Patients as Partners in Clinical Research

Reflections and themes

US conference: March 20–24, 2024, Philadelphia, PA

European conference: May 14–16, 2024, London, UK

Can we say research is a ‘partnership’ yet, and is it making a difference?

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The 2024 Patients as Partners in Clinical Research meetings were held in Philadelphia in March and London in May, and were attended by pharmaceutical and regulatory industry representatives, patient advocates, patients, and care partners eager to continue progress and share experience related to engaging patients more directly in research and clinical development.

The conference underscored a notable shift from discussing the rationale behind patient-focused drug development (PFDD) to exploring practical implementation strategies which signifies a growing emphasis on sharing best practices across advocacy groups and industry stakeholders.

Lumanity staff in attendance at the events:

Amanda Sergison-Main
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Heather Nyce
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Sarah Lucas-Eaton
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Key themes at this year's congresses

The discussion is progressing from 'why' to 'how' to 'now' when involving patients in clinical research design

More consistent and earlier approach to PFDD

Equitable access to research including health literacy, DE&I

Measuring impact of patient engagement in research

PED supports better decision making

Personalized data release and pre-competitive collaboration

Improving the sponsor, site and study participant partnership

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Key: DE&I - diversity, equity & inclusion; PED - patient experience data; PFDD - patient focused drug development

Patients, caregivers and patient advocates led discussions, presentations and calls for change

“Understand me first, then my disease”

– Patient speaker

Key themes

A more consistent and cross-functional approach to PFDD means better R&D

PFDD supports a transformational shift from research-based scientific and clinical endpoints, to patient-centric development focused on quality of life and what matters most to patient communities.

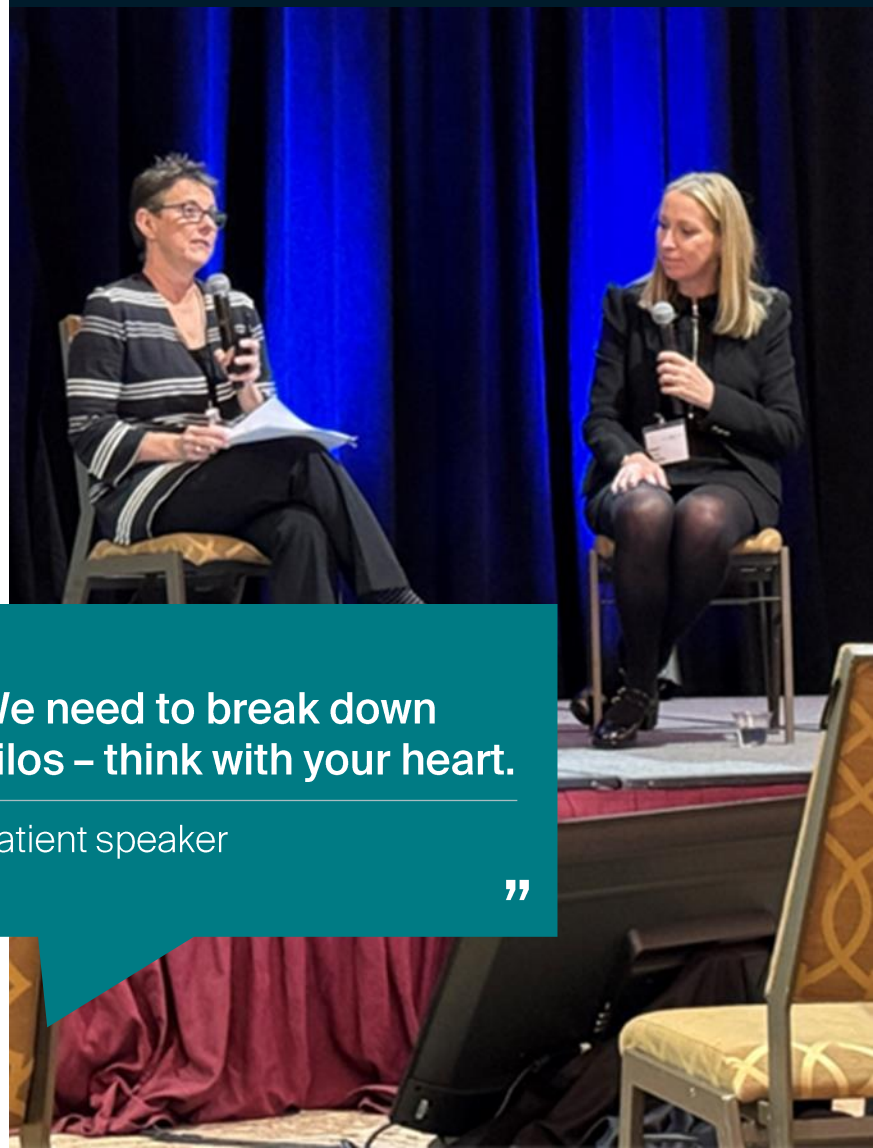
Cross functional frameworks for early engagement of patients in the drug development process, starting from the translational phase, are key and require buy-in and guidance from senior management.

