A Playbook for **Patient Engagement** in MedTech in the U.S.

A Framework for **Executing Successful and Sustainable Patient Engagement Strategies** Across the Lifecycle Management of Medical Diagnostics and Devices

AliraHealth

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Contributors

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Authors

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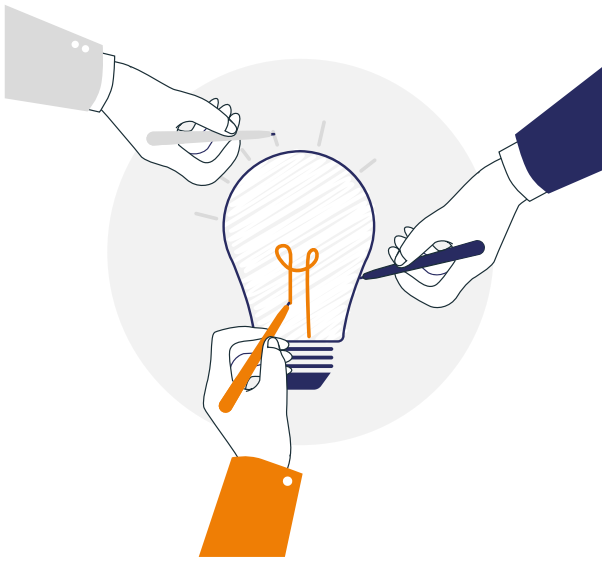


1. Executive Summary

The Patient Engagement Think Tank in MedTech was established in 2021 by Alira Health in partnership with MassMEDIC, The Center for Patient Advocacy Leaders (CPALs), and MedTech industry participants. The goal is to deliver actionable insights on how the MedTech industry can successfully incorporate patient engagement activities in medical device and diagnostic development — at every stage of the product lifecycle.

The Importance of Patient Engagement

The U.S. healthcare system is complex, with countless stakeholders, settings, and providers. Patients often represent the only consistent factor within the patient journey, inherently making patients essential partners to delivering high quality care effectively and efficiently. Evidence increasingly shows that patient engagement can improve patient knowledge, patient–provider relationships, healthcare utilization and satisfaction, treatment adherence, health outcomes, and healthcare costs.[1,2]

We know from the success of patient engagement in the pharmaceutical industry that these activities also add significant financial value for sponsors at each stage of the product lifecycle — from avoiding protocol amendments to enhancing clinical trial enrollment and clinical trial retention.[3] And while the MedTech industry recognizes the importance of patient engagement, it currently lacks the expertise, infrastructure, and standardization protocols to implement sustainable patient-centric strategies. In a 2021 survey of the MedTech industry, 60% of respondents said that patient perspective is important, but only 15% had fully implemented patient engagement initiatives as part of their product development.[4]

The slow adoption of patient engagement and lack of MedTech industry expertise in this area drove the establishment of the Patient Engagement Think Tank. Over the course of one year, the Think Tank brought together patient advocates and industry players to explore how patient engagement can be successful in key aspects of the product lifecycle: clinical development, regulatory, market access, and pre- and post-commercialization.

1. Patient Engagement. Health Policy Brief. 2013.
2. Gagliardi AR, et al. "Factors constraining patient engagement in implantable medical device discussions and decisions: interviews with physicians" International Journal for Quality in Health Care, 2017, 29(2), 276–282. Accessed July 11, 2022.
3. Levitan B, et al. "Assessing the Financial Value of Patient Engagement: A Quantitative Approach from CTTI's Patient Groups and Clinical Trials Project". Therapeutic Innovation and Regulatory Science. 2018;52(2):220-229. doi:10.1177/2168479017716715.
4. de Maria, Annabel, Ouensanga, Aude. A Survey to Assess US MedTech Patient Engagement (Unpublished). Boston: Alira Health, 2021.

Results and Insights

Creating a clear, industry-standard definition of "patient engagement" was the first step in helping MedTech companies think about how to create effective and successful strategies. The Think Tank defines patient engagement as:

> *The systematic approach to ensure that patients' experiences, perspectives, and priorities are captured and meaningfully incorporated into the whole lifecycle management by establishing a bidirectional partnership between manufacturers and patients with the goal of generating products and solutions that serve patients' needs, increase access, and improve outcomes."*

With this common definition, the Think Tank was able to define the challenges, opportunities, and value propositions of patient engagement activities throughout the product lifecycle.

