Q&A PUBLICATION

From Past to Future:

The Evolution of Patient Engagement and Excellence in Patient Partnerships

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How has patient engagement evolved over time, and what lies ahead? In this piece, we explore the journey towards patient partnerships of today and tomorrow, with three experts in the field:

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You have extensive experience working in patient engagement and patient advocacy. What has changed from your early times in this space? What is different today?

Alastair Kent: I started in patient advocacy nearly 40 years ago, and it was a very hierarchical system. Patients were expected to defer to doctors, and there was little interest in patient engagement from the pharmaceutical industry. Patients were at the bottom of the hierarchy and expected to follow what was deemed best for them, although there were some differences across specialties.

Over time, there has been a growing recognition of the importance of the patient and family perspective. Today, the system is much more of a matrix, where different stakeholders bring different types of expertise to the development and delivery of healthcare. There is also greater awareness that while diseases may have defined causes, their trajectories vary significantly between individuals, requiring more tailored approaches. The rise of patient organisations has played a key role in this shift. While their level of influence still varies, there is now a much broader acceptance of the importance of engaging people with lived experience.

Danielle Drachmann: When I began, patients were often invited in to "give input." Today, I see patients shaping agendas. That is a profound shift. In the rare disease organisation I founded, families did not wait to be included. We initiated research ourselves and partnered with clinicians as equals. We did not have the luxury of waiting, because there were few experts, limited funding, and the published literature did not reflect patients' reality. Our children were deteriorating. The patient community took control and drove the research forward: Parents became organisers, literature reviewers, fundraisers, data contributors, and co-authors. We invited clinicians and scientists to collaborate. The synergy of these two different kinds of knowledge allowed us to advance by leaps and bounds.

What began as urgency in rare disease has since inspired broader patient engagement across therapeutic areas. This has demonstrated something powerful: When different forms of expertise – experiential, clinical, and methodological – are treated as complementary rather than hierarchical, research moves faster, becomes more relevant, and restores trust. The rare disease community showed what is possible when patients are not positioned as participants in research but as drivers of it.

In my role as Senior Patient Partnering Manager at PPD, part of Thermo Fisher Scientific, I see the industry shifting from "involving" patients after the fact to proactively co-creating with them from the beginning, integrating patient insight into protocol design, feasibility, and endpoint selection.

Today, the difference is that patients are no longer asking for a seat – we are building the table.

What are the most important evolutions and trends of the past years?

Alastair: One of the most important shifts has been a growing recognition of the value of lived experience, both at the level of individual patients and through patient organisations. Another key trend is the changing relationship between industry and patient organisations towards more balanced partnerships. At the same time, expectations on healthcare systems and industry have evolved. In assessing new therapies, industry must demonstrate that the benefit for the patient is significant, and new therapies are not assessed solely based on physiological benefit, but also on the broader societal value. Finally, the way patients access information is changing. With the rise of digital tools and social media, patients may no longer rely on patient organisations in the same way as before. This creates both challenges and opportunities, reinforcing the need to engage both at the community level and directly with individuals to capture nuance.

Lara Bloom: We have moved from tokenism to transformation. Ten years ago, patient engagement often meant a listening exercise. Today, people with lived experience are co designing trials, shaping endpoints, sitting on regulatory committees, and influencing global policy. The language has shifted from "including the patient voice" to recognizing lived experience as expertise. We are also seeing far more structure, with frameworks, metrics, and compensation models emerging. It is not perfect, but it is progress. The most important evolution is that engagement is no longer a side conversation. It is becoming central to how decisions are made.

Danielle: First, the rise of co-production. Lived experience is increasingly recognised as shaping research agendas. Second, the conversation is slowly shifting toward Return on Engagement (ROE) – not just

asking where the short-term financial return on investment is but whether engagement meaningfully changes outcomes. For too long, patient engagement has been evaluated by short term return on engagement (ROI) and activity metrics: number of advisory boards, number of patients consulted, number of workshops held. ROE challenges that thinking. It asks harder questions: Did patient insight alter the protocol? Did it reduce participant burden? Did it improve recruitment or retention? Did it shape endpoints that truly reflect lived experience? Did it build trust that makes future research possible? ROE recognizes that the real return is not measured only in funds saved but in better science, stronger relationships, and outcomes that matter to the people the research is meant to serve. Third — and this matters deeply to me — we are starting to acknowledge not just the benefits but the risks of patient engagement. Our research has shown that passion and hope, when not managed, can quietly turn into burnout. For example, parents and medical experts can feel pressured to work long hours, disrupting their personal lives and damaging their own physical and mental health. To help reduce this risk, we call for funding for patient-led organisations and the creation of ethical guidelines for patient engagement.

