THE Sumaira FOUNDATION

Embedding patient partnership in clinical research practice

A toolkit for clinicians & researchers

The Sumaira Foundation

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ABOUT THIS TOOLKIT

Overview

This toolkit is intended for researchers, clinicians, healthcare professionals, industry partners, and other stakeholders seeking practical guidance on embedding patient partnership within clinical research practice. Throughout this resource, the terms *patient*, *patient partner,* and *patient partnership* are used inclusively to refer to both patients and caregivers who contribute lived experience and experiential knowledge to research.

A companion toolkit has been developed for clinicians, academic researchers and others seeking to partner more effectively with patient organizations and patient partners. Please contact contact@sumairafoundation.org to request a copy.

About The Sumaira Foundation (TSF)

The Sumaira Foundation (TSF) is a patient-founded, patient-led organization dedicated to advancing rare neuroimmune research and supporting individuals affected by neuromyelitis optica spectrum disorder (NMOSD), myelin oligodendrocyte glycoprotein antibody disease (MOGAD), and related neuroimmune conditions.

Through our work across research, education, support, and advocacy, we partner with a global network of patient communities, clinicians, researchers, and industry partners to accelerate scientific progress and strengthen patient partnership across the rare disease ecosystem.

Acknowledgements

We extend our sincere gratitude to the patients and caregivers whose lived experiences, advocacy, and leadership continue to shape the evolution of patient partnership in research. We also thank the patient partners, clinicians, academic researchers, and community collaborators who contributed to this toolkit, developed by Alanna Yee, Leyla Herbst, and Michael Devlin as part of The Sumaira Foundation's research initiatives.

Disclaimer

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The views, statements, and opinions presented in this toolkit are the sole responsibility of The Sumaira Foundation and do not necessarily represent the views of PCORI®.

SECTION 01

BACKGROUND & CONTEXT

THE CASE FOR PATIENT PARTNERSHIP IN CLINICAL RESEARCH

Overview

Patient engagement and patient partnership are rapidly emerging as defining features of contemporary clinical research. People with lived experience are moving beyond traditional participant or limited advisory roles into positions where they actively shape how clinical research is conceptualized, designed, and implemented.

This section outlines the context driving this shift, including limitations of traditional research models, growing evidence of the impact of patient engagement, rising expectations from funders and regulators, and the evolving role of patient communities and patient advocacy organizations (PAOs) as partners across the research lifecycle.

CASE STUDY: THE SUMAIRA FOUNDATION (TSF)

In 2025, The Sumaira Foundation (TSF) secured $9 million in PCORI funding to lead a five-year international clinical trial in NMOSD. This achievement was made possible through foundational partnership infrastructure developed in the years prior.

A 2024 PCORI Engagement Award enabled us to build research capacity and strengthen the systems needed to support meaningful patient partnership in research. Through this initiative, we established a multi-stakeholder research council, trained patient partners in patient-centered research, and developed a community-driven research agenda for NMOSD and MOGAD.

This work was complemented by our existing organizational strengths, including our global Medical Advisory Board and a TSF Ambassador network spanning patients, caregivers, clinicians, and researchers across more than 35 countries.

Throughout this toolkit, we highlight elements of this experience as a case study to illustrate how intentional patient partnership can yield fundable research concepts, strengthened study design, and scalable partnership models capable of supporting large-scale research initiatives.

1.1 From Study Subjects to Research Partners

Limitations in traditional research approaches

Historically, clinical research has been designed and led primarily by clinicians and academic investigators, with patients engaged as study *participants* or subjects. Within this model, individuals enroll in studies, complete procedures, and contribute data essential to evidence generation, but have limited involvement in shaping research priorities, study design, or implementation strategies.

While this model has produced important scientific advances, it also carries limitations. Research questions may not fully reflect the lived burden of disease, and study protocols may overlook feasibility considerations such as symptom fluctuation, travel demands, caregiver responsibilities, or cognitive fatigue. These factors influence recruitment, retention, and participant diversity, shaping who is able to enroll and remain engaged through study completion. Outcome measures may also prioritize clinical or biomarker endpoints while underrepresenting impacts on daily functioning and quality of life, limiting the real-world applicability of findings.

Many of these challenges stem from how research has traditionally been structured. Investigators define priorities, develop protocols, and interpret outcomes largely through clinical and scientific expertise, with participants expected to fit within the systems created. Although methodologically rigorous, this model often lacks the experiential knowledge that patients and caregivers bring. Their day-to-day insight into living with health conditions can illuminate feasibility barriers, unmet needs, and outcome priorities that may be less visible with a clinical or academic lens alone.

The emergence of patient partnership

In recent years, there has been a growing shift toward recognizing patient partnership as a core component of high-quality study research. This is visible across the research ecosystem, including funders, academic institutions, biopharmaceutical sponsors, regulatory agencies, and health technology assessment bodies, all of whom are placing greater emphasis on integrating patient perspectives across the research lifecycle.

In practice, this means engaging patients earlier and more consistently, and in some cases integrating them into research team governance structures. Patients are contributing to priority setting, study design, and implementation planning, helping teams anticipate feasibility challenges and better align study goals with real-world participation and outcomes.

This shift reflects a broader recognition of the value of experiential knowledge. Patients navigate their health condition(s) every day, managing symptoms, treatments, healthcare systems, and the wider social and functional impacts of illness. When engaged as research *partners* rather than subjects, this perspective helps ensure studies remain methodologically rigorous while also being meaningful, feasible, and positioned to drive real-world impact. Research designed with patient partnership is more likely to generate outcomes that matter to affected communities and that translate more effectively into clinical care and health decision-making.

Exhibit 1: The Evolution of Clinical Research Practice

Research Dimension	Traditional Research Model	Emerging Partnership Model
Role of patients	Study participants / subjects	Research partners contributing experiential expertise
Research priorities	Defined by clinicians and investigators	Informed through patient partnership and priority-setting
Governance & decision-making	Investigator-driven governance structures	Shared governance through advisory boards, steering committees, and councils
Study design	Limited patient input	Protocols informed by lived experience and participation realities
Recruitment strategies	Institution-led outreach	Community-informed recruitment through PAO and partner networks
Compensation practices	Limited or inconsistent compensation	Structured compensation recognizing lived expertise

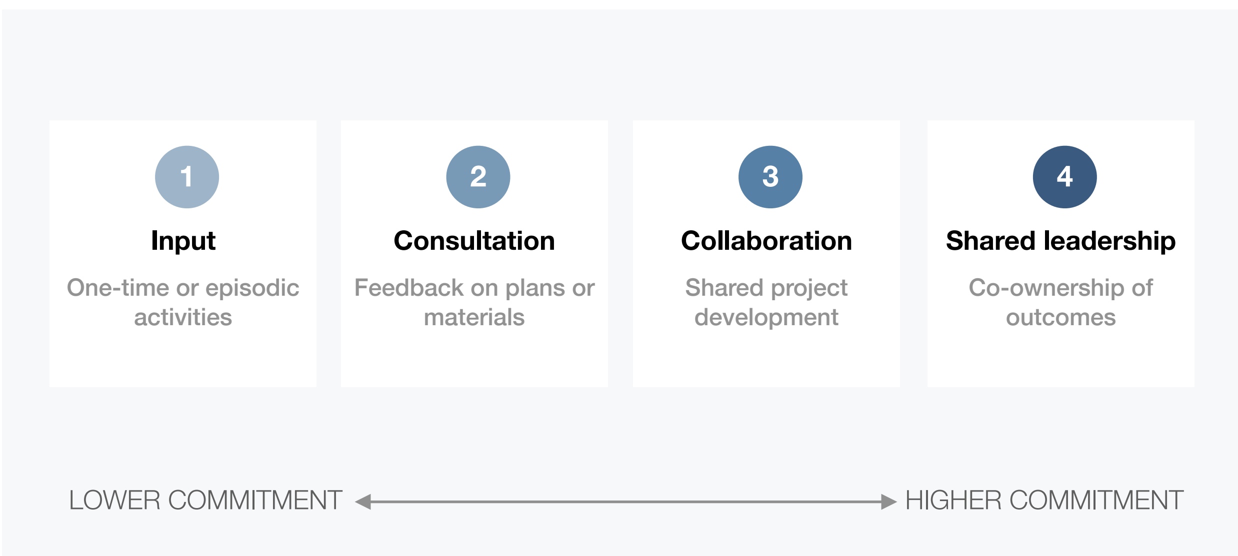
The spectrum of engagement

Patient engagement exists along a spectrum of involvement. While activities such as surveys or focus groups provide important input, they do not necessarily constitute *partnership.* Partnership implies shared influence, shared responsibility, and shared ownership of outcomes.