Clinical Development

While an overall cultural shift is occurring around the patient's role in healthcare, this shift is not happening at the same pace in clinical research. The current sentiment in clinical research is more that it is "performed on patients, not with patients," positioning patients as a "source of data" rather than an active participant in the process."[5]

However, including patient, caregiver, and advocate insights can help researchers plan and execute clinical trials with more feasible protocols, improved inclusion/exclusion criteria, and meaningful endpoints — saving time and financial resources while also yielding more impactful results and addressing unmet medical needs.

Regulatory and Market Access

Patient engagement in the regulatory process has historically been dictated by the U.S. Food and Drug Administration (FDA), whose current published guidance encourages incorporation of patient-reported outcomes, experiences, and patient preferences into regulatory filings.[6,7,8] The Centers for Medicare and Medicaid Services (CMS) and private payers, in contrast, have issued disparate policies and unclear expectations for patient engagement in market access considerations and health technology assessments (HTAs).

Even with this disconnect, there are many logical and pragmatic benefits that, though not easily quantifiable, are understood based on the increasing desire of regulators and payers to receive patient insights and data. Utilizing patient-generated insights and data can improve regulatory approval, time to market, return on investment (ROI), market access, coverage, and payment determinants.[9]

5. Sacristan J, Aguarón A, Avendaño-Solá, et al. Patient involvement in clinical research: why, when and how. Patient Prefer Adherence. 2016;10:631-640.
6. Weldring, Theresa, and Sheree M.S. Smith. "Patient-Reported Outcomes (PROs) and Patient-Reported Outcome Measures (PROMs)." Health Services Insights 6 (August 4, 2013): 61–68.
7. CMS, "Patient Reported Outcome Measures", (2021).
8. "Evolution of Patient Engagement at the FDA." FDA, July 8, 2021. https://www.fda.gov/patients/evolution-patient-engagement-fda.
9. This value proposition was workshopped over the course of the 3rd Think Tank meeting to come to a conclusion that was satisfactory for all participants.

Pre- and Post-Commercialization

MedTech's commercialization shortcomings are largely due to its reliance on only one or two patient communication channels and inadequate access to patient data. The result is limited presentation rates and lack of patient awareness around diagnosis, treatment, and quality of life improvement.[10] The COVID-19 pandemic exacerbated these pre-existing challenges, with patients postponing visits and delaying treatment.

Engaging patients through mobilization efforts, education initiatives, support systems, and partnerships with patient advocacy organizations can have widespread impact on improving patient presentation rates and awareness — benefiting patients with unmet needs as well as the commercial success of the medical device or diagnostic.

What's Next for MedTech?

Patients, as well as caregivers, providers, and patient advocacy partners, are uniquely positioned to contribute to medical device and diagnostic development. Properly executed patient engagement strategies can serve patient needs, improve outcomes, and maximize market access. It is time for leaders in MedTech to establish industry-wide expertise and infrastructure and standardize sustainable patient-centric strategies. Leveraging the strategies outlined in this playbook will help establish industry awareness of patient engagement activities throughout the product development lifecycle, accelerate patient engagement efforts, and drive commercial success while meeting significant unmet needs.

10. Bhatnagar, Sudhanshu, Shepley, Tanya. How to bridge patient engagement capability gaps for MedTech commercial success. ZS. January 11, 2021. Accessed July 20, 2022. https://www.zs.com/insights/how-to-bridge-patient-engagement-capability-gaps-for-MedTech-commercial-success.

