, we had six meetings, but actually, out of that x, y, z happened, and it was really positive." The term 'value-impact' was coined during this study to capture these positive interpretations.

Patients involved in patient engagement projects want to influence change that will deliver real and tangible outcomes. There is a clear sense of urgency and empathy as participants discussed: the impact of community advisory boards that they had helped set up; contributions made to lay language disease information; coordinating delivery of educational events for patients.



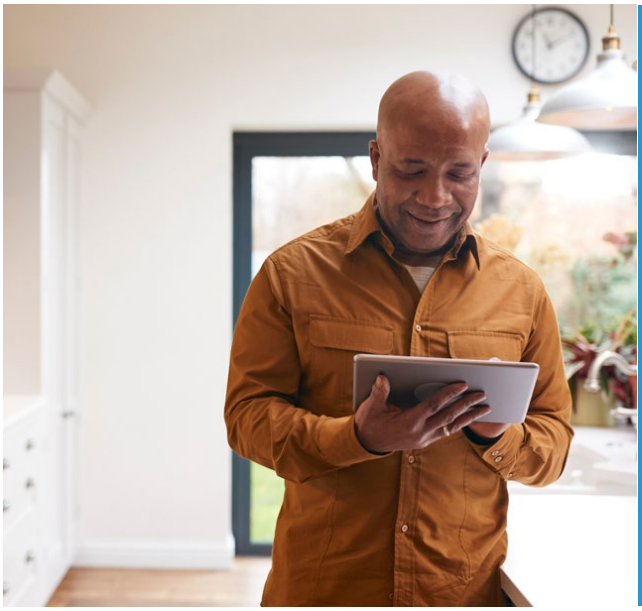
> "[We want to know] how would this affect patient outcomes, not just it's a nice-to-have, actually what's the tangible? What is this going to actually achieve? And I don't want to achieve it in 20 years, I want to achieve it now."
>
> *Anonymous study participant*

Patient participants also highlight the importance of impact from patient engagement projects on industry itself. For instance, routinely working with the patient community helps shift attitudes, learn about the right language to use with the collaborating patients, and helps companies know and understand the emotional story in order to better engage with the patients.

Industry not taking the lead on early impact discussions

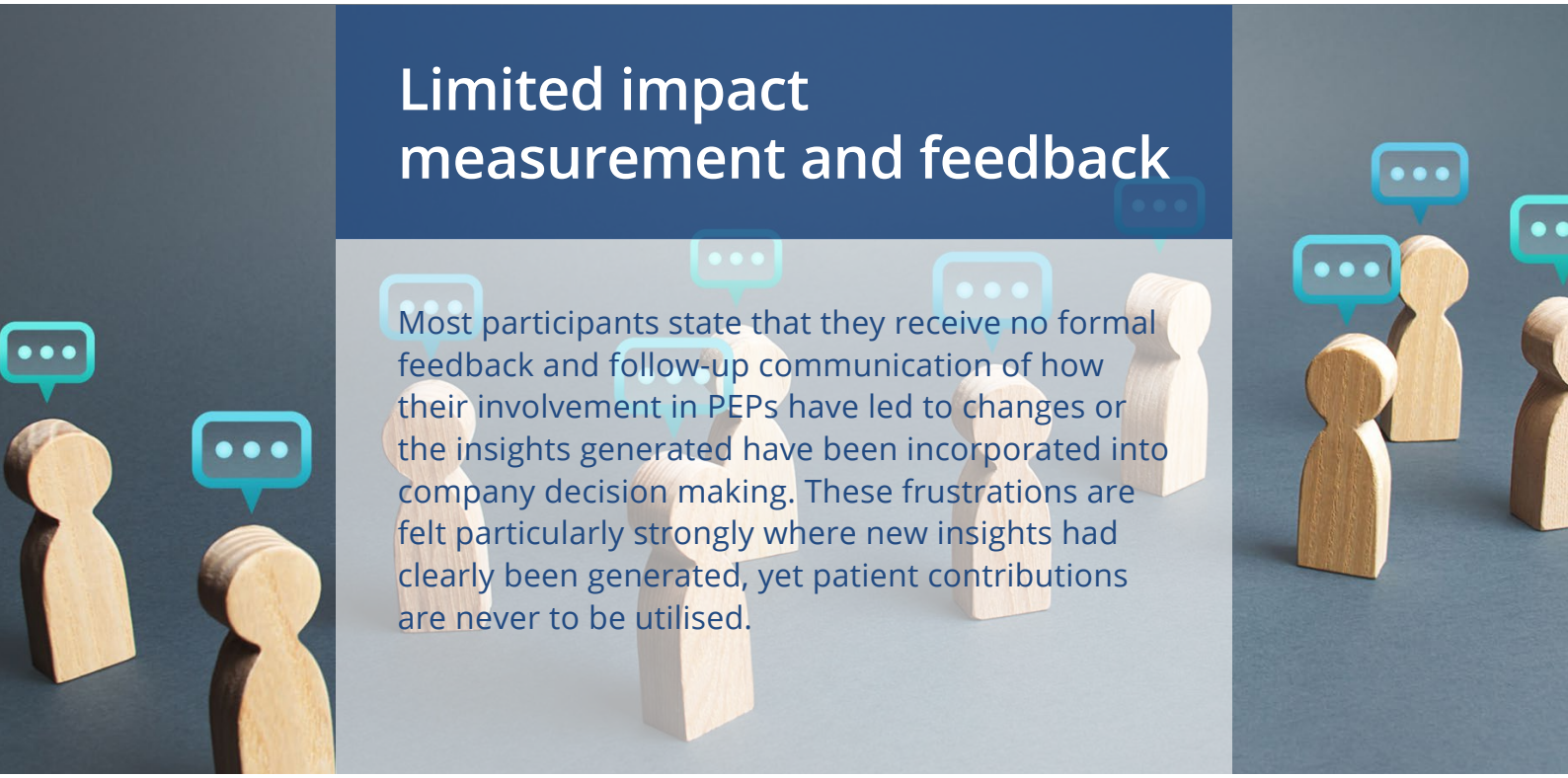
There is limited evidence of early discussions, or any expectations, being set by pharmaceutical companies about the potential impact from PEPs. This is an important first-step discussion that needs to happen alongside the contract process and when patients' role and responsibilities on the PEP are being clarified.

