UNIVERSITÀ LUISS GUIDO CARLI

Department of Business and Management

Master's Degree in Strategic Management

Optimizing Patient Engagement (PE) initiatives through organizational integration to enhance drug development quality and relevance

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Contents

1. Introduction

In the past few decades, patient involvement in clinical development for medicines has undergone a total paradigm shift. Patients are no longer limited to the passive object of trials but are instead becoming increasingly recognized as proactive stakeholders whose voices and personal experiences are rich sources of inputs throughout the entire product life cycle of medicines. At the focal point of the paradigm shift is the concept of Patient Engagement (PE) defined as the systematic and substantive participation of patients in decision-making that informs drug development, ranging from research prioritization in early phases through post-marketing activity.

Increased enthusiasm about PE is driven equally by ethic and practical motives. Ethically, PE is a fairer, and more even-handed kind of innovation in healthcare. It is a manifestation of the ethic of "nothing about us without us" institutionalizing fairness, accountability, and legitimacy in those decisions with existential meaning for patients' lives. Practically, patient engagement makes clinical research and development more relevant and acceptable, easier to identify areas of unmet need, optimizes conduct and design of trials, and ultimately optimizes clinical and development efficiency.

As PE is increasing through demand, it is no longer regarded as an optional approach but increasingly as an integral element of a people-centric and outcomes-oriented innovation system. Nevertheless, whereas there is increasing consensus regarding its importance, there remain primary challenges to structurally integrating PE within internal processes of the pharmaceutical sector. Amongst these primary challenges are those founded upon lack of mutually defined definition and code of practice by all stakeholders concerned, lack of mutually held standards for assessment, and decentralized implementation of PE practices by diverse organizations.

Current guidelines, while valuable, often fall short of covering the full scope of PE across the medicine development lifecycle. Implementation practices vary widely, shaped by organizational cultures, regulatory environments, and resource constraints. Moreover, as recent literature highlights, many PE initiatives remain ad hoc, lacking institutional anchoring, and tend to focus on isolated moments of interaction rather than fostering continuous, bidirectional relationships.

The COVID-19 pandemic further underscored the urgency of embedding patient perspectives into rapidly evolving trial modalities, including decentralized and hybrid models supported by digital technologies.

Recent work has clarified definitional variety, effects, and instruments of PE. Clinical improvement possible, financial improvement possible with the use of PE, and streamlining of trials possible are clear from the literature. Though it simultaneously recognizes significant challenges as representativeness biases, cultural inertia, and instrumentalization of PE for regulatory purposes, there is a supplementary literature explaining change indicative of organizational transformation and explaining why sustainable integration of PE requires cultural transformation, interdisciplinarity, and alignment strategy at companies. Much less is researched about organizational arrangement of internal work that pharma companies need for sustaining such integration with duration and persistence.

This discrepancy between theoretical concordance as it pertains to organizational change and minimal empirical evidence as it pertains to ways in which said change is being operationalized is the starting point for this thesis. Through literature review discovery, what this research undertaking embarks on is an experimental and exploratory position in trying to witness ways in which PE is being operationalized amongst those already established drug firms with said practice. In embarking on a mixed-method study with structured survey and semi-structured interview with a few key informants amongst selected Italian drug firms, the research endeavours to witness ways in which internal functions, procedures, governing process and interdepartmental collaboration are run with regards to applying PE.

By doing so, it aims to chronicle current practices but to distill trends and crucial success factors to inform organizational designs for the future. By connecting the evolving conceptual and normative environment for PE to its operationalization in concrete terms within companies, the thesis offers a more informed and actionable description of how it is possible to embed patient engagement with drug development's DNA.

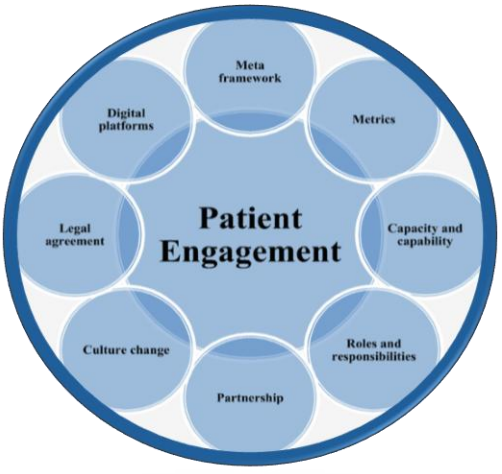
The following chapter begins this journey by analyzing the diverse definitions, frameworks, and theoretical underpinnings that shape the current understanding of PE across different contexts.

2. Literature Review

2.1 Approaches to Patient Engagement (PE)

In pharmaceutical development, neither PE nor its definition is single or unchanging. Rather, PE constitutes a dynamic and heterogeneous patchwork of definitions, models, frames, and practical applications mediated by cross-cutting institutions as well as stakeholder views. Even as it is increasingly agreed that patients should be engaged in decisions impacting their own health, a persistent controversy remains about what "engagement" itself is as much as a practical operationalization of patient engagement. It is the goal of this chapter to execute a systematic review of the ways PE has been defined and implemented across recent scholarship.

Figure 2.1: Key Enablers of Patient Engagement [Source: Vat et al., 2021]



2.1.1 Definitional Diversity and Terminological Challenges

Extent of usage of the volume of terms utilized both theoretically as well as practically is one of the early impediments to establishing a harmonized definition of physical education. In 20 researches, more than 40 definitions for PE were found, with a manifestation of conceptual fragmentation across branches. Overuse of such terms as "patient participation," "patient involvement," "patient-centered," "partnership," as well as "co-creation" with absolutely no discernible distinctions causes confusion academically as much as pragmatically.(Auwal et al., 2023)

Such linguistic flexibility is more than a question of semantics; it also speaks to deeper conflicts regarding the epistemic role to be taken up by the patient. For example, "partnership" or "co-creation" imply a more equitable distribution of decision-making authority, while "participation" may suggest a more passive, consultative role. Deeper structural differences between industry-driven demands for efficiency and innovation, academic aspirations toward democratization, and top-down regulatory expectations are reflected in the lack of agreement.(Zvonareva, 2023)

The notion that PE entails the "meaningful and active collaboration" of patients at different stages of the drug development process is becoming more widely accepted despite the semantic differences. Patients' participation "as partners in research," whose contributions are acknowledged as experiential and epistemic, is emphasized in a definition from the Patient-Centered Outcomes Research Institute (PCORI). This acknowledgement represents a shift from considering patients as merely research subjects to appreciating their lived experiences as important knowledge sources that guide the planning and execution of research.

2.1.2 Thematic Models of Engagement

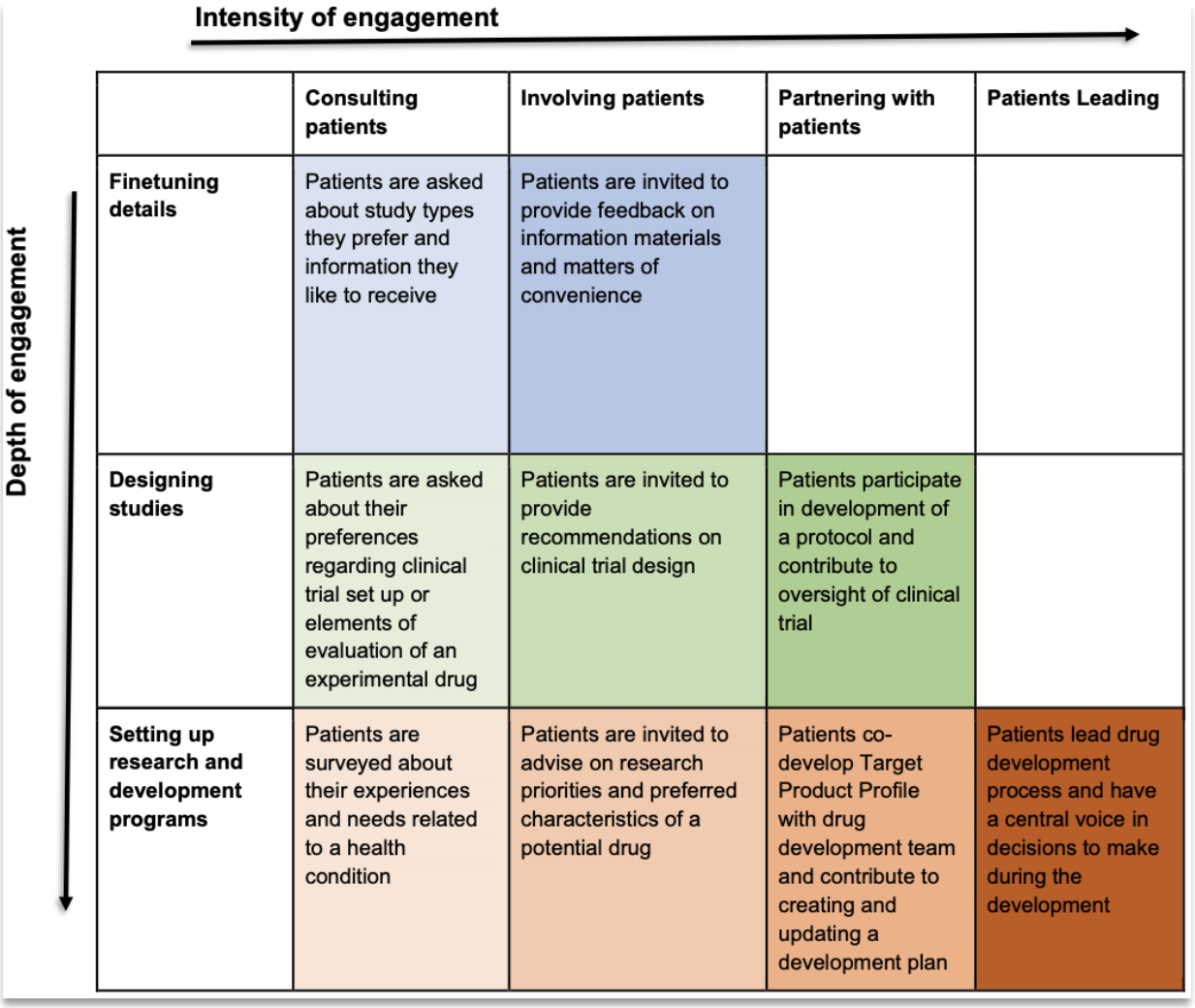
A conceptual model that identifies eight thematic components required to achieve meaningful engagement attempts to systematize this complexity. Trust, respect, representativeness, openness, co-learning, flexibility, continuity, and shared decision-making are a few of these. These

components are portrayed as interrelated elements that together define the quality of engagement rather than as discrete principles.

The analytical framework delineating patient participation at five critical points in the drug development process - (1) research priority setting, (2) clinical trial design, (3) regulatory review, (4) post-marketing surveillance, and (5) health technology assessment - provides evidence to this thematic framework. The stages all have clear points at which patient views can contribute to decisions. In practice, patient participation during these stages is uneven. As institutional actors continue to dominate subdomains such as regulatory decision-making or post-marketing surveillance, participation is biased towards clinical trial design, i.e., endpoint selection and recruitment strategies. (Zvonareva at al., 2022)

A more thorough examination demonstrates how Patient Engagement programs can be mapped along two crucial dimensions, as shown in *Figure 2.2*: the implementation stage and the level of engagement. From unidirectional information provision (the lowest level) to full co-production of research (the highest), intensity describes the extent of patient influence in the process. The term "stages of implementation" describes the phases of the development process, from pre-clinical research to market authorization, where PE takes place.

Figure 2.2: Framework for Depth and Intensity of Patient Engagement in Drug Development [Source: Zvonareva et al., 2022]



Note here too is their exercise in cartography indicating that whereas there is growing discussion relating to co-creation, hands-on practices are all too frequently to be located towards the consultation or limited cooperation end of the spectrum. Certain drug firms limit their ability to influence key aspects of trial design or regulatory strategy by only engaging patients after critical decisions have already been made. Questions regarding representativeness and inclusion are further posed by the prevailing tendency for these interactions to rely upon a small number of very senior, English-speaking patient experts.

2.1.3 PE as a Strategic Resource for Innovation

Engagement is understood as a tool of strategy and might trigger innovation in products and processes and as a mechanism of participation. Asserting that patients' experiential knowledge can be incorporated into R&D and open new forms of value creation, authors build up their argument upon organizational theory and research on innovations. In detail, there is an argument that physical education might allow what the authors refer to as "collaborative epistemic practices," that is, an integration of experiential and scientific forms of knowledge.

