White Paper

Three Key Elements of Patient Centered Research

How pharma can use validated measures and strategies to engage patients at every stage in the drug/device development lifecycle.

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Introduction

Patient-centric drug development is top of mind with regulators, payers, healthcare professionals, and patient advocacy organizations everywhere. It would be remiss of a drug or device developer not to ride this wave of generating information for the patient – ultimately the end user of their product or treatment.

After decades of being treated as passive participants in the research process, patients are demanding to be heard. They want their feedback and insights to be incorporated into research strategies and industry stakeholders are beginning to move towards this shift.

Initially, biopharma may have seen demands for the patient voice in clinical research as burdensome. But over time they've come to recognize the pivotal role patient feedback can play at key points throughout the drug/device development lifecycle:

- In the early stages, sponsors can gather valuable insights from patients about living with the disease, their unmet treatment needs, and outcomes of relevance, to help them select molecules, devices, doses and administrations of interest and shape their research strategy.
- During trial planning, patients can offer insights on trial design that can eliminate unnecessary obstacles, accelerate recruiting and improve engagement.
- During the trial, feedback from patients about their experience can provide deeper understanding of the treatment's safety, efficacy and perceived value, which can help secure approval and payer support, and can enhance prescribing confidence.
- Once a drug is on the market, ongoing feedback from patients provides insights about their real world experience with the treatment, and can help sponsors craft more targeted marketing and patient support materials.

Leading companies are leveraging a number of strategies to capture patient feedback in more standardized ways through a combination of qualitative and quantitative methods (otherwise called mixed methods) and through the use of digital tools. This combination provides them with rich and interpretable data that can inform a framework to incorporate the patient voice in drug development, and to prove to regulators, payers and providers that they treat the patient perspective as a valued part of this process.

In this paper, we will further explore IQVIA's perspective from lessons learned about how we help clients to optimally capture meaningful patient data through relevant patient-centered research, utilizing technology, and engaging directly with patients throughout the drug development lifecycle.

"After decades of being treated as passive participants in the research process, patients are demanding to be heard."

Part 1: Speaking to patients and families is the best way of generating disease insights

MAKE SURE YOU FULLY UNDERSTAND WHAT IT MEANS TO LIVE WITH A DISEASE AND ITS TREATMENT

Sponsors who want their new drugs and medical devices to be approved, covered and prescribed need to know what matters to people living with a disease before designing and implementing trials to measure improvement. Only a person living with a disease can reliably and comprehensively explain their symptoms and the impact that the disease has on their quality of life. This is not always the person who has the diagnosis; speaking with people who care for those with the disease (parents, spouses, dependents) and those who treat the disease (healthcare professionals) can offer additional important insights.

Understanding this is key to developing a strategy for demonstrating potential benefits of drugs and medical devices in development. It may not matter to a person living with a disease that their tumor has shrunk if they still can't walk, go to work, or live a pain-free life. For patients, the reduction of physical symptoms and improvement in aspects of their quality of life are often the measures that matter.

To understand this, we must speak directly with people living with the disease. Taking a semi-structured approach and using specially trained interviewers allows the most robust insights to be generated. These insights can then be the basis from which a structured strategy is defined to measure what matters to people living with a disease in a trial.

DESIGN PATIENT-FRIENDLY TRIALS TO GET THE MOST RELEVANT AND COMPLETE DATA

Patient interviews can be equally valuable in designing the trial itself. Collecting insights about why a patient would agree to participate in a trial – and what's holding them back – helps sponsors build more engaging trials that maximize recruitment and retention. It seems obvious, but sponsors may not involve patients in trial design, which is a missed opportunity to create better trials and a more invested patient community.

Co-designing trials with patients brings a number of benefits for sponsors.