2. Background and Playbook Initiative

Prioritizing Patient Engagement a Potential Driver for MedTech Growth

Stakeholders in the pharmaceutical industry, including sponsors, regulators, and patient groups, have recognized the importance of patient engagement more clearly in recent decades compared to the MedTech industry, as demonstrated by ongoing initiatives throughout lifecycle management. These initiatives include regulatory agencies and various public and private collaborations developing frameworks, recommendations, and other resources to engage patients and integrate patient perspectives. In fact, patient engagement activities in the pharmaceutical industry have revealed the ability to add significant financial value for sponsors by avoiding protocol amendments and/or enhancing enrollment, adherence, and retention for clinical trials and beyond.[11]

The MedTech industry has the opportunity to build an awareness of and appreciation for the value of patient engagement and patient perspectives in establishing successful business models for driving growth. Patient engagement in MedTech is definitely ripe for expansion: in our review of the industry, we identified only a few pockets of expertise, infrastructure, and implementation standards for sustainable patient-centric strategies.

The Evolving Role and Importance of Patient Engagement for MedTech and Its Stakeholders

The U.S. healthcare system is complex, including diverse stakeholders, settings, and providers. Patients and caregivers sometimes represent the only consistent factor in the health care journey, and are also important partners in the provision of care. Evidence increasingly demonstrates that engaging patients in the development process can build trust between patient and provider, and improve patient knowledge, healthcare utilization and satisfaction, treatment adherence, health outcomes, and healthcare costs.[12,13] As a result, private and public stakeholders are employing strategies to successfully engage patients in their care and treatment options.

11. Levitan B, et al. "Assessing the Financial Value of Patient Engagement: A Quantitative Approach from CTTI's Patient Groups and Clinical Trials Project". Therapeutic Innovation and Regulatory Science. 2018;52(2):220-229. doi:10.1177/2168479017716715.
12. "Patient Engagement | Health Affairs Brief." Accessed November 7, 2022. https://www.healthaffairs.org/do/10.1377/hpb20130214.898775/full/.
13. Gagliardi AR, et al. "Factors constraining patient engagement in implantable medical device discussions and decisions: interviews with physicians" International Journal for Quality in Health Care, 2017, 29(2), 276–282. Accessed July 11, 2022.

› FDA Seeks to Directly Engage Patients in Medical Device Development and Approvals

Regulators at the FDA have made strides in recent years to directly engage patients into product development and approval processes. Since 2008, initiatives and programs collecting patient-reported outcomes, patient preference information, and medical device adverse events were launched to better include patient-generated data into the regulatory process for medical device and diagnostics development. In 2015, the FDA Center for Devices and Radiological Health (CDRH) established the Patient Preference Initiative to provide guidance on incorporating the patient perspective into the medical device lifecycle.[14] That same year, the FDA announced the creation of the Patient Engagement Advisory Committee (PEAC), the first expert advisory body of patients, caregivers, and representatives of patient organizations providing patient perspective to "help inform device innovation, development, evaluation, and access."[15]

In January 2022, CDRH released final guidance concerning patient engagement in the design and conduct of medical device clinical studies targeted to the MedTech industry, FDA staff, and other stakeholders. Within the realm of medical device clinical studies, the guidance is intended to (1) help sponsors acknowledge how they can leverage patient engagement to gather relevant information from patient advisors, (2) emphasize the pros of engaging patient advisors early on in the medical device development process, (3) explain which patient engagement activities do not constitute research in the eyes of the FDA or an activity subject to FDA's regulations, and (4) address questions and misconceptions about collecting and submitting patient engagement information to the FDA.[16]

Additionally, the FDA has partnered with the Medical Device Innovation Consortium (MDIC) to improve the inclusion of patient perspectives and/or patient preferences in the development, pre-market approval, and post-market evaluation of medical devices.[17] At the same time, the FDA has focused on incorporating patient-reported outcomes (PROs) into device studies.[18,19] In "Section 5: Patient Engagement in Regulatory and Market Access," the FDA's involvement in patient engagement is explored further.

› CMS Declaration on SDM Models in Medical Device Implantation

CMS has recently demonstrated their focus on patient engagement and activation through two separate declarations pushing for shared decision-making models in the cardiovascular space. In February 2016, CMS issued a declaration requiring documented use of a "formal shared decision-making (SDM) interaction" with patients on oral anticoagulation prior to percutaneous left atrial appendage (LAA) closure therapy.[20] Two years later, CMS issued a similar declaration, requiring use of shared decision-making interaction prior to implantation of implantable cardioverter defibrillators (ICD) for certain patients.[21] These requirements highlight CMS's acknowledgement of the importance of patient engagement in clinical decision-making and of aligning treatment choices with patient values/preferences within medical devices and related procedures, specifically.[22]