Such epistemological re-interpretation thereby suggests a paradigm shift of what patient inputs as understood and utilized by drug product manufacturers are. Innovation-oriented strategy views patients as co-developers whose contextual knowledge might modify the configuration and orientation of issues being posited, what will become of issues at the solution endpoint, and what products will emerge instead of looking at patients as distant validators or end-stage consultants. Two examples of taking that kind of an approach are conceptualizing new digital tools to track symptoms or re-defining trial endpoints along those issues patients are concerned about regarding their therapeutic outcomes.

The strategic resource perspective also sheds light on the means whereby PE facilitates organisational learning. Organisations can introduce new ways of considering value and evidence with maintenance of a partnership with patients. Yet, as the authors note, much greater reform is needed than simply the blending of patient voices; organisational structural reform such as updated incentive configurations, cross-discipline workgroups, and new communication methods are instead required. (Albulushi et al., 2024)

2.1.4 Operational Frameworks: From Principles to Practice

Turning theoretical goals into practical processes is one of the biggest challenges to the advancement of PE. This section notes the increasing number of Patient Engagement frameworks, that range from generic sets of ethical principles to narrow, practical toolkits, that have been created with the aim of facilitating implementation. For instance, the European Patients' Academy

on Therapeutic Innovation (EUPATI) toolbox makes tools and training accessible with the intention of supporting patient engagement in scientific discussions and the Patient Focused Medicines Development (PFMD) framework makes an orderly process available for including patient input as part of development cycles.

The multi-stakeholder framework created by the PARADIGM (Patients Active in Research and Dialogues for an Improved Generation of Medicines) consortium is among the most extensive attempts in this direction. It comprises 15 context variables and more than 80 metrics to evaluate the effectiveness and impact of PE programs. Instead of imposing a single evaluation standard, this framework promotes customization according to project goals and organizational requirements. Metrics for input (like resource allocation), process (like transparency and trust), learning (like stakeholder understanding), and outcomes (like trial efficiency, product quality, and patient empowerment) are all included.(Auwal et al., 2023)

Adopting such frameworks is essential for two reasons. First, in quantifying and making explicit the work of PE, they give it credence. They provide a foundation for organizational accountability and future development as well. The literature cautions against applying metrics thoughtlessly, however, as it might hide the emergent and relational nature of engagement.

2.1.5 Limitations and Future Directions

There are still several conflicts in spite of the advancements in PE theory and structure. One is the instrumentalization of participation. Such utilitarian approaches run the risk of undermining the emancipatory promise of engagement as a democratizing force in medicine development, even though using PE to increase recruitment rates or regulatory compliance may have immediate benefits.

The uneven distribution of engagement across populations and stages is the subject of a second tension. Participation is often biased toward urban, English-speaking, and digitally literate patient populations, excluding marginalized groups that might encounter the biggest obstacles to participating in research and obtaining healthcare. This calls into question the fairness and equity of PE's implementation.

Organizational inertia is least discussed here. The internal infrastructures to facilitate frequent, high-quality interaction are yet to be established by majority pharma companies. This is also true for employee education, alignment with KPIs and fitting with business plans by specialized PE teams.

The texts mention that cultural change and structural innovation are going to have to take place for PE to mature in decades ahead. It is going to have to develop an ambition to transfer power and redefine experience-based learning at the cultural level. It is going to require durable infrastructures, flexible frameworks, and enduring funding models structurally.

2.2 Impact of Patient Engagement

In addition to being realized as a moral imperative, patient engagement (PE) is also seen as a strategic advantage that has the potential to lead to measurable gains. Clinical, organizational, and economic domains have seen high impacts emanating from incorporation of patient contributions to frontline views within medication development practices. Herein, an in-depth review is undertaken to specify such impacts beyond broad brushstrokes to look into particular ways PE influences development outcomes. In an examination relying upon industry reports as well as empirical literature, a multi-dimensional picture is seen wherein patient engagement is one area impacting allocation of resources, innovation and successful tests, and organizational learning. (Faulkner et al., 2023; Levitan et al., 2018)

2.2.1 Clinical Impacts: Enhancing Relevance, Feasibility, and Quality in Research

Internal validity and regulatory compliance have historically been given top priority in clinical research, especially randomized controlled trials (RCTs). However, patient acceptability and real-world relevance have frequently suffered because of these priorities. By reorienting clinical development toward patient-centred outcomes, trial viability, and inclusivity, patient engagement corrects this imbalance.

2.2.1.1 Improving design of clinical trials

The enhanced clinical trial design is one of PE's most well-established clinical advantages. Contributions from patients during protocol development aid in identifying endpoints that might not be consistent with their lived experience, restrictive inclusion/exclusion criteria, and unduly burdensome procedures. This leads to research that is both logistically possible and scientifically sound. Two industry-sponsored clinical studies on uncommon neuromuscular disorders, such as Myotubular Myopathy and Spinal Muscular Atrophy, provide a particularly clear and well-documented example of this dynamic. Children and young adults, who have severe mobility limitations, are frequently affected by these conditions.

Early in the design phase, the research sponsors implemented a patient engagement strategy because they understood how difficult it would be to recruit and retain participants with such burdens. This required several rounds of consultation with people who had the targeted disorders, caregivers, and patient advocacy groups. Patients actively reviewed and helped guide key parts of the study including visit schedules, testing procedures, and endpoint definitions rather than restricting their contributions to level-surface information.

Amongst most significant contributions towards logistical practicability for the trial. Burden of frequent face-to-face visits to specialty centers, which could imply protracted duration of travel with high physical exertion, was explained by caregivers as also by patients. Trial team responded to it by offering participants scheduling flexibility options that enable stretching out visits as per energy. Even home visits were contemplated occasionally for critical evaluations.

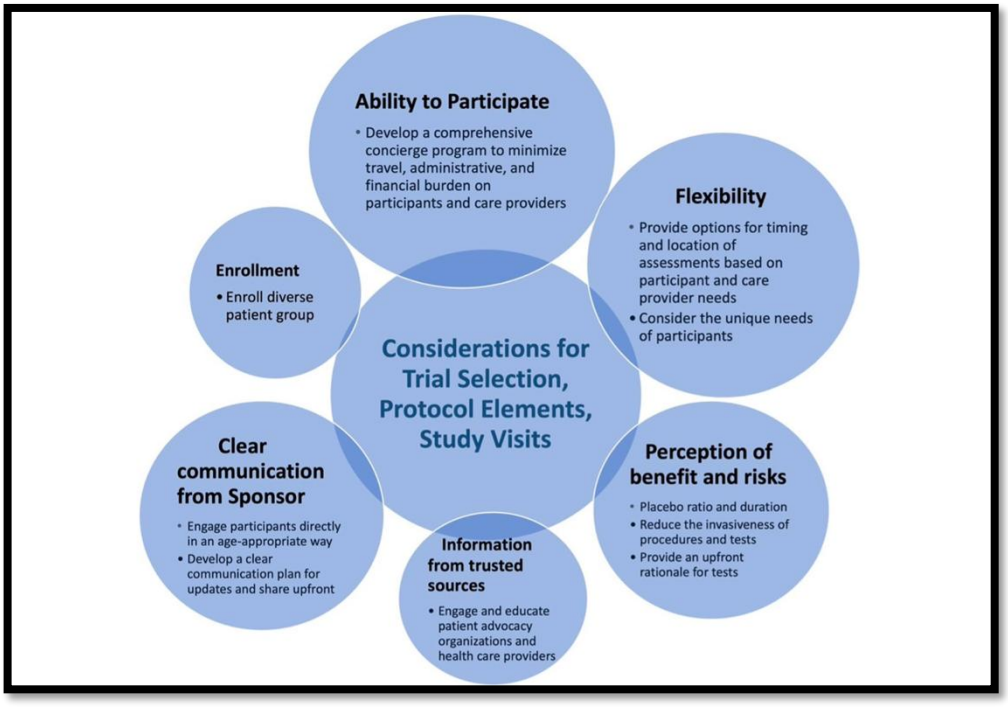
Similar concerns were also noted by patient representatives about invasive procedures such as several biopsies of muscles or spine punctures taps originally used for mechanistic monitoring. They noted how traumatic and unnecessary such procedures were. Priority then shifted to non-invasive imaging procedures and sampling of biomarkers whenever feasible with the new protocol. These adjustments enhanced participant satisfaction and ethical acceptability as well as diminished procedural risk.

These outcome measures also underwent a novel adaptation. The complex, dynamic aspect to everyday functioning in rare neuromuscular diseases was perceived to be outside the realm of standard performance-based outcomes, such as the six-minute walk test or clinician-administered motor scales. Virtual health instruments, including wearable sensors and mobile-based trackers of fatigue, were implemented into a paradigm of a trial consistent with patient preference. These allowed a standardized picture of treatment efficacy by providing low burden but frequent assessment of energy and functional mobility levels within daily settings.

Interestingly enough, such design modifications had an impact that went beyond process enhancement. Higher than anticipated levels of recruitment as well as a significantly lower level of dropouts were realized by the trials by early as well as relevant integration of patient views. Despite extensive testing, participants were urged to stay part of as well as to stick to study

protocols due to feelings of ownership developed by participation design. Additionally, the resultant data were more indicative of patient-specified outcomes, an aspect which came to be useful for subsequent payer as well as HTA (Health Technology Assessment) negotiations as well as regulatory evaluation. (Faulkner et al., 2023)

Figure 2.3: Considerations for Trial Selection, Protocol Elements, and Study Visits
[Source: Furlong et al., 2024]



2.2.1.2 Improving recruitment and retention in clinical trials

Building on this, patient engagement also has a significant impact on patient recruitment and retention, which in turn has a big impact on the validity and cost of drug development. Excessively complicated protocols, strict inclusion criteria, and procedures that don't fit with everyday routines are common problems in trials that aren't guided by patient perspectives. These elements jeopardize statistical power and external validity by delaying enrolment and raising dropout rates.

Trials created in conjunction with patients, on the other hand, are more likely to demonstrate a practical balance between participant burden and scientific ambition, which results in better recruitment schedules and higher adherence. This is especially true for rare or chronic diseases, where patient eligibility is restricted and trial participation is frequently hampered by significant logistical or physical obstacles.

As Clinical Trials Transformation Initiative (CTTI) has mentioned in their report, early and extensive patient participation has a direct positive effect on retention by rendering participants more relevant. Patients would be more likely to stay enrolled for their entire participation period if they know not only what they have been asked to do but also why, and if participants' wishes as well as their restrictions have been considered by their respective trial designs.

This assertion is supported by comparative implementation studies, which show that trials with protocols co-developed with patient communities had significantly lower attrition rates than those developed by researchers alone. In one instance, a multi-site trial that included people with a rare genetic disorder decreased dropout by more than 40% after incorporating patient feedback on visit frequency, transportation reimbursement, and communication materials. These were among clinical trials of DMD and DM1. Regardless of having a low running cost, they significantly enhanced participants' willingness to stick with the protocol and finish it as scheduled.

In addition, participation makes it possible for support networks centering on a trial such as reminder systems or peer support forums to be established, and these reduce risk of withdrawal and strengthen feelings of community. These are especially useful with long-term trials. In these,

patient trust in a research team is central, and retention is a relational as much as a methodological problem. (Levitan et al., 2018)

2.2.1.3 Improving inclusivity and representativeness in clinical trials

By extending the conversation, PE has helped to make trials more inclusive and representative. Patient groups have also been critical to identifying socioeconomic, linguistic, and geographic barriers that discourage underrepresented groups from participation in a study. An example of a multi-center clinical trial undertaken by the CTTI to review integration of telemedicine and mobile health technologies into study design is a very good demonstration of how participation by patients can seriously increase inclusivity for a trial. In such a case, early planning stages involved consultative meetings with structured involvement by patients about logistical barriers and daily realities among participants who were concentrated in remote or underserved areas.

To overcome this latter criticism, the research team embraced a hybrid strategy, combining telehealth consultations and remote data collection with mobile technology with conventional site-based assessments. Thus, remote frequent check-ins were exchanged for secure video conferencing, wearable sensor devices and mobile phone software facilitating remote monitoring of core health parameters including heart rate, activity level, medication taking, and symptoms. To allow for continuation and integrity of clinical measures despite remote location reasons for participation or mobility issues or transport problems after participation completion, participants were also able to upload data directly from home, thus eliminating face-to-face attendance.