Most importantly, it provides the patients' perspective of what it is like to participate in a trial while living with their condition, and where hidden obstacles may lay. From inconvenient appointments and excessive trial site commutes, to confusing paperwork, off-putting advertisements, and painful procedures or side effects, clinical trials can be full of physical and emotional land-mines for patients. While sponsors can't eliminate all

"Only a person living with a disease can reliably and comprehensively explain their symptoms and the impact that the disease has on their quality of life."

of the barriers, they can use that feedback to reduce unnecessary burdens – like too many site visits or excessive blood tests – and educate patients about the need for others. This engagement will lessen their fears and make them feel valued and heard.

Sponsors are also adopting virtual elements in trial designs to ease data collection and make trials more patient-friendly. Online surveys, telemedicine visits, and wearable devices that automatically capture patient data can all enhance participation and reduce patient burden while keeping patients engaged. Virtual trial elements also improve follow-through and accountability for sponsors and demonstrates to patients that their input is valued.

When seeking a more patient-centric trial environment, co-designing trials using scientific techniques, such as patient panels in which a group of experts reach a consensus on design decisions, can ensure consistency in approach across trials and ensure high quality and actionable input.

Taking the time to engage with patients during trial design and customizing the trial design based on their feedback will result in more appealing trials that are easier to recruit and more likely to retain patients. That translates to better, faster, and more reliable trial data.

HOW TO ENGAGE PATIENTS IN QUALITATIVE RESEARCH

- **Start with patient advocates.** Patient advocacy groups can communicate your interest in talking to patients throughout their network. This can be an efficient way to connect with patients who want to play a role in the research environment and have strong opinions to share. These patients will bring an informed perspective and help you build grassroots support and awareness of your research.
- **Talk to research-naïve patients.** To create a full understanding of disease and a truly engaging trial you also need to talk to people who've never considered trial participation and are less educated about their condition and treatment options. These patients can be harder to find and may be more skeptical of your advances, but they also offer a different set of insights.
- **Truly listen.** In these conversations the patient is the expert, so let them tell you what they need, then use their feedback to shape or alter your understanding and the trial design to make it more patient friendly. You will not get value from this process if you spend the whole time explaining what you think or want.

Part 2: COAs bring structure to the measurement of patient insights

Insights from qualitative research bring real value to a program, but these insights need to be formulated into a scalable strategy to measure what matters in a robust, reliable, valid, structured and systematic way. In other words, asking a friend who has diabetes what they'd like to see in a trial, or asking the marketing team to do a patient survey, is not usually a reliable source of patient feedback.

This is where Clinical Outcome Assessments (COAs) become vital to the research experience. COAs are scientifically rigorous instruments used to capture unfiltered data directly from the patient about a specified area of interest in a clinical study. They can take one of four forms[i]:

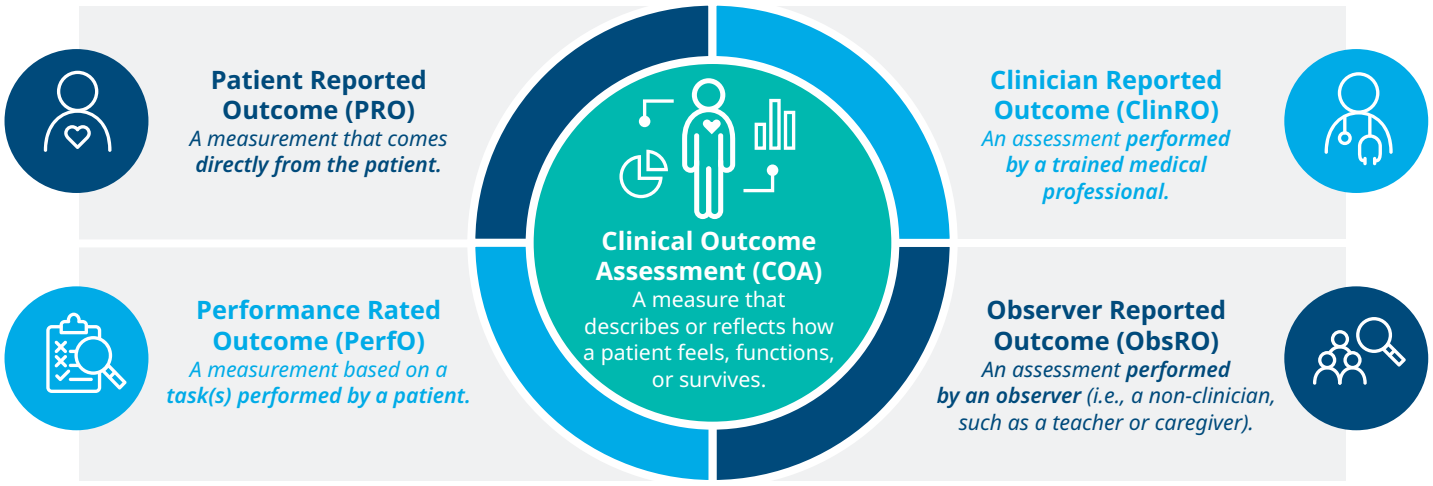
PROs capture feedback directly from the patient about the status of their health condition, their symptoms, and their quality of life, without amendment or interpretation by a clinician or anyone else. A PRO can be self-reported or recorded through an interview.