14. Patient Preference Information – Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and De Novo Requests, and Inclusion in Decision Summaries and Device Labeling. U.S. Food and Drug Administration. May 18, 2015. Accessed July 11, 2022. https://www.fda.gov/media/92593/download.
15. CDRH Patient Advisory Committee. U.S. Food and Drug Administration. April 13, 2022. Accessed July 11, 2022. https://www.fda.gov/about-fda/cdrh-patient-science-and-engagement-program/cdrh-patient-engagement-advisory-committee.
16. Patient Engagement in the Design and Conduct of Medical Device Clinical Studies. U.S. Food and Drug Administration. January 26, 2022. Accessed July 7, 2022. https://www.fda.gov/media/130917/download.
17. Science of Patient Input. Medical Device Innovation Consortium. July 13, 2022. Accessed August 24, 2022. https://mdic.org/program/science-of-patient-input/.
18. Hunter NL, O'Callaghan KM, Califf RM. Engaging Patients Across the Spectrum of Medical Product Development: View From the US Food and Drug Administration. JAMA. 2015;314(23):2499–2500. doi:10.1001/jama.2015.15818.
19. Hurst FP, et al. Stimulating Patient Engagement in Medical Device Development in Kidney Disease: A Report of a Kidney Health Initiative Workshop. American Journal of Kidney Diseases. 2017;70(4):561-569. https://doi-org.ezproxy.bu.edu/10.1053/j.ajkd.2017.03.013.
20. Final Decision Memorandum for Percutaneous Left Atrial Appendage Closure (LAAC). The Centers for Medicare and Medicaid Services. February 8, 2016. Accessed July 11, 2022. https://www.cms.gov/medicare-coverage-database/view/ncacal-decision-memo.aspx?proposed=NandNCAId=281.
21. National Coverage Determination for Implantable Cardioverter Defibrillators (ICDs). The Centers for Medicare and Medicaid Services. February 15, 2018. Accessed July 11, 2022. https://www.cms.gov/medicare-coverage-database/view/ncacal-decision-memo.aspx?proposed=NandNCAId=288.
22. Knoepke CD, Allen LA, Kramer DB, Matlock DD. "Medicare Mandates for Shared Decision Making in Cardiovascular Device Placement" Circ Cardiovasc Qual Outcomes. 2019 July ; 12(7): e004899. doi:10.1161/CIRCOUTCOMES.119.004899. Accessed July 11, 2022.

› MedTech Demonstrates Proliferations in Patient-Centered Solutions:

The MedTech industry itself is also undergoing a patient-centric evolution as exemplified by the proliferation of wearable technologies, remote monitoring solutions, and digital health tools focused on engaging and empowering patients to regulate their own health.[23] Furthermore, a shift from the hospital to the home as a care setting is anticipated in certain therapy areas such as infusion therapy and dialysis.

These trends demonstrate MedTech's shift from viewing the patient as a source of data to considering the patient as an informed, equal partner in their health and treatment options. For their part, patients increasingly demand more open and transparent healthcare systems; they expect to have access to information about their health and to have an active say in their care (although individuals may vary substantially in their desired level of involvement).[24]

› The COVID-19 Pandemic as a Catalyst for Patient Engagement:

Finally, the pandemic served as a catalyst for patient engagement in MedTech. Patient empowerment exploded during the COVID-19 pandemic; as patient access was widely disrupted, patients assumed the primary role of decision-making regarding when and how to receive care. Barriers to digital forms of patient engagement prior to COVID-19, such as geography, inaccessibility, and cost, no longer represented significant hurdles, as methods such as virtual care/telemedicine and home monitoring became commonplace. It is feasible that the pandemic will have permanent effects on patient engagement and activation, although work remains to be done for marginalized groups who remain "digitally-excluded".[25]

Survey of MedTech to Validate Assumptions

To validate the assumption that MedTech has progress to make in developing and executing patient engagement strategies, in August 2021, the Patient Engagement Think Tank administered a survey of 949 MedTech industry participants to gauge their competency in and experience with patient engagement.

