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Early Patient Engagement in Drug Development: Guiding Principles for Meaningful Collaboration

Bristol Myers Squibb®

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Foreword

Despite broad recognition of the value of patient, care partner, and advocacy input in research, engagement often occurs late or without a clear connection to early scientific and development decisions. Perspectives may be gathered after key assumptions are set or operational parameters and feasibility constraints are already in place, limiting their ability to influence decisions and outcomes.

Closing this gap requires more than intent. It requires consistent expectations for when engagement occurs, how insights inform decisions, and how collaboration is sustained across the research and development lifecycle. Early engagement is not a standalone activity. It is an ongoing practice that strengthens development quality, feasibility, and relevance when applied systematically.

At Bristol Myers Squibb (BMS), this has meant establishing a clear structure for when and how patients, care partners, and patient advocacy groups are engaged from early stages. Grounding decision-making in lived experience strengthens development decisions and requires sustained partnership with clear follow-through on how insights are used.

This white paper reflects a collaborative effort between BMS teams and patient advocacy leaders, who co-authored the work, to define what early engagement looks like in practice and how it can be applied in routine development. The intent is to provide a practical reference that helps translate patient experience into research decisions and practices that are meaningful and durable.

Momentum around early engagement continues to build across industry, advocacy, and regulatory communities. As expectations evolve and experience grows, early engagement is increasingly recognized as a core capability for responsible and effective research. The opportunity now is to move from intention to consistent implementation.

Josée Pelletier, Executive MBA
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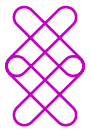
Executive Summary

Embedding lived experience early in research and drug development is increasingly recognized as essential to developing treatments that are relevant, feasible, and equitable. When engagement occurs late in development, barriers related to participation burden, access, and representation may be more difficult to address.

Patients and care partners provide direct insight into the daily realities of disease and treatment. Patient advocacy groups contribute aggregated, community-informed insight and practical expertise that can help anticipate feasibility challenges, inform more inclusive trial design, and strengthen recruitment and participation approaches. These contributions are most effective when integrated alongside scientific, clinical, and operational evidence.

This white paper reflects the outcomes of a workshop that brought together BMS leaders, patient advocacy groups, and cross-functional partners to align on how early engagement should function in practice and how roles and expectations can be clarified across stakeholders. The outputs from this collaboration are intended to guide a consistent and experience-informed approach to embedding early engagement across the research and development lifecycle.

In this paper, early engagement refers to structured, ongoing collaboration that begins during preclinical research and continues through Phase 3 clinical development. The paper presents guiding principles for early engagement, organized into three domains, each further defined by three core components. Each domain informs and reinforces the others.



Domain A: Community Engagement and Collaboration
Building and sustaining trusted, reciprocal relationships with patient communities and patient advocacy groups through clear communication, tailored education, and ongoing two-way exchange.

Domain B: Development Strategy and Trial Design
Integrating patient, care partner, and advocacy perspectives into preclinical thinking, trial prioritization, design decisions, and active trial conduct.

Domain C: Accessibility, Metrics, and Data
Ensuring engagement is understandable, measurable, transparent, and ethically grounded, including responsible data practices and closed feedback loops.

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Introduction: The Importance of Early Engagement

Current state: Where engagement typically starts today

In many research and development settings, engagement with patients, care partners, and patient advocacy groups occurs after key scientific and operational decisions have already been made. Input is often sought once programs are underway, protocols are drafted, or feasibility parameters are largely fixed. While downstream engagement can improve communication and support participation, it limits the ability of patient and advocacy perspectives to influence foundational decisions.[1]

> *Too often, patient and advocacy perspectives are brought in after key decisions have already been made.*
>
> **Courtney Bugler,** ZERO Prostate Cancer

Defining early engagement

Early engagement is structured, ongoing collaboration that begins during preclinical research and continues across clinical development. It involves patients with lived experience, care partners, and patient advocacy groups representing the broader patient community. Fundamentally, it is embedded across development stages rather than limited to one-off consultations.

Credible engagement requires synthesis of insight across multiple sources and community touchpoints over time. Patient advocacy groups contribute aggregated, community-informed perspectives. Patients and care partners contribute lived experience that grounds decisions in day-to-day realities.

Early engagement positions lived experience and advocacy expertise as sustained inputs alongside scientific, clinical, and operational evidence, informing decisions while they remain adaptable.[1]

Why earlier matters

Engagement that begins after core program decisions have been made limits the potential for patient, care partner, and advocacy input to influence trial direction. By that stage, endpoint priorities, eligibility criteria, visit schedules, burden assumptions, recruitment strategies, and trial locations may already be substantially defined, limiting opportunities to address feasibility, inclusivity, and alignment with patient-identified unmet needs.[1,2] This increases the potential for recruitment challenges, operational delays, and additional participant and care partner burden once trials are underway, when redesign is costly and flexibility is reduced.[1,3]

Embedding engagement during preclinical and early planning phases shifts collaboration to a point where it can meaningfully inform priorities and design. Patient and advocacy input can validate whether research questions reflect meaningful unmet needs rather than assumptions disconnected from lived experience. It can inform methodological decisions, including recruitment approaches, digital tools, visit structures, and trial site strategy, so proposed designs are appropriate for the communities they aim to include.

Addressing structural drivers of inequitable participation is more feasible at this upstream stage. Practical barriers such as travel burden, inflexible scheduling, care partner responsibilities, language and digital access constraints, health literacy, and socioeconomic factors are difficult to mitigate once embedded in trial frameworks.[3–5] Early engagement can also uncover patterns of historical exclusion from research that continue to influence trust, participation, and representativeness.[6,7]

Regulatory guidance and multi-stakeholder frameworks increasingly emphasize the importance of incorporating patient perspectives early to support both scientific rigor and equitable access.[8–12] Published analyses[13–15] and case examples[16,17] have associated earlier engagement with improvements in trial operations, recruitment, and retention.

Scope and context of this paper

This paper focuses on early engagement from preclinical research through Phase 3 clinical development. It is intended primarily for pharmaceutical organizations and patient advocacy groups seeking to embed patient and care partner experience earlier and more consistently into development strategy and trial design. It may also be relevant to other stakeholders involved in clinical research.

Approaches to early engagement are context-dependent and may vary by geography, therapy area, regulatory environment, and community landscape. A practical approach recognizes existing local relationships, governance requirements, and differences in healthcare systems while maintaining a consistent commitment to structured, meaningful collaboration.