The appropriate level of engagement should be calibrated to the specific goals of the research (Exhibit 2). At one end of the spectrum, engagement may involve seeking *input* through one-further along, *consultation* may include offering feedback on draft materials or dissemination strategies. Deeper engagement occurs through *collaboration*, for example through co-developing materials, contributing to study design, or supporting implementation. At the highest level, *shared leadership* positions patients as equal partners in governance and decision-makers, shaping strategic direction and sharing responsibility for project outcomes.

Importantly, patient partnership does not replace scientific rigor or investigator leadership. Instead, it expands the expertise informing research. Understanding engagement as a spectrum helps research teams make intentional decisions about when, where, and how patient partnership can add the greatest value across the lifecycle of a study.

Exhibit 2: The Spectrum of Engagement



1.2 Evidence of Impact on Study Feasibility & Research Outcomes

Patient partnership impacts

The case for patient partnership is often framed in terms of values: patient-centeredness, inclusion and respect. While these principles matter, they do not fully capture why partnership is indispensable to research. Equally important is its impact on scientific quality.

Initiatives led by organizations such as the Patient-Centered Outcomes Research Institute (PCORI) demonstrate that meaningful patient engagement can strengthen the acceptability, feasibility, rigor and relevance of research studies, improving elements such as:

- How feasible study protocols and recruitment criteria are in real-world settings
- Selection of outcomes that matter most to patients
- Clarity, accessibility, and cultural sensitivity of study materials
- Trust between research institutions and patient communities
- Dissemination and translation of research findings into real-world practice

Patient engagement is increasingly recognized as more than symbolic or optional, with impact that can be seen across the research lifecycle. However, the value generated depends heavily on how engagement is approached.

Moving beyond compliance

When treated primarily as a compliance exercise, engagement tends to become performative. Activities are completed, documented, and reported, but generate limited operational or scientific value relative to the effort invested. Approached in this way, engagement can feel disproportionately burdensome, both for research teams and for patient partners, because it generates limited operational or scientific value relative to the time invested.

Genuine partnership, by contrast, creates compounding returns. Patient partners who feel respected, valued and meaningfully integrated often develop a strong sense of shared investment in the success of the project.

Over time, they become:

- *Advocates* who build credibility and trust within patient communities
- *Problem-solvers* who help troubleshoot recruitment and retention challenges
- *Protocol advisors* who surface feasibility barriers early
- *Bridges to community networks* that support outreach and enrollment

While the effort required to build meaningful patient partnerships is front-loaded, the operational, scientific, and translational benefits accumulate over time. This mitigates risk, optimizes study execution, and increases the likelihood of sustained, real-world impact.

Table 3: Levels of Patient Engagement and Their Impact on Research Design & Execution

Engagement Level	How Patients Are Engaged	Primary Influence on Research	Resulting Value to Study Execution
Input	One-time activities such as surveys, focus groups, or listening sessions	Early insight into patient experiences and priorities	Informational context to guide exploratory thinking
Consultation	Feedback on draft protocols, study materials, or dissemination strategies	Refinement of materials, communication approaches, and feasibility considerations	Improved clarity, accessibility, and participant experience
Collaboration	Ongoing partnership in study design, implementation planning, and project activities	Protocol development, recruitment approaches, operational planning	Strengthened feasibility, recruitment, and retention performance
Shared Leadership	Integration into governance, steering committees, or co-investigator roles	Strategic direction, priority setting, and study oversight	Sustained partnership infrastructure and translational impact

The necessity of patient partnership in rare disease research

Rare conditions present a distinctive research context in which patient partnership is not merely beneficial but essential to feasibility and success. Small, highly connected patient populations mean recruitment viability, participant retention, and long-term research sustainability are closely tied to how institutions engage and are perceived within the community.

The conditions themselves introduce additional complexity. Diseases such as NMOSD and MOGAD involve unpredictable relapses that can abruptly alter a patient's capacity to participate. A patient who enthusiastically commits to an advisory board may experience a severe attack that limits mobility, cognition, or stamina for extended periods. Study protocols designed without patient input may inadvertently exclude the very populations you need to reach—such as travel requirements that are difficult for individuals with mobility impairments, or cognitive demands that may not align with brain fog or fluctuating neurological symptoms.

1.3 Funder & Regulatory Expectations

Funder requirements

As patient partnership becomes more embedded across the research ecosystem, expectations for engagement are becoming increasingly formalized.

Major research funders increasingly require evidence of meaningful patient engagement within grant applications, study design, and governance structures. Organizations such as the Patient-Centered Outcomes Research Institute (PCORI), the National Institutes of Health (NIH), Horizon Europe, and other international funding bodies have embedded patient partnership expectations into funding criteria, evaluation metrics, and reporting requirements.

Regulatory integration

Regulatory agencies – including national and regional bodies responsible for approving therapies and providing regulatory oversight of clinical research, such as the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) – are also placing increasing emphasis on incorporating patient input into regulatory decision-making. This includes engagement in clinical trial design, endpoint selection, and benefit–risk assessment, as well as the growing use of patient preference data, patient-reported outcomes, and lived experience insight to inform regulatory decision-making.

1.3 Understanding Patient Communities & Advocacy Organizations

Contextualizing patient experience in research

Effective partnership begins with understanding the context patients bring into research environments – not just their symptoms, but their histories, their concerns, and the context that shapes how they experience research participation. Rare neuroimmune patients often enter research spaces with backgrounds that differ meaningfully from those of individuals living with more common conditions.

Lived experience and experiential knowledge

Many rare neuroimmune patients spent months or years navigating evolving symptoms, multiple specialist consultations, and periods of clinical uncertainty before receiving an accurate diagnosis. During this process, some may experience misdiagnoses, delayed recognition, or attribution of symptoms to psychological or nonspecific causes.

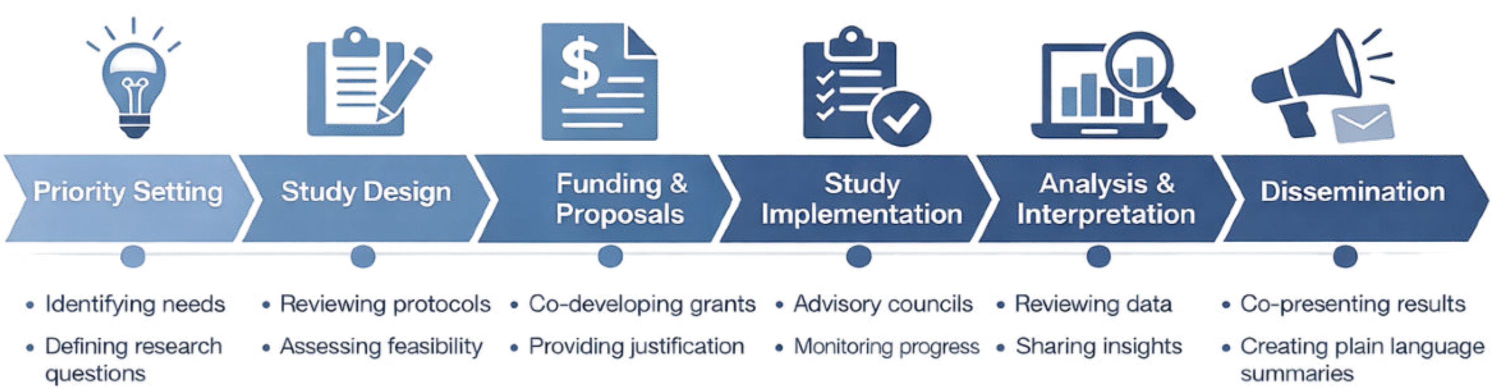
These experiences shape how patients engage with research and medical institutions. Many bring both respect for clinical expertise and a firsthand awareness of its limitations, informed by their lived experience. This can surface as thoughtful questioning, detailed lived insight, and a strong desire for research that advances understanding of their condition. When engaged constructively, this perspective can strengthen study design discussions, feasibility planning, and interpretation of findings. Researchers who acknowledge uncertainty transparently and engage patients as knowledgeable partners tend to build deeper trust and more productive collaborations.