This ensures that patient needs and preferences are considered and integrated into trial design from the outset.

Several examples of patient engagement frameworks were shared across the US and European conferences:

- AstraZeneca developed a Patient Centricity framework following internal survey showing inconsistent patient engagement practice across the business; now measuring impact and have shown that 100% of studies now have patient engagement review
- Pfizer have implemented 6-theme framework based on 'input, output and value' to demonstrate greater R&D efficiency and more rapid access
- Novartis: co-created a framework with six measurement themes that enables patient engagement activities to be measured for impact at a societal level

Lumanity panel presentation:
Implementing a Patient Focused Drug Development Framework for Enhanced Patient Centric Strategies



> "We need to break down silos – think with your heart."
>
> Patient speaker

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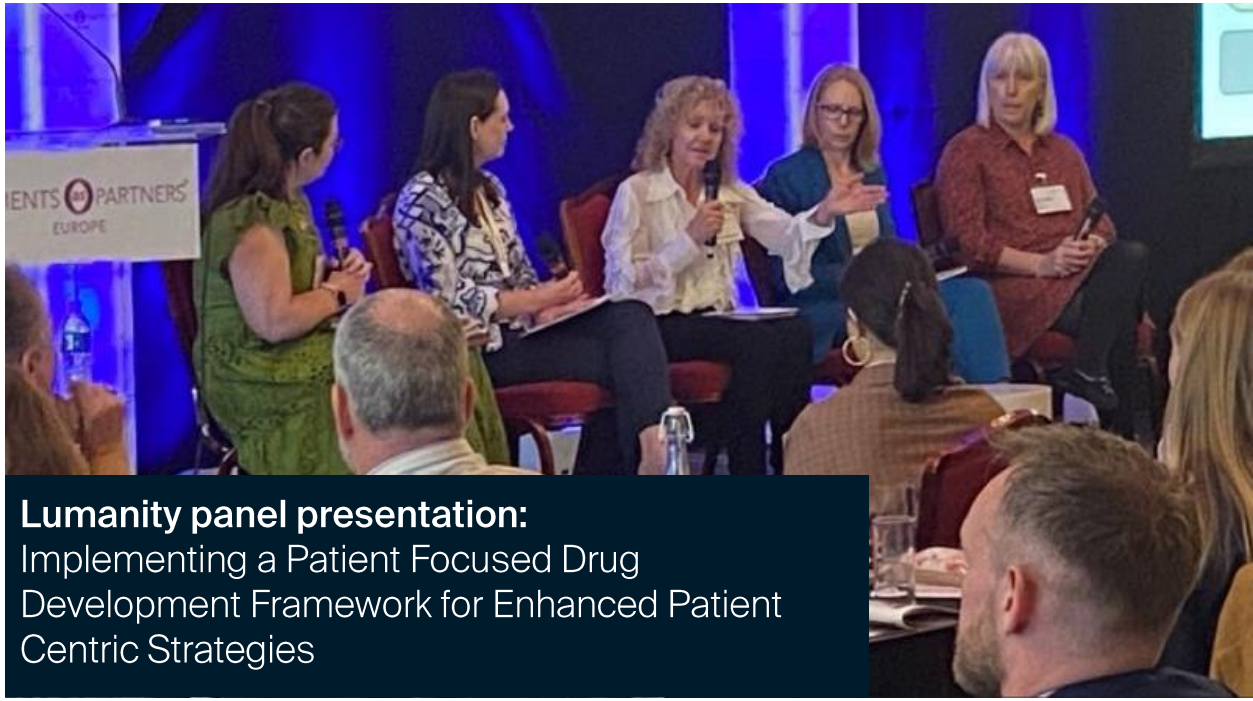
Key: DE&I - diversity, equity & inclusion; PED - patient experience data; PFDD - patient focused drug development