> *No one disease or organization has all the best practices, so learning from each other is an essential part of the process.*
>
> **Mellanie True Hills,** StopAfib.org

How this paper was developed

The principles outlined in this paper were developed through a structured, multi-stakeholder workshop that brought together BMS teams and patient advocacy leaders, drawing on their accumulated experiences across research and development activities. The workshop was designed to examine how early engagement should function in practice, with a focus on identifying barriers, enablers, and operational considerations across the development lifecycle.

Discussions were conducted using guided exercises to capture both individual and collective perspectives. Insights were systematically consolidated and thematically analyzed to identify recurring patterns, areas of alignment, and points of tension between patient-centered principles and real-world operational constraints. This process enabled the integration of advocacy-led insight grounded in lived experience with cross-functional industry expertise.

Through this process, participants identified nine core components of early engagement, grouped into three domains, which together describe what consistent practice requires.

How early engagement can inform research and development across the lifecycle

From early strategy to real-world impact, patients, care partners, and patient advocacy groups can help ensure development reflects what matters most to the communities they represent. Engagement begins early and is continuous across research and development, with insights informing decisions at every stage.

Indication, Asset, and Program-Level Insights

+ Understanding unmet need grounded in real patient burden and lived experience
+ Identification of symptoms and outcomes that matter most to patients and care partners
+ Insights into disease heterogeneity and populations that may be underserved or underrepresented in research
+ Early feedback on target product profiles

Strategic Approach to Development Plans

+ Perspectives on acceptable benefit-risk balance based on lived experience
+ Preferences around treatment modalities, dosing, and administration that reflect real-world use
+ Guidance on endpoints that reflect quality of life, daily functioning, and outcomes meaningful to patients
+ Identification of barriers to participation and adherence

Clinical Trial Design and Protocol Review

+ Feedback on protocol feasibility and participant burden, as well as operational considerations
+ Recommendations to strengthen inclusivity and representation in trial design
+ Input on visit schedules, procedures, and endpoint relevance
+ Optimization of patient-facing materials and informed consent

Active Trial Optimization

+ Ongoing feedback on patient experience and trial burden during study delivery
+ Identification of recruitment and retention barriers
+ Insights into adherence challenges and engagement gaps
+ Recommendations to improve patient-facing communications and support throughout trial participation

Post-Launch Insights

+ Real-world feedback on treatment experience and outcomes
+ Insights into adherence and support needs in routine care settings
+ Identification of access barriers and health system challenges affecting treatment use
+ Ongoing input to inform lifecycle management and future development decisions

Late Engagement in Practice

Delayed or absent patient and advocacy engagement can create avoidable cost, complexity, and timeline pressure. Evidence from clinical development shows consistent operational impacts when patient and advocacy input is not incorporated early.

Budget inefficiency and downstream rework

Trials developed with patient input have been associated with lower-than-planned budgets, including reports of protocol budgets approximately 28% below plan and fewer substantial protocol amendments.[14]

More complex and less feasible protocols

Protocols developed without early patient input may include unnecessary endpoints and procedures that increase participant burden and operational complexity. Protocols developed with patient input have been associated with approximately 30% fewer total endpoints,[14,18] and 10% fewer distinct procedures.[19]

Weaker alignment with regulatory and decision-making expectations

Regulatory guidance increasingly emphasizes demonstrable patient involvement in development and evidence generation.[19,20] Clinical trials focused only on technical regulatory requirements may lack patient-relevant outcome measures, limiting understanding of treatment benefit in real-world use.[21]

Greater likelihood of protocol amendments and delayed access

Study designs that are not optimized for participant experience can create unnecessary burden for participants, lead to avoidable protocol amendments, and delay access to new medicines and technologies.[21]

Slower trial initiation and longer timelines

Trials developed without early patient input may experience slower initiation and extended development timelines. Patient engagement has been associated with shorter timelines, faster enrollment, and improved performance against planned study milestones.[14]

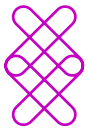
Less effective materials, tools, and patient-facing resources

Materials and tools developed without patient input may be less relevant, harder to understand, and less likely to support appropriate use. Incorporating patient perspectives into the development of medical apps, guidelines, and information materials has been associated with improved relevance, usability, comprehension, and user behavior outcomes.[22–24]

2

The Core Components of Early Engagement

The nine components are grouped into three domains. While each domain addresses a distinct aspect of effective early engagement, the domains are intended to work together in practice. Within each component, the paper outlines why it matters, best practices, and roles in practice. Selected case studies are provided later in the paper to demonstrate how aspects of early engagement may be applied in practice.



Domain A

Community Engagement and Collaboration

Focuses on building and sustaining trusted, reciprocal relationships between pharmaceutical organizations, patient advocacy groups, and patient communities.

Domain B

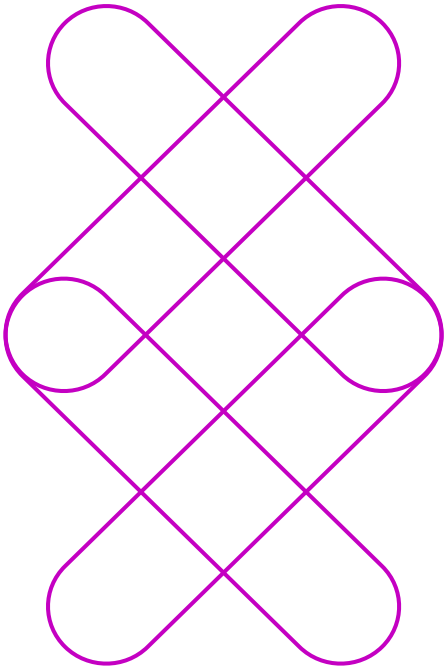
Development Strategy and Trial Design

Focuses on incorporating patient and advocacy perspectives into scientific strategy, trial prioritization, and design decisions early enough to shape direction.

Domain C

Accessibility, Metrics, and Data

Focuses on ensuring engagement is understandable, measurable, transparent, and ethically grounded, including accessibility, outcomes, and data-sharing practices.



Domain A
Community Engagement and Collaboration

This domain covers the relationship foundations that make early engagement credible and usable. It focuses on how trust-based relationships are built and sustained through clear expectations, consistent communication, and visible follow-through. It also addresses how education and practical support are structured to enable participation across different communities, including attention to health literacy and accessible language.

01

Patient Community Relationships

Why this component matters

Without sustained, trust-based relationships, engagement risks becoming episodic and unrepresentative. Input may skew toward individuals or organizations with the time, resources, or proximity to participate, reinforcing existing inequities rather than addressing them. When engagement is transactional or short-term, insights are less likely to reflect the breadth of patient and care partner experience across disease stages, geographies, and socioeconomic contexts.

Long-term relationships enable aggregation of perspectives over time and across communities, improving the credibility and usability of insights. They also create the conditions for feedback, learning, and accountability, which are essential for translating patient and advocacy input into research decisions that improve feasibility, inclusivity, and relevance.