It was also a technological development but a planned answer to what the patients were calling for. It did allow an expansion of geographic reach with additions of these digital tools that were prompted directly by patients' feedback. It did benefit in particular low density as well as remote areas where specialist site coverage would be unattainable. Accordingly, a more heterogeneous but more representative universe of participants were approached by the study including participants who would be excluded by virtue of practicalities.

Finally, the case illustrates how incorporating patient voices into operational planning for clinical trials can yield more transparent and equitable models of research. Inclusive research was augmented by enhancing participant satisfaction, and data collection efficiency improved by

eliminating real-world access barriers that were individually cited by participants. These also contribute to improved retention and data quality over the duration of a trial.(Chegini et al., 2021)

2.2.2 Organizational Impacts: Transforming Culture, Processes, and Decision-Making

Patient engagement has significant organizational consequences that go beyond stakeholder governance to organizational practice, organizational culture, and knowledge management. PE is an epistemological disrupter that disturbs the long-standing separation of scientific knowledge and experiential knowledge and requires pharmaceutical companies to revise their epistemic hierarchy.

PE re-designs learning at organizational level too. Involving the workforce to respond to patients' priority agenda and communicate their language, having "patient-in-residence" or patient advisor positions generating learning as an ongoing process, there are indeed initiatives for training workforce staff with coproduction and communications skills. These suggest culture shift with patients themselves as coproducers of clinical value and not as subjects for testing and data points.

Participatory practices got further standardized by patient engagement. Such models as the PARADIGM multi-stakeholder model, offering metrics and contextual factors to determine PE by phases, are being accepted by ever-growing numbers of companies. Process measures (transparency, satisfaction), input quality measures (availability of resources, representativeness), and result measures (ethics of studies, data quality, institutional trust) are a few of them. These frameworks support an enterprise's mission to continuously improve by letting it determine PE initiatives' impact and consistency.

As would be expected, PE has an impact upon risk management and crisis management as well. The ones with matured patient engagement infrastructures permitted them to easily adapt procedures to suit decentralized trial models and remote monitoring once pandemic from COVID-19 broke out. Crisis management and rapid feedback loops amidst unpredictable times were eased by open lines and trust fostered by earlier initiatives with PE.

Nevertheless, there are some challenges when incorporating PE into organizational processes. Implementation may be hampered by departmental silos, institutional inertia, and ambiguous

accountability structures. Successful businesses have demonstrated top-down commitment by establishing specialized PE offices or incorporating PE KPIs into executive scorecards to address these issues.

However, there is still much knowledge void in the literature on what an organizational system ideally PE-oriented should be, even with growing prevalence of engagement-related practices. Instead of offering complete organizational designs that organically embed patient engagement in governing and operating processes, most of the historical contributions are centered on single intervention mechanisms or auxiliary mechanisms.

Because of this, there is very little guidance on reorganization of internal workflows, incentive systems, and pharmaceutical firm hierarchies in a way that would significantly empower PE as an institutional logic facilitating the entire innovation process as opposed to a collection of activity. There are many such initiatives that are relying upon ad hoc champions rather than systemic change because of this lack of structural coherence, and it disables engagement scalability and sustainability.

2.2.3 Economic Impacts: Maximizing Return on Engagement

The economic implications of Patient Engagement (PE) are only now starting to receive systematic attention, even though its ethical and procedural benefits are well established. According to recent financial analyses, PE can provide significant returns on investment when applied strategically and early, upending the long-held belief that it is an ancillary or cost-driving activity.

Levitan et al. made a significant contribution in this field as part of the Clinical Trials Transformation Initiative's (CTTI) Patient Groups and Clinical Trials (PGCT) Project. Their work is among the most systematic attempts to use metrics that pharmaceutical stakeholders are familiar with to model the financial value of PE. They specifically concentrated on Expected Net Present Value (ENPV), a common drug development tool that evaluates clinical program profitability by combining anticipated revenues, expenses, risks, and timelines.

Two engagement scenarios in oncology were simulated by Levitan and colleagues: one at the pre-phase II stage, right after safety was established in Phase I, when the study's efficacy and design are being considered, and one at the pre-phase III stage, right before the start of trials intended to

obtain regulatory approval. Their results were convincing. According to their estimates, a small investment of about $100,000 in structured PE activities (such as advisory boards, consultation workshops, and co-development of materials) could result in phase II ENPV increases of $35 million and phase III ENPV increases of up to $75 million. These numbers show returns that are hundreds of times higher than the initial investment, establishing PE as a low-cost, high-impact R&D pipeline intervention.

The framework is risk-adjusted and based upon cross-sector stakeholder input, including clinicians, trialists, and patient representatives, yet based upon simulation and not actual trial data. As such, it is a good and useful framework to connect financial decision-making with patient-based values. This work reformulates PE as a sound economic investment at the strategic level with direct benefits to efficiency in development, portfolio optimization, and follow-on market access downstream, and less as an ethical or procedural improvement only.

Overall, Levitan and colleagues' research shows early engagement can create out-of-proportion value from relatively modest investment and supports the belief that PE can and ought to be viewed as a source of drug innovation's competitive and financial advantage and not as a cost centre. (Levitan et al., 2018)

2.3 Tools and Methods for Implementation

From a theoretical aim to a practical operational goal, integration of Patient Engagement (PE) into pharmaceutical R&D has evolved. Firms have begun utilizing various strategies that leverage various methods and web-based tools to transition from haphazard participation to deliberate engagement. Based on recent trends from patient-led initiatives to industry-driven programs to multi-stakeholder platforms, this section considers key tools and paradigms that facilitate implementation of PE.

Emphasis is given to practical facilitators of PE such as community forums, mobiles, sophisticated analytics tools, and patient advisory boards. In addition to facilitating a bidirectional exchange of information, these strategies introduce patient views into pivotal phases for a design of protocols as well as definition of outcomes. Industry stakeholders can take a step further to a superior paradigm for drug development by implementing such strategies into organizational practices and tailoring them to local as well as regulatory requirements. (Furlong et al., 2024)

2.3.1 Structured Advisory Platforms

Conducting PE via structured advisory forums is one of the most efficient ways to implement PE. These include thematic workshops, Community Advisory Boards (CABs), and Patient Advisory Boards (PABs), and it includes bringing experts, caregivers, and patients to offer helpful comments during planning and conduct phases of trials. Instead of waiting for sporadic points of feedback, these forums help drug entities to have constant interactions with patient communities.

These rare neuromuscular disease trials are a notable case in point. They adopted a dual-engagement approach, collaborating with the Duchenne CAB, an accepted group of veteran patient representatives, and engaging with intensive workshops during development. These consultations greatly minimized participants' burden by fitting trial components to patients' reality.

Patients and their caregivers talked about practical issues regarding participation in a trial during workshops. In reply to their remarks, the research team modified the protocol to vary muscle biopsy frequency, to be specific regarding what is expected about placebos, and to revisit activity

related to video assessment. Age-sensitive information materials with pictorial information created for the adolescents and children who were to participate in the studies were included as a result of these consultations. As a standing consultation forum, the Duchenne CAB also contributed continuous commentary throughout a trial's lifespan, with comment regarding informed consent forms, letters to participants, and study names.

These consultative systems work because they are cyclical. They posit constant feedback loops rather than restricting patient input to a single design stage so that shifting trial strategy and patient experience align. In addition, via integrated remunerated participation models, such systems can reward time and experience of patient contributions, a factor which increases inclusivity and legitimacy. (Coran et al., 2019)

2.3.2 Cross-Functional Teams

Clinical experiments with Duchenne Muscular Dystrophy (DMD) and Myotonic Dystrophy type 1 (DM1) present a vivid example of effective cross-functional collaboration to enable good Patient Engagement (PE). Interprofessional collaboration from within various units such as clinical operations, regulatory affairs, patient advocacy, medical writing, and data science were not only helpful but a necessity in such experiments to transform patient comments into usable changes to protocols.

Patients and caregivers, for instance, also raised serious concerns during our initial engagement workshops about placebo use beyond long-term trials, particularly where rare diseases with few treatment alternatives were involved. What they cited were moral and psychological costs to long intervals where active treatment is withheld. Instead of dismissing or reject these concerns, they held several internal working meetings with scientific and regulatory communities to determine whether placebo arms might be altered without compromising data integrity.

Following these cross-functional discussions, early escape criteria were added to the trial protocol, enabling participants to transition from placebo to active treatment in the event that specific thresholds for clinical deterioration were reached. Additionally, the medical writing and communication teams worked together to close a significant transparency gap found during the

workshops by making sure that patient-facing materials adequately described the goal, duration, and safety precautions of the placebo component.

The internal engagement team also raised concerns with the appropriate clinical and operational units regarding the burden of frequent site visits and invasive procedures like muscle biopsies. As a result, the frequency of these evaluations was decreased, and the study design was modified to incorporate alternate data collection techniques like caregiver-reported outcomes and wearable remote monitoring. It is only through complete interdisciplinary cooperation that such revisions were brought to reality, thereby making revisions centred on patients technically and scientifically sound.

This is an example of how cross-functional PE teams have a function that is more than facilitation; they translate and operationalize patient insights so that experiential knowledge is brought into complex clinical and regulatory systems. Most of the data gathered within engagement activity would be unused or overlooked as incompatible with operational constraints without such internal aligning.

2.3.3 Mobile and Digital Technologies

Clinical studies have been revolutionized considerably with a large-scale implementation of mobiles and information and communications technologies, primarily in relation to patient engagement (PE). Therein, timely observation of a patient's condition has replaced location-based information collection with assistance from such tools as remote sensing devices and mobile-based applications. It is an extension of a wider aim to ensure that clinical trials would be more centred towards patients.

Through enabling patients to participate from their own sites, such technologies increase efficiency for a single trial but also enable equity, as defined by the Clinical Trials Transformation Initiative (CTTI). In rare disease trials, where participants can be geographically distant or physically unable to travel, this is especially relevant. Mobile tools eliminate critical participation barriers and create new avenues to inclusion through simplifying remote monitoring and minimizing visit requirements to a site.

Practically speaking, wearable technologies such as activity trackers or smartphone-based devices with concomitant bands can be utilized to gather longitudinal information regarding parameters such as activity or heart rate with feedback regarding treatment effects with a reduction to a minimum in disruption to the patient. In a way that does not necessitate hospital-level hardware, home-based remote sensors can monitor biomarkers such as respiratory function. Compared with evaluations solely based in clinics, such technologies help with setting up endpoints that better realistically reflect patients' real-world experience.

One example is a trial for Parkinson's disease where participants recorded vocal samples and tapping exercises using a smartphone app. Machine learning was used to assay these digital biomarkers to monitor speech and motor function changes, excellent and germane proxies for disease progression. This was conducted at home and only took a few minutes but reduced patient burden to enough of an extent that a complete data set for near real-time analysis was acquired. Likewise, oncology clinical trial patients recorded daily symptoms such as nausea and fatigue using a smartphone app. Automatic notifications went to clinical study monitors at serious symptom limits to permit timely follow-up and therapy.

In data gathering, mobile technologies are central to maintaining interaction and communication. Two-way commentary between clinical teams and their patients can be facilitated with apps, alongside providing individually tailored educational information. Patients can access their own monitoring and data through dashboards and portals, encouraging a degree of empowerment. These electronic interfaces can be utilized to facilitate collaborative decision-making throughout study design and design a more user-centric trial.

Of particular interest with adaptive design for trials is that incorporating digital technologies permits enhanced responsiveness and flexibility. In real-time, adjustments to protocols can be steered with information from trackers for symptoms or sensors to reduce visit frequency if stability is gained or to initiate other assessments if a loss of function is observed. In aligning interventions better with individual trajectories, such a function benefits patient safety as much as scientific validity to the trial.

These digital tools have also been proven to be useful for increasing participant retention. High attrition is common with longitudinal trials because of attrition through participant fatigue or lack of perceived value. With individual notification tools and peer support forums, mobile tools can reduce these hazards and facilitate maintenance of participation and motivation over longer intervals. These strategies are especially helpful with rare diseases or chronic diseases where months- to years-long studies are necessary.