Insights increase the impact of patient engagement in clinical research

Early engagement offers the greatest impact on outcomes and better trial experience.

Engaging patients and advocacy groups in early research phases is crucial to ensure patient needs and preferences are considered and integrated into design.

Patients involved in early design recounted how the experience encouraged a more trusting and transparent partnership and allowed them to better support other members of the community who may want/need to become involved in research. Peer-to-peer support is a powerful tool.



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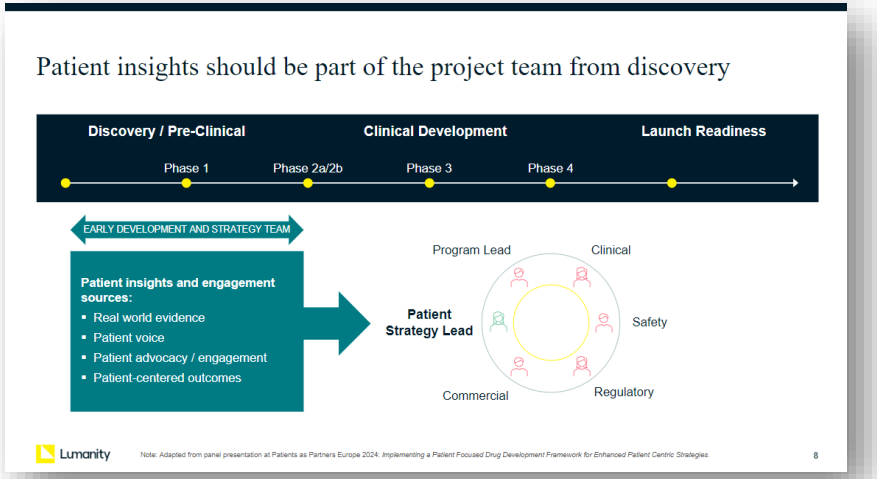


Key themes

Health equity, access and inclusion

Enhancing health literacy

Efforts are needed to improve health literacy to ensure patients can fully understand and engage with the information provided to them throughout the trial process and beyond. AI tools are increasingly being used, such as to improve readability of materials.