The role of patient advocacy organizations (PAOs)

Researchers may be most familiar with PAOs through their roles in patient education, awareness campaigns, or support services. But they are increasingly playing a role in bridging patient communities and research ecosystems. This positions them a strategic partners in facilitating research participation and partnership, particularly due to their deep, trust-based relationships within patient communities built over years of engagement

PAOs may support priority-setting, partner identification, recruitment outreach, dissemination, and interpretation of findings. They often hold community-level insight, engagement infrastructure, and communication channels that researchers cannot easily replicate independently. Partnering with PAOs enables research teams to engage patients more credibly, inclusively, and efficiently while strengthening alignment between study objectives and community priorities.

Exhibit 4: Patient Partnership Across the Research Lifecycle



SECTION 02

GETTING STARTED

PREPARING RESEARCH TEAMS FOR PARTNERSHIP

Overview

Many research teams already hold strong foundations for patient partnership, including clinical expertise and established research infrastructure. As you move from traditional study models toward structured partnership, it is important to put dedicated processes and supports in place to ensure engagement efforts are both feasible and meaningful.

This section provides practical frameworks and reflection tools to help you clarify research partnership objectives, assess institutional readiness, scope priority initiatives, and plan the resources needed for you to engage patient partners effectively within research activities.

2.1 Clarifying Partnership Objectives

Why goal clarity matters

Clarifying partnership objectives early helps ensure patient engagement activities are purposeful, strategically aligned, and positioned to meaningfully inform research. Research teams may identify multiple opportunities for partnership, but prioritizing where engagement will have the greatest impact helps prevent tokenistic involvement or partnership that exceeds available capacity or infrastructure.

Exhibit 5: Informing Research Objectives Through Patient Engagement

Objective	Engagement Activities	Outputs Generated	Research Application
Community insight capture	Listening sessions, community discussions, focus groups	Lived experience insight	Identifies unmet needs and participation realities
Structured data collection	Surveys, needs assessments, symptom mapping	Quantified patient experience data	Supports feasibility assessment and hypothesis development
Consensus development	Delphi panels, advisory councils, working groups	Prioritized research questions	Informs research agendas and focus areas
Grant & study integration	Protocol workshops, co-development sessions	Partnership-informed study concepts	Strengthens feasibility, relevance, and funder alignment

CASE STUDY: THE SUMAIRA FOUNDATION (TSF)

Since 2014, The Sumaira Foundation (TSF) has engaged the global NMOSD and MOGAD communities through education, advocacy, and research initiatives. our TSF Ambassador network, Coalition for Health Equity, TSF Spark Research grants, and other programs, recurring lived experience insights emerged around diagnostic delays, care access, treatment burden, and unmet research needs. TSF's PCORI Engagement Award translated these insights into a patient-centered NMOSD and MOGAD research agenda, demonstrating how structured patient engagement can inform and guide mult-stakeholder research objectives.

Aligning internally before engaging externally

Before initiating partnership activities, it is important to ensure alignment within the research team and institution. Clarifying the intended purpose, scope, and expectations of engagement helps establish shared understanding and prevents misalignment once patient partners are involved.

Exhibit 6. Reflections to Support Internal Alignment

Consideration	Reflection Questions	Why It Matters
Research alignment	How does patient partnership support our study objectives or research program priorities?	Ensures engagement strengthens, rather than diffuses, scientific focus
Study & program integration	How will patient partnership inform study design, implementation, or dissemination?	Promotes intentional integration rather than ad hoc consultation
Leadership & team buy-in	Do investigators and study staff share understanding and support for partnership activities?	Enables coordinated implementation and realistic workload planning
Partner expectations	How will we communicate roles, scope, and influence to patient partners?	Builds transparency and trust from the outset

2.2 Assessing Institutional Readiness

Understanding your starting point

It is not necessary to have every resource in place before initiating patient partnership activities; many research teams build infrastructure iteratively as experience grows. At the same time, meaningful partnership introduces real operational demands, including staffing, coordination, training, and administrative oversight.

Taking stock of your institutional readiness helps research teams leverage existing assets while planning for the support needs to engage patient partners effectively over time.

Exhibit 7. Assessing Capacity For Patient Partnership

Capacity Area	Signals You May Need Additional Support
Staffing & leadership time	Engagement responsibilities are added on top of existing roles without dedicated time or ownership
Partnership coordination	Communication, scheduling, and follow-up with partners feel ad hoc or difficult to sustain
Training & preparation	Study staff or partners feel unsure about engagement processes or expectations
Administrative infrastructure	Contracts, compensation, or documentation processes are unclear or time-intensive to manage
Financial resources	Engagement costs are not yet build into study budgets
Sustainability	Participation depends on short-term enthusiasm rather than institutional planning

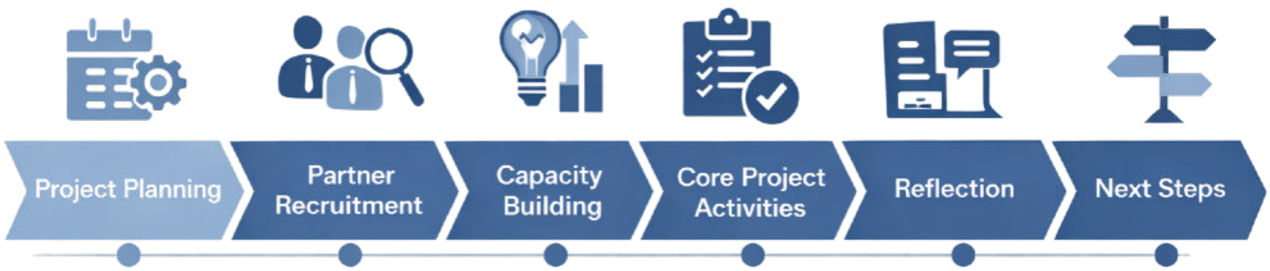
2.3 Integrating Patient Partnership into Study Design

Planning your project

Once your patient partnership objectives are identified, the next step is translating them into an actionable plan. This includes defining engagement objectives, establishing milestones and deliverables, identifying coordination needs, and determining roles and responsibilities across the study team.

While partnership approaches vary, many follow similar operational phases—from early planning and partner recruitment to onboarding, capacity building, core engagement activities, dissemination, and reflection.

Exhibit 8. Typical Operational Phases of Engagement Initiatives



Involve patient partners early as you plan your project. Early engagement allows partners to shape foundational elements – including protocol feasibility, outcome selection, and recruitment strategies – before key decisions are finalized.

We recommend capturing your plan in a plain language project summary that patient partners can return to throughout the project. This shared reference point helps keep everyone aligned on purpose, scope, roles, and timeline, and makes it easier to course-correct early if expectations start to drift.

Engaging patients early in study development

The most common mistake is involving patients only after key decisions have been made. You've designed the study, written the protocol, perhaps even received IRB approval – and now you want patient input. At this stage, meaningful changes are difficult, and patients recognize that their role is to validate decisions rather than shape them.

Early partnership, by contrast, allows patient insight to influence fundamental choices: which outcomes to measure, what burden the protocol places on participants, how to frame recruitment materials, what the findings will mean for the community. These early decisions lock in constraints that are costly to change later. Patient input is most valuable precisely when you have the most flexibility to act on it.

Working with IRBs

Institutional Review Boards (IRBs) vary in how they interpret patient engagement activities. Clarify your IRB's requirements before you've promised patients a role you cannot deliver.