60% of respondents stated that including patient's perspectives in the lifecycle management process is either important or highly important. However, when asked about their current level of experience with patient engagement at their current organization, responses ranged from having no experience in patient engagement (12%), to initiation of planning (30%), to early stages of implementation (14%), to ongoing implementation (29%) and finally, fully implemented patient engagement initiatives and ongoing collection of real-world evidence (RWE) (15%).[26]

Of work currently initiated or ongoing at these organizations, the majority was reported as taking place during Evaluation and Dissemination (e.g., regulatory advice, study reporting) (53%), Research Design and Planning (e.g., clinical trial design) (50%), and Pre-Launch (e.g., patient journey insights, mobilization programs) (31%).[27]

23. Finnegan, Gary. "Is MedTech ready for patient engagement?" Patient Focused Medicine Development. November 23, 2022. https://patientfocusedmedicine.org/is-MedTech-ready-for-patient-engagement/.
24. Patient Engagement: Technical Series on Safer Primary Care. World Health Organization. 2016. Accessed July 11, 2022. https://apps.who.int/iris/bitstream/handle/10665/252269/9789241511629-eng.pdf.
25. Denegri S, Starling B. COVID-19 and patient engagement in health research: What have we learned? CMAJ. 2021 Jul 12;193(27):E1048-E1049. doi: 10.1503/cmaj.210998. PMID: 34253547; PMCID: PMC8342012.
26. de Maria, Annabel, Ouensanga, Aude. A Survey to Assess US MedTech Patient Engagement (Unpublished). Boston: Alira Health, 2021.
27. Multiple responses allowed.

Challenges preventing effective implementation of patient engagement initiatives proved to be multi-factorial, including[28]:

42%
Patient engagement inconsistent with the schedule of device/ diagnostic lifecycle management

33%
Challenges finding the "right" patients

27%
Limited internal expertise in patient engagement

25%
Defining a meaningful role with the patient

26%
Resources (including time and people)

Establishment of the Patient Engagement Think Tank Initiative

Given these current conditions in the industry and that patients are uniquely positioned to play a more active, meaningful role in medical device and diagnostic development, Alira Health and MassMEDIC created a Patient Engagement Think Tank, together with CPALs and our industry partners.

We assert that properly executed patient engagement strategies can lead to improved service of patient needs, improved outcomes, and maximized market access. For these reasons, patient engagement must be elevated to the attention of top MedTech management. The purpose of the Patient Engagement Think Tank is to identify MedTech's challenges and opportunities in implementing sustainable patient-centric strategies, with the formulation of a playbook designed for MedTech industry players outlining how to successfully incorporate patient engagement throughout lifecycle management.

28. Multiple responses allowed.

3. Methodology and Key Definitions

Organization of the Patient Engagement Think Tank Meetings

Alira Health in partnership with MassMEDIC, The Center for Patient Advocacy Leaders (CPALs), and its MedTech industry participants established a Patient Engagement Think Tank. This initiative was structured as a four-part symposium conducted over one year in which patient advocates and industry participants met quarterly to provide their insights on the challenges and opportunities for patient engagement in MedTech, based on their own experience.

First, the Think Tank reached a common definition of the lifecycle management of medical device and diagnostics, around which the proceeding Think Tank meetings would be organized (*Figure 1*). The topics of subsequent Think Tank meetings were grouped by phase to enable sequential focus on the device and diagnostic lifecycle, as illustrated in Table 1. Meetings were organized into plenary and break-out sessions. During plenary sessions, findings from secondary and primary research were shared by the Alira Health team with the members of the Think Tank. During the break-out sessions, Think Tank participants discussed the contents of the plenary session in smaller groups to discuss and debate findings and bring clarity and meaning to concepts and processes.

