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WHITE PAPER

# Making Clinical Research Better for Patients with Patient Centricity by Design

By Alicia C. Staley

# Introduction

A 2017 BMJ Innovations journal article defines patient centricity as "Putting the patient first in an open and sustained engagement of the patient to respectfully and compassionately achieve the best experience and outcome for that person and their family."

Although the journal Applied Clinical Trials started reporting on patient centricity in 2014, there is still no universally accepted definition of a patient-centric trial and no standardized way to determine whether a protocol design is optimal for patients. Lacking those tools, many sponsors struggle to design and conduct studies that accommodate the perspectives and preferences of patients and caregivers.

Companies often capture patients' insights for a specific trial by convening an advisory board, conducting feedback sessions, launching a marketing campaign, and employing patient-facing technologies such as digital health and mobile apps. While these techniques can be helpful, they don't represent an integrated or systematic approach. Sponsors are left to balance the preferences and priorities of patients and caregivers with those of the sponsor team, including medical directors and statisticians, and site personnel like investigators and study coordinators while meeting scientific and regulatory requirements. But these occasional sessions often lack follow-up and preclude consistent relationships, so patients rarely hear whether, how, or why their feedback is being used, which can erode trust in the process and make future collaborations less likely.

At Medidata, we wanted a gold-standard methodology to embed patient centricity into the software we build to run clinical research. We call our process Patient Centricity by Design (PCbD), a spin on the concept of privacy by design (PbD) developed by IT pioneer Ann Cavoukian.

> PCbD is a powerful methodology that integrates patients' views and guides the creation of patient-facing solutions to improve the clinical trial experience, accelerate development timelines, and bring therapies to market faster. It comprises an analytical framework and stepwise process that integrates patient perspectives into the software development life cycle, allowing us to create technical solutions that improve the patient experience in clinical research.

Before launching a trial, we subject all proposed patient-facing technologies and prototype solutions to the PCbD process.

Our process begins with our patient insights board, a diverse panel of patient experts gathering insights in multi-day face-to-face or virtual workshops, post-event surveys, and retrospective sessions. From there, our product designers and engineers develop and implement proposed enhancements with full accountability and traceability to patients' insights. The process is iterative and relies on a sustained, dynamic dialogue between patients, disease experts, and trial designers.

# Understanding the patient's journey

The starting point for any patient-centric design is empathy for and an understanding of the patient's journey, from the initial disease diagnosis to clinical trial participation to receiving study results when the trial is completed. The "customers" of trials are patients, not sponsors, sites, trial designers, software coders, data-entry technicians, or analysts. This is a paradigm shift for the industry.

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We recently worked with a pharmaceutical company to review its informed consent form (ICF) to test whether patients found it easy to understand and complete. The patients told us that the quality and clarity of the ICF affects their willingness to join the trial. One said, "If I can't get through the informed consent process, how could I get through the trial?"The client wanted to know how it could transform its consent process from a confusing, tedious, document-heavy chore into an informative, engaging, empowering interaction that better prepared patients for participation.

After putting the client's ICF through the PCbD process and presenting alternative ICFs to a group of cancer patients, we recommended that the client:

- **Restructure the ICF.** Patients wanted practical information—such as how much time the trial would take and the logistics of procedures—up front, not buried deep in the document.
- **Edit the language.** The ICF sometimes used technical jargon. "Early escape," for example, was used to describe moving patients out of ineffective treatment groups once certain criteria are met, but patients thought the phrase sounded negative, as if they were escaping from "clinical trial jail." They emphasized that words matter. "One wrong word could scare people away," said one. "You can't unsee it."
- **Prioritize clarity and navigability.** Patients said the length of an ICF is less critical than how easily they can navigate it.

## Core Principles

## Elevating patients to equal partners

Patients are equal, integral partners in the PCbD process, not just workshop attendees. Full collaboration demands that dialogue start in the early stages of trial design and continue to the end, with patient feedback and suggestions analyzed and adopted when feasible. If feedback is rejected, the reasons should be transparent and promptly communicated to patients. These design decisions are traceable and allow for greater trust and transparency.

A clinical trial's target population determines fundamental design and operations. Will the study be enrolling patients with recently diagnosed advanced cancer or with a mild, asymptomatic respiratory disorder? Will it enroll young children cared for by multitasking parents with limited time, or elderly individuals who may prefer unrushed, in-person interactions with the investigative site staff?

We recently used PCbD techniques to evaluate a client's design for an online pretrial registry seeking to enroll patients with a heart condition. Such registries can engage, educate, and empower patients to prepare for participation in clinical research.