Invariably PO representatives provided their own notions as to why patients' perspectives need to be captured. One US participant outlined a number of innovative ways in which her PO engages with pharmaceutical companies and emphasised the need for patient insights to impact decision-making:



"When you talk about patient engagement, it's one thing to be able to provide the patient voice but it's really important for us to understand how the regulatory people weigh that [...We] want to know that it's useful, otherwise it's an exercise in futility."

Anonymous study participant



Limited impact measurement and feedback

Most participants state that they receive no formal feedback and follow-up communication of how their involvement in PEPs have led to changes or the insights generated have been incorporated into company decision making. These frustrations are felt particularly strongly where new insights had clearly been generated, yet patient contributions are never to be utilised.

"There was one project, there was a round of patient experts [...It] was great work, and I was convinced in the beginning that this project would have a high impact [...] 'never' heard anything [...They] do it in a black box and they store it a big warehouse."

Anonymous study participant

Some companies only provide feedback reactively, for example, when POs request follow up information. Impact measurement, however, seems more likely to occur where there are larger-scale, longer-term, and deeply established relationships developed, e.g., one UK-based participant had a particularly strong relationship with one pharmaceutical company that was oriented around a single medicine (for use in rare disease) with a dedicated team attached to the PO.

"If you're asking me to rate from all the impact you have given over the years – maybe 30% has had an impact and the rest I have a feeling has gone, it's somewhere up in the air."

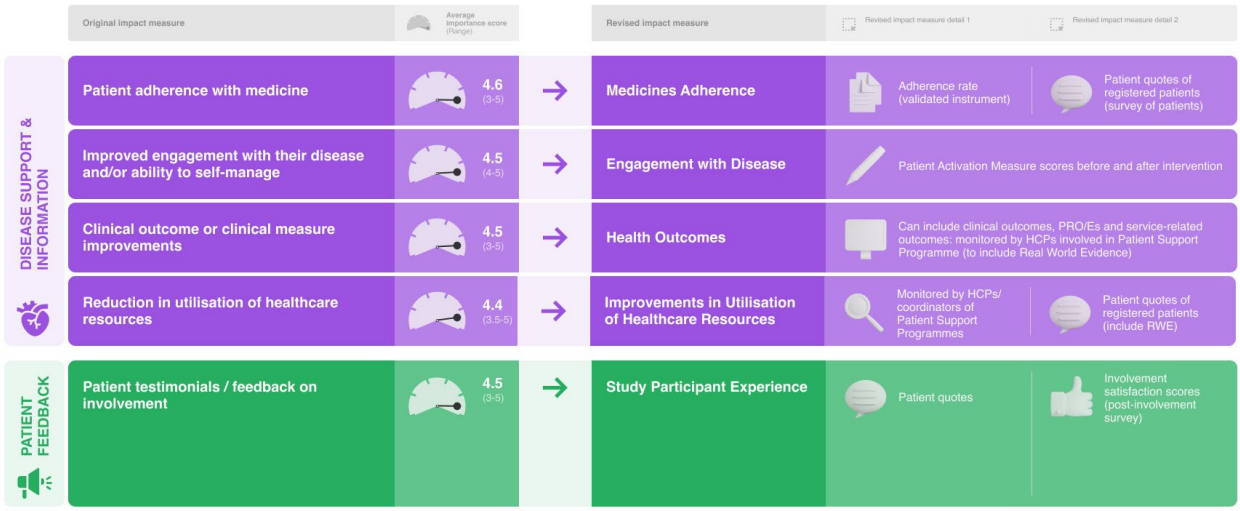
Anonymous study participant

Patients say impact measures should:

(1) Capture relevant context

(2) Capture how patients influenced decisions

(3) Use patient-friendly terminology



Impact measures should capture relevant context

We heard that capturing the necessary context around impact is considered particularly important. For example, this could mean including 'percentage' in addition to the 'number' of study participants not complying or withdrawing from a study. However, most people said that additional 'qualitative' context, such as descriptions, patient quotes, and satisfaction, is needed.

This tendency for a deeper and more nuanced understanding is also seen in patients' strong preference to be involved in developing Patient Reported Outcome and Experience Measures, recording the 'day-to-day' experience of their condition and the value delivered by a medicine. The support for the 'patient feedback' impact measure is a further endorsement of a more descriptive understanding of the impact of involvement in PEPs.

The parallels between the patient-centred method and qualitative inquiry have been acknowledged previously; both adopt a holistic, naturalistic, and empathic understanding of the phenomena being investigated.

There was a sense that they are not only interested in the 'how many' but also in the subjective and interpretive reporting of impact, including capturing patients' experiences of newly developed support programmes or appropriately contextualising a poor hospital visit experience during clinical study participation that may otherwise unfairly reflect on the biopharmaceutical company's broader research programme.

This call for 'greater context' reinforces recent work that a "set of coherent measures" is needed rather than the application of single measures. The present study now provides the rationale for this claim, crucially, from the patient's perspective.

Capture what influence patients had

How patients 'influenced' key aspects of early R&D and clinical trial design is another dimension of impact that needs to be made clear. Patients interviewed asked for internal R&D personnel to provide an objective description of how patients influenced target value profile (TVP) development, asset development plan/strategy, and clinical trial delivery such as the development of patient-facing documents or development of clinical outcomes.

In addition, post-involvement feedback will capture patients' opinions and sentiments about the degree to which they felt they were 'genuinely' able to influence decision-making processes.