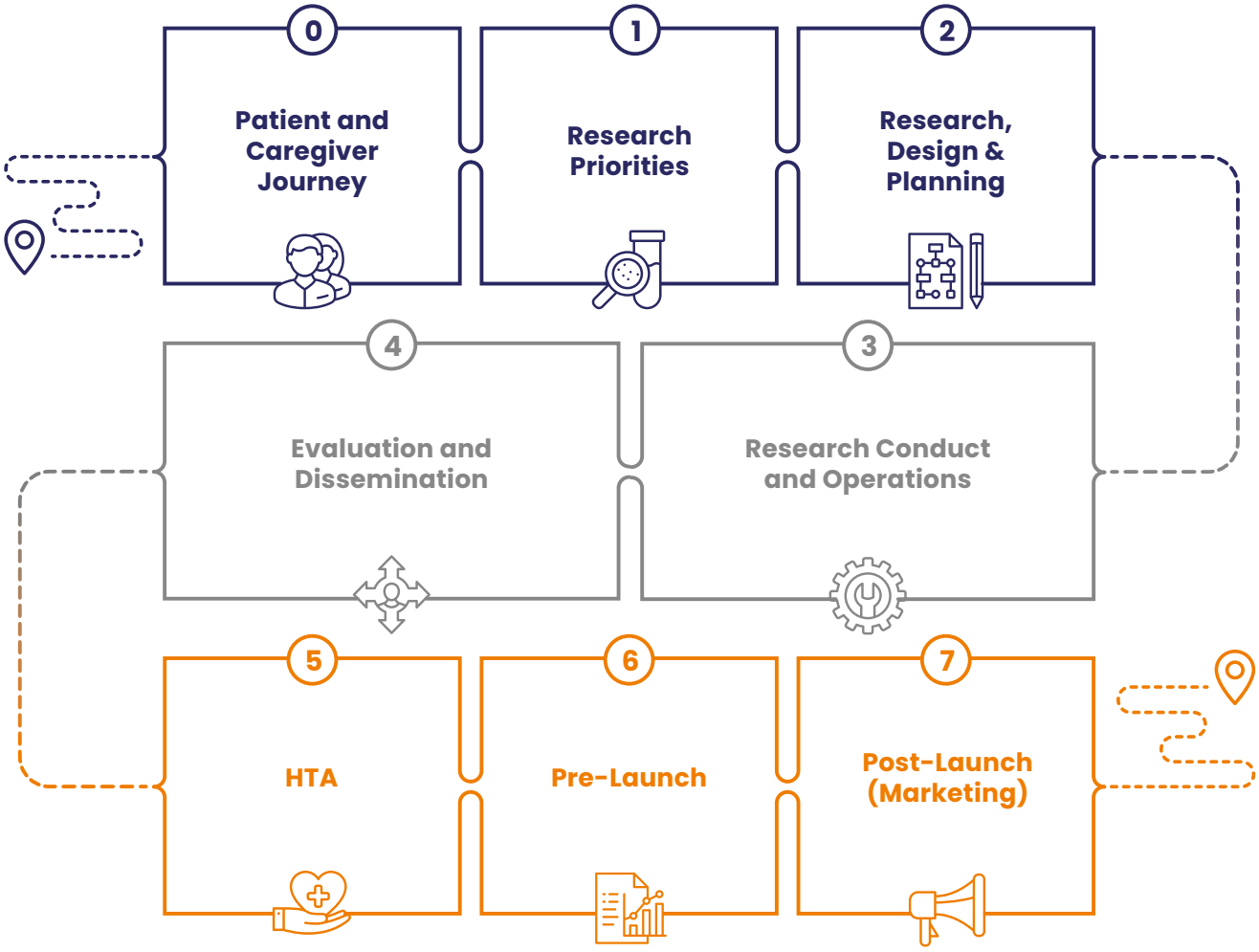


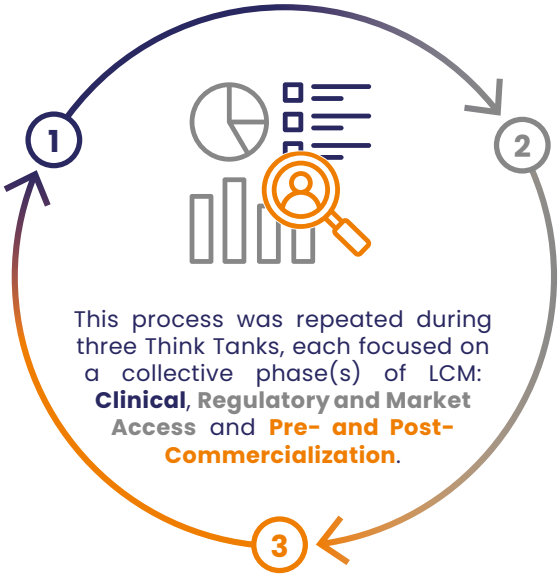
Figure 1. *Definition of MedTech Lifecycle Management as agreed upon by the Patient Engagement Think Tank*