ClinROs capture feedback from trained health-care professionals observing the patient's condition. Most involve a clinical judgment of observable signs, behaviors, or other manifestations related to the condition, such as a rash, or limited range of motion.

ObsROs capture feedback from caregivers about observable signs, events or behaviors related to a patient's health condition. They are particularly useful for infant, children, and patients who cannot report for themselves.

PerfOs capture data on standardized tasks performed by a patient, such as a timed walk or memory test. They can be administered by a trained individual or independently completed.

Clinical Outcome Assessments (COAs) can take one of four forms



eCOA platforms intelligently automate capturing a patient's experiences through robust and reliable electronic measures, providing real-time insights that inform trial progress and demonstrate results.

[i] https://www.fda.gov/drugs/drug-development-tool-qualification-programs/ddt-glossary

COAs allow investigators to capture data from patients in a consistent and integrated format. The science of COA development and use is now well established and it has become an expectation that COA data will be presented for regulatory review, and to an increasing extent, payers are seeking them out when deciding which drugs and medical devices to support and at what price point. Physicians are also using PRO data to support prescription decision-making and to have more meaningful conversations with patients about their treatment options

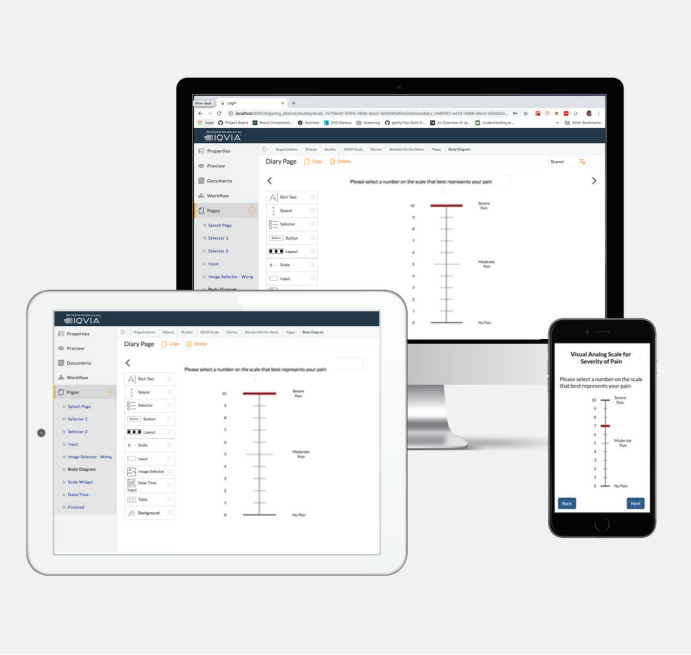
Being able to provide information about clinical outcomes using COAs will therefore ultimately influence regulatory approval, payer reimbursement, and rates of prescriptions. Pharma companies can generate further value from these COA data sets to develop targeted patient support programs that enhance usability, adherence and persistence.