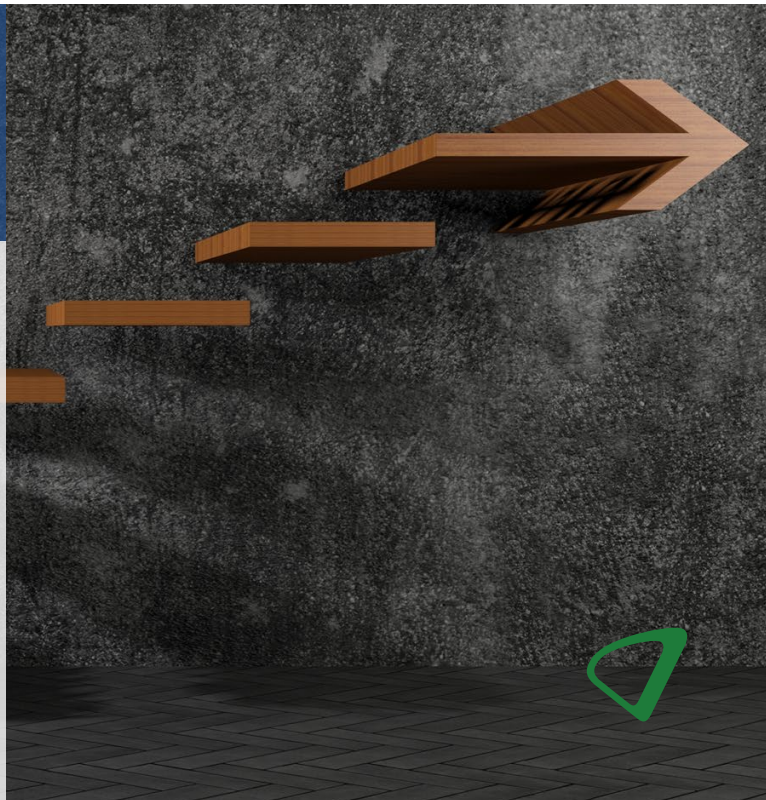
Use patient-friendly terminology for impact measures

Some of the original impact measures contained words or phrases that patients felt were less patient-friendly and recommended alternatives. For example, patients' 'critique' of evidence [...] amended to patients' 'evaluation' of evidence [...] and 'patient adherence' to 'medicine adherence'.

This last change demonstrates how patient insight into co-developing impact measures can reframe companies' thinking, shifting the emphasis to developing medicines and support programmes that patients value and which enable adherence rather than attributing lack of adherence solely to patients' behaviour.

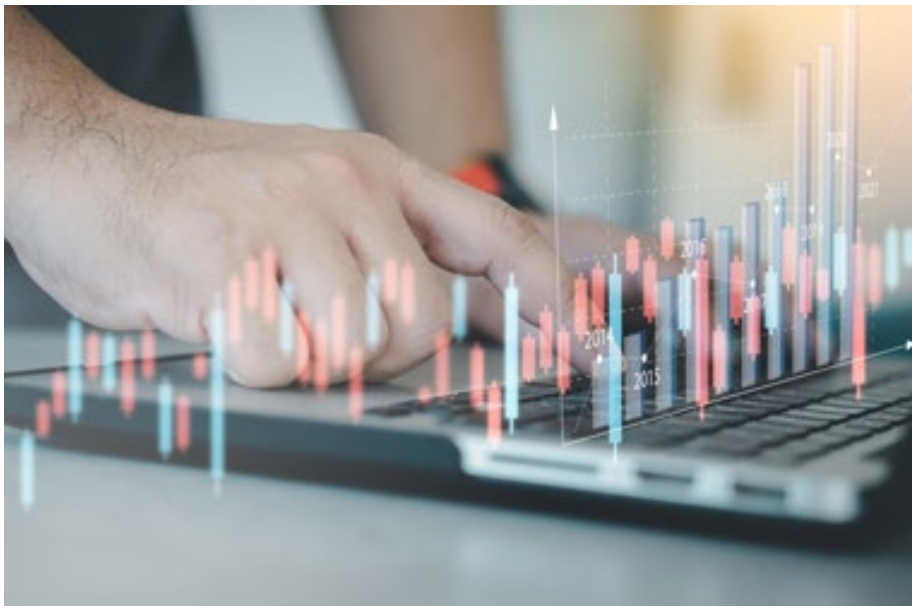
Next steps to implement impact

Patients expect a meaningful relationship with the biopharmaceutical industry, along with transparent dialogue before, during, and after a project. Discussions about planned impact must take place much earlier, ideally at project initiation, with expectations set for all stakeholders.



Outputs must not be put in a 'black box'. How value-impact was delivered, incorporated into decision-making, and operationalised should be proactively shared with the patient community to avoid those partnered with feeling frustrated about potential wasted opportunities. Establishing long-term, sustainable partnerships with patient experts and POs is another critical success factor.

This study provides additional considerations for the development of new impact measures: ensure greater context, capture how patients have influenced the project, and use patient-friendly terminology. Impact measures should also be tailored to the stage of a medicine's lifecycle, in which patient engagement projects are being undertaken.



Having co-developed a list of impact measures, the next step for companies is to consider how best to implement them in practice. Immediate practical considerations are to develop the right qualitative and quantitative data collection tools, assign new roles and responsibilities within the company, and develop data visualisation both internally and externally.

It will also be important to further involve patients in the implementation process ensuring, for instance, newly (co-)developed data collection tools capture the various aspects of value-impact.

About the authors

Dr Oleksandr Gorbenko, MD, PhD, BCMAS

Dr Oleksandr Gorbenko is an expert in public health, medical affairs, patient affairs, and community engagement with overall 22 years' experience in pharma industry. He graduated as a medical doctor (general practice) in O.Bogomolets' National Medical University and Shupyk National Medical Academy of Postgraduate Education and later worked on his PhD dissertation in the Ukrainian Institute of Public Health MOH.

Since 2019, Dr Gorbenko has a role of global patient affairs director at Ipsen, with the focus on developing necessary capacities/capabilities cross-functionally, advice-seeking and insights gathering with patient experts throughout the Medicines Development Continuum, patient experience mapping, partnership with several patient organisations, substantiation and inclusion of the specific PRO/PCO measurements as endpoints, defining the patient value attributes within the Target Value Profiles (TVP) for new medicines, KPIs and impact indicators on patient centricity for big pharma and other programmes. Board Certified Medical Affairs Professional (ACMA, 2020), he is a member of MAPS Focus Area Working Group on Patient Centricity.

Dr Sandeep Bagga

Sandeep Bagga is research and operations lead at MediPaCe. He is both a qualitative researcher and pharmacist, with senior-level experience in hospital, community, and business settings.

Sandeep has a broad portfolio of patient research from accurately mapping patient pathways, lived experiences of disease and treatment, through to using digital ethnography to identify normalised unmet needs. Throughout, he co-creates research design and delivery with patients themselves to ensure the project is respectful, compassionate, and that outcomes are accurately measured, informative, and relevant.

About Ipsen

At Ipsen, we're dedicated to improving people's lives and health outcomes. Our 4,500 employees around the world are committed to tackling areas of high unmet medical need to improve the quality of life of patients and caregivers.

Ipsen's vision is to be a leading global mid-size biopharmaceutical company, with a focus on transformative medicines in three key therapeutic areas: oncology, rare disease, and neuroscience.