However, they present serious technical and legal issues with their usage. First, to have valid measurements that are clinically relevant, device validation should be performed. There should be activity or gait changes relevant to disease endpoints captured with reliability by a wearable that is step-based. These regulatory authorities like FDA or EMA may reject such data if they prove invalid.

Secondly, interoperability and standardization of data continue to be major impediments. It is challenging to aggregate gathered data into central systems such as electronic case report forms (eCRFs), as most devices have proprietary software. It is more challenging to compare data that is scattered in different data forms because data is less reusable overall, and comparisons are more difficult. Projects like CTTI offer methodological advice for overcoming impediments by making recommendations regarding long-term data governance, device selection, and integration.

Moreover, maximum caution is to be exercised regarding laws concerning data protection and privacy. Issues regarding data ownership and transparency arise with frequent passive or continuous collection of highly sensitive personal health information through mobile instruments. Clarification about purposes behind data usage, storage, and transfer to participants is necessary to moral deployment. From a legal standpoint as well as from a trust establishment point of view, compliance with such protocols as the General Data Protection Regulation (GDPR) across some parts of Europe or HIPAA across parts of the United States is necessary.

Information technology holds promises to an inclusivity agenda but also has the potential to widen ingrained inequities if it is not regulated. Even if their introduction is able to widen coverage for clients who would be underserved or who reside some ways from cities, remote systems remain an issue. To offset this, several studies have proposed hybrid participation models to achieve balanced and equitable participation by providing paper-based alternatives, onsite onboarding, or caregiver assistance with digital reporting. (Coran et al., 2019)

2.4 The Organizational Transformation Driven by Patient Engagement

The incorporation of Patient Engagement (PE) into pharmaceutical companies is a paradigm shift in the way value and innovation are conceptualized, not merely a reaction to changes in regulations or societal expectations. This section examines where the implementation gap still exists, how human-centred design is promoting cultural change, and how the patient-centric paradigm is changing strategic priorities.

2.4.1. Embracing the Patient-Centric Paradigm: A Strategic Reconfiguration

Strategic DNA of drug company is being re-engineered along the lines of patient centricity. Pharmed value proposition was based on scientific excellence driven through innovation emanating out of the marketplace and therapy-driven reasoning on prescriber preference. The patient fits best with the old paradigm as volunteers in clinical trials or even as active recipients of therapy.

But the current reengineering puts patients at the center as an active participant of the whole life cycle of medicines. It is a overhauling of the business model at the primary level to prioritize rather than a lexical update. Lead from the front are some such stellar businesses such as LEO Pharma and UCB.LEO Pharma created patient co-creation platforms for prototyping services and tools and drug development and UCB made the appointment of a Chief Patient Affairs Officer and patient value creation one of its key strategic pillars.

Patient-centricity is about using patient value throughout and along the entire breadth and depth of the R&D continuum, from post-marketing surveillance all the way back to trial protocol building and target selection. Even KPIs are redefined: alignment with patient preference, patient experience with therapy, etc., are applied as indicators of success other than time-to-market and return on investment.

However, the transformation is yet unequal throughout the entire sector. Even as surveys report that more than 80% of pharma enterprises are aware of the strategic value of PE, there are plenty

that fail to implement it in quantitive and consistent ways with their operating models. This gap between the operating reality and the strategic intent is an indication that there is yet required structural transformation. (Auwal et al., 2023)

2.4.2. Culture Shift and Human-Centered Design: The Engine of Organizational Change

Reorientation alone is insufficient without re-alignment of culture. Emotionally and experientially rich dimensions of patient care have been de-prioritized long enough by pharmaceutical firm culture. Organizational and new-governance designs are required for integration of PE, so is a core redefinition of "what is valued" and "how things are done" throughout the firm.

To achieve such a cultural shift, Human-Centered Design (HCD) is offering an approach and an attitude. Utilizing immersion techniques like ethnographic observation, co-design workshops, cultural probes, and diary studies, HCD attempts understanding the daily lives of patients and all the stakeholders. These methods recognize the psychological and practical challenges encountered by patients.

Instead of focusing on purely pharmacological efficacy, that is, organisations are in a position to implement HCD and construct services and therapeutic experiences based upon human needs. "Patient personas," "journey maps," and "care ecosystems," for example, are used to translate qualitative knowledge into concrete artifacts capable of communicating with cross-functional groups.

But research on organisations tells us that culture shift is hardest of all to engineer. It must work simultaneously at three levels: systemic (institutional policies and reward systems); interpersonal (team norms and relationships); and personal (individual values and behaviour). PE is in danger of being a one-off project and not an over-arching organisational principle unless co-ordinated activity proceeds across these levels. (Zvonareva et al., 2022)

2.4.3. Innovation Through Patient Knowledge: Reframing the Epistemic Hierarchy

The creation and validation of knowledge by drug companies is another key component of change. Regulatory acceptability and scientific rigor are emphasized in traditional pipelines of R&D. Even if these criteria can never change, patients' tacit knowledge is frequently left out.

This epistemic hierarchy is further questioned by PE's rise to prominence. By deeming patient experiences as independent wellsprings of innovation themselves, PE creates pluralization of expertise. Where standard evidence is frequently lacking, as it is with rare diseases or pediatric trials, where the patient perspective can inform design of trial, this cognitive re-channeling is most effective.

However, there is still uneven incorporation of this knowledge at the formal decision-making levels. According to recent research evidence, most organizations still come to patients at the termination point of the process, usually at the recruitment/dissemination stage, and miss out on actual co-creation and value capture.

2.4.4. Structural Barriers and the Implementation Gap

Although the pharmaceutical industry now widely acknowledges the strategic importance of patient engagement (PE), its actual operationalization is still symbolic rather than systemic. This disparity draws attention to a crucial mismatch between the concrete realities of organizational design and the aspirational rhetoric of transformation.

The absence of consolidated ownership and control is the initial and most frequent obstacle. PE accountability is fractionated among medical affairs, regulatory units, public affairs, market access, and patient advocacy at large for most pharmaceutical companies. Varied goals and lack of institutional memory build-up or create lasting cultural change are some consequences of this fractionalization. As a result of this scenario, PE all too frequently takes a reactive or peripheral role and not a strategic lever integrated with mainstream activities.

Second, internal accountability and learning across systems are delayed by insufficient common metrics. Even though instruments like the Public and Patient Engagement Evaluation Tool

(PPEET) and PARADIGM's framework for evaluation are worthwhile beginning points, implementation is still restricted and inclusion in standard performance management tools is rare. Decision dashboards have yet to formally adopt key indicators like impact on trial adherence, patient satisfaction with participation, or protocol change based on recommendations by patients. For this reason, it is hard to justify resource investment and show return on engagement.

Another systemic limitation is infrastructure inflexibility. PE processes tend to be appended to established protocols with no upstream consideration where patients can influence strategic choices. PE is seen as a compliance-focused step but is not a driver of organizational flow redesign. It then loses its power to transform and becomes a tactical instead of a strategic input.

Theoretically, digital innovation could serve as a facilitator. New avenues for gathering real-time patient insights are provided by devices like wearables, patient-reported outcome apps, e-consent platforms, and decentralized trials. However, if design principles based on empathy and contextual relevance are not applied, technology by itself cannot ensure meaningful engagement.

Furthermore, learning mechanisms are still in their infancy. Many businesses lack the specialized infrastructure needed to internally record PE experiences. Despite their success, case studies are rarely institutionalized. As a result, new projects are frequently "piloted" without taking advantage of past learning, creating a cyclical pattern that wears down organizations and raises doubts about their scalability. As a result, PE remains in a state of perpetual experimentation without moving past the point of structured implementation.

Overall, a glaring structural deficiency is revealed. Few pharmaceutical companies have been able to successfully incorporate PE as a systemic practice into their organizational structure, despite the large number of declarations.

The need for a more grounded understanding of the organizational enablers that can facilitate the shift from isolated engagement efforts to a patient-integrated operating model is further supported by this ongoing misalignment between aspiration and execution. In order to transform PE from a symbolic endeavor to a structural element of pharmaceutical innovation, it is imperative to determine how leadership, incentives, workflows, data systems, and interdepartmental coordination can be redesigned. This goes beyond conceptual advocacy.(Vat et al., 2021)

3. Methodology

3.1 Research Methodology

3.1.1 Epistemological Foundations and Justification of the Qualitative Approach

In this study, analysis of the integration, perception, and organizational structure of Patient Engagement (PE) is undertaken with qualitative research methodology. Assumption about epistemology that knowledge about organizational behaviour and human behaviour is better understood from an interpretation perspective of setting and meaning as opposed to measurement and prediction is an assumption that is arrived at in the process of decision (Greenhalgh et al., 2016). It is with an intent of gaining an understanding of what is perceived, enacted, and interpreted about PE in major internal stakeholders rather than determining up to what extent it is integrated.

It is a qualitative research specialty to study emergent, context-specific, and culturally embedded phenomena like PE. Qualitative methodology helps us study the nature, occurrence patterns, and subjective meaning of subtle organizational routines outlined by Ugwu and Eze (2023). When it investigates patient-oriented practices against proscribed top-down pharmaceutical development systems, it reveals much about people building meaning, making decisions, and navigating organizational systems.

Phenomenological-interpretive paradigm with aim of investigating a phenomenon as it is lived and interpreted by people themselves from their own perspective is particularly appropriate in this study (Finlay, 2009). In the scenario of PE, it is regarding being responsive to what insiders perceive goals, benefits, and outcomes of working with patients as part of research and development. Quantitative methods would not let in similar degrees of closeness, adaptability, and reactiveness regarding emergent themes even with their supportiveness in other contexts. Qualitative methods, as described by Busetto et al. (2020) are best applied while carrying out a study on organizational innovation and organizational change in health systems where stringent measures normally cannot adequately account for complexity of changes taking place.

Moreover, PE is a relational as well as a cultural construction by definition; values, leadership, and interpersonal relations all have a central role to play to its implementation alongside policies and structures (Vat et al., 2021). From a frontline perspective, this inquiry hopes to understand not only "what" and "how" PE is implemented but "why" as well. For that purpose, a qualitative methodology is not only suitable but necessary to achieve the purposes of the inquiry.

3.1.2 Purpose and Design of the Study

Achieving a holistic, situational understanding of how senior executives in a multinational pharmaceutical company think about, conduct, and perceive PE is the general aim of this study. At a particular level, the study explicitly considers four crucial issues: (1) internal motivators and historical development of PE adoption; (2) cross-functional integration processes facilitating its usage; (3) organizational cultural inhibiting and facilitating factors; and (4) perceived PE effects on organizational learning as well as medicinal development processes.

It employs a qualitive case study design with in-depth semi-structured interviews due to the explorative as well as interpretative aims. Despite being capable of retaining thematic coherence between participants, it allows one to garner in-depth narrations. It is possible for one to examine meanings, emotions, as well as rationalities in very defined settings and it has been extensively used among organizational as well as health scholarship (Adams, 2015; Alkhoraif & McLaughlin, 2018).

With the application of open yet directive interviews, it is possible to request clarification or description without limiting conversation. In a similar vein as observed Domecq et al. (2014), there is a crucial role played by interviews in observing goals, tensions, and decision-practices forming implementation projects regarding patient engagement. Applicable in such a situation is consideration of PE as a mindset whose execution is a product of negotiation throughout multiple organizational levels, as opposed to a practice.

3.1.3 Participant Selection and Recruitment Strategy

Purposive sample, a non-probabilistic approach commonly utilized in qualitative research to choose people with particular knowledge or experience about the phenomenon being investigated,

was used to invite participants (Palinkas et al., 2015). We attempted to acquire rich, insightful information from informants intentionally chosen rather than extrapolating to a population.

A total of eight professionals who work within the healthcare and pharmaceutical sectors were randomly sampled to take part into the investigation. Because of confidentiality concerns, names of organizations together with participants have been disguised. Respondents include pharmaceutical firms that are multinational in scope, health service providers, together with patient organizations to represent a complete as well as complementing set of perspectives about what is being researched. The participants are referred to by their organizational roles: Global Patient Advocacy Manager (large Italian pharmaceutical multinational), Medical Director (large Italian pharmaceutical multinational), Product Manager (pharmaceutical multinational based in Milan), General Practitioner (territorial healthcare setting), Manager of Digital Health & Innovation (international pharmaceutical multinational), Representative of a dermatology patient association (national advocacy organization), Board Member (mid-size international biotech/pharma), and Medical Director (mid-size local hospital).