When frame engagement activities, distinguish between study *participants* and patient *partnership*. Patient partners reviewing recruitment materials are collaborators contributing to research quality, not human subjects generating data. This distinction matters for IRB purposes, and muddying it creates unnecessary complications. Where needed, existing engagement guidance from organizations such as PCORI can support IRB discussions around compensation, roles, and governance.

2.4 Planning & Budgeting for Patient Engagement

Resourcing partnership activities

A common oversight is under-budgeting for patient engagement. Partnership costs should be planned proactively rather than retrofitted once other budget items are established.

Consider partner compensation, capacity training, coordination needs, accessibility supports, and dissemination involvement when developing study budgets. Early resourcing ensures partnership activities are feasible, equitable, and sustainable. Refer to Section 4.2 for more detailed guidance.

Capacity development and training supports

Effective partnership requires investment in capacity development for both patient partners and research teams. Patients may benefit from orientation to research processes, study design fundamentals, governance structures, and the specific scientific context of the initiative. This preparation supports confidence, clarity of role, and more meaningful contribution over time.

Research teams also benefit from training in patient partnership practices, including facilitation approaches, accessible communication strategies, and working within shared decision-making environments. Building this mutual readiness establishes a common language, clarifies expectations, and strengthens collaboration across stakeholder groups throughout the research lifecycle

Refer to section 3.4 for more detailed guidance.

SECTION 03

BUILDING PARTNERSHIPS

STRUCTURING PATIENT PARTNERSHIP WITHIN RESEARCH TEAMS

Overview

Effective patient partnership extends beyond engaging individual patients alone. While lived experience perspectives remain foundational, high-functioning partnership models operate at the intersection of experiential insight, clinical expertise, and research methodology.

This section focuses on how research teams can assemble multi-stakeholder partnership structures, define meaningful roles, cultivate trust-based collaboration, and support patient partners to contribute confidently within research environments.

3.1 Identifying & Engaging Patient Partners

Balancing experiential knowledge & technical expertise

Patient partnership is most effective when experiential insight and scientific expertise are integrated rather than siloed. Research teams with a diverse range of patients, caregivers, clinicians, academic investigators, and other stakeholders bring complementary perspectives on disease burden, care delivery, feasibility, and implementation realities. Bringing this expertise together strengthens the design and execution of research by ensuring initiatives are informed by both lived experience and methodological rigor.

Exhibit 9. Complementary Expertise in Patient-Partnered Research

Patients & Caregivers	Clinicians & Researchers
Lived experience with the condition	Scientific and methodological expertise
Insight into unmet needs and care gaps	Understanding of diagnostic and treatment pathways
Perspectives on treatment realities and quality of life	Study design and endpoint development expertise
Awareness of participation barriers and accessibility needs	Knowledge of feasibility, safety, and regulatory considerations
Community trust and connection	Access to clinical populations and research infrastructure

Direct recruitment pathways for patient partners

Direct recruitment pathways may include:

- Clinical site referrals
- Existing study participants
- Institutional patient advisory boards
- Registries and natural history studies
- Community outreach events

These approaches can be effective for identifying individuals with relevant lived experience. However, direct recruitment alone may present limitations, including restricted diversity of perspectives, limited community reach, or insufficient infrastructure to support sustained engagement.

The single patient problem

Recruiting one patient representative to a large team of researchers creates structural imbalance that good intentions cannot overcome. That patient faces pressure to speak for an entire community, disagree with multiple experts simultaneously, and contribute without backup when they're outnumbered ten to one. Even the most confident patient partner will self-censor in this environment.

Multiple patient partners create safety in numbers. They can validate each other's perspectives, push back collectively, and offer diverse viewpoints that no single person could provide. The minimum for genuine partnership is two patient partners; three or more is better. If your budget or timeline cannot accommodate multiple partners, that's a signal to reconsider the structure of your engagement.

Partnering through PAOs

PAOs offer established partnership infrastructure that can complement or extend direct recruitment pathways. Rather than recruiting individual partners ad hoc, research teams can engage through organizations that already convene, train, and support patient communities.

PAOs may provide:

- Pre-engaged patient partner networks
- Experience supporting advisory councils and engagement initiatives
- Community trust and established communication channels

- Infrastructure for recruitment, onboarding, and facilitation
- Insight into community priorities and unmet needs

Engaging through PAOs can streamline partnership development, reduce recruitment burden on study teams, and strengthen alignment between research activities and community priorities.

Designing diverse & representative teams

As partnership structures are assembled, it is important to consider whose perspectives are represented and whose may be missing. Patient experiences vary across disease stage, geography, culture, race and ethnicity, socioeconomic context, disability, and healthcare access.

Ensuring diversity of lived experience strengthens the relevance and applicability of research outputs and helps avoid over-reliance on the perspectives of individuals who are most visible or easiest to engage

Exhibit 10. Reflections to Support Building Diverse & Representative Teams

Consideration	Reflection Question	Practical Strategies
Representation	Does our partner group reflect the diversity of the populations we aim to study?	Recruit across geographies, care settings, and demographics
Range of lived experiences	Are multiple disease stages and care journeys represented?	Include both newly diagnosed and long-term patients
Caregiver perspectives	Are caregivers included where disease burdens affect family systems?	Engage family partners to broaden insight
Skills and readiness	Do partners feel prepared to contribute in research settings?	Provide onboarding, orientation, and mentorship
Equitable outreach	Are we reaching populations facing participation barriers?	Partner with community organizations and PAOs

Refer to Section 4.1 for recruitment implementation guidance.

Selecting collaborators for partnership readiness

When identifying clinician, researcher, or stakeholder collaborators, consider their prior experience working within patient-partnered environments and their openness to integrating experiential knowledge into research processes.

Effective collaborators communicate in accessible language, engage patient partners as peers within research dialogue, and value lived experience as a complementary form of expertise. Early partnerships often benefit from engaging collaborators who already demonstrate alignment with patient-centered research principles.

3.2 Clarifying Roles & Expectations

Structuring meaningful participation

Convening partners is only the first step; meaningful collaboration requires intentional structuring from the outset. This includes clarifying responsibilities, defining expected contributions, and establishing how decisions will be made throughout the project lifecycle.

How patient partners are positioned within this structure matters. If involvement is limited to reviewing materials or providing feedback after key decisions have already been made, opportunities for stronger research outcomes are lost. Involvement in priority-setting, protocol development, material design, and dissemination strengthens both research quality and partnership depth.

Establishing role clarity early supports coordination, accountability, and sustained engagement across stakeholder groups.

Refer to Section 4.3 for guidance on designing governance and leadership structures.

Avoiding tokenism

Tokenism refers to the inclusion of patient partners in ways that are symbolic rather than substantive. In these instances, patient involvement tends to be reduces to a procedural requirement or reputational signal rather than an integrated component of research design and decision-making. Tokenistic engagement undermines trust, represents a poor use of partner time and expertise, and limits the scientific and implementation value partnership is intended to bring.

Signs that engagement may be tokenistic include:

- Involvement after key decisions are already finalized
- Having a single patient representative on a large team
- Lack of compensation
- Soliciting input that does not actually influence outcomes.

Meaningful engagement requires structural commitment:

- multiple patient partners (not one isolated voice)
- engagement throughout the research lifecycle (not just recruitment)
- clear mechanisms for patient input to shape decisions
- transparent communication about how input was used—including when it wasn't followed and why.

Recognizing contributions and authorship

Authorship and acknowledgment considerations should be discussed early when defining partnership roles. When patient partners contribute to engagement outputs – such as resource development, priority-setting activities, data collection, communications materials, or dissemination efforts – their contributions should be appropriately recognized.

Clarifying expectations around credit, authorship criteria, and acknowledgment practices helps ensure contributions are recognized equitably and prevents misunderstandings as projects evolve.

TSF CASE STUDY

TSF established a multi-stakeholder research council composed of patients, clinicians, and researchers. Roles and expectations were defined from the outset, with members co-developing community surveys, collaborating on research priority-setting activities through a modified Delphi survey process, supporting translation of materials into multiple languages, building consensus around the NMOSD and MOGAD research agenda, and authoring plain language communications. Council members participated in facilitated meetings and collaborative work sessions tied to specific outputs. This structure positioned patient partners not only as contributors, but as active collaborators and leaders while building confidence and skills for future research partnership.