The life sciences industry is (re)structuring and rethinking how it engages with people with lived experience, to deliver more sustainable and impactful partnerships. Are we heading in the right direction? What could be improved or done differently?

Lara: We are moving in the right direction, but we are still learning. Many organizations are building patient engagement functions and embedding them earlier in development, which is encouraging. The next step is cultural, not structural. Patient partnership cannot sit in one department. It has to influence clinical, regulatory, access, and commercial strategy. What could be improved is consistency and long-term commitment. Relationships built only around a product lifecycle will never be truly transformative. Partnership must be ongoing, transparent, and rooted in shared goals.

Danielle: Yes — but we need courage. From both nonprofit and industry perspectives, I see real intent to partner meaningfully, but structural change must follow cultural change. Engagement cannot sit in a slide deck — it must sit in governance, budgets, and decision checkpoints. If engagement influences million-dollar development decisions, then it deserves million-dollar-level seriousness — through measuring ROE, by funding infrastructure, and by protecting people.

Alastair: There is now a clearer understanding that relationships with patient organisations need to be established at the early stages of clinical development.

Industry must also think more radically about what "engagement" means - it does not just mean sitting around a table and chatting.

Partnerships must be realistic and sustainable. While they are generally willing to work with industry, patient organisations often operate with limited resources, and engagement should consider their capacity. They strive to respond to the current needs of their community, address their priorities and make space to engage with industry.

What advice would you give to organizations early in their patient partnership journey?

Danielle: Start early. Start humbly. And start structurally. Engage as soon as you have a research question. If you want to work with patient partners or advocacy groups, you need to listen, be respectful and embed engagement across all layers of the organisation.

Do not invite patients in after the protocol is written. Do not take advantage of people with lived experience and be respectful of their time, notably through offering compensation.

Lara: Start by listening, but do not stop there. Build trust before you build programs. Be clear about your intent, compensate people fairly, and create feedback loops so contributors know how their input shaped decisions. Do not aim for perfection on day one. Aim for authenticity and humility. And most importantly, engage diverse voices, not just the usual suspects. The communities most affected by inequity are often the least represented in decision making.

Alastair: It is important to start by recognising both your ambitions and your limitations — including the level of engagement that is possible and channels through which the engagement can happen.

Patient organisations need to be careful not to overcommit, especially in the early stages of relationships. Even well-intentioned opportunities must be sustainable over time, otherwise there is a real risk of

damaging long-term relationships with industry, healthcare systems, and the communities that these organisations serve. Engagement from all parties should be manageable and realistic, so that organisations can deliver on their commitments.

There also needs to be a commitment from healthcare systems, from industry, to support patient organisations in participating effectively. This includes helping them understand how regulatory and policy processes work, how to contribute, and supporting them in structuring their input so that it resonates with decision-makers.

What is "excellence" in patient partnerships? How does it look like in real life?

Danielle: Excellence is when patient partners are fully trusted and allowed to lead, when the patient partner is seen as the expert because of their lived experience. I saw this in practice during a recent industry interaction. I joined a call during which, from the very beginning, the patient principal investigator was introduced and took the lead, defined the questions, and was the subject matter expert within the company. Seeing that level of trust in his lived experience and his expertise was a powerful example of what patient-driven partnership can look like. That gave me hope that we are moving to something where lived experience can take the lead.

Lara: Excellence is when people with lived experience are not invited into the room as guests but as equal partners — at the right time. In real life, it looks like co-created research priorities; patient reported outcomes that truly reflect daily impact, transparent data sharing, and shared accountability. It looks like meetings where lived experience shifts the direction of a discussion. It looks like long-term relationships built on respect, not transactions. Excellence is not a badge or a framework. It is a culture. It is inviting lived experience to help build the table, not just sit around it.

Alastair: Excellence is defined by whether all parties feel they have had a meaningful opportunity to contribute and make a difference. It is about being recognised as bringing legitimate expertise, and about participating in a system that is equitable, timely, user-friendly, and grounded in both science and lived experience. What excellence looks like will vary depending on the disease area, the level of scientific understanding, and the available expertise. What works in one context may not work in another. From a patient perspective, excellence means feeling that the system is there for you. That nothing is too complex, too rare, or too challenging for the system to respond to, and that your needs are taken seriously. The most successful collaborations have been at the level of policy or public engagement on the part of the industry or healthcare systems.

How are health equity considerations influencing partnership frameworks?