The rationale behind this matching was to mirror organizational views that are complementary to one another. The Patient Advocacy role has a relational and ethical focus and outward interaction with patient communities, whereas the group Medical Affairs is generally responsible for clinical, regulatory, and scientific alignment. Both jobs together embody "technical-scientific" and "relational-strategic" aspects of PE implementation.

Due to the networking by the researcher himself/herself, participants were solicited with access to veteran experts who otherwise would never consent to participate. This form of recruitment is familiar to qualitative research with experts or elites as participants. The "information power" principle takes precedence over small sample size by suggesting that when interviews are information-rich, when narrow goals exist, and when participants are critically relevant to the research question, participant numbers can remain smaller.

3.1.4 Data Collection Procedure and Interview Protocol

In July and August of 2025, the interviews were done remotely using Microsoft Teams. Every session was held in English and lasted roughly thirty minutes. The conversations were recorded on audio and subsequently verbatim transcribed for analysis with prior consent. A semi-structured protocol that had been pre-tested and modified in light of literature-based insights was used for the interviews (Domecq et al., 2014; Vat et al., 2021).

The five main open-ended questions in the protocol, as shown in *Table 3.1*, were each accompanied by probes intended to promote more in-depth thought and make it easier to clarify important ideas.

Table 3.1: Interview Protocol Overview

Core Question	Analytical Aim	Example Probes
How did Patient Engagement start and evolve in your organization?	Explore the historical trajectory and initial motivations	Was there a specific trigger or turning point? Who championed the initiative?
How is PE coordinated across departments or teams?	Analyse governance and cross-functional collaboration	What departments are involved? Are there tools or formal processes?
What cultural elements support or hinder PE?	Identify organizational values, norms, and resistance	Have you encountered internal pushback? How was it handled?
What has been the impact of PE on drug development?	Understand perceived outcomes and organizational learning	Has PE influenced clinical trial design, patient experience, or strategy?
What is still needed to strengthen or scale PE in your organization?	Explore gaps, needs, and future perspectives	What's missing: skills, resources, leadership support?

To evaluate the organization's ability to strike a balance between local cultural and legal peculiarities and global PE initiatives, an extra question was added.

3.1.5 Methodological Strengths and Relevance to This Research

Various gains clearly out of the scope of this dissertation are taken from embracing such research process. Specifically, it captures PE as an organizational and cultural innovation that cannot be achieved through accepting performance indicators and/or measures. Qualitative research is justified as posited by Finelli and Narasimhan (2020) in uncovering latent power relations, internal resistances, and value-based conflict during organizational changes, specifically where Participatory Values are at stake.

Secondly, interviews guarantee that what is basically the perspective of individuals who bear direct accountability for PE is not simply heard but comprehended in terms of their own institution's role and surroundings. That is required to be understood about the ways in which various actors not merely conduct PE but also apprehend and reproduce it.

Lastly, the qualitative design offers a framework for descriptive understanding. Findings can lend credence to managerially pertinent decision making and theoretical consideration through light shed on organizational enablers and inhibitors, value judgments, and future needs.

3.2 Method of Data Analysis

Braun and Clarke's (2006) created thematic analysis method that Braun and Clarke (in later work; 2014; 2021) perfected for coding research data gathered for purposes of this research study. It is one such framework chosen with a systematic yet non-stringent system for differentiating and interpreting text-based patterns (themes) of data with sufficient latitude for thought on the part of a researcher with tailoring for a specific inquiry objective. Since it allows researchers to transcend a descriptive report of interview accounts to an interpretative integration linking individual-level tales with higher organizational-level as well as movement-related culture, although, thematic analysis was one amongst the most-used methodology adapted in management as well as health studies. Because an enduring, context-specific practice wherein strategy, governance, culture, and innovation intersect is PE (Patient Engagement), adaptability with regards to corresponding (Vat et al., 2021; Zvonareva, 2023).

Braun and Clarke (2006) outlined six processes to perform while conducting thematic analysis: (1) familiarizing with data; (2) generating primary codes; (3) searching for themes; (4) re-examining themes; (5) labeling and interpreting themes; and (6) writing the report. Each step offers methodological direction while retaining freedom in tailoring based on the study design. There was exact adherence down to the letter of the six steps in the present study while there was some practical adaptation. For instance, coding was preferred over software-assisted coding and intercoder reliability was ruled out by the single-researcher study.

3.2.1 Phase 1: Familiarization with the Data

Immersion in the data was the first step. Following transcription and anonymization of the interviews, the transcripts were read several times to become acquainted with both overt and covert meanings. Following each reading, preliminary memos were written to document initial thoughts, recurring keywords, and potential links to the conceptual framework. At this point, for instance, it was common to hear mentions of cross-functional cooperation, leadership sponsorship, and regulatory drivers.

According to Braun and Clarke (2006), this phase was iterative, with transcripts being reviewed whenever new information surfaced later on. This ensured a cyclical rather than linear familiarization process.

3.2.2 Phase 2: Generating Initial Codes

Transcripts were manually coded line by line in the second phase. Codes were succinct labels that summarized text passages. According to Braun and Clarke (2014; 2021), the majority of the coding was inductive, but it was still sensitive to ideas taken from the literature and applied here as sensitizing ideas. For example, the process was informed without being constrained by categories like financial justification (Levitan et al., 2018), cultural barriers (Chegini et al., 2021), and institutionalization of PE (Vat et al., 2021).

Codes varied from interpretative ("leadership as cultural enabler," "compliance as both safeguard and constraint") to descriptive ("budget constraints," "digital portals," "training gaps"). Prior to being abstracted into higher-level categories, initial codes remained close to the participants' wording. Since the dataset was small and manual coding allowed for a closer interaction with the content, no CAQDAS software was used (Marshall et al., 2024).

3.2.3 Phase 3: Searching for Themes

Following their creation, codes were grouped into potential themes that reflected more general meaning patterns. For instance:

- *Lack of resources*, *absence of training*, and *no dedicated structures* → **Resource Constraints**.
- *Compliance frameworks*, *regulatory guidance*, *governance rules* → **Compliance and Governance**.
- *Collaboration across Medical Affairs, Market Access, and Communication* → **Cross-functional Coordination**.

To investigate connections, such as how leadership sponsorship affects cultural receptivity to PE, preliminary thematic maps were created. Instead of being refined into software-based diagrams, these maps were used heuristically.

3.2.4 Phase 4: Reviewing Themes

These potential themes were then tested against the data to ensure that it was original and made logical sense. This phase required testing themes against the entire dataset and against coded excerpts, as Braun and Clarke (2006) recommended.

There were multiple themes that overlapped; for instance, "digital tools" was first noted as a discrete theme but later merged with Cross-functional Coordination as participants so often positioned digital platforms as mediating collaboration. In contrast, "global-local tension" was re-subsumed as a sub-theme of Challenges of Scalability, as with some research with very rare diseases where localized practice was determined by response of patients (Furlong et al., 2024).

3.2.5 Phase 5: Defining and Naming Themes

Themes were defined, delineated, and labeled after they were refined. To prevent conceptual overlap, each was carefully limited. For instance, the transition from episodic sponsorship of patient associations to planned, strategic patient involvement throughout the R&D lifecycle was referred to as the Evolution and Institutionalization of PE (Vat et al., 2021; Zvonareva, 2023).

The five final themes were:

1. **Evolution and Institutionalization of PE**
2. **Cross-functional Coordination**
3. **Cultural Barriers and Enablers**
4. **Impacts on R&D Outputs**
5. **Challenges for Scalability**

These themes mirror the structure of Chapter 3 (Findings) and ensure coherence between method and results.

3.2.6 Phase 6: Producing the Report

The final step was combining themes with a narrative that was meaningful and backed with direct quotations. This step, as Braun and Clarke (2006) drive home, is an interpretative integration that connects empirical research with theoretical discourses rather than providing descriptive reporting. The Findings chapter of the current thesis exemplifies the step by listing themes with exemplary quotations of interview respondents and integrating them into the broader body of research concerning patient advocacy, organizational reform, and pharmaceutical digitization (e.g., Finelli & Narasimhan, 2020; Marshall et al., 2024; Zvonareva, 2023).

3.2.7 Adaptations and Simplifications

Although Braun and Clarke's six-phase model was closely followed, some adaptations were made:

- **Manual coding** over CAQDAS software, to preserve closeness to the text.
- **Single-coder design**, common in single-author research, mitigated through iterative checking and dialogue with the literature.
- **Heuristic thematic mapping**, used for internal reflection but not formalized.

3.2.8 The Significance of Thematic Analysis

Other methods were entertained but not used. Because the aim was interpreting expert opinion in dialogue with pre-existing frameworks rather than theory building, grounded theory (Glaser & Strauss, 1967) was not appropriate. Content analysis would have turned interpretation into frequency counts, while framework analysis (Ritchie & Spencer, 1994) was needlessly rigid.
Considering this, the most appropriate technique was thematic analysis, as it gave us the required flexibility to identify emergent dynamics while retaining systematic rigor (Braun & Clarke, 2006; 2014; 2021).

4. Findings

4.1 Overview of the Results Section

The six canonical phases of Braun and Clarke's (2006) thematic analysis (familiarization with the data, code generation, theme identification, review, definition, and report writing) were followed in the qualitative data analysis. Finding recurrent patterns and opposing viewpoints was made possible by this approach, which proved especially appropriate for investigating the perspectives of various stakeholders (pharmaceutical industry, clinical world, and patient associations).

Eight semi-structured interviews, each lasting 20 to 30 minutes on average, make up the dataset. The interviewees' diverse backgrounds offer a nuanced viewpoint on patient engagement (PE), as shown in *Tab 4.1*:

Tab 4.1: The interviewees

Global Patient Advocacy Manager: large Italian pharmaceutical multinational
Medical Director: large Italian pharmaceutical multinational
Product Manager: pharmaceutical multinational based in Milan
General Practitioner: operating in a territorial setting
Manager of Digital Health & Innovation: international pharmaceutical multinational
Representative of a dermatology patient association: national advocacy organization
Board Member: mid-size international biotech/pharma
Medical Director: mid-size local hospital

The interviews, as shown in *Tab 4.2*, explored:

1. The evolution of PE
2. Internal coordination dynamics
3. Enabling and hindering cultural factors
4. Perceived impacts on project quality and relevance
5. Future needs and scalability challenges
6. Global–local balance

Tab 4.2: Emerging Themes

Protocol Question	Objective	Theme Emerging from Thematic Analysis
1. PE's development and changing priorities	Recognize the origins and development of the organization	**Theme 1: Evolution and Institutionalization**
2. Structure and coordination of PE across functions	Examine cross-functional cooperation and governance.	**Theme 2: Cross-Functional Coordination and Compliance**
3. Organizational culture: obstacles and facilitators	Examine enabling values and resistances.	**Theme 3: Cultural Factors**
4. Perceived impacts on quality and relevance	Elicit useful implications for clinical practice, research, and organization	**Theme 4: Impact**

5. Future needs and consolidation	Determine the leadership, procedures, and resources that are required.	**Theme 5: Challenges for Scalability**
Extra question – Global–local balance	Examine cultural and regulatory variations in various markets.	**Theme 5: Global–Local Harmonization**

4.2 From Coding to Final Themes

It was thought to be helpful to report the matrix that shows the analytical path: from the initial codes, which emerged from the reading and segmentation of the interviews, to the intermediate sub-themes, and finally to the final themes that structure this chapter. This ensures methodological transparency and consistency with Braun and Clarke's (2006; 2014; 2021) approach. *Table 4.3* illustrates the process of interpretation that resulted in the creation of analytical categories, but it does not take the place of the narrative. To put it another way, it emphasizes how the transition from unprocessed data to more abstract ideas was not random but rather derived from the interviewees' conversations and then arranged into logical conceptual groups.