Under the overarching theme of 'Focus. Together. For patients & society', Ipsen is focusing on four strategic priorities to accelerate our innovation and drive positive impact for patients, employees, shareholders, and society – a strategy for the short and long term:

1. Bring the full potential of our innovative medicines to patients.
2. Build a high-value, sustainable pipeline.
3. Deliver efficiencies to enable targeted investment and growth.
4. Boost a culture of collaboration and excellence.

About Research Partnership

MediPaCe is a values-first, purpose- and data-driven, patient engagement and research consultancy to the global Life Sciences and Medical technology industry. Our services fall into three main categories: strategic consultancy, delivery of patient engagement and involvement, and patient research.

Our approach is to integrate these areas to design optimised solutions that are delivered with strong methodology, open communication, and to gather impactful results that can be measured. MediPaCe was founded on the core belief that, to achieve the outcomes that matter most to patients, you must work in equal partnership with patients themselves. This foundational principle is the driving force behind our purpose-driven commitment to provide our clients with co-created insights that consistently deliver results.

The future of clinical research in neurology must be driven by community voices

Strong patient partnerships are more important than ever. In the pursuit of a personalised future for neurological care, collaboration and co-creation should be at the forefront of all research and development. That way, everything we do for patients, we do with patients.

By working directly with each distinct community, made up of people with a certain neurological variance, their families, supporters, and advocates, it is easier to gather different perspectives and address unmet needs. The community should be included in important decisions about their health, particularly in the early stages of clinical trial design to ensure research is designed to address unmet needs and in a way that encourages participation.

"The first step is recognising that within many neurological conditions, the community is diverse. Continuing to work with these communities is therefore crucial to ensure that we understand their needs and work alongside them to create meaningful tools and resources."

Caroline Averius, senior global patient partnership director, Roche

At Roche, this extends across Multiple Sclerosis (MS), Spinal Muscular Atrophy, Neuromyelitis Optica Spectrum Disorders, Alzheimer's disease, and autism, among others. Research is based on first-hand insights gathered from within these communities, which helps inform the initial stages of clinical trial design and drug development. Their voices need to be heard to understand the evolving needs of each therapeutic area, emerging expectations, and gaps they experience within their care.

The first step is recognising that, within many neurological conditions, the community is diverse. For clinical trials, this means that participants may have a range of needs that should be considered to develop truly inclusive research. Exploring these differences and celebrating nuances within the community requires a cultural shift – the pharmaceutical industry needs to embed inclusivity in all areas of the clinical trial process, from trial design and site selection to post-study communications.

"We cannot truly make clinical trials participant-focused until we begin to understand and take into consideration the needs of the community. Small changes can make a big difference to making participants feel seen, heard, understood, and supported, which in turn can increase clinical trial retention. That is why it is important that the industry continues to make strides in including participants in every part of the conversation."

Christine Eighteen, global patient partnership director, Roche

Making clinical trials participant-friendly isn't always complicated and can often include small details that make the study participant feel valued and comfortable. For example, ensuring there is on-site parking, that the site selection is not intimidating, or that staff can answer difficult questions can help ease negative feelings towards clinical trials.

Being considerate of the correct language to use when communicating with different neurological communities is vital. Many communities do not consider themselves as patients. People at different stages of a condition may also have different outlooks and attitudes.

For example, within the Alzheimer's community, people may have periods of wandering or forgetfulness, and may act differently depending on their mood and cognitive ability that day. This can make it harder to gain clear consent for somebody to take part in a trial. It is through close collaboration and in-depth discussion with the community that these potential issues can be addressed.

"I think it's very important to have a human approach. In this aspect, opening the [clinical trial] process with the family and the participant in a human way is of the utmost importance because you need to gain their trust and maintain it along the whole way."

Alzheimer's community representative

Co-creation of materials can help address the unique barriers these communities face

In neurology, every person requires a different approach to their management and care in a clinical trial. Neuroscience is an area that needs to see more of a participant-first approach emerging from the pharmaceutical industry. It is a must to make sure that the participant's and study partner's needs are put first, and to ensure that this is done correctly, by learning from the very same community the trial is serving.

Whilst some considerations that need to be made are general and apply to all research design, there are specific considerations depending on the therapeutic area being studied and neuroscience is a highly complex field where the individual needs of each and every member of the community vary. Given this, Roche has been working with representatives across autism, Alzheimer's, MS, and beyond to collaborate on a series of guidebooks for those designing clinical research, focused on improving the clinical trial experience.

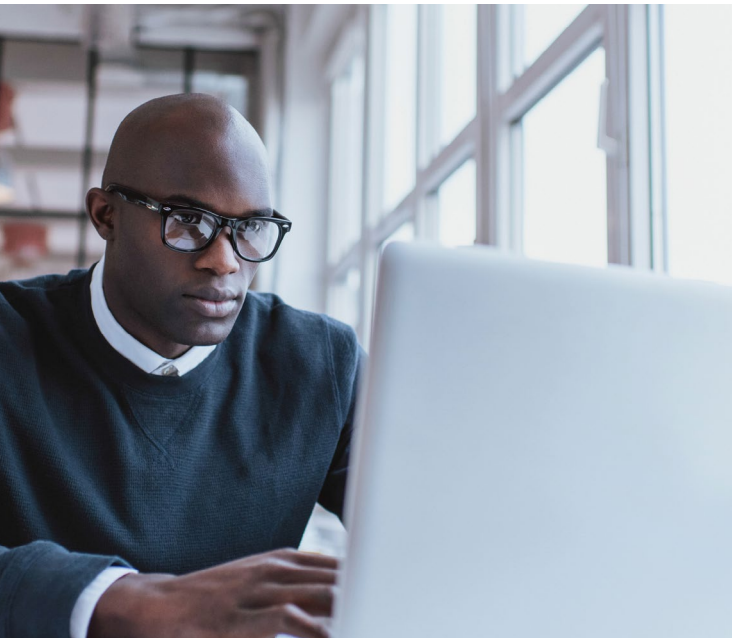
"Alzheimer's disease is one of the biggest public health challenges of our time, impacting millions of people around the world. The care and support for people living with Alzheimer's disease and their care partners is extremely complex and its provision is challenging. In this context, clinical research in Alzheimer's disease is more important than ever. We need to ensure that we are approaching clinical trials with open minds, and to not be scared to take risks or challenges to encompass the needs of participants."