For communities with historical reasons for skepticism, structured and transparent relationships are the foundation for everything that follows.

Upal Basu Roy
LUNGevity

What good looks like in practice

Effective relationships are long-term and grounded in transparency, mutual respect, and accountability. They are sustained through continuity of partnership and visible follow-through over time, so patient and advocacy partners can see how insights inform decision-making.

Central to this work is the recognition of contributions. This includes acknowledging input, closing feedback loops, and explaining how insights were used, or why they could not be acted upon. Regular updates reinforce confidence that engagement is meaningful rather than symbolic and support deeper contribution over time.

Roles in practice

Pharma
+ Maintain continuity of engagement across development stages
+ Provide regular updates and demonstrate follow-through on partnership commitments
+ Close feedback loops, recognize contributions, and share outcomes where appropriate

Patient advocacy groups
+ Provide aggregated, community-informed perspectives across multiple touchpoints
+ Contextualize lived experience for development decisions and real-world application
+ Shape dialogue, frame priorities, and advise on interpretation of insights
+ Support engagement that reaches appropriate and representative patient populations

Patients and care partners
+ Contribute lived experience through structured feedback and information-sharing activities
+ Provide insight into day-to-day realities
+ Inform how trial design and communication choices affect daily life

Shared accountability
+ Uphold respect, transparency, and follow-through
+ Align upfront on how insights will be used
+ Maintain open dialogue and reinforce trust through consistent feedback

02

Pharma, Patient Advocacy Group, and Community Communications

Why this component matters

Poorly coordinated communication can undermine even strong relationships. Inconsistent, delayed, or unclear information can erode trust and lead to misunderstandings around research intent, trial expectations, and progress, limiting meaningful participation. Gaps in communication can also contribute to misinformation and disengagement from research, particularly in communities with historical reasons for mistrust.

Clear, structured communication supports informed engagement. Once relationships are established, effective information exchange enables advocacy groups to contextualize information accurately for their communities, align expectations across stakeholders, and support transparency throughout the research lifecycle.

What good looks like in practice

Effective communication is proactive, structured, and bidirectional. It is important that pharma teams and patient advocacy groups share information in a timely and coordinated way, rather than reactively or without alignment across stakeholders. Communication continues as programs evolve, not only at fixed milestones, and includes opportunities for clarification and dialogue.

Meaningful communication involves both timing and substance. Pharma must clearly articulate information about investigational programs, trial progress, and participation expectations in formats appropriate for community dissemination. Risk and potential benefits are presented together, with clear framing of what is known and what remains uncertain. Investigational treatments are clearly distinguished from available options.

Defined points of contact reduce duplication, align priorities, and manage capacity. Regular, structured touchpoints, such as standing meetings or working groups, reinforce alignment and help ensure communication remains consistent, transparent, and appropriately resourced.

> *Good communication is more than sharing information — it means making space for questions, listening, and working together with patients and communities throughout the research journey.*
>
> **Jasmine Greenamyer**
> Bristol Myers Squibb

Roles in practice

Pharma

+ Coordinate clear, timely, and compliant communication
+ Establish cadence, clarify roles and responsibilities, and maintain message consistency
+ Sustain open feedback loops that support ongoing dialogue

Patient advocacy groups

+ Interpret, contextualize, and refine messages to ensure relevance and comprehension
+ Advise on framing so information reflects patient priorities and real-world needs
+ Support dissemination through trusted community channels while actively shaping dialogue

Patients and care partners

+ Inform tone, clarity, and content based on lived experience
+ Identify areas where messaging does not reflect real-world concerns or community needs

Shared accountability

+ Align on communication goals, cadence, and boundaries
+ Maintain regular forums for exchange and reinforce transparency and responsiveness
+ Clarify expectations around time, expertise, and contribution, with appropriate recognition and resourcing

03

Personalized Education and Support

Why this component matters

Patient advocacy groups vary widely in capacity, reach, and community needs. Without tailored education and support, early engagement may unintentionally favor well-resourced organizations while excluding smaller or underrepresented communities. Standardized approaches can overburden some advocacy groups or fail to address real-world barriers faced by others.

Personalized education and support strengthen equitable engagement by aligning research-related materials and practical assistance with community realities. In this context, support includes both educational enablement and practical assistance that reduce barriers to research participation. When support is calibrated to patient need, this improves comprehension, lowers participation barriers, and supports broader inclusion, increasing patient engagement in research and development.

Here, education and support are limited to research engagement and clinical trial participation. Patient advocacy groups maintain independence over their broader disease education. Pharma involvement may include funding for the development of research- and clinical trial-related educational materials, scientific clarification, or assistance improving the clarity of research-related content but does not extend to directing general community content.

> *When materials are only written for the most educated audience, we've already excluded the communities who need access to information the most.*
>
> **Robin Tuohy**
> International Myeloma Foundation

What good looks like in practice

Personalized education and support are co-created with patient advocacy groups and grounded in community realities. Research and trial-related materials are developed using structured input from patient advocacy groups. Feedback is captured, consolidated, and applied to refine language, format, and delivery, with updated materials reviewed to confirm clarity and usability. Materials reflect patient language, appropriate literacy levels, and accessibility needs, with attention to cultural context and community-specific factors that influence understanding and participation. Materials and approaches are refined over time to ensure they remain usable and relevant.

Education is directed not only to patients, but also to investigators, trial site staff, and healthcare professionals. This strengthens awareness of patient experience and participation barriers, including social determinants of health, geographic constraints, and care partner responsibilities that influence access and engagement.

Operational support complements educational efforts. Where needed, this includes responsive, high-touch assistance such as logistical coordination, navigation of trial processes, and hands-on participation support, often described as a white-glove service. The type and level of support is calibrated based on what communities identify as necessary to engage effectively and consistently, rather than delivered as a standard approach.