MEETING	DATE	TTOPIC
Think Tank 1	September 2021	Introduction, Defining the State of Patient Engagement (PE) in MedTech and analyzing the results of the survey
Think Tank 2	January 2022	PE in Clinical Phases (*Figure 1, 0 – 3*)
Think Tank 3	March 2022	PE in Regulatory and Market Access (*Figure 1, 4 and 5*)
Think Tank 4	June 2022	PE in Pre- and Post- Commercialization (*Figure 1, 6 and 7*) Guest Speaker: **Michelle Tarver**, *MD, PhD, FDA Director of Patient Science & Engagement Program (CDRH) on FDA's commitment, role and activities in PE in clinical investigations*

Table 1. *Organization, timing, and content of Patient Engagement Think Tank Meetings*

Project Methodology

The plenary session content was established through a uniform methodology for each Patient Engagement Think Tank meeting (*Figure 2*). First, the **pains**, or challenges, associated with successfully executing patient engagement strategies for the corresponding phase of the lifecycle management (LCM) were defined. Secondly, the **gains**, or potential benefits, derived as a result of successful patient engagement strategies were defined as well. Thirdly, the **value proposition** describing the potential benefits of prioritizing patient engagement along the device/diagnostic lifecycle was defined. To conclude the exercise, for each phase, **activities** were defined to execute patient engagement strategies along each phase of the device/diagnostic lifecycle. To create an overall conceptual framework for patient engagement, the session content and process activities for each phase were developed to be consistent with the concepts and definitions of the previous phase and to build to the next phase.



1. Define and prioritize the **pains** associated with Patient Engagement
2. Define and prioritize the **gains** associated with successful Patient Engagement
3. Define **value propositions** for MedTech to incorporate PE into device/diagnostics lifecycle and associated activities to achieve them

Figure 2. *Methodology employed to define patient engagement strategies along the device/diagnostics lifecycle*

Developing the Playbook

This playbook represents the output of four Patient Engagement Think Tank meetings, each focused on collective stages of the device/diagnostics lifecycle management. It was written by a cross-functional team of strategy consultants and patient engagement experts at Alira Health from July through November 2022. The playbook was shared with all participants of the Think Tank to receive their input and commentary prior to publication in January 2023.

Key Definitions

The following terminology and abbreviations are referenced throughout the playbook:

› Device/Diagnostic Lifecycle Management (LCM): the series of all phases in the life of a medical device or diagnostic, spanning from clinical (*Figure 1, 0 – 3*), to regulatory (*Figure 1, 4*), market access activities (*Figure 1, 5*), and finally pre- and post-commercialization (*Figure 1, 6* and *7*).

› Gains: the potential benefits derived from the results of an activity. In the context of this initiative, gains describe the potential benefits to MedTech participants and their stakeholders (including patients and caregivers) in successfully incorporating patient engagement strategies across the medical device/diagnostics lifecycle.

› Pains (or "pain points"): a persistent or recurring problem (as with a product or service) that causes frequent inconvenience or annoyance. In the context of this initiative, pains describe the persistent or recurring problems encountered by MedTech participants in trying to execute on patient engagement strategies across the medical device/diagnostics lifecycle.

› Patient Engagement (PE): a systematic approach to ensure that patients' experiences, perspectives, and priorities are captured and meaningfully incorporated into the medical device/diagnostics lifecycle by establishing a mutually beneficial partnership between manufacturers and patients to generate products and solutions that serve patients' needs, increase affordable access, and improve outcomes and/or quality of life (QoL).[29]

› Patient Preference Information (PPI): captures the value that patients place on the aspects of a medical device, accounting for the benefits and risks that come with using that device or treating a condition.