3.3 Addressing Power Dynamics in Research Teams

Structuring inclusive participation

Multi-stakeholder research partnerships bring together people with differing expertise, professional backgrounds, institutional authority, and familiarity with research environments. It is important to recognize that these differences can shape how comfortable people feel participating in discussions and decision-making processes.

Clinicians and researchers may be more accustomed to technical discourse and formal meeting structures, whereas patient partners may be newer to research terminology or group dynamics in academic settings. Structuring discussions to reduce these barriers supports more balanced participation.

Simple structural choices – such as pacing discussions, clarifying terminology, explicitly inviting patient perspectives, and ensuring space for reflection and questions – can help reduce participation barriers and support more equitable contribution across the group.

Fostering trust and inclusive dialogue

Addressing power dynamics is closely linked to building trust within research partnerships. Trust develops when partners feel their perspectives are respected, taken seriously, and reflected in decision-making. Fostering reciprocal relationships grounded in co-learning, transparency, honesty, and trust can also help balance inherent power differences across the group.

Creating an approachable and productive collaboration environment requires consistency in communication, openness to differing viewpoints, and a shared commitment to mutual learning across stakeholder groups. Thoughtful coordination and facilitation play an underestimated but central role. Simple practices such as inviting patient perspectives early in discussions, using accessible language, and creating space for reflection or small-group dialogue can support more balanced participation and ensure conversations are not dominated by academic or institutional voices. Approaches such as moderated discussion rounds, breakout groups, or guided reflection can further help ensure contributions are not limited to those most comfortable speaking in professional or technical settings.

Facilitators help shape how dialogue unfolds. Establishing shared group values – including respect for diverse perspectives and openness to challenge – creates space for constructive disagreement and deeper insight. Over time, these practices strengthen trust, improve decision-making, and support more effective multi-stakeholder collaboration.

Exhibit 11. The Engagement–Trust Feedback Loop

3.4 Developing & Supporting Patient Partners

Trauma-informed approaches

Rare diseases often involve sudden onset, diagnostic uncertainty, and potential health instability. As a result, research participation may surface difficult experiences related to illness, healthcare interactions, or loss of functioning. Trauma-informed engagement refers to partnership practices that recognize these realities and intentionally design participation environments that prioritize psychological safety, autonomy, and flexibility.

Trauma-informed approaches include:

- Recognizing that some partners may have had negative experiences with the healthcare system
- Creating participation environments where individuals are comfortable sharing as much or as little personal experience as they choose, and in varying formats (e.g. live group setting or anonymous written feedback)

- Avoiding triggering language, imagery, or scenarios in research materials and discussions
- Building flexibility into engagement expectations to accommodate fluctuation and health variability

Building research literacy and engagement skills

Patient partners bring critical lived experience and experiential knowledge, offering insight grounded in real-world care realities and community priorities. At the same time, many may be newer to research concepts and processes. Building research literacy – including familiarity with study design fundamentals, terminology, and evidence interpretation – supports more confident and substantive engagement.

Creating structured opportunities for skill development further supports the growth of informed, confident partners equipped to help guide research initiatives and represent community priorities. Capacity-building may include foundational orientation to research processes and study design, alongside skill-building in areas such as document review, meeting participation, public speaking, and collaborative decision-making.

Supporting leadership pathways and peer mentorship

Considering the motivations, skills, and strengths of partners can help identify pathways to deepen partnership over time. Cultivating a broad base of capable patient partners – rather than relying on a small number of experienced advocates – strengthens long-term research infrastructure and institutional capacity.

Capacity-building should be approached as an ongoing process rather than a one-time event, with learning integrated into meetings, supported through self-directed resources, and reinforced through peer mentorship models. Whenever possible, learning should be paired with immediate opportunities for application – for example, reviewing a study document after an orientation to protocol design, or practicing feedback techniques within live project discussions. Pairing newer partners with experienced members further supports confidence-building, knowledge transfer, and peer learning while creating space to translate new knowledge into practice.

Anticipating collaboration challenges and sustaining participation

Even thoughtfully structured partnerships will require adjustment as the work unfolds. Multi-stakeholder research initiatives operate within real-world constraints, with tight project timelines, competing priorities, and evolving partner availability. Approaching partnership with a mindset of responsiveness rather than rigidity allows teams to adapt as needs and circumstances emerge.

Participation may shift as patient partners balance engagement alongside health needs, caregiving responsibilities, employment, or other life demands. Similarly, the intensity of research activities may rise and fall across project phases, with some periods requiring more concentrated input than others. Building flexibility into how and when partners contribute, as well as planning for continuity across project phases, can help maintain stability even as individual involvement fluctuates.

Exhibit 12. Anticipating Partnership Challenges in Research Teams

Challenge	Potential Manifestation	Strengthening Approaches
Project pacing & timelines	Limited time for partners to review materials or provide feedback	Build in realistic input windows and clearly signal priority areas for partner feedback
Role clarity & scope of influence	Uncertainty about where input is expected or how it informs decisions	Define roles, decision points, and engagement expectations early and revisit throughout the project
Power dynamics in group settings	Academic or clinical voices unintentionally dominating discussions	Use facilitated dialogue, structured input methods, and inclusive meeting practices
Emotional labor of sharing lived experience	Fatigue or discomfort related to recounting health experiences	Offer flexibility, content advisories, and supportive facilitation approaches
Representation burden	Individuals feeling responsible for representing an entire community perspective	Clarify that partners are contributing perspective, not serving as sole representatives of the entire community
Sustaining participation over time	Engagement fluctuating due to health, caregiving, or employment demands	Offer multiple participation formats and plan for transitions to maintain continuity if involvement changes over time

SECTION 04

OPERATIONALIZING PARTNERSHIPS

IMPLEMENTATION IN CLINICAL RESEARCH PRACTICE

Overview

With the foundations of multi-stakeholder partnership established, this section turns to how patient partnership is implemented in practice. This includes embedding partnership into the operational workflows across the research lifecycle

While study designs vary, the day-to-day work of meaningful patient partnership tends to follow similar systems, processes, and coordination needs. This section outlines practical execution approaches that support effective, sustainable integration of patient partners within research teams.

4.1 Partner Recruitment & Onboarding

Inviting and onboarding patient partners

As outlined in Section 3.1, recruitment should be intentional and aligned to partnership objectives. Patient partners may be identified through clinical referral pathways, registries, patient advocacy organizations, advisory boards, or prior research participation.

Recruitment outreach should be clear, transparent, and grounded in assessing mutual fit. Early conversations are an opportunity to share the purpose of the work, why the individual's perspective is being sought, and what participation will involve.

Key considerations to communicate include:

- Anticipated time commitments
- Meeting cadence and duration
- Scope of decision-making influence
- Compensation and available participation supports

Recruitment dialogue should create space for prospective partners to ask questions, share motivations, and raise accessibility or capacity considerations. Establishing this clarity early supports alignment on both sides and lays the foundation for more sustainable, long-term participation.

TSF CASE STUDY

To recruit patient partners, we engaged members of our TSF Ambassador program: a community network of over 100 patients, caregivers, and clinicians who serve as peer leaders, educators, and regional connectors. Ambassadors support and lead awareness campaigns, support groups, and other initiatives across diverse geographies and care contexts. Engaging this network provided a meaningful starting point for identifying partners bringing lived experience alongside demonstrated commitment to partnership roles.

Planning for engagement continuity

Over multi-year projects, shifts in patient availability and engagement are expected. This may be due to health changes, caregiving demands, or evolving personal priorities. Planning for continuity – for example, by lightly over-recruiting advisory groups – helps maintain continuity if participation fluctuates.

4.2 Compensation & Participation Supports

Recognizing lived experience as expertise

Unlike clinicians or researchers, patient partners are usually not participating as part of their paid professional role. Engagement often requires stepping away from work, caregiving responsibilities, or personal time in order to contribute.

Without compensation, participation may unintentionally be limited to those who have the time, financial flexibility, or health stability to volunteer. Providing compensation that reflects fair market value affirms patient partnership as valued expertise rather than voluntary contribution.