Tab 4.3: Initial Codes, Sub-themes, Final themes

Initial Codes (extracted from interviews)	Sub-themes	Final Themes
Financial sponsorship to associations	From sponsorship to co-creation	Evolution and Institutionalization
Regulatory pressure	Regulatory origins	Evolution and Institutionalization
Patient involvement in rare diseases	Origins in rare diseases	Evolution and Institutionalization
PE function located within Medical Affairs	Variability in organizational positioning	Evolution and Institutionalization

Cross-functional task forces	Interdepartmental integration	Cross-Functional Coordination and Compliance
Use of digital platforms	Digital as an enabler	Cross-Functional Coordination and Compliance
Compliance as a constraint	Compliance as a constraint	Cross-Functional Coordination and Compliance
Compliance as a guarantee	Compliance as a catalyst	Cross-Functional Coordination and Compliance
Inclusive leadership	Leadership sponsorship	Cultural Factors
Skepticism: "the doctor prescribes, not the patient"	Internal awareness gap	Cultural Factors
Non-independent associations	Conflicts of interest	Cultural Factors
Younger patients more informed	Generational empowerment	Cultural Factors
Active role of caregivers	Caregiver involvement	Cultural Factors
Revision of packaging and instructions	Communication and support	Impact
Reduction in injection volume	Product improvement	Impact
Development of ergonomic device	Device improvement	Impact
Advocacy lobbying for access	Advocacy and access	Impact
Lack of physicians' time	Time as a barrier	Challenges for Consolidation and Scalability
Insufficient budget	Dedicated resources	Challenges for Consolidation and Scalability
Need for KPIs and incentives	Strategic recognition	Challenges for Consolidation and Scalability
Global/local differences	Global–local harmonization	Challenges for Consolidation and Scalability

Too many fragmented associations	Fragmentation of representation	Challenges for Consolidation and Scalability

As the table illustrates, the original codes made it possible to isolate specific elements (such as "insufficient budget," "reduction in injection volume," and "younger patients more informed"), which were then grouped into more general conceptual sub-themes (such as "Dedicated resources," "Product improvement," and "Generational empowerment") before coming together to form the five final themes that make up the Findings chapter's interpretive framework.

4.3 Theme 1: Evolution and Institutionalization of Patient Engagement

4.3.1 Sub-theme 1.1: From Sponsorship to Co-Creation

The **Manager in Digital Health & Innovation** emphasized the acceleration brought about by the pandemic:

"We moved from simple awareness campaigns to continuous listening platforms, with online communities and personalized content. Today, digital has made PE a structural part of strategies."

From the patient associations' side, the same change emerged. The **organization representative** observed:

"Patient Engagement has now become a cornerstone for companies, institutions, and associations. It is no longer just consultation: today it means participation in decision-making tables and co-design of care pathways."

This trajectory confirms the literature: Vat et al. (2021) describe the shift from symbolic forms of engagement to structured partnerships; Carman et al. (2013) emphasize the concept of a "continuum" leading from mere listening to actual co-creation; Marshall et al. (2024) document the positive impact of this paradigm shift on clinical studies.

4.3.2 Sub-theme 1.2: Diversity of Origins and Sectors

The evolution of PE was not linear but followed different paths across contexts. The **Board Member of a mid-size biotech** highlighted how it all started particularly in rare diseases:

"Patient Engagement originates from the initiative of patients themselves, who organized because they had no available treatments. Associations built databases, pressured the system, and created real leverage over companies."

The **Medical Director of a small local hospital** described a more recent change in the clinical context:

"If in the past the patient was seen as the end consumer, today they are an active part of care. This improves adherence and satisfaction, and in many cases caregivers play a central role."

The literature confirms this plurality of trajectories: Chegini et al. (2021) show that PE develops differently depending on organizational maturity and therapeutic area, while Zvonareva (2023) stresses the specific push from rare diseases, where the lack of alternatives made the protagonism of associations indispensable.

4.3.3 Sub-theme 1.3: Variability of Organizational Positioning

Finally, a recurring point concerns the positioning of the PE function within companies. The **Medical Director** noted:

"In the past, Patient Engagement was managed by Medical Affairs. Today, however, many companies have dedicated functions, though located in different departments: Market Access, Public Affairs, Medical."

The **Product Manager** added:

"Positioning varies, and this reflects the level of maturity and priority the company assigns to PE. There isn't a single model."

Chegini et al. (2021) confirm this observation, noting that the absence of a standardized placement makes PE a "hybrid" function, often in search of stable legitimacy.

Tab 4.4: Summary of Theme 1: Evolution and Institutionalization of Patient Engagement

Sub-theme	Description	Illustrative Quotes	Literature References
Sponsorship → Co-creation	From symbolic activities to shared and structured projects	*"The term Patient Engagement wasn't used; we only talked about adherence"*(General Practitioner) *"We moved from awareness campaigns to continuous listening platforms"*(Digital Health Manager) *"Today it means participation in decision-making tables"* (Patient Association)	Vat et al., 2021; Carman et al., 2013; Marshall et al., 2024
Differentiated origins and sectors	PE born from specific needs (e.g., rare diseases) and progressively integrated in hospitals and clinics	*"PE originates from the initiative of patients themselves"* (Biotech Board Member) *"Today the patient is an active part of care"* (Local Hospital Director)	Chegini et al., 2021; Zvonareva, 2023
Organizational positioning	Variable placement of PE function within companies	*"Initially managed by Medical Affairs, today dedicated teams exist"* (Medical Director) *"Positioning varies, no single model exists"* (Product Manager)	Chegini et al., 2021

4.4 Theme 2: Cross-Functional Coordination and the Role of Compliance

4.4.1 Sub-theme 2.1: Integration in Interdepartmental Projects

Patient Engagement is described by all interviewees as an activity requiring contributions from multiple functions, with varying degrees of formalization depending on the context.

The **Global Patient Advocacy Manager** described PE as a "bridge" function:

"Our role is to bring the patient's voice into the corporate conversation, even when the project seems to concern only scientific or market areas."

The **Product Manager** confirmed that today PE involves not only Medical Affairs, Market Access, and Communication, but also digital functions:

"We manage portals and platforms for patient feedback, in coordination with compliance and communication. This makes PE part of dedicated task forces, with regular meetings and shared objectives."

The **Manager in Digital Health & Innovation** emphasized how coordination has now become more fluid:

"Digital, Marketing, Communication, and Patient Advocacy now work together on a regular basis. We use shared dashboards and social listening tools to monitor in real time what works and what doesn't."

From the clinical side, experiences also emerged. The **General Practitioner** recalled the electronic registry for diabetic patients in his region, developed with the contribution of associations and industry:

"It was clear that it had been designed with and for patients: intuitive, useful, capable of facilitating conversations between doctor and patient."

Finally, from the patient associations' perspective, it emerged that the more structured companies are those that more easily establish joint working groups:

"The main point of contact remains the Patient Advocacy function, but real collaboration requires companies to have dedicated processes and roles. Associations cannot deal with ten different figures—clarity is essential."

These statements are in line with Vat et al. (2021), who contend that credible PE requires cross-functional governance, and Finelli & Narasimhan (2020), who emphasize how digitalization promotes quicker and easier collaboration.

4.4.2 Sub-theme 2.2: Compliance as a Safeguard and Catalyst

The role of compliance was recognized as crucial from multiple perspectives.

The **Medical Director** emphasized the regulatory constraints imposed by Farmindustria and other authorities:

"These are not obstacles, but necessary rules to maintain transparency in relationships and reduce reputational risks."

The **Manager in Digital Health & Innovation** added that the biggest challenge in digital concerns privacy:

"The real critical point is ensuring ethical and safe interactions. We have developed standard formats and clear guidelines precisely to protect patients and reduce risks."

From the associations' side, however, the opposite problem emerged: overly bureaucratic procedures risk excluding less structured groups:

"If an association lacks the resources to handle complex contracts, it risks being left out of discussions, and this is a paradox because PE should be inclusive."

The **Hospital Medical Director** noted that in smaller hospitals compliance is perceived more as a constraint than a lever:

"PE is not structured; it depends a lot on the individual doctor or department. Bureaucracy often slows things down more than it helps."

These results align with the views of Vat et al. (2021), who support multi-level guidelines that can strike a balance between rules and inclusion, and Chegini et al. (2021), who emphasize the dual nature of compliance as both enabling and limiting.

Tab 4.5: Summary of Theme 2: Coordination and Compliance

Sub-theme	Description	Illustrative Quotes	Literature References

Interdepartmental integration	Cross-functional involvement of roles and stakeholders	*"Dedicated task forces with shared objectives"* (Product Manager) *"Shared dashboards and social listening"* (Digital Health Manager) *"Electronic registry developed also with patients"* (General Practitioner)	Vat et al., 2021; Finelli & Narasimhan, 2020
Compliance as safeguard and catalyst	Rules as protection, but also a risk of exclusion	*"Necessary safeguards for transparent relationships"* (Medical Director) *"Privacy as a critical point in digital"*(Digital Health Manager) *"Less structured associations risk being excluded"* (Patient Association)	Chegini et al., 2021; Vat et al., 2021

4.5 Theme 3: Enabling and Hindering Cultural Factors

4.5.1 Sub-theme 3.1: Leadership Sponsorship

Support from top management was cited by several interviewees as a fundamental lever. The **Global Patient Advocacy Manager** emphasized:

"My manager fully supports this vision: it's not just communication, it's strategy."

The **Manager in Digital Health & Innovation** confirmed:

"There is a natural curiosity for new tools and strong support from leadership. This has made it easier to integrate patients' perspectives into activities."

The **Hospital Medical Director** added that in hospitals, sponsorship must translate into clinical culture:

"We need leaders who promote empathy, active listening, and interdisciplinarity. Without this, PE remains just a label."

These data connect to Chegini et al. (2021), who identify inclusive leadership as the main cultural enabler.

4.5.2 Sub-theme 3.2: Internal Awareness Gaps and Resistance

Resistance also emerged. The **General Practitioner** recalled:

"Many colleagues, especially years ago, were skeptical: 'the patient should just follow the prescription.' Even among patients, some prefer to delegate everything."

The **Product Manager** reported a recurring phrase among colleagues:

"It's the doctor who prescribes, not the patient! This was the initial attitude, overcome only by showing concrete cases of benefits."

The **Board Member** warned that there is also a risk of instrumentalization:

"Some associations are not entirely independent, because they were created or funded by a single company. This undermines the credibility of Patient Engagement."

These observations recall Johnson et al. (2021), who identify lack of knowledge and risks of conflicts of interest as two of the most widespread obstacles to PE.

4.5.3 Sub-theme 3.3: Patient Empowerment and Generational Change

Many interviewees observed a cultural shift among patients. The **General Practitioner** noted:

"Younger patients come in informed, ask questions, want to understand. Sometimes they bring confusion, but it's a positive sign: there's more willingness to participate."

From the hospital side, the **Hospital Medical Director** highlighted the role of caregivers:

"In caring for frail elderly patients, caregivers are active participants in Patient Engagement. We can no longer think only of the patient as isolated."

The **organization representative** stressed that associations try to interpret this new demand:

"We try to represent patients' real needs, not only clinical but also quality of life. This changes the culture of dialogue with companies and institutions."

These observations align with the work of Marshall et al. (2024), who demonstrate that patient empowerment is particularly increasing among younger populations and in digital contexts, and Carman et al. (2013), who characterize PE as a continuum spanning from the informative level to partnership.

Tab 4.6: Summary of Theme 3: Cultural factors

Sub-theme	Description	Illustrative Quotes	Literature References
Leadership sponsorship	Support from management as a cultural lever	*"It's not just communication, it's strategy"* (Global Patient Advocacy Manager) *"Strong support from*	Chegini et al., 2021

		leadership" (Digital Health Manager) *"We need leaders who promote empathy and listening"* (Hospital Medical Director)	
Gaps and resistance	Initial skepticism, conflicts of interest	*"The patient should just follow the prescription"*(General Practitioner) *"It's the doctor who prescribes, not the patient"*(Product Manager) *"Some associations are not independent"* (Board Member)	Johnson et al., 2021
Empowerment and generational change	More informed patients, active caregivers	*"Young patients want to understand"* (General Practitioner) *"Caregivers are active participants"* (Hospital Medical Director) *"We represent quality-of-life needs"* (Patient Association)	Carman et al., 2013; Marshall et al., 2024

4.6 Theme 4: Impact of Patient Engagement

4.6.1 Sub-theme 4.1: Improvement of Products and Devices

Interviewees provided concrete examples of how patient involvement has had tangible effects on the development of products and devices.

The **Global Patient Advocacy Manager** shared a case in the immunology field:

"Patients found the injection too painful due to the high liquid volume. We reformulated the drug to reduce the volume and improve tolerability. This was not a detail: it changed the therapy's acceptability."