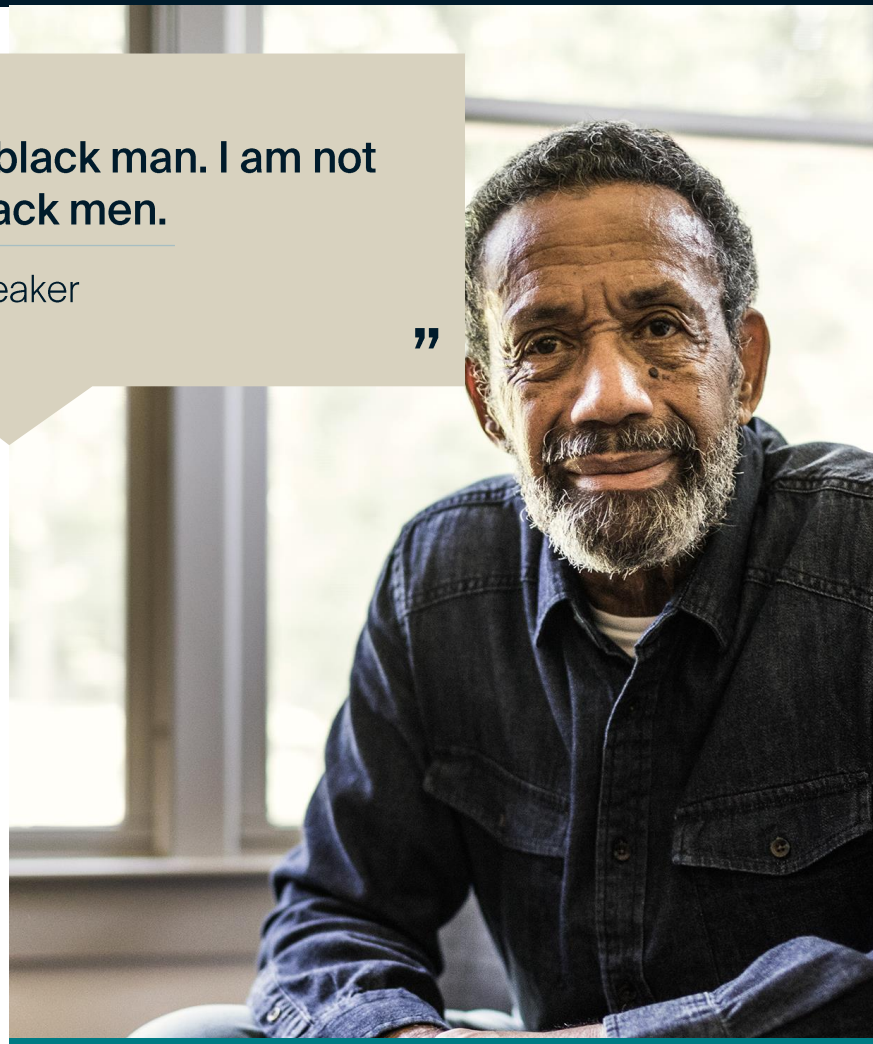
Diversity and inclusion

Patient advocacy groups continue to push for better representation in research, specifically to ensure study populations include cohorts that match the community and include those at risk. Attitudes toward research differ significantly across cultures and those within the culture are best placed to help with education and misinformation.

Capacity building for advocacy groups

The need for capacity building and support for patient advocacy groups was highlighted, including providing resources, education, and templates to empower them to contribute meaningfully to drug development processes and data collection.

> **“I am a black man. I am not ALL black men.”**
>
> PAG speaker



Key takeaway

PAGs are being asked to contribute more to research based activity, while also trying to support and include diverse communities; the burden is high on stretched organizations.

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Key: AI - artificial intelligence; PAG - patient advocacy group

Key themes

Patient experience data supports better decision making

The value and use of PED[1] is increasing as regulators and industry more consistently use it for decision making

Growing collaboration between regulatory bodies, industry and the patient community: the amount of PED is increasing in marketing applications (FDA)

- The FDA shared examples in treatment-resistant depression and to demonstrate how PED was used to evaluate the patient perspective of disease during review
- Co-created clinical outcome assessments are being used as endpoints
- PED is increasingly used in regulatory reviews to understand patient perspectives in benefit-risk assessments

EFPIA presented results from a survey that demonstrated **strong support from industry for the development of an EU reflection paper** to provide guidance on regulatory submissions, review and benefit-risk assessments

The value of **precompetitive collaboration** was highlighted. By working together on shared goals, stakeholders can leverage expertise, and work with patient communities to build collective PED databases and clinical outcome assessment instruments, which reduces the 'ask' on patient communities

Key takeaway

Evidence demonstrates increasing use and utility of PED; highlights the need for more routine, collaborative and consistent collection.