Roles in practice

Pharma

+ Resource and enable personalized education and support, including funding, training, and operational assistance where needed to support participation, such as coordination of logistics or navigation of trial processes
+ Collaborate with patient advocacy groups on research- and trial-participation materials that support informed decision-making
+ Ensure compliance alignment and adapt approaches based on feedback
+ Support research engagement collaboration rather than designing tools for dissemination in isolation

Patient advocacy groups

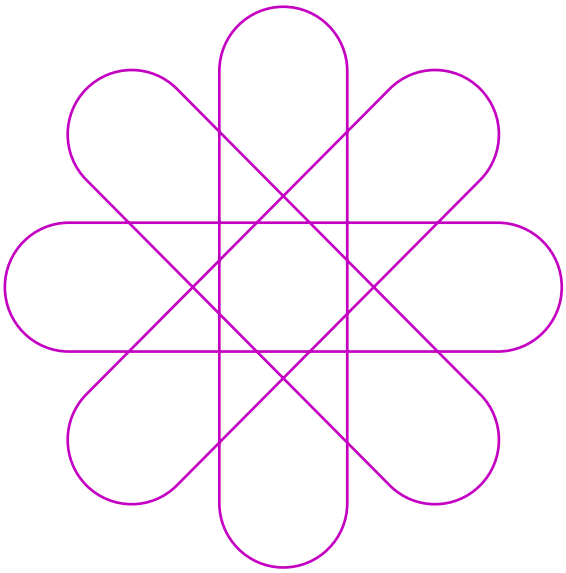
+ Review and advise on research- and trial-related educational tools and resources
+ Ensure materials are readable, accessible, and tailored to the needs of patient communities
+ Advise on language, cultural context, and practical participation realities that may require additional support
+ Maintain independence over broader disease education and determine how external materials are adapted or shared

Patients and care partners

+ Participate in educational initiatives related to research and trial participation
+ Provide direct feedback on clarity, relevance, and usability
+ Identify practical barriers that affect understanding or participation

Shared accountability

+ Align on what effective and equitable support looks like for different communities
+ Review reach, usability, and impact over time
+ Refine education and support approaches based on feedback
+ Monitor whether operational support remains appropriate, proportionate, and responsive to community need



Domain B
Development Strategy and Trial Design

This domain focuses on how patient and advocacy perspectives are integrated into development strategy and trial decisions from preclinical planning through active trial conduct. It emphasizes structured input at decision points that shape feasibility, inclusivity, and relevance, rather than limiting engagement to downstream communications. It also highlights continuous feedback as a practical mechanism for improving trial execution without undermining governance or protocol integrity.

04

Preclinical Insights

Why this component matters

Decisions made during preclinical development shape the trajectory of research long before patients are enrolled in trials. Without early patient and advocacy input, programs may advance based on assumptions that do not reflect lived experience, real-world care pathways, unmet need across affected populations, or structural barriers that later influence who can realistically participate in research. These misalignments can surface downstream as recruitment challenges, protocol amendments, or limited applicability of results.

Early engagement helps identify blind spots while scientific flexibility still exists. At this stage, patient and advocacy insight may include understanding real-world experiences of disease and treatment, tolerance for assessments or interventions, practical realities of disease progression, and factors that shape what meaningful benefit looks like in daily life.

By informing how unmet need is understood, how potential benefit is framed, and which questions are prioritized, early engagement supports development strategies that are more relevant, inclusive, and aligned with real-world context. Early insight can also highlight disparities in disease burden, access, trust, and geographic distribution, helping inform future decisions about which populations are prioritized and where research efforts may need focused attention.

What good looks like in practice

Patient and advocacy input are incorporated into early scientific thinking, supporting early alignment and patient relevance. Patient advocacy groups can be engaged as expert contributors who help define unmet need, frame potential benefits, and identify early concepts for further exploration. Their input informs early scientific thinking by adding lived-experience context and community insight, alongside scientific, clinical, and operational considerations.

Engagement also includes collaboration on language and framing. Scientific concepts are translated into patient-meaningful terms through co-development of materials and engagement tools. Language and imagery are reviewed with patient and advocacy partners, with input captured and used to refine content so that it reflects real-world experience and avoids abstract or idealized representations disconnected from daily life. Revisions are iterated until materials are considered clear, usable, and aligned with how patients and communities describe their experiences.

Preclinical engagement is intentionally resourced and planned. Dedicated funding, time, and preparation enable meaningful participation, including structured dialogue and, where appropriate, exploratory surveys or rapid-cycle feedback activities with shared ownership of approach and interpretation. Purpose, scope, and intended use of insights are clarified upfront to ensure alignment and transparency.

Roles in practice

Pharma

+ Share early scientific priorities, hypotheses, and open questions
+ Resource and plan engagement, including funding and internal coordination
+ Involve advocacy partners, patients, and care partners in shaping early concepts, language, and exploratory approaches

Patient advocacy groups

+ Contribute expert, community-informed perspectives shaped by lived experience, ongoing engagement, and, in many cases, formal research advocacy training
+ Co-develop patient-friendly approaches and tools so early scientific thinking reflects real-world relevance
+ Where exploratory surveys or rapid-cycle feedback mechanisms are used, collaborate on question design and contribute to interpretation of findings

Patients and care partners

+ Provide early input through surveys, advisory activities, discussion forums, and other coordinated engagement channels with advocacy groups
+ Help refine early concepts, test assumptions, and identify practical considerations not visible through scientific or clinical lens

Shared accountability

+ Align up front on purpose, scope, and intended use of engagement
+ Agree roles in language development, tool design, interpretation of insights, and feedback processes

05

Trial Design and Feasibility Planning

Why this component matters

Decisions about which research questions are pursued, which populations are studied, and which trials receive investment shape who can participate and the outcomes that are measured. Without early patient and advocacy input, trials may unintentionally exclude certain populations, impose avoidable burdens, or focus on endpoints that do not reflect what matters most to patients and care partners.

Engaging early helps ensure patient-relevant outcomes, including symptoms, functional impact, and quality of life, are considered alongside clinical and regulatory endpoints. It also identifies feasibility, equity, and access considerations before they become embedded in protocols. Incorporating lived experience at this stage supports designs that are more representative, inclusive, and applicable in real-world care settings, strengthening both scientific validity and relevance of the evidence generated.

Trial feasibility can't be assessed in a vacuum – if the people most affected by a disease can't realistically participate, the science itself is at risk

Andrew Whitehead
Bristol Myers Squibb

What good looks like in practice

Trial design and feasibility planning build on insights generated during preclinical engagement, translating them into practical decisions that shape eligibility criteria, endpoint selection, visit schedules, and site strategy. Advocacy input is sought early to validate areas of focus, identify high-impact opportunities, and highlight barriers that may limit access, relevance, or recruitment.

Engagement continues through trial design, with decisions reviewed against real-world disease burden and participation realities across different communities and care settings.

Scientific innovation is considered alongside whether proposed trial designs are accessible and feasible for patients facing different barriers to participation. Trial site selection and operational design account for geographic coverage, scheduling flexibility, transportation needs, and care partner responsibilities to reduce avoidable burden and minimize systematic disadvantages for underserved populations.