PCORI's Financial Compensation Framework recommends aligning compensation for patient and caregiver partners with the time, effort, expertise, and responsibilities associated with their role. In practice, TSF has seen rates begin within the $50-100 per hour range, with higher levels for roles involving greater responsibility, preparation, or leadership.

Before designing compensation structures, it is important to consider how payments may intersect with disability or income-dependent public assistance benefits. Offering flexible options – or inviting patient partners to share preferences around compensation structure – can help support participation in ways that are responsive to individual circumstances.

Exhibit 13: Example Partner Compensation Models

Engagement Activity	Compensation Considerations
Advisory or council participation	Per-meeting stipends or term-based honoraria
Document review or feedback	Hourly compensation or flat review fees
Governance or leadership roles	Expanded stipends reflecting level of engagement (refer to Exhibit 3)
Conference presentations or dissemination activities	Speaker honoraria and travel coverage
Research collaboration (e.g., co-investigator roles)	Contracted compensation aligned with scope of work

Resourcing participation supports

Standard reimbursements and accessibility supports are equally critical to consider and discuss with partners upfront. These may include:

- Travel and lodging for in-person meetings
- Internet access, technology stipends, or software access,
- Accessibility accommodations like captioning, assistive technology, or mobility supports
- Translation or interpretation services
- Caregiving or respite reimbursement

Putting these supports in place and clearly communicating their availability helps create inclusive conditions where partners can engage meaningfully and participate on more equitable footing with other stakeholders.

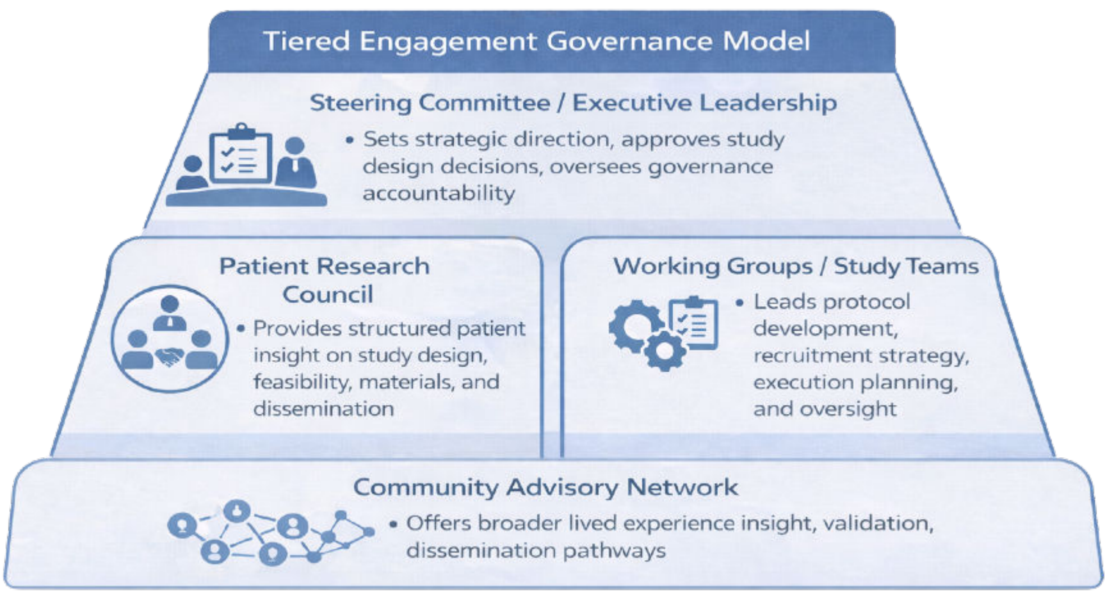
4.3 Governance & Leadership Structures

Embedding patient partners within formal structures

It is important to establish clear project governance/leadership structures early to ensure that patient partners are embedded within initiatives rather than positioned on the sidelines. Governance design should clarify where influence sits, how input informs decisions, and how accountability is distributed across stakeholders.

Many projects involve a tiered leadership model. This may include a core decision-making council, smaller working groups responsible for specific deliverables, and a broader community engaged for consultation or dissemination. Structuring engagement in this way allows patient partners to engage at varying levels of influence aligned to their skills, aptitudes, desires, and capacity.

Exhibit 14: Example Tiered Engagement Governance Model



Clarifying representation and authority

Governance planning should address:

- How patient representation is embedded across decision bodies
- Scope of authority and responsibility of each body
- Participation expectations and term structures
- Alignment with compensation and resourcing models

Establishing these structures early helps manage expectations and ensures patient perspectives are integrated into decision-making processes rather than layered on as an afterthought.

Integrating patient input into decision-making

Governance structures should clarify how patient partner input is incorporated into study decisions. While not all recommendations can be implemented, transparency regarding how perspectives were considered – and the rationale behind final determinations – reinforces trust and ensures engagement remains meaningful rather than symbolic. Establishing clear pathways for how input is reviewed, documented, and integrated helps partners understand where their contributions influence study direction and where constraints may shape final outcomes.

Conversely, soliciting input without demonstrating how it informs decisions can erode confidence in the partnership process. Engagement that is perceived as performative—or disconnected from tangible influence – risks being experienced as a poor use of partner time and expertise. Patient partners generally recognize that scientific, regulatory, feasibility, and resource considerations will limit the incorporation of some recommendations. What sustains trust is not universal adoption of input, but transparent communication about how perspectives were evaluated and why certain decisions were made. This clarity strengthens accountability and supports more durable, credible collaboration over time.

4.4 Meeting Planning & Inclusive Engagement Practices

Accommodating patients with variable health

Standard meeting practices often assume participants can commit to fixed schedules and remain engaged for extended periods. Rare disease patients may not be able to do either. Symptoms fluctuate unpredictably; a patient who confirmed attendance yesterday may be unable to participate today due to fatigue, pain, or cognitive fog.

It can be helpful to think of planning for engagement meetings as a three-step process: preparation, facilitation, and follow-up.

Exhibit 15: Health-Responsive Meeting Design

Health & Participation Consideration	Engagement Design Implications
Symptom fluctuation affecting attendance	Offer multiple meeting times, rotating schedules, and asynchronous participation options. Build flexibility into attendance expectations to account for health variability rather than treating absence as disengagement.
Fatigue and energy variability	Design shorter sessions, incorporate breaks, and avoid scheduling critical input discussions late in meetings or across back-to-back sessions.
Cognitive fatigue and processing load	Pace discussions thoughtfully, minimize dense real-time information processing, and allow time for reflection before input is requested.
Preparation needs due to health variability	Share materials in advance so partners can review content at their own pace and engage when cognitively or physically able.
Participation modality preferences	Offer camera-optional participation and multiple engagement formats (verbal, chat, written feedback) to support sustained involvement.
Care context and support needs	Allow caregiver presence where helpful to support communication, comprehension, or participation comfort.
Psychological and medical trauma considerations	Use trauma-aware framing, avoid distressing language or imagery, and allow partners discretion in how personal experiences are shared.

Preparing inclusive meeting environments

Engagement does not occur simply because patients are present; it occurs when collaboration is intentionally prepared for, facilitated, and followed up on in ways that enable all partners to contribute their expertise. Agendas that are overly technical, rushed, or dominated by academic voices can limit participation. In contrast, well-designed meetings create space for reflection, clarification, and dialogue, allowing patient insights to more directly inform research activities and decisions.