The **Medical Director** mentioned the experience of the *Cinzia* device developed with an ergonomic design company:

"Patients with rheumatoid arthritis could not use thin pens. We designed a larger device with a rubber grip. It was a success: patients used it with less difficulty and greater continuity."

From the associations' perspective, the **organization representative** recalled the impact of PE in psoriasis:

"Patients told us that topical therapies were too demanding. From there emerged the urgency for simpler solutions, such as oral drugs. This guided development."

These examples confirm Lavallee et al. (2022), who document how patient feedback has led to clinical studies with more realistic protocols and more user-friendly devices.

4.6.2 Sub-theme 4.2: Communication, Packaging, and Support Services

PE has had effects not only on products but also on communication and services.

The **Global Patient Advocacy Manager** noted:

"Even the packaging and instructions were revised: patients told us they were incomprehensible, so we simplified the language and modified the formats."

The **Product Manager** emphasized the impact on clinical protocols:

"Thanks to PE, we now have more realistic and better-tolerated protocols, with lower dropout rates. It's not only the drug that changes, but the entire patient experience."

The **General Practitioner** confirmed an indirect effect in daily practice:

"When campaigns or tools are developed with patients, I notice it because my patients come in with new questions: they ask not only if the drug works, but whether it will let them work or sleep well."

The **Hospital Medical Director** also linked PE to better adherence to treatment:

"In cardiology, we observed a direct impact on adherence: patients are more motivated to follow treatments when they feel they have contributed to the protocols."

These findings align with Chegini et al. (2021), who stress the importance of clear communication and language adapted to patient needs.

4.6.3 Sub-theme 4.3: Advocacy and Access to Care

Another significant impact concerns advocacy and improved access.

The **Medical Director** cited the Spanish case:

"Associations pressured to remove prescribing restrictions on triple therapies for asthma and COPD. Eventually, authorities expanded the pool of physicians authorized to prescribe them. The result was broader access."

The **Board Member** noted that associations can also play a critical role in reducing prices:

"Some lobby to lower costs, making medicines more accessible. This is a real impact, even if it can affect company cash flow."

The **organization representative** confirmed that joint discussions help improve patients' quality of life:

"Thanks to our contributions, some companies revised their development strategies, making treatments easier to follow. This has a concrete impact on people's daily lives."

These observations resonate with Johnson et al. (2021), who highlight how PE can have financial and regulatory impacts in addition to clinical ones.

Tab 4.7: Summary of Theme 4: Impact of Patient Engagement

Sub-theme	Description	Illustrative Quotes	Literature References
Product and device improvement	Reformulation and redesign for usability and acceptability	*"We reduced the injection volume"* (Global Patient Advocacy Manager) *"We developed an ergonomic device"*(Medical Director) *"Psoriasis highlighted the need for simpler solutions"* (Patient Association)	Lavallee et al., 2022; Vat et al., 2021
Communication, packaging, and services	PE as a driver for language, protocols, and adherence	*"We now have more realistic and better-tolerated protocols"* (Product Manager) *"My patients come with new*	Chegini et al., 2021; Lavallee et al., 2022

		questions"(General Practitioner) *"In cardiology there is more loyalty to treatment"* (Hospital Director)	
Advocacy and access	Associations' pressure on policy and pricing	*"Associations pressured authorities"*(Medical Director) *"Some lobby to lower costs"* (Board Member) *"We made treatments easier to follow"*(Patient Association)	Johnson et al., 2021; Vat et al., 2021

4.7 Theme 5: Challenges for Consolidation and Scalability

4.7.1 Sub-theme 5.1: Dedicated Resources

The need for specific resources was one of the most recurring points.

The **Global Patient Advocacy Manager** stated:

"Budgets, time, and dedicated people are needed; otherwise, Patient Engagement risks remaining just a label."

The **Product Manager** added:

"Beyond budget, cross-functional training is required: if colleagues do not understand the value of PE, it risks being confined to a few people."

The **General Practitioner** confirmed that lack of time is an obstacle:

"If the visit lasts ten minutes, there will never be real engagement. Dedicated spaces and times are needed."

The **Hospital Medical Director** reiterated that without structural investments PE remains dependent on individual goodwill:

"In small hospitals it depends on the doctor or department: without budgets and specific incentives, PE never becomes systemic."

These testimonies reflect Vat et al. (2021), who identify financial resources and skills as prerequisites for sustainable PE.

4.7.2 Sub-theme 5.2: Strategic Recognition

Recognition of PE as a strategic lever was another highlighted condition.

The **Global Patient Advocacy Manager** explained:

"The patient's voice must reach leadership tables. If it remains confined to an operational role, it loses impact."

The **Hospital Medical Director** stressed the need for KPIs and incentives:

"We need sponsorship from top management, process standardization, clear indicators. And rewards linked to results must be planned to encourage consistent behaviors."

From the digital perspective, the **Manager in Digital Health & Innovation** suggested a more structured approach:

"A shared playbook would help standardize languages and practices across countries and internal teams, strengthening coherence in Patient Engagement."

These observations are consistent with Chegini et al. (2021), who see leadership support and process formalization as two pillars for consolidating PE.

4.7.3 Sub-theme 5.3: Global–Local Harmonization

The challenge of balancing global guidelines with local adaptations was emphasized by several interviewees.

The **Manager in Digital Health & Innovation** highlighted the usefulness of digital as a rapid adaptation tool:

"Principles are global, but language and channels must always be adapted. Digital allows us to test and calibrate quickly without losing coherence."

The **Medical Director** recalled the need to respect local regulations even in centralized projects:

"If you organize an international event, you still need to follow the host country's rules. Local partners are the ones who ensure compliance."

The **Board Member** warned that fragmentation among patient associations can hinder harmonization:

"If too many associations exist for the same condition, it becomes difficult to understand who really represents patients and to ensure independence."

These observations align with Johnson et al. (2021), who note that regulatory and organizational fragmentation hinders PE scalability at the international level.

Tab 4.8: Summary of Theme 5: Challenges

Sub-theme	Description	Illustrative Quotes	Literature References

Dedicated resources	Budgets, training, time, and staff	*"Budgets, time, and dedicated people are needed"*(Global Patient Advocacy Manager) *"If the visit lasts ten minutes, real engagement is impossible"* (General Practitioner) *"In small hospitals it depends on individuals"*(Hospital Director)	Vat et al., 2021
Strategic recognition	Integration of PE into priorities and corporate KPIs	*"The patient's voice must reach leadership tables"* (Global Patient Advocacy Manager) *"KPIs and incentives are needed"* (Hospital Director) *"A shared playbook would help standardize practices"* (Digital Health Manager)	Chegini et al., 2021; Vat et al., 2021
Global–local harmonization	Balance between global principles and local adaptations	*"Digital allows rapid adaptation"* (Digital Health Manager) *"Local partners ensure compliance"* (Medical Director) *"Too many associations fragment representation"*(Board Member)	Johnson et al., 2021; Vat et al., 2021

4.8 Thematic Map of the Results

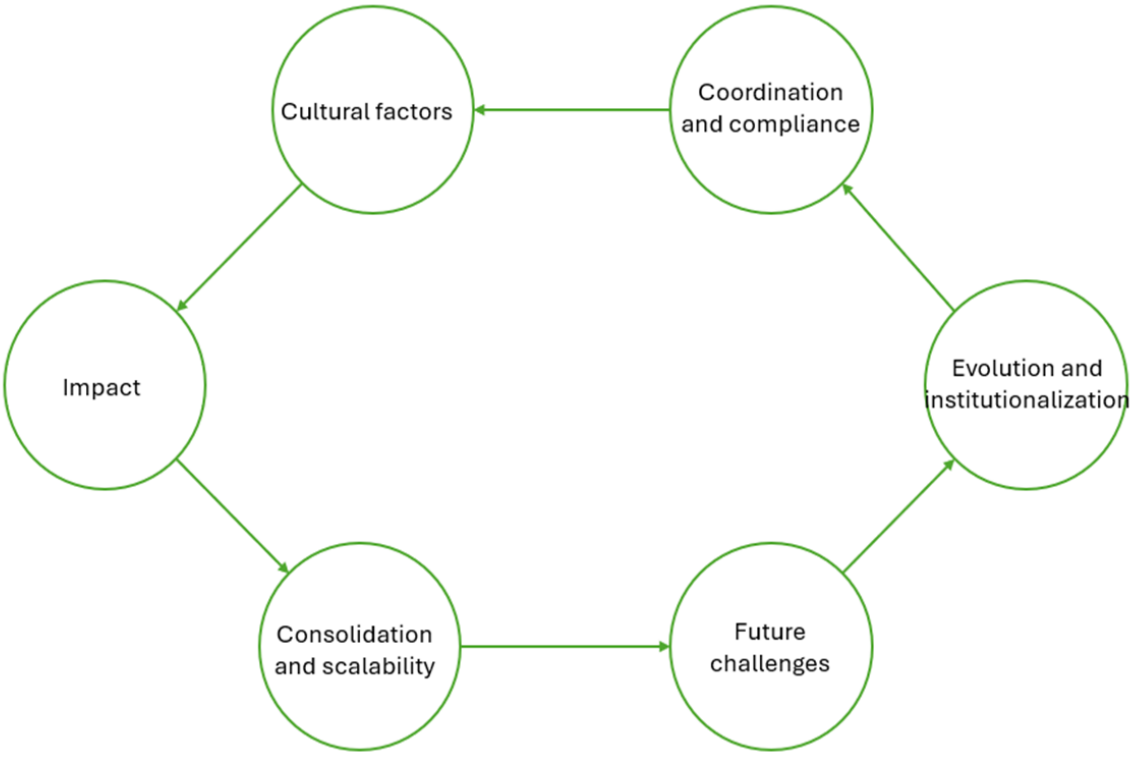
The map shown in the *Figure 4.1* visually represents the relationships among the themes that emerged from the analysis. A circular representation was chosen to emphasize the cyclical and iterative nature of Patient Engagement (PE), which cannot be understood as a linear process but rather as a dynamic that is constantly evolving.

The analysis shows that the historical evolution and institutionalization of PE enabled the subsequent development of cross-functional coordination, supported and regulated by the role of compliance. Coordination, in turn, contributed to modifying internal cultural factors, reducing resistance and fostering the empowerment of patients and caregivers.

This cultural and organizational change translated to concrete implications for product and device excellence, planning for clinical protocols, and access-to-care policy. These implications reinforced PE's reputation as a strategic lever, generating pressure to institutionalize its adoption and to upscale its practices.

At this stage, the main challenges emerge, related to the availability of dedicated resources, recognition within leadership processes, and the need to harmonize global and local practices, which close the cycle and feed back into the future evolution of PE, pointing to new directions for development and continuous improvement.

Figure 4.1: Thematic map



4.9 Final Summary of the Results

The analysis of the eight interviews provides a broad and multi-level picture of Patient Engagement (PE).

Tab 4.9: Final summary

Evolution	from sporadic activities and sponsorships to structured co-creation practices, driven by both external factors (regulators, patient associations) and internal factors (corporate leadership, digital transformation).
Coordination	PE emerges as a cross-functional activity, integrating multiple corporate units (Medical, Market Access, Communication, Digital) and interacting with associations, general practitioners, and hospitals. Compliance acts both as a constraint and as a catalyst.
Cultural factors	leadership support and patient empowerment have reduced skepticism, although resistance and risks of instrumentalization remain.
Impact	PE has produced concrete improvements in more realistic clinical protocols, more user-friendly devices, clearer communication, and broader access to care.
Challenges	the main critical issues concern scarcity of resources, the need for strategic recognition,

	and fragmentation between global and local levels.

The findings shown in *Tab 4.9* are in line with research from around the world (Vat et al., 2021; Chegini et al., 2021; Lavallee et al., 2022; Johnson et al., 2021; Marshall et al., 2024; Finelli & Narasimhan, 2020; Zvonareva, 2023). They also demonstrate that, even though patient engagement is now widely acknowledged as a crucial component of drug development, organizational, cultural, and structural obstacles must be removed before it can become a truly systemic and scalable practice.

5. Discussions

Interpreting the results of Chapter 3 in the context of the study's goals and the larger scholarly discussion of Patient Engagement (PE) in pharmaceutical development and healthcare institutions is the aim of this discussion. While the previous chapter explained how PE has changed over time, how it is coordinated across functions, what cultural factors influence it, what effects it produces, and what obstacles still stand in the way of its consolidation and scalability, the current section aims to make sense of these insights by relating them to theoretical frameworks and empirical data from the literature. It enables a holistic investigation into how PE is implemented into organizations, how it transforms procedures internally and externally, and what conditions should be met for it to become a systemic and sustainable capability.