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Key: FDA - US Food & Drug Administration; PED - patient experience data

Note: [1]PED is data gathered using several different methodologies to collect patients' experience of their health status, symptoms, disease course, treatment preferences, quality of life and impact of health care

Patients, caregivers and patient advocates led discussions, presentations and calls for change

“I am more than a datapoint in a clinical trial; I have given you my blood, my story and my journey; what are you giving me back?”

– Patient speaker

Key themes

Personalized data release requires collaboration and communication

The calls to share individual participant data and test results are getting louder; several trial sponsors have created transparent platforms to share certain data without compromising scientific integrity of research.

Clinical trials generate vast amounts of data but a small percentage of this is published or released. There is an increasing call for sponsors to return data to patients, both as trials are in progress and after the trial has concluded.

From a patient perspective, sharing results of trial interventions such as imaging and bloodwork can avoid unnecessary duplication of testing for patients and reduce resource and costs in primary care situations.

Issues such as return timelines for patient data and the need to ensure patient access to their personal information need to be addressed to foster transparency and empower patients.

Key takeaway

There is likely to be increased pressure on study sponsors to share data with participants and communities.

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“The average oncology Phase 3 clinical trial collects over 6 million data points.”

– Tufts Center for the Study of Drug Development

What is the patient burden linked to this, and is the data being optimally used?

Key themes

Optimizing the sponsor, site, and study participant partnership

Consensus based and co-created resources and solutions will help change how partners collaborate for better research.

- Protocols have become increasingly complex in the last decade (3.6 million data points per average Phase 3 study) adding pressure to sponsors to meet speed and efficiency goals[1]
- The burden on patients physically and mentally increases with the amount of unnecessary and complex data collection and interventions; There is an inverse relationship between the amount of data collected and clinical trial experience[1]
- Caregivers have an important role; their 'trial experience' can be different but no less critical to the success of the trial

Clinical site teams are the 'face' of a trial, but sponsor focus may be in favor of lead investigators rather than teams who have most direct interaction with patients and caregivers.

Time to thoughtfully plan the site experience, training of staff and information cascade in a 'patient centric' way is critical.

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Reference: [1]Ken Getz, Tufts Medical Center, Tufts Center for the Study of Drug Development

Regulatory updates: Patient Engagement at the FDA

FDA guidance documents on PFDD have been well received and increasingly accepted as best practice (2 of 4 modules still in 'Draft' status). Plans for an International PFDD guidance document are in progress.

Increasingly, companies are scheduling pre-development meetings with regulators to plan data generation, including PFDD.

PFDD guidance series

Spread the news: FDA Patient Listening Sessions with sponsors and advocacy groups help the Agency inform medical product development, clinical trial design, patient preferences, and shape regulatory thinking.

Summaries are available online and the diversity and number of these sessions are increasing; more than 40 currently scheduled for this year.

Patient listening sessions

The FDA has 2 active committees, the Patient Engagement Collaboration (PEC) and the new Patient Engagement Cluster (FDA, EMA and Health Canada partnership) that advise on issues of education and communication related to medicinal product research.

Patient Engagement Collaborative

Patient Engagement Cluster

Digital health is an increasingly important area of focus for the FDA and it has created a Digital Health Advisory Committee (DHAC) supporting guidance and resource development.

Digital Health Advisory Committee

The Total Product Lifecycle Program (TAP) launched a pilot phase late last year to demonstrate the feasibility and benefits of process improvements to the FDA's early interactions with participants and stakeholders in medical device development.

Total Product Lifecycle Program (TAP) pilot

Key: DHAC - Digital Health Advisory Committee; EMA - European Medicines Agency; FDA - US Food & Drug Administration; PFDD - patient-focused drug development

Consensus: Common themes across the US and EU meetings

Measuring impact

Efforts have been made to measure the impact of patient engagement initiatives, including the development and sharing of toolkits. However, there is a need for standardized metrics and better dissemination of results to demonstrate the value of patient involvement.