Roles in practice

Pharma

+ Define trial priorities and designs within scientific, feasibility, and resource constraints
+ Share rationale, timelines, and constraints transparently to enable informed input
+ Invest in appropriate training and enablement of advocacy partners

Patient advocacy groups

+ Contribute aggregated, community-informed perspectives to validate relevance and unmet need
+ Advise on inclusion and exclusion criteria, trial location, and participation barriers
+ Identify unintended consequences or feasibility concerns based on real-world care pathways
+ Raise focused questions on specific design elements during structured patient engagement to assess likely participation, burden, and feasibility

Patients and care partners

+ Provide lived experience input through surveys, focus groups, and advisory activities
+ Highlight practical constraints, outcome priorities, and participation considerations

Shared accountability

+ Ensure transparency around how advocacy input informs trial design and feasibility planning decisions
+ Communicate back to communities on how input was applied and what decisions were made

06

Active Trial Optimization

Why this component matters

During active trials, patient advocacy groups play an important role in raising trial awareness, supporting recruitment and participant matching, and helping participants navigate practical aspects of trial participation, supporting sustained engagement once a study is underway. Even with this support in place, well-designed trials can encounter unforeseen challenges once active. Trial sites may face resource constraints, staffing pressures, or operational variability. Without structured mechanisms for ongoing feedback, issues affecting participant understanding, burden, recruitment, or retention may go unrecognized until they impact delivery or data quality.

Ongoing engagement during trials provides a structured way to identify and address emerging challenges during trial conduct. Feedback from patient advocacy groups may highlight reasons for limited interest or perceived barriers among prospective participants, while structured input from enrolled participants can surface issues affecting understanding, burden, or retention. Addressing these insights within defined operational boundaries strengthens participant experience and recruitment while preserving protocol integrity.

> *Launching a trial is not the finish line – continuous live feedback helps identify barriers early and improve the experience.*
>
> **Jeff Anderson**
> Bristol Myers Squibb

What good looks like in practice

Active trial optimization relies on ongoing feedback mechanisms throughout trial delivery. These mechanisms capture community-level insights related to recruitment and perceived barriers, as well as feedback from enrolled participants on clarity of study information and participation expectations, burden, and trial experience.

Feedback can be used to adjust communication, clarify misunderstandings, and strengthen support without altering core protocol elements. Any adjustments should be feasible within trial site realities and avoid introducing additional operational burden.

Advocacy input typically reflects themes emerging from conversations with prospective participants and community members, rather than confidential site-level operational detail. For enrolled participants, trial navigators or centralized support services may help address practical challenges, such as scheduling or reimbursement questions, to support continued engagement.

Input from trial site coordinators and healthcare professionals can complement participant feedback by identifying operational constraints and improvement opportunities that can inform both current delivery and future trial design.

Roles in practice

Pharma

+ Establish appropriate feedback mechanisms during active trial conduct
+ Relay insights to relevant development and operations teams
+ Implement feasible communication or support adjustments within protocol and regulatory boundaries
+ Maintain confidentiality and blinding requirements while promoting transparency where appropriate

Patient advocacy groups

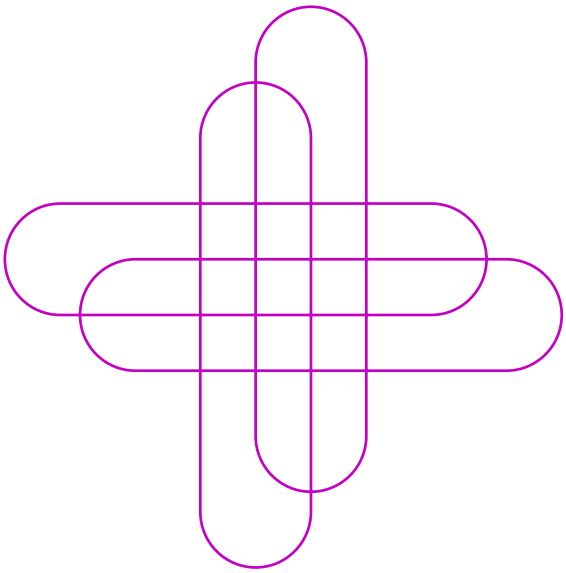
+ Surface recurring issues related to recruitment interest, perceived burden, clarity of information, and reasons for non-participation
+ Identify and contextualize emerging themes raised within their communities during trial conduct
+ Provide community-level insight to inform appropriate communication and support responses

Patients and care partners

+ Support trial awareness, recruitment engagement, participant navigation, and sustained participation throughout trial conduct
+ Provide structured feedback and contextualize recurring issues related to recruitment interest, perceived burden, clarity of study information and expectations, and reasons for non-participation
+ Inform improvements to participant-facing communication and support

Shared accountability

+ Align upfront on how feedback will be gathered, interpreted, and used
+ Communicate changes transparently where appropriate to reinforce responsiveness and trust



Domain C
Accessibility, Metrics, and Data

This domain focuses on the practices that make early engagement visible, trackable, and sustainable. It addresses how information is communicated clearly, how outcomes and impact can be measured, and how data exchange is governed and protected. The components in this domain support transparency and ethical practice across the research and development lifecycle. They also enable continuous improvement by linking engagement activities to observable outputs, decisions, and outcomes.

07

Clarity and Accessibility

Why this component matters

Clear, accessible communication is essential throughout the research process, particularly when patients and care partners are considering whether to participate in clinical trials. Patients and care partners encounter a range of research-related information, including trial education materials, awareness and recruitment communications, participation guidance, and ongoing study information. These materials shape understanding, expectations, and decision-making at multiple points across the research experience.

Informed consent is one critical application within this broader set of patient-facing materials. Its purpose is to ensure that patients and care partners receive sufficient information about the research, potential risks and benefits, available alternatives, and participation expectations so they can make voluntary and informed decisions about participation.

The consent process operates within established ethical and regulatory requirements designed to protect research participants.[25–27] However, regulatory compliance alone does not guarantee understanding; lengthy, technical documents may meet requirements yet still fail to support comprehension.

Complex language, unclear expectations, and poorly framed information can create barriers to research participation. These barriers disproportionately affect individuals with lower health literacy, limited English proficiency, cognitive impairment, or constrained time and resources, narrowing participation and limiting representativeness.

Clarity and accessibility function as equity mechanisms by presenting information in ways that support understanding, informed decision-making, and equitable participation in research.

> *Clarity isn't something you build once and hand over – it must be co-created with patients, tested in the real world, and refined as the conversation evolves.*
>
> **John Dwyer**
> Global Alzheimer's Platform Foundation

What good looks like in practice

A shared, patient-meaningful lexicon between industry and patient advocacy groups is established early and refined over time. Language is tested for clarity and adapted to reflect patient experience, cultural context, literacy levels, and accessibility needs.