COA data can be collected using a paper form at clinic visits. However, there are notable issues with paper completed COAs and in the past decade there has been a significant investment in electronic modes of collecting COA data (eCOA). This increases reliability and validity of the assessment, allows for time-stamped completion between site visits as relevant, and maximizes measurement precision.

And now, the IQVIA eCOA Solution enhances this further, providing an advanced study build and execution platform to help optimize real-time, direct-from-patient data collection. The platform features a library of assessments, which gives sponsors instant access to more than 750 eCOA instruments and growing. This platform cuts months from the development process.

IQVIA eCOA

Advanced study build and execution platform to optimize real-time, direct-from-patient data collection in clinical research and real world studies



The simple and intuitive interface provides advanced patient assessments that enhance the patient experience and improve data quality – amplifying the patient's voice to accelerate outcomes.

Key customer benefits:

- **Reduced cycle times –** enables set up of eCOA up to 3x faster than traditional methods while improving quality
- **Increased efficiency –** eliminates manual efforts through auto-generated project documentation and an extensive library of pre-built assessments
- **Improved data quality –** enhanced reliability and protocol compliance through simplified patient engagement
- **Real-time insights –** cloud-based visual eCOA configuration for greater transparency, control, and collaboration

These technology advancements are an important step, but the mode of collecting the data is heavily intertwined with the instrument itself. There is no point in having a really good instrument without a robust way to collect data, and vice versa.

HOW TO BUILD/SELECT COAs THAT DELIVER RELEVANT DATA

- **Build a conceptual model.** The conceptual model should summarize the patient experience and the relationships between the disease etiology, patient symptoms, and immediate impacts of these symptoms. It should also explore more general impacts on the patient that relate to the condition and treatment, such as depression or loss of independence. This conceptual model should tell us what is most important to patients in defining treatment benefit.
- **Choose/build a COA.** The conceptual model will guide the selection of the COAs, and help sponsors determine whether an existing instrument should be used, modified, or if a new COA should be developed. Sponsors need to verify that the selected, modified, or new COA will collect data in a format that can be scored and analyzed in a way that will be meaningful to regulators, payers and other relevant stakeholders.
- **Show the COA works.** The COA should be examined for reliability and validity of measurement and research should be undertaken to understand what sort of change is meaningful to patients. If a label claim is the ultimate goal, a defined series of regulatory agency meetings should be a part of the development process to ensure alignment on key aspects of instrument modification or development with the agencies in question. Once the COA has been defined, sponsors should test it with patients in the target population to ensure that they understand the instrument, that the data collected aligns with the concepts being measured, and that the design does not create undue burden on patients.

Although not traditionally considered as COA, two other types of structured data capture are emerging as important in understanding disease and treatment; patient reported experience (PRE) measures, through which patients can offer valuable insights about trial experience and perceptions about the efficacy, safety and risk/harms of a medicine or device; and digital health technology tools (DHTT) which can collect active or passive data directly from wearable and non-wearable technology, making it easier for patients to record and share data in real time.