Exhibit 16: Meeting Preparation Considerations

Preparation Area	Key Questions	Practical Approaches
Scheduling & Timing	Are meeting times workable across health, caregiving, and work realities?	Consider variable partner needs when scheduling meetings, and explore options such as rotating or standing times to support participation.
Session Length & Pacing	Is the meeting length realistic for participant stamina and focus?	Limit duration, build in breaks, and prioritize high-value discussion items.
Participation Format	Can partners engage in ways that suit their capacity and preferences?	Offer virtual, in-person, and hybrid options; include opportunities for asynchronous participation where possible e.g., written feedback, surveys, document comments, recorded briefings
Advance Preparation & Context	Do partners have adequate time and context to prepare and engage in discussion?	Offer optional pre-meeting briefings or primers, particularly for new partners or technical topics. Share agendas, slide decks, and background materials in advance (ideally ≥72 hours).
Clarity of Requested Input	Is it clear what type of feedback or expertise is being sought?	Clarify whether the discussion is exploratory, feedback-focused, or decision-oriented so partners can prepare accordingly.
Language & Content Accessibility	Are materials understandable to non-technical participants?	Use plain language, define terminology, and provide glossaries or briefing notes to reduce jargon barriers.
Accessibility & Accommodation Planning	Have participation supports been proactively addressed?	Arrange captioning, translation, interpretation, disability accommodations, and confirm needs in advance.
Technology Readiness	Do partners have the tools and supports needed to participate fully?	Provide tech orientation, troubleshooting support, and alternative dial-in options.

Refer to Section 3.2 for more conceptual guidance on preparing for meaningful participation.

Facilitating inclusive dialogue in practice

Facilitation quality directly shapes partnership effectiveness. Skilled facilitation helps balance participation, encourages contribution from all partners, and ensures patient perspectives are actively surfaced and integrated into group discussion and decision-making.

Exhibit 17: Meeting Facilitation Considerations

Facilitation Area	Key Questions	Practical Approaches
Opening & Context Setting	Are partners oriented to the purpose of the discussion?	Begin with a brief overview of objectives, agenda flow, and where partner input is most needed.
Balanced Participation & Accessible Dialogue	Are all partners able to engage meaningfully in discussion?	Invite input across stakeholder groups, use round-robin prompts, define terminology in real time, and encourage clarifying questions.
Power Dynamics & Psychological Safety	Do partners feel comfortable sharing candid or dissenting perspectives?	Actively invite patient perspectives, validate contributions, and redirect discussion if certain voices dominate.
Breakout Discussions	Are smaller formats being used to support deeper participation?	Use breakout groups or paired discussions to create space for reflection and encourage input from quieter participants.
Engagement Methods & Participation Tools	Are varied formats being used to support different communication styles?	Incorporate live polls, guided reflection, chat input, icebreakers, or visual exercises to broaden participation.
Real-Time Synthesis & Capture	Are partner insights being captured and reflected accurately?	Summarize discussion themes, confirm interpretations, and reflect back key points before moving forward.
Facilitation Support Roles	Are team roles structured to support effective dialogue?	Assign facilitators, note-takers, timekeepers, and technical support to ensure discussions run smoothly.

Refer to Section 3.3 for more conceptual guidance on building trust-based, inclusive partnership environments.

Translating discussion into action

Follow-up is where partners determine whether their participation truly mattered. How insights are captured, communicated, and acted on after meetings shapes whether engagement feels meaningful or merely symbolic. Clear documentation, visible next steps, and transparent communication all play a role in sustaining momentum and reinforcing trust in the partnership process.

Follow-through also helps preserve what your institution is learning through engagement. Capturing not only decisions, but the discussions and reasoning behind them ensures that knowledge is retained over time. This makes partnership more sustainable, transferable to future work, and less dependent on any single staff member or partner remaining involved.

Exhibit 18: Meeting Follow-up Considerations

Follow-Up Area	Key Questions	Practical Approaches
Documenting Insights & Decisions	Were discussion themes, partner contributions, and decisions accurately captured?	Compile synthesized notes reflecting key insights, areas of consensus, open questions, and agreed decisions.
Capturing Additional Reflections	Were partners given space to share input not raised live?	Offer asynchronous follow-up channels such as surveys, written reflections, or email feedback opportunities.
Sharing Materials & Tracking Commitments	Do participants have access to meeting outputs and clarity on responsibilities moving forward?	Distribute slide decks, recordings, summaries, and supporting documents alongside action logs, assigned leads, deliverables, and timelines.
Archiving & Institutional Knowledge	Is engagement documentation preserved for continuity and learning?	Store notes, outputs, and engagement artifacts in centralized repositories to support onboarding, institutional memory, and long-term partnership infrastructure.
Sustaining Engagement Continuity	Do partners understand how their input informed direction and remain connected between meetings?	Share post-meeting recaps outlining decisions, rationale, and next steps. Provide interim updates and progress reports to sustain engagement and visibility into project momentum.

Refer to Section 3.4 for broader conceptual guidance on building long-term partnerships.

4.5 Continuous Engagement Between Study Milestones

Maintaining and growing momentum through feedback and reflection

Meaningful partnership requires ongoing attention between formal meetings. Without intentional touch points, collaboration can become episodic, reducing alignment, and making it harder to identify emerging needs as initiatives progress.

Continuous engagement creates space to understand how partners are experiencing the work, surface challenges early, and reinforce shared investment in the initiative. Building in opportunities for reflection and demonstrating openness to adjusting engagement approaches over time helps ensure partnership remains responsive, adaptive, and positioned for long-term success.

Exhibit 19: Continuous Engagement Considerations

Engagement Area	Key Questions	Practical Approaches & Tactics
Ongoing Partner Check-Ins	Are partners being supported between meetings?	Conduct periodic 1:1 or small-group check-ins to understand partner experience, workload, and emerging needs.
Partnership Feedback & Reflection	Do partners feel comfortable sharing candid feedback?	Use surveys, reflection prompts, pulse polls, and milestone discussions to gather input on meeting experience, communication, and partnership dynamics.
Adapting Engagement Approaches	Are partnership structures being refined based on partner input?	Use feedback to adjust not only meeting practices, but also project activities, priorities, and partnership approaches as the initiative evolves.
Sustaining Communication & Partner Value	Do partners remain informed, connected, and motivated?	Share interim updates, highlight how input is shaping decisions, acknowledge contributions, and celebrate wins between meetings.
Transition & Exit Learning	When partners step back, is learning being captured?	Conduct brief exit conversations to understand partner experience and identify improvements for retention and future recruitment.

SECTION 05

SUSTAINABILITY

MAINTAINING & SCALING PARTNERSHIPS

While effective meeting design, facilitation, and follow-up are essential to operationalizing patient partnership, longitudinal and sustained engagement involves additional planning and support. As partnership activity expands, research teams must consider how relationships are maintained, how engagement learning is preserved, and how partnership infrastructure is resourced to support continued collaboration

Sustainability emerges through the intentional cultivation of partnerships, knowledge infrastructure, and resourcing pathways that support engagement to persist, evolve, and scale in impact. This section outlines practical approaches for sustaining partnership momentum and embedding patient engagement as durable research infrastructure.

5.1 Sustaining Long-Term Partnerships

Strengthening trust, visibility, and continuity

Long-term partnership is reinforced when patient partners experience their contributions as valued, visible, and impactful. When engagement is structured meaningfully, partners are more likely to remain involved and to bring forward new ideas, networks, and collaboration opportunities that strengthen research initiatives over time.

Recognizing contributions through authorship, co-presentation, advisory roles, and public acknowledgment supports sustained engagement while advancing patient partner leadership within research environments. These practices reflect equitable partnership and affirms the value of experiential expertise within academic settings.

TSF CASE STUDY

As our partnership infrastructure strengthened through the course of our PCORI Engagement Award, we worked with partners to identify funding opportunities aligned with community-defined priorities. When PCORI released a targeted rare disease comparative effectiveness research (CER) funding opportunity, TSF executive including patient leaders worked alongside TSF's Medical Advisory Board and other collaborators to develop the proposal, integrating feedback from our multi-stakeholder research council to ensure the study concept, outcomes, and engagement approach reflected real-world needs. This groundwork ensured the application reflected both scientific rigor and lived experience perspectives, resulting in TSF securing $9 million to lead a five-year international clinical trial.

Partnership continuity systems

Sustaining partnership capacity requires investment in continuity structures. Maintaining partner registries, tracking areas of expertise and interest, and offering recurring engagement opportunities helps reduce reliance on episodic recruitment and enables partners to remain engaged across multiple initiatives. This continuity strengthens institutional memory, streamlines onboarding for new studies, and allows partners to deepen familiarity with research processes, expectations, and team dynamics.