Alastair: Health equity is a critical consideration across healthcare systems, industry, and patient organisations. Healthcare systems must think of equity between conditions and diseases, but even within a single healthcare system, there can be significant variation in access to expertise, treatments, and timely care. There also needs to be a consideration of equity between different sections of society, for example links to ethnicity, language, culture, and religion. Patient organisations, often volunteer-led and resource-constrained, may struggle to reach all segments of their communities. Yet, a global overview and the individual patient's nuance are vital. We should find ways of combining both the overview with the specifics through the use of a variety of engagement techniques.

Danielle: I can attest to the importance of health equity. Knowledge and influence should not be gated by mobility. I know from experience, mobility is influenced by who can travel and afford to attend, who has the health and ability to sit through full-day meetings, who has childcare, and who can take unpaid leave without consequences. For example, when I was homebound caring for my children, conferences weren't realistic. In rare diseases especially, the people who know the most about the condition are often the ones least likely to be in the rooms where decisions are made. We are currently unable to truly incorporate health equity into patient partnering. For example, when we are recruiting patient advisors for an advisory board in Europe, we often only choose patient partners who are fluent in English and able to participate. This means that we are only targeting an elite population of often expert patients and caregivers. We need asynchronous models. We need compensation. We need transparent access. We need multiple entry points. We need intentional outreach. We need to take advantage of technology so that people can speak comfortably in their native language and gain access so that they can influence decisions. If we are serious about equity, we need to stop asking who can show up — and start asking who the system prevents from showing up.

Lara: Health equity is prompting us to confront uncomfortable truths. Who is not at the table. Whose data is missing. Whose outcomes are worse. Partnership frameworks are increasingly prioritizing diversity, accessibility, and community-based engagement. This means investing in translation, digital inclusion, culturally competent approaches, and reaching beyond traditional advocacy networks. Equity is not an add-on. It should shape who we engage, how we engage, and what success looks like.

What would you like policy, regulatory and corporate decision-makers to truly understand about the value of investing in patient involvement?

Alastair: Healthcare systems are built on a social contract, particularly in Europe where they are grounded in solidarity. For these systems to function effectively, the public needs to feel that resources are being used fairly and that the system is trustworthy. This is where the patient voice is important: It helps ensure that healthcare delivery reflects real needs and experiences, reinforces public confidence that decisions are both fair and meaningful, while helping to demonstrate value for money in healthcare, at a time where there is increasing pressure.

Danielle: Many families – especially in rare disease – have been dismissed, misdiagnosed, or disbelieved. People die or suffer because they do not get the treatment they need. People are traumatized by their interactions with the healthcare system. This also creates epistemic injustice in the healthcare system – where lived experience is treated as lesser knowledge.

In today's development landscape – with increasing protocol complexity, recruitment delays, and regulatory scrutiny – failing to integrate patient insight early and throughout the product lifecycle risks not meeting the patient community's needs and therefore developing products that nobody wants.

Decision makers need to think about the concept of ROE. They need to think about the long-term strategic and financial impact instead of only the short-term financial impact.

Lara: Patient involvement is not a cost. It is risk mitigation, innovation, and trust building all at once. When you involve people with lived experience early, you design better trials, reduce protocol amendments, improve recruitment and retention, and create solutions that people actually use. Beyond that, it is about legitimacy. Health systems and industries that serve people must be shaped by people. Investing in patient involvement is investing in relevance and sustainability.

On a more personal note, what continues to motivate you in your daily work?

Alastair: The sense that it is possible to influence the system in ways that can deliver meaningful benefits. Even small contributions can help improve outcomes, and at the very least ensure that changes are thoughtful and unlikely to cause harm. There is value in knowing that you have had the opportunity to contribute to a system that aims to be fair, effective, and responsive to real needs.

Danielle: I started Ketotic Hypoglycemia International because I was desperate for answers for my own children. What began as a cry for survival became science. Then community. Then systemic change. Today, I stand in two worlds – leading a global patient organization and working inside a global CRO. What drives me now is helping those worlds move toward each other.

When lived experience shapes research design – and research infrastructure actively values and integrates that experience – science becomes more precise, more humane, and more relevant.

I will keep working until patient engagement is no longer powered by good intentions alone – but by structure, investment, and long-term commitment.

Lara: I live with one of the conditions I advocate for - EDS. I know what it feels like to be dismissed, misdiagnosed, and told it is all in your head. I also know the power of finally being heard. What motivates me is the belief that no one should have to fight alone to be taken seriously. Every time a patient tells me they feel seen, or a policymaker changes course because of lived experience shared in a room, I am reminded why this work matters. The patient voice is powerful. When we amplify it together, we change systems.

This publication is part of a DGA series about patient engagement.

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