Understanding is actively confirmed rather than assumed. Trial materials are co-developed, tested, and refined to support patients and care partners in understanding their role, expectations, potential risks and benefits, and areas of uncertainty. Comprehension checks, particularly in consent processes, help ensure materials function as intended in real-world settings and support informed decision-making. Applying plain language principles, layered presentation of information, and visual aids improves usability while preserving scientific rigor across consent and other patient-facing materials.

Scope and boundaries are defined upfront with patient advocacy groups to avoid misinterpretation and reinforce transparency. Materials and communication approaches are reviewed periodically and updated based on feedback, comprehension findings, and changes in study design or participant needs.

Roles in practice

Pharma

+ Co-develop shared terminology and accessible materials with patient advocacy groups
+ Implement alternate formats, such as layered consent documents, short and extended versions, or visual and video-based content where appropriate
+ Resource accessibility early rather than treating it as a late-stage adaptation
+ Ensure alignment with regulatory and compliance requirements

Patient advocacy groups

+ Validate readability, language, and accessibility for diverse communities
+ Advise on framing of research-related information and participant expectations
+ Ensure materials reflect real-world experience and community context

Patients and care partners

+ Participate in usability and comprehension testing
+ Identify areas where materials are unclear, unrealistic, or disconnected from lived experience

Shared accountability

+ Align on clarity standards and how understanding will be assessed
+ Review and refine materials over time
+ Communicate adjustments transparently

08

Metrics and Outcomes

Why this component matters

When engagement outcomes are not defined or tracked, early engagement risks being perceived as symbolic rather than consequential. Lack of visibility makes it difficult to assess impact, learn from experience, or justify continued investment, both internally and with external partners.

Clear metrics support accountability and continuous improvement. By defining and sharing outcomes that reflect both progress and influence, organizations can demonstrate how engagement informs decisions and improves development processes. Outcomes may reflect influence across multiple aspects of engagement, including development strategy and trial design, recruitment and participation experience, partnership continuity across development programs, and the timeliness and effectiveness of feedback processes. Transparent reporting also reinforces trust with patient advocacy groups and communities by showing how input is used in practice.

What good looks like in practice

Metrics are defined to reflect both implementation and impact, proportionate to the maturity and scope of engagement.

Process measures assess whether engagement is occurring as intended. These may include breadth of communities reached, continuity of engagement, timeliness of feedback loops, responsiveness to patient and advocacy input, and effectiveness of communication and partnership processes.

Outcome measures assess what changed as a result. These may include documented influence on research questions, endpoint strategy, protocol design, communication approaches, operational adjustments, recruitment and participation experience, awareness and engagement reach, partnership continuity across development programs, and responsiveness of feedback processes. Recruitment and retention signals may also be monitored, with careful attention to attribution.

> *Metrics aren't just about accountability to sponsors – they're how advocacy communities know whether their time and trust are actually translating into better outcomes for patients.*
>
> **Michael Sapienza**
> Colorectal Cancer Alliance

Taken together, these measures support course correction. They help identify gaps in reach, barriers to participation, and where engagement is not translating into meaningful change, so approaches can be refined.

Insights are captured and synthesized consistently, using secure digital tools where appropriate. Outcomes are communicated transparently to internal teams and, where feasible, to external partners.

A practical example of documenting and tracking engagement activities is included later in the paper in the Patient Expert Engagement Resource (PEER) case study.

Roles in practice

Pharma

+ Define, track, and report engagement metrics across relevant functions
+ Use findings to inform course correction and continuous improvement
+ Share outcomes transparently where appropriate

Patient advocacy groups

+ Advise on which outcomes are meaningful from a patient perspective
+ Provide context to interpret engagement findings within community realities
+ Track and share engagement or program metrics developed and maintained by patient advocacy groups, where appropriate
+ Support responsible communication of engagement findings and metric outcomes to patient communities

Patients and care partners

+ Provide lived experience input to assess whether changes driven by engagement are working as intended in practice
+ Highlight any missing perspectives or community experiences not captured by formal metrics

Shared accountability

+ Agree up-front on which metrics will be tracked, how they will be interpreted, and how results will be shared
+ Review findings together where appropriate and clarify implications
+ Use insights to refine engagement approaches over time

09

Data-Sharing Logistics

Why this component matters

Data sharing is central to early engagement and involves the exchange of research information, patient population insights, and community-informed data between pharma and patient advocacy groups. When the purpose, standards, or intended use of these data are not clearly defined, risks can emerge, including inefficiencies, ethical concerns, or loss of trust. Clear, responsible data-sharing arrangements, aligned early on purpose and scope, ensure that information exchange is transparent, proportionate, and aligned with regulatory expectations, enabling insights to meaningfully inform development.

What good looks like in practice

Good practice begins with shared clarity on why data are being exchanged, how they will be used, who will have access, and for how long. Scope, limitations, and intended application are defined upfront.

Pharma teams and patient advocacy groups align on the value of different data types. Quantitative datasets may include structured information such as trial results shared with communities, patient population data from registries or surveys, or aggregated datasets used to support advocacy publications or future analysis. Qualitative insights may include feedback gathered through patient advisory discussions, community engagement activities, or narrative accounts of lived experience that help interpret findings and inform engagement approaches. Governance agreements define access permissions, retention periods, oversight structures, and safeguards to protect confidentiality and prevent misuse.

Data exchange is bidirectional and proportionate. Quantitative outputs suitable for broader dissemination, such as congress presentations or publications, are complemented by high-level qualitative insights that add context without compromising confidentiality. Clear standards outline what data are collected, by whom, and at what points in time, alongside an agreed feedback cadence, so insights are shared back consistently.

All data-sharing activities align with applicable ethical and regulatory guidance and are communicated transparently, so patient advocacy groups and communities understand how their information is protected, governed, and applied.

Roles in practice

Pharma

+ Define required datasets and intended use in alignment with development objectives
+ Establish formal data-sharing agreements and confidentiality protections, including defined approval and authorization processes
+ Ensure ongoing governance, ethical, and regulatory compliance throughout data exchange
+ Clarify how data will inform decisions and how insights will be communicated back

Patient advocacy groups

+ Share relevant aggregated and blinded data where appropriate and feasible
+ Engage legal, privacy, or governance support as needed to review data-sharing agreements and confirm appropriate protections
+ Provide qualitative insight to contextualize quantitative findings
+ Align on interpretation so shared data reflect lived experience and community priorities

Regulators

+ Provide guidance on data standards and evidentiary expectations for regulatory submissions, supporting fit-for-purpose datasets and efficient review