Patient-reported outcomes

Patient experience data provides evidence of the impact on life, not only medical symptoms but the impact on a patient's ability to go to work or school, and their quality of life.

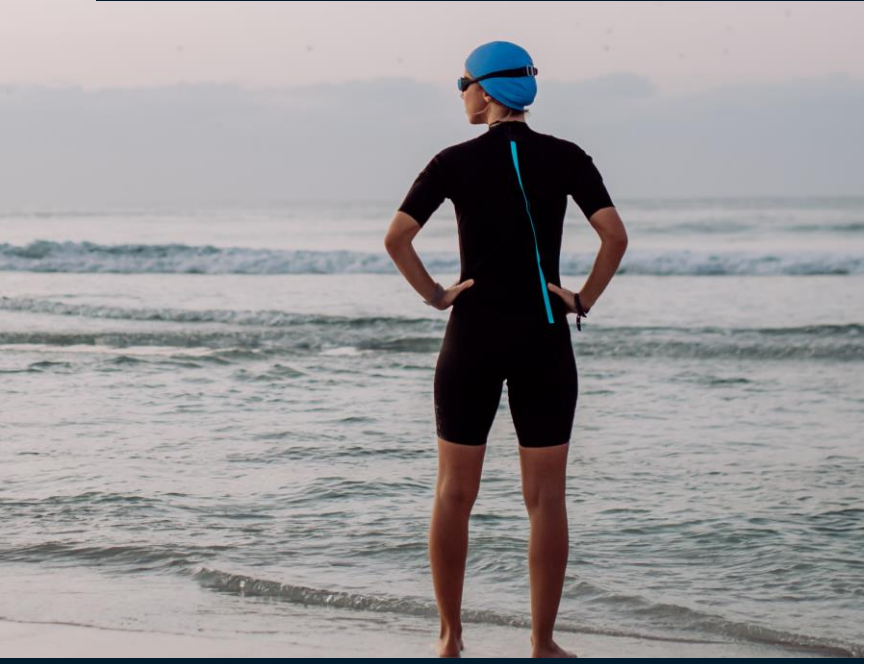
Corporate social responsibility

Companies are increasingly recognizing the importance of corporate social responsibility (CSR) and patient engagement in enhancing their reputation and fulfilling regulatory expectations. This includes partnering with patient communities and demonstrating commitment to societal goals.

Compensation for patient involvement

There was discussion around compensating patients for involvement in clinical trials, and authorship in publications. While some patients may expect compensation, others prioritize having a seat at the table and contributing to the process.

While the meetings provided valuable insights and discussions on patient-centric initiatives, there are some topics we'd like to see more of next year.



Where does the partnership go from here?

More focus on solutions

- We would like to see more nuanced exploration of the challenges and especially their potential, practical solutions. We are hoping to see **more concrete strategies** and **practical guidelines** to bridge the gap between the conceptual and its real-world application

Wider coverage of different therapeutic areas

- This year's meetings had a heavy emphasis on oncology and neurology, potentially at the expense of insights from other therapeutic areas
- While oncology is undoubtedly a critical field with significant patient impact, a more balanced representation of diseases and conditions would provide a richer and comprehensive understanding of patient experiences

Greater diversity of stakeholders

- The pharmaceutical industry and patient advocacy groups were well represented, to create an 'ecosystem' approach, the other players, like policymakers, clinical operations colleagues, HCPs, and employers should also be at the table

Key: HCP - healthcare professional

Patients are our purpose, Hear My Voice™ is our promise

The importance of the patient voice in the development of treatments and health services cannot be overstated. We believe that active and ongoing patient engagement will result in more effective and beneficial treatments, and ultimately help improve patient outcomes and overall health.

Hear My Voice enshrines our commitment to weaving the voice of the patient into the fabric of new treatment development.

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Thank you

If you have any questions about the themes outlined in this article or wish to discuss the challenges you're facing in your patient engagement strategy, please contact us.

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