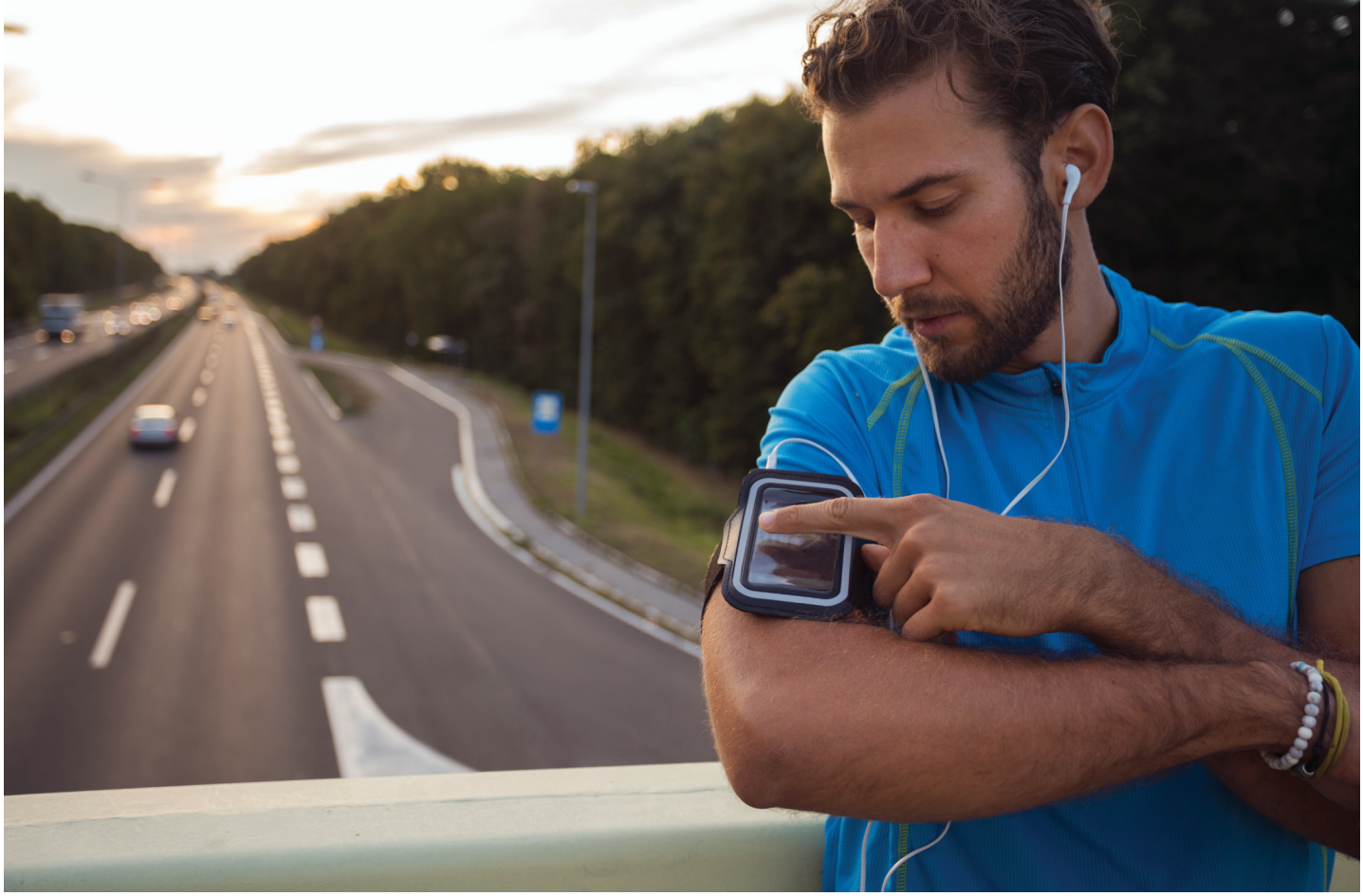
Part 3: COA data should not only be used in drug and medical device development

In the real world, patients face myriads of obstacles in their care journeys, including payment challenges, transportation issues, confusion about dosage, and other issues. Collecting data about the real world treatment experience using COA, PRE and DHTT helps pharma craft more targeted information about treatment benefits and use, and devise support programs that solve real patient problems, rather than investing in projects based on assumptions that often miss the mark.

This can be particularly valuable for treatments where there is a post-authorization commitment (safety, efficacy), or that use a novel mode of administration.

RECENT EXAMPLE

We recently worked with a pharma company that spent millions of dollars developing a patient reminder app in an effort to increase adherence, only to find out later that the real problem patients faced was their discomfort with a new injection mechanism. Had they gathered patient insights through qualitative research or COAs at the outset of the project, they would have made a more patient-centric plan.



There is an increased emphasis on including the patient perspective in evidence-based decision-making to support patient communication and enable shared decision-making, and capturing this can be facilitated through the use of technology – specifically through the use of eCOA, ePRE and DHTT to gather the patient perspective in the healthcare practice.