Shared accountability

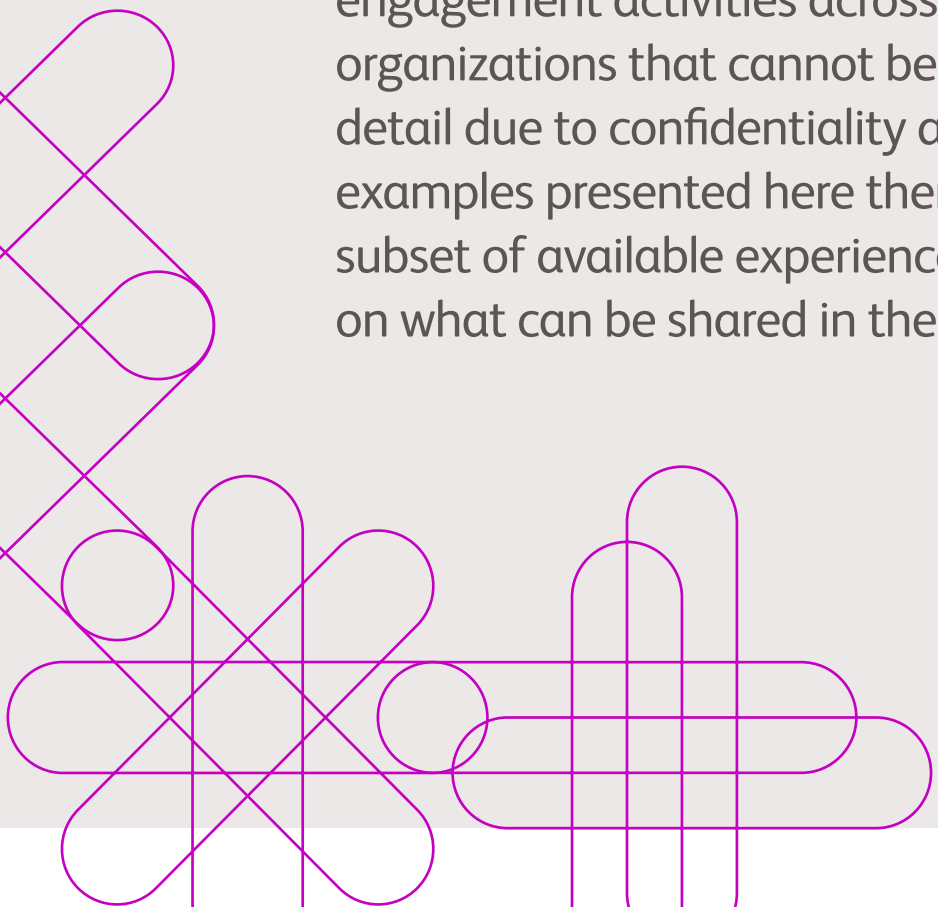
+ Agree up front on purpose, scope, governance, and feedback cadence
+ Safeguard ethical practice and confidentiality
+ Communicate outcomes in ways that reinforce transparency and trust

3

How Early Engagement Shapes Research and Development Decisions

The following case studies illustrate how early engagement can be applied in practice across different contexts. Most examples are drawn from BMS to ensure consistency of detail and transparency in what can be shared publicly.

Participating patient advocacy organizations and co-authors have contributed to additional engagement activities across multiple organizations that cannot be described in detail due to confidentiality agreements. The examples presented here therefore represent a subset of available experience, selected based on what can be shared in the public domain.



BMS early advocacy engagement in Alzheimer's disease

Alzheimer's disease research presents a particularly challenging development environment. Trials often involve complex eligibility criteria, frequent visits, and significant cognitive and logistical demands on participants and care partners, contributing to high screen failure and slow enrollment. Communities disproportionately affected by the disease, including Black and Hispanic populations, have been historically underrepresented in research, limiting generalizability. Effective engagement therefore requires sustained trust, long-term commitment, and structured input from patient advocacy groups and care partners.

Early engagement with Alzheimer's disease patient advocacy groups and care partners informed research and clinical development at the protocol stage and throughout ongoing development activities. Engagement included ten protocol-level reviews, supported by follow-up surveys, advisory boards, and roundtables.

Advocacy groups and care partners provided input on diagnostic complexity, screening burden, visit schedules, outcome relevance, inequitable access to care, the need for more representative trials, and the cumulative impact of participation on patients and care partners. Feedback from these engagements highlighted the need for clearer and more consistent diagnostic criteria, streamlined and tiered screening approaches, and earlier use of less burdensome assessments to reduce screen failure and participant fatigue. Advocacy partners emphasized the importance of outcomes that reflect what matters most to patients and families, including neuropsychiatric symptoms and quality of life, alongside traditional cognitive measures. Engagements also identified additional considerations related to care partner burden, communication clarity, and the need for practical support during screening and participation. These insights were incorporated into protocol planning discussions and feasibility considerations, helping teams anticipate operational challenges, refine study procedures, and better align study expectations with the realities of participation for patients and care partners.

This case study illustrates how sustained, early relationships with patient communities can strengthen shared understanding and inform research planning in Alzheimer's disease.

BMS approach to improving health literacy through Universal Patient Language® [28]

Health literacy remains a persistent challenge in healthcare and research. Many patients and care partners struggle to understand complex or inconsistent medical information, which can limit their ability to engage confidently in care decisions or research participation. These challenges disproportionately affect people facing language barriers, cognitive burden, limited access to support, or systemic inequities. Addressing how information is structured and communicated is therefore a foundational element of meaningful engagement and equitable inclusion.

The Universal Patient Language® (UPL) is an evolving set of principles and practical resources co-developed with patients, care partners, patient advocacy groups, and healthcare providers. It helps turn complex science into clear, meaningful, and actionable information by translating concepts into language and images that reflect lived experience, practical realities, and what matters to patients and care partners. This approach uses plain language, supportive visuals, intuitive formatting, and accessible data displays to support confident understanding and use of information.

Across clinical development, UPL supports personalized education by providing shared guidance on terminology, structure, and framing that can be adapted to different communities and literacy levels. When reviewing content, such as clinical trial plain language summaries, informed consent forms, or other educational materials, UPL helps make information about disease, research, and trial participation easier to understand, navigate, and act on, reducing cognitive and informational burden.

Collaborating with patient advocacy groups is critical to the impact of UPL and to ensuring it continues to meet the educational needs of different communities. UPL evolves through feedback, enabling the ongoing development of tools and resources that address patient needs. Resources available via UPL.org provide practical tools that advocacy groups and healthcare teams can use to tailor communications across different formats and settings.

This case study illustrates how structured collaboration with patient communities can support the development of communication approaches that improve understanding, support informed participation, and strengthen trust in research through clearer and more accessible information.