Jannice Roeser, global patient partnership director, Roche

Co-created with the autism community, the Guidebook for Participant-Friendly Clinical Trials in autism highlights the diverse needs within the autism community, and how we can overcome or reduce the barriers participants face when taking part in a clinical trial. It provides both general and specific considerations for designing, conducting, and supporting participants in autism clinical trials.

"People with autism are not always included in the clinical trial development process, and large parts of the community with profound autism can often be excluded in conversations around their health. It is therefore very important that people with autism at all levels are informed about clinical trials and what it means for them."

Autism community representative



Paving the way for more successful research in clinical trials

A new guidebook for the Alzheimer's community, *Integrating the perspectives of people living with Alzheimer's disease and their study partners into clinical trial development*, provides a series of recommendations and key information focused on the complexity of the community.

Built with the Finding Alzheimer's Solutions Together (F.A.S.T) Council, it focuses on a range of considerations for the community and researchers. For example, it notes that people living with Alzheimer's are heterogenous in terms of symptoms and cognitive ability. They are sometimes psychologically vulnerable and may act differently day-to-day depending on their mood or willingness to participate in everyday life.

It is therefore important to provide information about a clinical trial upfront and be transparent throughout the process, sharing information about the trial design and study drug's mode of action in a way that is easy to understand. Study partners, who often do not know which challenge they will face from one day to the next, should be given clear expectations regarding their attendance during a trial and receive detailed information.

"It is driving personalised care in neuroscience, and ensuring all of our activities have a tangible, real-world impact."

Stephanie Ludwig, global patient partnership chapter lead, neuroscience, Roche

Co-created tools like these can pave the way for more successful research in clinical trials. It's up to pharmaceutical companies to embed community perspectives and insights into their core decision making. It is through these community insights that they will be able to deliver a truly personalised approach to neurological care.

About the authors

Caroline Averius

Caroline Averius, senior global patient partnership director at Roche, focuses on neurological communities such as the Autism community, and has worked in clinical development at several pharmaceutical companies (MSD, Actelion, & Roche) for 15 years. Over the last four years, she has been following her passion for inclusivity and equality, focusing on working with the communities in psychiatry to integrate their voices into the development of Roche solutions. She is also one of the organisers for the Roche-sponsored global platform, International Experience Exchange with Patient Organisations, that aims to inspire and equip patient communities to be strong and impactful partners with an equal voice in the healthcare ecosystem.

Christine Eighteen

Christine Eighteen, global patient partnership director focused on Multiple Sclerosis (MS), has over 20 year's experience working in the pharmaceutical and healthcare industry.

As a Canadian, she started her journey in medical communications before moving to Switzerland in 2008 to continue her journey at Roche. Here she has worked in various roles and it was when she was conducting market research with patients where she really learned about the impact of unmet needs that patients are facing today in healthcare. This ignited her passion to join the Global Patient Partnership team in 2019 where she is dedicated to raising the voice of the MS patient community and co-creating with them meaningful solutions in order to improve their lives and the lives of their supporters across the entire journey.

Stephanie Ludwig

Stephanie Ludwig has been with Roche since 2020 as principal global patient partnership director. In this function, she engages with multiple patient organisations, individual patients, caregivers, and other stakeholders to help generate the greatest value to people impacted directly or indirectly by neurological disorders. She has over 20 years of leadership experience in the research and consultancy industry for health-related projects and has trained global facilitators and moderators to interact with patient communities and patients. She holds an MA in educational science, psychology, and sociology and has worked as part-time lecturer at the Rheinische Friedrich-Wilhelms-Universität Bonn for psychoanalytical approaches in communication.

Jannice Roeser

Jannice Roeser has been a global patient partnership director at Roche since 2020, focusing on Alzheimer's disease. She has eight years combined experience in both healthcare communication and patient partnership. In her current role, she is facilitating the understanding and integration of the patient community perspective into the Roche activities in early development and beyond to improve the life of people living with Alzheimer's and their caregivers. Before joining Roche, Jannice was patient advocacy manager at Takeda France, covering disease areas such as multiple myeloma, lymphoma, and lung cancer. Jannice is native French and is passionate about her hometown Strasbourg. She has lived in Basel since 2020 and is now discovering the Swiss way of life.

About Roche

Founded in 1896 in Basel, Switzerland, as one of the first industrial manufacturers of branded medicines, Roche has grown into the world's largest biotechnology company and the global leader in in-vitro diagnostics. The company pursues scientific excellence to discover and develop medicines and diagnostics for improving and saving the lives of people around the world. We are a pioneer in personalised healthcare and want to further transform how healthcare is delivered to have an even greater impact. To provide the best care for each person we partner with many stakeholders and combine our strengths in diagnostics and pharma with data insights from the clinical practice.

The Roche Global Patient Partnership (GPP) team works across the company to embed patient perspectives and insights into core decision-making. By working with the very same communities the research serves, Roche aims to embed these perspectives in the early stages, such as through the development of the autism and Alzheimer's guidebooks. Both the autism and Alzheimer's guidebooks were produced and fully funded by Roche in collaboration with community representatives.

This article has been produced and funded by Roche.

Talent across borders: opening doors for refugee pharmacists in the UK

The idea of rebuilding your life from scratch, far away from the people, culture, and places you know and love, would be challenging for many people. Yet, for the growing number of individuals forced to flee conflict, natural disasters, and persecution, this is the reality of life as a refugee.

According to data from the United Nations Refugee Agency, the number of displaced people crossed the staggering milestone of 100 million in 2022. Among them are trained medical professionals who have much to offer healthcare services, if given the opportunity.

However, returning to practice can be a long and complicated process for refugee health professionals. Each health service has a specific way of working, and without education, employment opportunities, and support, even highly skilled individuals can struggle to navigate an unfamiliar healthcare landscape.

As part of efforts to help support displaced people on their journey to employment in the UK, on 11th August, IQVIA, in collaboration with RefuAid – a non-profit organisation that supports refugees and asylum seekers across the UK to access higher education and employment – invited 12 refugee pharmacists from across the country to attend the company's first employability event.

"We've always worked with organisations to support both healthcare opportunities and to really look at what we can do to aid, for example, people who may be working within the UK healthcare environment," says Melinda Morgan, associate HR director for IQVIA.