When pharma demonstrates a commitment to capturing this perspective it can be a differentiator for physicians and patients.

However, as with all measures, the tools used to capture patient feedback in real world settings must be validated to generate reliable and valuable data. Sponsors need to carefully consider what data should be measured, how it should be measured, and what format those measures will take to ensure appropriate, reliable, sensitive and valid results.

HOW TO GENERATE DATA IN CLINICAL PRACTICE

- **Don't treat patient feedback as a one-and-done activity.** Introducing COA measures in clinical practice creates an ongoing dialog between patients, physicians and other stakeholders. These real time insights can be used to adapt current engagement efforts, improve communication, and inform future trial designs.
- **Don't wait.** Too often pharma turns to patients for feedback or insight after a problem occurs. This adds time and cost to the process, and can leave patients angry, frustrated, and turning to other treatment options. Instead, talk to them first about what they need, or what obstacles they face in using your treatment then build your solutions around that. This human-centered design approach ensures the decisions you make have patients' best interests at heart.

Conclusion: The right way to collect patient data

There are many ways to capture the patient perspective in clinical research and beyond. The sponsors that embrace the need for patient insights, at every stage of drug development, and leverage validated tools to capture it, will be better able to maximize their investments and deliver value-driven treatments to market.

At IQVIA, we've learned valuable lessons about the right way to capture patient data through years of building and collecting COAs, utilizing technology to capture patient data and engaging with patient communities throughout the drug development lifecycle.

From our experience, we would propose three key lessons which should be applied whenever biopharma wants to integrate the patient perspective into their development and commercialization efforts:

1. **Use scientific methods.** As with all elements of clinical research, capturing patient data must be done with scientific rigor using validated tools and practices. To ensure the data you collect is relevant and reliable, include scientists in the design and validation of surveys and questionnaires, and train live interviewers in scientific methods to avoid the risk of bias, or pre-determined outcomes that can occur when data collection is conducted without consideration of scientific principles or practices.

2. **Bring in these methods early in the trial design process.** COAs and other patient data capture methods are most valuable when they align with specific endpoint results. Choosing and building these tools in the planning stages ensures the data you capture will measure the outcomes of value.

3. **Don't ask for too much.** COAs add valuable insights, but they can be overly burdensome for patients if the frequency or duration of assessments takes too much of their time. Before adopting any COAs, determine if the data is really necessary, if it adds value, and what you can do to make it easier for patients.

If you can adhere to these principles, then COA data – collected in a patient-friendly way – can provide valuable insight and outcome data which can shape review and approval, prescribing habits, and ultimately, patient health and well-being.

About the authors

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As vice president and head of the Patient Centered Sciences team at IQVIA, Jean Paty is a leader in effective strategies and practices of capturing patient experience data for use in clinical development and commercial success of new and existing medical products.

Jean has been published extensively in the areas of Clinical Outcome Assessments (COA) and electronic COA (eCOA). He has deep expertise regarding regulatory guidance for development and implementation of eCOA, in addition to scientific, clinical, regulatory implications of COA data collection in clinical trials. Jean joined IQVIA in 2014 and has held a variety of leadership positions within the company.

Jean has a BS in Psychology from the University of Toronto, and an MS and PhD in Psychology from the University of Pittsburgh.

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As senior principal and head of Scientific and Analytic Research for the Patient Centered Sciences team at IQVIA, Matt provides scientific oversight and support to PCE consulting projects, and other PCE services that require scientific participation, with a focus on patient insight generation and COA.

Matt has extensive experience in COA development, implementation, analysis and interpretation.
He has worked across academic, consulting, clinical and industry settings and is an active leader in the COA science industry. Matt joined IQVIA in 2019.

Matt has a BS in Psychology, and an MS in Health Psychology. He is a Chartered Practitioner Health Psychologist and a Chartered Scientist. Matt has been awarded Fellowships by the Royal Society of Medicine and the Royal Society of Public Health and an Associate Fellowship by the British Psychological Society.

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