Pancreatic Cancer Action Network data platform enables research collaboration[29,30]

Pancreatic cancer research has historically been constrained by limited access to large, well-annotated patient datasets, slowing progress in understanding disease biology and developing effective therapies. To address this challenge, the Pancreatic Cancer Action Network (PanCAN) established Know Your Tumor, a precision medicine service that provides molecular profiling for patients and generates clinical and molecular data to support more personalized treatment approaches. Building on this foundation, PanCAN developed SPARK as a centralized data and analytics platform to enable responsible secondary use of these data for research and development.

SPARK integrates de-identified patient data generated through PanCAN initiatives into a single, cloud-based platform. The data include clinical information, molecular and multiomic data, imaging, treatment details, and outcomes, structured to support analysis while protecting patient confidentiality. Access is governed by formal data use agreements aligned with patient consent, regulatory guidance, and ethical standards.

The platform includes a defined workflow through which qualified researchers, including those from commercial and for-profit organizations, can request access to datasets. Governance and review processes clarify intended use and support responsible data access and handling, with oversight from an advisory committee representing academia, industry, and data science to ensure the platform continues to meet the needs of the pancreatic cancer research community. In practice, SPARK has been used to support exploratory analyses of molecular subgroups, treatment response patterns, and potential biomarkers, enabling more efficient hypothesis generation and early research insights. PanCAN has publicly announced partnerships in which SPARK data have been used to support drug discovery and biomarker research.[31]

This case study illustrates how nonprofit-led data platforms, supported by clear data standards, transparent access pathways, and structured governance from the outset, can enable responsible collaboration with industry while reinforcing transparency and trust with patient communities.

BMS early advocacy engagement in ZERO Prostate Cancer informs oral therapy presentation decisions

Oral therapies play an important role in prostate cancer treatment and are commonly administered at home rather than in clinical environments. Responsibility for medication management therefore rests largely with patients and care partners, making practical aspects of treatment use, such as supply duration and packaging design, relevant to daily routines. Considering these factors during development helps ensure that product presentation decisions reflect daily treatment use.

Early engagement with the patient advocacy group ZERO Prostate Cancer was conducted through BMS's Patient Expert Engagement Program. Patient advocacy input was sought ahead of stability studies for commercial supply, at a defined decision point in development to inform product presentation choices. A virtual session was held with representatives from ZERO Prostate Cancer, where participants reviewed proposed options and provided feedback in response to specific questions.

Discussion focused on supply duration, container configuration, and capsule color for the planned product presentation of gel capsules. Participants provided input based on practical experience managing medication in everyday settings. Feedback indicated that a 30-day supply would be preferable to reduce the burden associated with more frequent prescription refills and pharmacy visits, and this informed the selection of a 30-day supply over a 15-day supply. Participants also emphasized the importance of packaging that is easy to access and handle, particularly for older adults who may be managing conditions such as arthritis. This feedback informed the selection of bottle packaging over blister packs for use in the United States and a preference for a bottle without an outer box rather than a bottle packaged within a box. Color options were presented, and a blue capsule color was selected over white or teal.

This case study illustrates how patient advocacy input can inform practical product presentation choices while addressing everyday usability alongside clinical and manufacturing requirements.

BMS approach to structured early advocacy engagement through the Patient Expert Engagement Resource (PEER) Program

Meaningful early engagement requires more than individual interactions. Organizations also need structured approaches that create opportunities for patient and advocacy perspectives to inform decisions consistently and at appropriate stages across research and development activities.

BMS established the Patient Expert Engagement Resource (PEER) Program in 2020 to support a more systematic approach to engaging patients and patient advocacy groups across development activities. PEER was designed to facilitate engagement across the full product development and commercialization continuum — from discovery and early development through clinical trial design, launch, and post-market activities. As part of implementation, BMS decided to require that all pivotal Phase 3 clinical trial protocols undergo PEER review before proceeding.

Since launch, PEER has worked with over 100 patient advocacy groups spanning more than 30 disease areas and supporting over 200 engagements. Approximately 70% of engagements focused on research and development or health literacy. In 2025, 72% of protocol reviews conducted through PEER resulted in changes informed by advocacy input.

Feedback and insights generated through PEER are captured within the Patient Experience Hub, an internal repository designed to support documentation and organizational learning across engagement activities, while helping track how input is considered and communicated back to patient advocacy groups. As part of the broader Early Advocacy Engagement Framework at BMS, PEER has increasingly been applied earlier in development to help create opportunities for patient and advocacy perspectives to inform trial design choices. Feedback from patient advocacy groups also supports continued evolution of the approach and helps ensure engagement processes remain responsive to community needs and experiences.

The growth and adoption of PEER has been supported by leadership endorsement, dedicated program oversight, established standard operating procedures, training, and ongoing investment. Patient advocacy organizations are also engaged to provide feedback that supports the continued evolution of the program.

This case study illustrates how structured approaches to early advocacy engagement can support timely incorporation of patient and advocacy perspectives, strengthen transparency around how insights are used, and help sustain ongoing collaboration across research and development activities.

4

Conclusion: From Insight to Impact

Early patient, care partner, and advocacy engagement are core capabilities for modern research and drug development. Embedding lived experience earlier and more systematically ensures that decisions are informed while direction, feasibility, and impact can still be shaped.

The nine components outlined across three domains describe what consistent practice requires. Together, they move engagement beyond isolated activities toward a structured, repeatable approach that can be adapted across therapy areas, geographies, and stages of development. The examples presented illustrate how these principles can be applied in practice to inform real development decisions.

This work represents a shared starting point rather than a static model. Its value depends on how it is applied, tested, and refined in practice. Sustained impact requires transparency, feedback, and visible follow-through so that engagement is demonstrably linked to decision-making.

Progress depends on commitment across stakeholders.

For pharmaceutical organizations, this means treating patient and advocacy insight as a strategic input to development decisions and resourcing it accordingly.

For patient advocacy groups, it means continuing to contribute aggregated community insight, challenge assumptions, and shape feasible and relevant research approaches.

For patients and care partners, it means participating in structured engagement that grounds decisions in real-world experience.

For the broader ecosystem, including regulators, funders, and academic institutions, it means recognizing and reinforcing upstream engagement practices that strengthen scientific rigor and equity.

Above all, meaningful early engagement depends on the insight, expertise, and sustained involvement of patients, care partners, and advocacy communities. Their insight, expertise, and willingness to engage early make better research possible.

> *This white paper demonstrates how multi-stakeholder input can be integrated across all phases from conceptualization to study design, execution, and beyond... and provides a clear framework for how to implement that throughout the entire lifecycle.*

Shilpa Venkatachalam, Global Healthy Living Foundation

Progress now depends on implementation.

Organizations should examine where early engagement currently begins, how consistently it informs decision-making, and whether outcomes are visible and measurable in practice.

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