"With the RefuAid partnership, knowing that the individuals who came were qualified, experienced pharmacists who just needed to qualify for the UK put a different spin on it. We wanted to provide a day that we felt was meaningful for participants and utilised our skills at IQVIA."

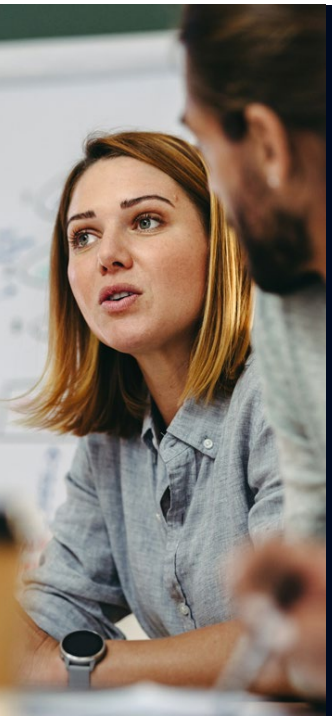
Designing an impactful event for pharmacists

This was not the first partnership that IQVIA has embarked upon to address areas of unmet need. The company has a rich history of partnering with external and non-profit organisations to advance public health efforts and improve access to healthcare around the world, having previously collaborated with Medicines for Malaria in Uganda, the American Medical Association's Task Force to Reduce Opioid Abuse, and the CEO Roundtable on Cancer, to name but a few.

As Morgan highlights, during initial collaboration discussions it quickly became clear that RefuAid and IQVIA shared similar goals and values. By combining the former's access to participants and experience of supporting refugees as they seek employment with the latter's skills and in-house knowledge, the companies could develop a programme that benefitted both participants and hosts.

"It seemed like an absolute match," she explains. "We could see RefuAid's work in supporting refugees to access higher education and employment, and we knew that we have the skills, in-house knowledge, and employability support through our TA and HR experience."

But having the right foundations in place was only step one in the process for the two organisations. For all involved, providing in-depth, useful information was paramount. As Morgan explains, "It was really important for us to make sure that the attendees came out of the day feeling that it had been a really valuable experience and increased their knowledge of the UK healthcare market."



While RefuAid took on the task of identifying suitable participants for the event, IQVIA led the development of each session, with team members across the organisation working together to create the structure and content for the day. As such, employees from IQVIA's Interface team of clinical pharmacists were able to utilise their specific skills in an unusual setting.

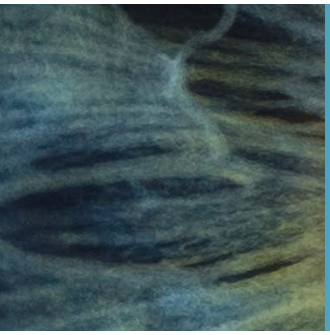
Knowing that each of the 12 attendees would come to the event with a high level of scientific knowledge, the organisational minds behind the sessions focused on supporting them in making effective applications.

To set the scene, IQVIA's team opened the event with an overview of the NHS and the opportunities available for those with a pharmacist background.

"The NHS, as we all know, is quite an interesting world to navigate, and understanding all the different pharmacy roles within is really valuable," says Morgan. "For example, there are lots and lots of opportunities within IQVIA, which need their pharmacist background, but they don't have to have necessarily requalified in the UK to be eligible.

"A lot of people weren't aware that, for example, you might use your pharmacy experience within medical information or pharmacovigilance or even our sales roles as well."

Building on this foundation of information about the UK health system, IQVIA then invited attendees to take part in a series of interactive workshops, including CV writing activities and mock interviews, to demonstrate how pharmacists can use these skills in a professional job application.



"We focused quite a lot on UK expectations of CVs and interviews because refugee pharmacists come from a variety of different countries and backgrounds," says Morgan. "For example, some people are used to putting salaries on a CV, which isn't typically done in the UK."

But while education and employment may have formed the main dishes of the day, for Morgan the relationship-building opportunities facilitated by the event offered an equally important element.

"One of the great things that we hadn't recognised before that day was that the clients from RefuAid had never actually all met or been able to all congregate together as a group of pharmacists," she explains. "You could see everybody sharing phone numbers, getting connections for later, and it was just wonderful. We really should not underestimate how incredibly valuable it was to have time to be together as a group and network with people of the same professional background and shared personal experience. Therefore, we will build in more time for this in future sessions."

Fostering meaningful connections and partnerships

In the UK, where urgent demand for skilled health professionals is growing, supporting programmes to help refugee health professionals realise their full employment potential can open doors for a previously untapped workforce to enter the healthcare system.

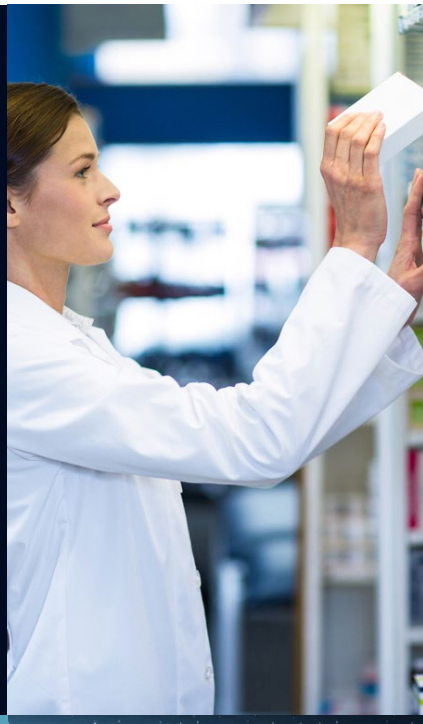
“There are a lot of unusual barriers for people at the moment, such as accessing the right level of language training,” explains Morgan. “The more industry knows about these issues, the more they can do to help these individuals.”

By partnering with RefuAid, IQVIA team members were given a unique opportunity to develop relationships with each pharmacist. These are not just casual acquaintances, but valuable networking opportunities that can be mutually beneficial for both potential employees and those already working in the sector.

“This gives us access to more individuals who’ve got great skills and engagement and will be absolutely brilliant employees, but who maybe wouldn’t find it as easy to access our opportunities without that,” says Morgan. “Through our work with RefuAid, we opened up the opportunity and the doors, if you like, to a new group of people who maybe we didn’t automatically think of being able to approach for roles.”

But fostering these relationships requires continued support. As such, Morgan notes, IQVIA was keen to extend engagement activities beyond the initial event.