Long-term sustainability also requires cultivating future patient research leaders. Training, mentorship, and progressively more complex engagement opportunities build partner capability while expanding institutional partnership capacity. Consider inviting experienced partners to serve as advisors, co-investigators, or peer mentors to newer partners. These leadership pathways strengthen continuity, distribute engagement responsibilities, and embed patient partnership as a durable component of research infrastructure

5.2 Scaling Partnership Across Research Portfolios

Embedding partnership within institutional systems and practices

As partnership activities expand, it is in a research team's best interest to embed engagement within institutional structures rather than relying on individual relationships alone. When partnership operates informally or project-by-project, it is harder to sustain, resource, and scale.

Institutional integration may include embedding patient partners within standing governance bodies, creating centralized engagement coordination functions, developing shared partner databases, and aligning partnership processes across study teams. Documenting facilitation practices, capturing partner feedback, and codifying engagement models supports replication across studies. Knowledge repositories and playbooks reduce the need to rebuild engagement infrastructure with each new initiative and contribute to continuous institutional learning. These approaches enable engagement to operate as a coordinated institutional function rather than a project-specific activity.

Continuously identifying evolving research priorities

Continue convening patients, caregivers, clinicians, and researchers to identify evolving unmet needs. The landscape changes constantly. New treatments emerge, patient priorities shift, research advances open new questions, and what was urgent five years ago may be less pressing today. Regular environmental scanning keeps your work relevant and responsive instead of locked into priorities that no longer reflect community needs. Identified priority gaps can inform pipeline development, feasibility assessments, and future grant positioning.

Use multiple methods to surface emerging priorities: focus groups with patients and caregivers, literature reviews, community surveys, ongoing council discussions. Look for gaps between what research is addressing and what patients say matters most. These gaps represent opportunities for work that others are not pursuing.

5.3 Leveraging Partnership for Funding & Impact

Demonstrating engagement impact

Funders and collaborators increasingly seek evidence that you are engaging in meaningful partnership approaches. Evaluating this through metrics such as recruitment outcomes, retention metrics, protocol modifications, partner satisfaction, and dissemination reach helps demonstrate the value of partnership infrastructure. These data can be leveraged in grant applications, institutional reporting, and collaboration discussions to substantiate engagement as a driver of research success.

Strengthening research competitiveness and collaboration pathways

Research teams with established partnership models may find that collaborators, sponsors, and funders increasingly seek to engage them based on demonstrated partnership capacity. Documenting and sharing engagement successes including through presentations, publications, and professional networks can strengthen institutional positioning within funding and collaboration ecosystems.

Integrating partnership into proposal development

Patient partnership is most impactful when it begins at the proposal stage rather than limited to study execution. Engaging partners early in concept development helps ensure that research questions, outcomes, and study procedures reflect real-world priorities and feasibility considerations.

Early partnership also strengthens partner investment in the project's success and fosters a sense of shared ownership deliver patient-centered research in practice, which often enhances proposal competitiveness.

Exhibit 20. Sustaining & Scaling Partnership Infrastructure

Infrastructure Area	Key Questions	Practical Approaches & Tactics
Knowledge Continuity & Field Leadership	Is partnership learning being retained, shared, and extended across studies?	Document engagement processes, decisions, and outputs in centralized repositories. Pair experienced partners with newer members to transfer knowledge. Translate learnings into trainings, advisory roles, and peer support for other organizations.
Visibility & Evidence Dissemination	Are partnership activities and outcomes visible and contributing to the broader field?	Publish engagement methods, share webinars, reports, newsletters, toolkits, conference presentations, and publications to communicate impact and sustain engagement interest.
Priority Alignment	Are emerging partner and community needs being identified?	Conduct advisory discussions, consensus initiatives, and landscape assessments.
Research Integration	Is partnership embedded across broader study portfolios?	Integrate partners into design, feasibility, recruitment, and dissemination activities.
Network & Collaboration Growth	Are external collaborators engaged in partnership models?	Build relationships with advocacy groups, research networks, and health system partners to support joint initiatives and shared learning.
Resourcing & Funding Pathways	Are resources in place to sustain and grow patient partnership work?	Include engagement costs in grants; pursue partnership-focused funding.
Institutional Capacity	Does the research have the infrastructure to support scale?	Assess staffing, governance, systems, and leadership capacity; invest in operational supports and professional development to sustain expansion.

CLOSING REFLECTIONS

THE FUTURE OF PATIENT PARTNERSHIP

The learnings captured in this toolkit reflect the ongoing work of embedding patient partnership within clinical research practice. What often begins as a single engagement initiative can evolve into sustained relationships, strengthened partnership infrastructure, and expanded opportunities for research impact.

As this evolution continues, research teams are increasingly recognizing that patient partnership is not ancillary to scientific work, but integral to how research is conceived, designed, and implemented. Investigators, institutions, and sponsors are moving beyond episodic consultation toward models in which patient insight informs governance structures, study design, feasibility planning, and dissemination. This shift is becoming more visible across the research landscape, with patient partnership shaping not only individual studies but broader research ecosystems.

At The Sumaira Foundation (TSF), this evolution is being operationalized through practice-based partnership. Research engagement efforts initiated in 2024 have focused on building partnership infrastructure, training patient partners, and co-developing a research agenda for NMOSD and MOGAD. This groundwork directly supported TSF in securing $9 million to lead a five-year international clinical trial funded by PCORI, with TSF serving as study sponsor and leading trial coordination while embedding patient partnership throughout implementation.

This progression illustrates how sustained investment in patient partnership can strengthen research infrastructure and support more effective and equitable health systems. When lived experience is systematically integrated alongside technical expertise, research becomes more relevant, more responsive, and more capable of generating meaningful real-world outcomes.

Sustaining this momentum will require ongoing collaboration across patient communities, clinicians, researchers, funders, policymakers, industry partners, and beyond. No single individual or organization advances this work alone. Progress emerges through shared learning, durable partnership, and a collective commitment to ensuring research reflects the needs, priorities, and lived experience of the communities it is intended to serve.

Looking for additional support?

The Sumaira Foundation continues to build research partnership infrastructure, train patient leaders, and advance multi-stakeholder collaboration across the field of rare diseases. To learn more about collaboration opportunities, visit sumairafoundation.org or email contact@sumairafoundation.org.

REFERENCES

REFERENCES & ADDITIONAL RESOURCES

Clinical Trials Transformation Initiative. (2019). Patient Group Organizational Expertise and Assets Evaluation Tool.

Israel T, Farrow H, Joosten Y, Vaughn Y. Community Engagement Studio Toolkit 2.0.

Patient-Centered Outcomes Research Institute. (2018). The value of Engagement. Available at: pcori.org/engagement/value-engagement

Patient-Centered Outcomes Research Institute. (2015). Financial Compensation of Patients, Caregivers, and Patient/Caregiver Organizations Engaged in PCORI-Funded Research.

Zimmerman EB, Cook SK. The SEED Method Toolkit for Stakeholder Engagement in Question Development and Prioritization. Richmond (VA): Virginia Commonwealth University; 2017.

PARADIGM. (2020). Recommendations on the required capabilities for patient engagement.

Domecq JP, et al. Patient engagement in research: a systematic review. BMC Health Services Research. 2014;14:89.

Forsythe LP, et al. Patient and stakeholder engagement in the PCORI pilot projects: description and lessons learned. Journal of General Internal Medicine. 2016;31(1):13-21.

Concannon TW, et al. A new taxonomy for stakeholder engagement in patient-centered outcomes research. Journal of General Internal Medicine. 2012;27(8):985-991.

Frank L, et al. Conceptual and practical foundations of patient engagement in research at the patient-centered outcomes research institute. Quality of Life Research. 2015;24(5):1033-1041.

Sheridan S, Schrandt S, Forsythe L, Hilliard TS, Paez KA; Advisory Panel on Patient Engagement (2013 inaugural panel). The PCORI Engagement Rubric: Promising Practices for Partnering in Research. Ann Fam Med. 2017 Mar;15(2):165-170.

Harrison JD, Auerbach AD, Anderson W, et al. Patient stakeholder engagement in research: A narrative review to describe foundational principles and best practice activities. Health Expect. 2019; 22: 307–316.

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