Transitioning Patient Engagement from occasional and symbolic endeavors to more formalized and institutionally established practices is the subject of the first seminal finding. Interview participants would frequently comment about how firms would once only financially sponsor patient organizations but now more routinely participate in such co-creation projects as online forums, usability-influenced redesigning of medical equipment, or jointly developing patient support programs. Vat et al. (2021) signal transforming from symbolic participation to institutionally established practices embedded within organizational structures of governance, and Carman et al. (2013) theorize about having a continuum scale from information delivery to partnership.

These findings are highly consistent with each other. Zvonareva's (2023) contention that PE becomes essential where unmet needs are greatest and where patient communities themselves produce knowledge, databases, and advocacy pressure that influence businesses' strategies is particularly supported by the evidence from rare disease contexts. In this regard, the findings of the interviews demonstrate that institutionalization is a reaction to bottom-up pressure from empowered communities as well as a top-down organizational choice. By demonstrating that PE matures more quickly when demand-side capability is present in the ecosystem, this nuance deepens the body of existing literature.

The data also revealed the importance of compliance and cross-functional coordination as a major theme. According to interviewees, PE is a cross-cutting role that links teams in Medical Affairs, Market Access, Communication, and increasingly Digital. Dedicated patient advocacy managers frequently serve as boundary-spanners in this regard. This supports Vat et al.'s (2021) multi-stakeholder framework, which identifies interdepartmental governance as crucial for credible PE, and it echoes Finelli and Narasimhan (2020), who contend that digital transformation encourages new cross-functional models of collaboration. However, compliance was viewed in two ways: as a safeguard that ensures ethics, transparency, and legal certainty, and as a barrier that may exclude less professional or smaller associations that are unable to handle the complexity of bureaucracy. This dual role is also explained by Chegini et al. (2021), who point out that if rules intended to ensure fairness are not accompanied by proportionate mechanisms of inclusion, they may unintentionally reinforce inequities. The interviews also highlight how regulatory rigidity limits mid-cycle product adjustments, indicating that PE has the greatest influence upstream, in early protocol and device design, and downstream, in communication and service delivery. This finding is consistent with Lavallee et al. (2022), who demonstrate that early integration of patient input maximizes feasibility and retention.

The results also emphasize how crucial cultural elements are in determining the breadth and caliber of physical education. Leadership sponsorship was frequently mentioned as a key facilitator: teams align, commit resources, and take the patient perspective more seriously when senior management frames PE as strategy rather than communication. On the other hand, professional skepticism, summed up in the common adage "the physician prescribes, not the patient," demonstrates the enduring paternalistic mindsets that impede participatory methods. This is consistent with the findings of Johnson et al. (2021), who discovered that professional mindsets and cultural resistance continue to be among the most challenging obstacles to overcome. However, the data also suggests a generational shift: caregivers are becoming more involved in chronic and frail contexts, while younger patients arrive better informed, ask questions, and want to be involved in decision-making. These cultural changes are consistent with Marshall et al. (2024), who highlight how digitalization promotes shared decision-making and increases empowerment. However, the literature on the independence and representativeness of advocacy groups also emphasizes the risk that PE's credibility may be damaged if patient associations are financially reliant on a single

sponsor (Vat et al., 2021). This emphasizes how important plural representation and open governance are to maintaining legitimacy.

Impact upon medication development and delivery to healthcare is most persuasive. From modified-injection design with smaller volumes todevices created for patients with limited dexterity, from better-designedpackaging and instructions to protocol changes which alleviated burden and attrition, interview participants supplied vivid examples of enhancing therapy usability with patient insights. These improvements align with empirical literature showing that patient engagement yields more realistic trials, superior adhesion, and higher satisfaction (Lavallee et al., 2022; Marshall et al., 2024). Interview conclusions affirm that PE has an impact upon market forces and access policies to an equal extent as clinical design. In reports, organizations were successful negotiating reduced costs or expanded prescribing eligibility, which demonstrates that participation has fiscal and regulatory impacts alongside clinical. Such activism has an influence upon successful results from trials but also reimbursement decisions and public confidence among pharmaceutical companies, according to CTTI reports alongside Johnson et al. (2021). In doing so, PE becomes a generator of hard economic and reputational value alongside an ethical imperative.

Notwithstanding these developments, the results highlight the need to overcome enduring obstacles in order to consolidate and scale PE. Three requirements stand out: global–local harmonization (balancing global principles with adaptation to local regulations and cultural contexts), strategic recognition (leadership sponsorship, KPIs, incentives), and dedicated resources (budgets, trained staff, time). PE runs the risk of staying limited to driven individuals or pilot projects in the absence of these. These circumstances are in line with those of Vat et al. (2021), who advocate for standardization and capacity-building, as well as Chegini et al. (2021), who emphasize process formalization and leadership as prerequisites for sustainability. The global-local balance seems to be especially important: effective PE must be localized, respecting national laws, cultural norms, and the maturity of local advocacy ecosystems, even though global templates and ethical baselines can direct practice. This conflict is similar to what has been observed in multi-country trials and rare disease collaborations, where contextual adaptation is frequently necessary for central strategies (Furlong et al., 2024). To prevent escalating disparities, businesses and

institutions may need to make investments in capacity-building in areas where associations are dispersed or nonexistent.

When combined, these findings improve managerial and theoretical knowledge of PE in a number of ways. First, they affirm that PE should be viewed as an organizational capability that develops through interaction with external ecosystems rather than as a stand-alone project. Second, they propose redefining compliance as a design question, asking whether rules are applied in ways that increase or decrease participation, rather than just as a constraint. Third, they describe a practical "engagement–fit–outcome" chain in which early patient involvement improves how well protocols and products fit patients' lives, which in turn improves adherence, retention, and, eventually, practical efficacy. These comments present fresh facts from interviews alongside correspond with Marshall et al.'s (2024) empirical assessments and Vat et al.'s (2021) normative models. From a management point of view, they signal that firms should develop PE as a controlled competence with resources, practices, and metrics rather than handle it as an improvised activity. They also signal a requirement for leadership to synchronize PE with strategic agendas and incentive systems alongside balanced systems of compliance. On their part, authorities responsible for regulating as well as HTA authorities can go a step further to legitimize PE at the policy level by endorsing patient-generated evidence, facilitating early scientific consultation with participation by patients, as well as providing infrastructure to patient organizations.

Lastly, it can be asserted that the discussion is a reaffirmation to the idea that patient engagement is indeed a part of innovation for pharmaceutical sectors as well as health systems today but is no longer a voluntary or tokenistic exercise. However, associated hurdles including cultural resistance, lack of resources, as well as disparity between local knowledge and international standards still impede complete institutionalization.

A systemic strategy that incorporates governance, external ecosystems, and organizational culture is needed to address these problems. The industry can only shift from involving patients for development to involving them with and as part of development by pooling resources, strategically recognizing PE, and standardizing practices at all levels. By demonstrating how engagement is implemented in practice, where it has the biggest impact, and which structural levers are required

to turn it into a scalable and sustainable capability, the research adds to the body of literature in this way.

6. Conclusions

6.1 Practical Implications

It began with an appreciation for a conspicuous absence within dominant literature regarding patient engagement (PE). In recent years, an increasing number of publications have noted the value to be had from engaging patients with pharmaceutical development but most contributions to the literature have depicted PE as piecemeal, occasional, and fundamentally restricted to pilots or one-time collaborations with patient groups. What has been missing is a critical consideration of how PE could transition from an occasional activity to a perennial organizational function with the ability to impact pharmaceutical innovation's quality, relevance, and legitimacy.

To offset such a void, the thesis adopteda three-pronged approach. It first conducted a theoretical examination of the literature with a focus on dominant perspectives and new paradigms about PE. It then synthesized empirical commentary gleaned from qualitative interviews with industry participants, patient advocates, among others to chronicle how engagement is enacted and where organizational barriers persist. Finally, it developed a composite perspective with a footing in organizational design to explore how PE can be instilled with an alignment among strategy, structure, process, and culture.

From such integrated perspective, our results confirm that indeed PE is facing a transitional phase: fragmentation is still present but rising signs of institutionalization emerge. Some pharmaceutical companies have introduced cross-functional task forces including Medical Affairs, Market Access, Digital, and Communication, others have created special functions and positions. Compliance, although often perceived as bureaucratic but unavoidable, proved to be a relevant enabler through introducing transparent rules facilitating safe and transparent participation of patients. At the same time, patient organizations are becoming established institutional actors with a capacity to influence procedures as well as access policies, and local providers increasingly recognize a value to be gained from active listening.

These findings align with recent scholarship: Johnson et al. (2021) warn that if PE is not trained or culturally adapted, then PE is only superficial; Finelli and Narasimhan (2020) illustrate how digital transformation redraws collaboration; Marshall et al. (2024) document how patient-inclusive design of clinical trials is superior for retention and compliance; and Vat et al. (2021) document shared governance structures and metrics for measurement.

From an organizational design point of view, our analysis emphasizes integration as a core aspect. To transcend descriptive patient-centricity, companies ought to redesign their systems of coordination, facilitate cross-functional groups, clarify boundary-spanning functions, and adopt digital platforms to facilitate open decision-making and communication. In their absence, PE is intermittent and subject to individual efforts; with them, it is integrated into organizational habits. The cultural side is equally central. Leadership sponsorship became an inevitable prerequisite: if senior management positions PE as a strategic priority, then resources follow, teams organize, performance metrics signal such an orientation. But where parental models ("the doctor orders, the patient complies") dominate even to this day, integration is forestalled. But bottom-up influences alter the calculus: patient organizations reinforce bonds, caregivers play a more active role, younger patients anticipate real involvement.

Lastly, structured PE's tangible effects reveal its real-world effectiveness. The literature and empirical resources agree in revealing that PE enhances drug usability (e.g., smaller volume injection size, device design with an ergonomic fit), communicative clarity (e.g., easy-to-read instructions, easy-to-read labeling), and inclusivity (e.g., treatment access-enhancing campaigns). At an organizational and systemic level, PE promotes feasibility with respect to a clinical trial, causes fewer dropouts, prevents expensive protocol modification, and yields a good reputation as well as a regulatory benefit.

In brief, our core research question has a positive answer: organizational integration is the factor that transforms PE into a structural attribute and driver of relevance for pharmaceutical development. When organizational integration is implemented into strategy, process, and culture, PE is converted from an add-on to an option into a value-enhancing function for pharmaceuticals, health systems, and—above all—patients.

6.2 Limitations and Future Research

However, there are still a number of difficulties. PE runs the risk of being reduced to rhetoric in the absence of specific funding, protected time for professionals and clinicians, and cross-functional training. It cannot integrate into decision-making procedures without official strategic recognition, which is reflected in KPIs and reward schemes. It runs the risk of becoming fragmented and losing legitimacy if global principles are not reconciled with local adaptations. These issues are not coincidental; rather, they represent the traditional organizational design conundrums in which managing increasing complexity requires striking a balance between integration and differentiation.

Given these results, this study contributes to closing the gap in the literature, at least in part. Its contribution goes beyond simply restating the value of patient involvement; it also demonstrates that the ability of organizations to properly design it—that is, to create integrative roles, codify procedures, introduce impact metrics, and cultivate an inclusive leadership culture—determines how effective it is. In this way, PE can be viewed as a true organizational capability that needs culture, rules, and investments to develop into a reliable infrastructure.

Many areas still need to be explored in the future. Developing more advanced measurement tools, comparing governance models, conducting longitudinal analyses of PE practices, and evaluating the clinical, organizational, and economic impacts of these practices will all be critical. In terms of management, businesses will need to keep spending money on resources and expertise while experimenting with hybrid governance models that strike a balance between inclusivity and efficiency. In order to support patient associations and validate the data they produce, institutional regulators and public entities must acknowledge and reward PE.

In summary, the field of patient engagement is undergoing a metamorphosis and is now an organizational infrastructure that requires careful planning and consolidation rather than merely being a rhetorical or promise. PE can genuinely become the lever that connects scientific quality and social relevance in pharmaceutical development if businesses are prepared to view it as a transversal capability that can produce innovation and shared value.

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