“The pharmacists have set up mentoring support, which will be reviewed over the next few months, but the idea is that that would potentially go over a six-month period,” she says. “It is something that RefuAid know is very valuable to their clients, but actually the lovely thing was our pharmacists, including people who were not there to support on the day, are also going to be involved in that.”



“There’s a much greater group giving support going forward. It’s just lovely that everyone can share their knowledge and experience,” she says.

Laying foundations for future collaborations

For IQVIA, designing a programme specifically aimed at helping refugee pharmacists build employment skills was an unknown – but very welcome – challenge.

"We didn't know how much engagement there was going to be until we were looking for about 12 to 15 people, and RefuAid just came back instantly and said, 'yes, we absolutely can do this'," says Morgan.

For Morgan, establishing a solid partnership was an invaluable part of developing and delivering the employment event. By working closely together, IQVIA and RefuAid were able to leverage their strengths and provide support where needed.

"Any partnership is really based on good mutual outcomes and wanting the other party to succeed. Having that relationship in mind, regardless of whether it's for profit or not," she explains. "Being really open about timeframes and keeping in touch regularly is really vital for that. At IQVIA, we're also very aware that we are taking people away from a billable work or day-to-day job sometimes in investing our time in that, so we need to think about how much time it takes to set these things up, but frankly, once you've done it once, it's relatively easy to keep doing it in the future because we got all the baseline in there."

The overwhelmingly positive response from attendees, RefuAid, and internal team members was an encouraging sign that IQVIA's expertise and resources could provide considerable value for underutilised groups seeking employment opportunities.

"Everybody came out of it feeling that they had done something that was meaningful. The team was so happy that the individuals themselves were engaged on the day and could feel that they've positively contributed to other people, but equally were using knowledge and skills they had anyway," she concludes.

About the author

Eloise McLennan is the editor for pharmaphorum's Deep Dive magazine. She has been a journalist and editor in the healthcare field for more than five years and has worked at several leading publications in the UK.

About the interviewee

Melinda Morgan associate HR director, IQVIA

Melinda Morgan has 25 years of experience working in healthcare leading engagement projects, delivering large scale teams and project builds with a strong focus on employee engagement and wellbeing programs across EMEA. She has worked in both the private and public sectors, including with social impact programmes supporting communities experiencing barriers to employment.

As a senior HR business partner and global project lead for IQVIA's eight worldwide Employee Resource Groups, Melinda is highly experienced in driving talent strategies to maximise employee opportunities for development and project creation for opportunities to give back to the community.

About RefuAid

RefuAid is a non-profit organisation based in London, which supports refugees and asylum seekers across the UK to access higher education, requalification, and employment.

Visit https://refuaid.org/ to find out more.

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.

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Research Partnership
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Endometriosis: hidden suffering and disease burden

Improving patient outcomes starts by listening to and understanding their needs. Research Partnership's Living With reports give a voice to patients living with a variety of chronic conditions, by reporting on quantitative research undertaken directly with them.

Women's health has been under the spotlight in recent years, as a much under-resourced area of investment.

Here we share key insights from the 2022 Living with Endometriosis report carried out in the US with over 100 patients, which highlights the pain and suffering experienced by over 10% of American females and explores what the pharmaceutical industry can do to support them.

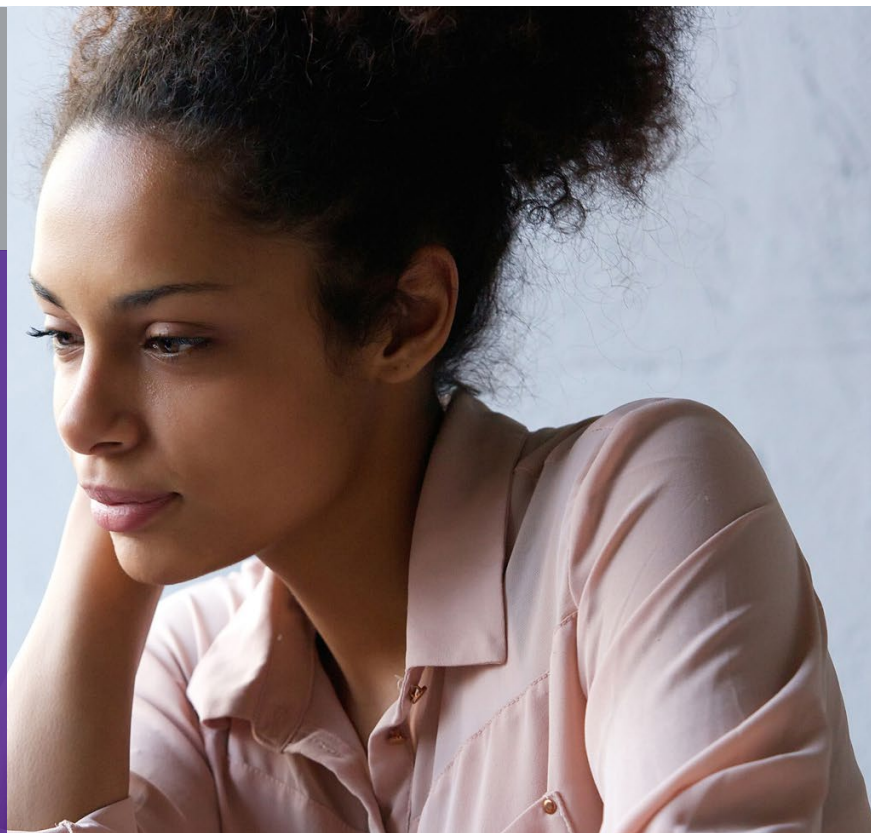
Women living with endometriosis

For most girls, starting their period is a significant event; a milestone which marks the transition between childhood and womanhood. Calendars are marked, protection is stored in purses and spontaneous trips, sleepovers and pool parties require more logistical planning than was considered before.

For women starting their period, it can be an empowering new chapter, and after becoming used to the process, is relatively manageable. However, for around one in ten US women, their periods are going to mark the start of something worse; not only will they need to manage their calendars around certain times of the month, but pain medication may also be required. Endometriosis is a condition where tissue, similar to the lining of the womb, starts to grow in other places, such as the ovaries and fallopian tubes.

Diagnosis

Endometriosis symptoms usually begin at puberty, and for some, from their first period. For more than 60% of endometriosis patients, symptoms start before the age of 20. Without easily identifiable biomarkers, pathogens or non-invasive diagnostic tests to diagnose endometriosis, patients usually have to wait between 4-11 years from symptoms to receive a definitive diagnosis.