One of our clients wanted to understand what motivates patients to join a patient registry and how they prefer to receive information about clinical trials for which they might be eligible. The 20 heart disease patients who participated in our design studio offered actionable insights, such as placing the "Register Now" link below the "Learn More" one rather than above it. They emphasized that the patient portal needs to explain how the registry might impact them personally: "What will it give me that will directly affect how I live my life with this disease?" They preferred images of real, diverse people over cartoonlike graphics.

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Patients said they were eager to hear about clinical trial options, but only from a "trusted source"—such as their physician or an online patient community—with trial sponsors clearly identified at the outset and eligibility criteria presented in plain language. Finally, patients wanted assurance that sponsors would report trial results to them at the end of the process. The client made changes based on this feedback and revised its communication plans to strengthen its messaging and outreach to patients.

## Creating solutions that align with patients' goals

Many technologies available today can help streamline clinical trial operations, including patient wearables, remote monitoring, teleconsultations, and digital therapeutics. But just because we can use these tools doesn't mean we always should. And when we do, we should think carefully about how we deploy them to align with the patients' goals.

Prior to the pandemic, sites were solely responsible for receiving, storing, dispensing, and returning drug supplies. One way that trial sponsors avoided shutdowns and delays was with direct-to-patient (DtP) drug shipments. Since DtP is appropriate for many trials and can reduce burdens for patients and clinical research staff, it's a pandemic-accelerated innovation that may persist.

At Medidata, we wanted to design a best-in-class DtP shipping software with patients in mind. We began by conducting a session with our patient insights board and learned that patients have specific needs and expectations regarding medication shipments. They want to:

- be able to diagnose, communicate, and resolve drug shipment issues quickly and efficiently so they can safely move forward with other trial activities
- know how and when drug shipments will arrive so they can plan their activities on those days
- use the communication channel—email, text, or phone—that best fits their lifestyle and activity level

With these insights from our PCbD process, we can better stage a design studio to help our software meet these goals while fulfilling regulatory and safety requirements.

## Meeting and exceeding patients' expectations

Patients participate in clinical trials to gain access to a therapy that might be better for them. But the rates at which they can be recruited and retained are heavily influenced by their expectations of the trial experience. It's critical to understand what patients want from a trial.

At Medidata, we're working on software and hardware solutions that enable wearable sensors and devices to collect high-quality data for clinical research. Usage of such devices is growing quickly, and sponsors often assume that patients are willing to wear them for sustained periods, recharge them, and troubleshoot them. To better understand how patients feel about sensors, we conducted a workshop on their expectations of digital health technologies. Most said they were willing to take on the additional burden if it gave them insights into their health and could benefit their treatment and health journeys.

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Because of their ubiquity in the consumer market, patients are familiar with wearables like Fitbit and Apple Watch. They expect to interact positively with sensors, so failing to meet that expectation can quickly damage their relationship with the trial sponsor. The industry has the opportunity to leverage these new consumer behaviors and apply them to clinical research.

Patients want to be able to customize and personalize their preferences via a minimally invasive user interface, and even more important, they expect access to the data the sensors collect. Patients on our insights board expressed a strong desire to interact with their sensor data and be able to grant or revoke others' access to it with relative ease. That gave us a chance to explain that revoking the sponsor's access might result in the patient being dropped from the study. We also learned that patients want to understand what the data means in the context of their health and identify behavioral changes that may improve their quality of life. These findings have profound implications for how to incorporate sensors into clinical trials.

## Verifying that solutions improve outcomes

One differentiating aspect of PCbD is the rigorous evaluation of solutions; if they do not improve the patient experience and outcomes in the relevant condition and population, they are not included in the design.

There's a widespread assumption that decentralized trials are inherently patient-centric, but replacing site visits with telehealth and home nursing, shipping medicines DtP, and collecting data with sensors and wearables are not more convenient for all patients. Some patients prefer to talk with their doctor in person. Others are reluctant to have medical personnel in their homes or may be intimidated by remote technologies and devices.

Patient centricity is not about a specific tool; it's about using the right tools to reduce the burden on each patient in the trial. If a fully decentralized trial is possible and warranted, the PCbD methodology would test whether that works in the real world for the trial's particular patient population. If not, a flexible protocol should incorporate other options.

Patient access to trials can be limited by the geographical location of sites, the willingness of healthcare professionals to collaborate on trials at competing sites, and travel expenses, among other factors. Restrictive eligibility criteria and misunderstandings about the overall purpose of clinical research can also impact patient enrollment and retention. PCbD empowers sponsors and trial designers to anticipate many of these complex issues and mitigate the risks.

Every successful company pays close attention to what its customers want, and researchers running clinical trials should do so too. Sponsors can't achieve patient-centricity just by offering televisits or home nursing. It is incumbent on us to listen much more closely to patients so that we can best address what they need to be comfortable with our trial designs. By partnering more closely with patients, we will advance the science of clinical research and bring new therapies more quickly to those who are waiting for them.

**ABOUT THE AUTHOR**
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