In our 2022 Living with Endometriosis US study, we spoke to 101 endometriosis patients, many of whom reported suffering for years before seeking healthcare professionals' (HCPs') advice. Endometriosis often runs in families: if a family member has been suffering, but hasn't had a diagnosis or sought medical help, it can mean young women follow on the same path. A 40-year-old patient explained: "I didn't see the doctor for years because my mom did not know any better. My mom was not going to take me to the doctor."

Based on our research, over eight in ten patients suffer from heavy, painful periods prior to their endometriosis diagnosis and manage their symptoms with over-the-counter (OTC) pain relief and heat pads. Periods tend to get progressively heavier, more painful and the disruption to school, employment, and family becomes too much to bear at which point, the sufferer usually then reaches out to the OB/GYN.

Impact

Even after diagnosis and prescribed treatment, endometriosis continues to place a significant burden on women and approximately seven in ten still suffer from heavy bleeding during their periods. However, when speaking to those living with endometriosis, the most challenging aspect is pain, which causes the greatest stress and takes a large emotional toll on women.

Around one in four women we spoke to have also been diagnosed with depression or anxiety. One 46-year-old described a day on her period feeling like: “I do not want to talk to anybody, I want to isolate myself. However, the more isolated I am, the more I get into my head. I start to have dark thoughts.” Women are highly anxious that their condition will worsen and how they will cope in the future.

Nearly half of women feel less confident in their body because of endometriosis and two in five feel the condition has a significant impact on their sexual drive, again affecting their relationships. One 25-year-old explained her mood as: “I’m just lacking in motivation, sometimes I just do not feel like being intimate due to the pain”.

Along with the mental and emotional impact, endometriosis also has a practical, educational, and sometimes financial impact. Most endometriosis patients are employed and four in five miss days from employment or study per month due to their condition.

Treatment

Most of the women we spoke to were receiving hormonal-based contraceptive or IUD treatment for their endometriosis. Around half were highly satisfied with their treatment, but many cited its main benefit as easy administration. Only one in three believed a reduction in their pain was a benefit of their prescribed treatment, and treated patients continue to rate their pain as severe. One 33-year-old patient told us: "[On treatment] I still feel my symptoms are severe enough for it to be debilitating sometimes or frustrating." To help alleviate their pain the majority of women in our study also relied on OTC pain relief.

With limited treatment options and few that directly treat pain, patients are often prescribed multiple types of birth control over the years. Birth control is not only relatively ineffective against pain, but side effects are common. According to patients in our study, side effects are the primary drawbacks of their treatment, and the main reasons for switching medications.

Our research identified that patients' greatest unmet need is a treatment that can reduce heavy bleeding and provide long-lasting symptom relief from pain. In 2018, the writer and actress Lena Dunham wrote an article for Vogue stating that after a decade of excruciating pain and eight prior surgeries, she took the drastic step of having a hysterectomy at the age of just 31. Patients often have multiple surgeries for their endometriosis, but it is usually not a permanent solution.

For 40%-50% of patients who undergo (non-hysterectomy) surgery, symptoms will recur within five years. Around one in five women we spoke to regretted not telling their doctor more information at their most recent visit, and their main concerns often centred around surgery. One 46-year-old women told us: "I always have the question of should I just get a hysterectomy? Should I just cut it all out? Am I ready internally and mentally to have a hysterectomy?"

Information and desired support

Patients are increasingly seeking information and support outside of their doctor's office, with nearly eight in ten looking to social media for endometriosis content. Awareness of the condition is increasing and celebrities such as Amy Schumer, Chrissy Teigen, and Halsey have spoken about the impact the condition has on their personal life, health, and fertility.

Of the women we spoke to, those who had been diagnosed within the last three years claimed to be more knowledgeable and knew a lot about the condition before diagnosis (vs. those diagnosed more than three years ago).

Increasing public awareness and education around how to recognise endometriosis symptoms are likely to lead to increased diagnosis. Pharma-sponsored PR could help to encourage young women to seek HCP help earlier, and not suffer in silence for years.

Health and wellness are a large part of many young women's lives, but its association with endometriosis is not yet well known. Filling this knowledge gap provides women with a chance of implementing lifestyle habits which can help ease their endometriosis symptoms. A 47-year-old stated: "I would like more holistic options, but I do not know that the science is there in terms of how things like lavender interact with the oestrogen system. I would like to see more dietary information and support."

To support the 6.5 million women in the US with endometriosis, pharma companies can not only develop more effective treatments, but also increase general awareness and facilitate more open dialogue between patients and their family, employers, and doctors about the condition. For endometriosis, no publicity is bad publicity.

About the authors

Mariel Metcalfe, director, head of Living With
Mariel is the Head of Living With and has 18 years of pharmaceutical market research experience in a wide variety of qualitative and quantitative methodologies, including global tracker research and segmentation. She has worked in a variety of therapy areas including infectious, autoimmune, respiratory and metabolic diseases. Mariel has extensive experience in patient insights and is committed to bringing the patient voice to life. She has written multiple articles and hosts the podcast 'Living with Chronic Illness' which is designed to give a voice to patients suffering from chronic conditions.

Nicole Syms, associate director, Living With
Nicole is an associate director within the Living With team at Research Partnership, with nearly ten years of pharmaceutical market research in primarily quantitative methodologies and with a particular focus on patient research. Nicole has conducted patient research in a variety of therapy areas, including many autoimmune conditions, women's health and other long-term or chronic diseases; providing a great understanding of how chronic disease impact patients' lives and what makes patients' experiences different from one another.

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Research Partnership
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Research Partnership, an Inizio Advisory Company, is a world-leading custom and syndicated global insights partner for health and life science companies. We optimise commercialisation success through evidence-based, story-told insights and recommendations that leverage custom market insights, syndicated real-world insights, and market access insights for the entire product lifecycle to empower better decisions and create long-term value for patients.

"Living With" is a series of reports based on quantitative and qualitative market research with patients, providing comprehensive and cost-effective insight into the patient journey from pre-diagnosis to stabilisation. We are continually expanding our reports to cover a large range of therapy areas and markets. To find out more information please visit:
researchpartnership.com/livingwith

Find out more about our Living with Endometriosis US 2022 report

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