› Patient Reported Outcomes (PROs): insights directly reported by a patient, without interpretation by a clinician, pertaining to the patient's health, QoL, or functional status associated with healthcare or treatment. The FDA identifies three key domains for PROs: health-related QoL (including functional status), systems and symptom burden (e.g., pain, fatigue), and health behaviors (e.g., smoking, diet, exercise).

› Patient Reported Experience Measures (PREMs): capture a patient's experience of receiving care, specifically the patient's perception of what happened during their care encounter and how it happened.

› Quality of Life (QoL): the patient's ability to enjoy normal life activities, as measured in PROs.

› Real World Evidence (RWE): the clinical evidence derived from analysis of real-world, often patient-generated data regarding the usage and potential benefits or risks of a medical device or diagnostic.

› Value Proposition (VP): a promise of value, typically stated by a company, that summarizes how the benefit of its product or service will be delivered, experienced, and acquired. In the context of this initiative, the term "value proposition" describes the promise of value for MedTech participants should they successfully integrate patient engagement strategies into the medical device/diagnostics lifecycle.

29. This definition of patient engagement was workshopped over the course of the four Think Tank meetings to come to a definition that was satisfactory for all participants.

4. Patient Engagement in Clinical Development

Background and Key Intelligence Questions

While a cultural shift is occurring in the way we understand the patient's role in healthcare, some patient engagement experts have not yet observed a similar shift in clinical research. This may be because patient participation in clinical research is not considered as critical as their participation in their own medical care. The cultural mentality of clinical research is that it is "performed on patients, not with patients," positioning the patient as a "source of data" rather than an active participant in the process."[30]

Patient, caregiver, and advocate insights can help researchers plan and execute clinical trials with more feasible protocols, improved inclusion/exclusion criteria, and meaningful endpoints, ideally saving time and financial resources and yielding more impactful results. This work is supported by the research model called Participatory Action Research (PAR). PAR differs from most other approaches to public health research because it is based on reflection, data collection, and action that aims to improve health and reduce health inequities through involving the people who, in turn, take actions to improve their own health.[31]

The following Key Intelligence Questions served as guides to structure this analysis:

1. What barriers exist from the perspective of patients, caregivers, advocates, industry participants, and others, in encouraging patient participation in clinical trials?

2. How can patient participation in clinical trials be optimized?

3. How can and should patients, caregivers, and/or advocates be engaged in the design of clinical trials?

4. What study endpoints are most important to patients and caregivers?

5. How can and should PROs, PREMs, and PPI be incorporated into study design?

Pains

Barriers to the successful implementation of patient engagement during clinical phases center around gaps in knowledge/understanding, perception, infrastructure, and logistics and can be segmented into those related to patients, clinical trial sponsors, and MedTech itself[32,33,34]:

> Patient-Related

- Patient perception that their input is not valued
- Low adherence to clinical trial protocols
- High drop-out rates (impacting timeline and cost)

> Sponsor/Site Investigator-Related

- Sponsors' limited awareness, resources, and time to participate in patient engagement activities
- Site investigators' reluctance to allow sponsors to engage with patients except as study/research participants

30. Sacristan J, Aguarón A, Avendaño-Solá, et al. Patient involvement in clinical research: why, when and how. Patient Prefer Adherence. 2016;10:631-640.
31. Baum, MacDougall, Smith. Journal of Epidemiology and Community Health, October, 60(10): 854–857, 2006.
32. Pains were drafted by Alira Health through primary and secondary research and internal expertise and were revised during the Think Tank with input by all Think Tank members to reach this list.
33. Patient Engagement in the Design and Conduct of Medical Device Clinical Studies. U.S. Food and Drug Administration. January 26, 2022. Accessed July 7, 2022. https://www.fda.gov/media/130917/download.
34. Maximizing Patient Input in the Design and Development of Medical Device Clinical Trials. Medical Device Innovation Consortium. April 5, 2021. Accessed July 5, 2022.

› MedTech-Related

- Costly resubmission of protocols
- Poorly designed clinical trials
- Poorly selected/designed clinical endpoints
- MedTech companies lacking defined PE role and internal practices working in silos
- Challenges finding patient advisors knowledgeable in clinical investigation methodology
- Challenges determining which patient advisors or organizations should be engaged
- Incorrect perception that the FDA does not allow patient engagement in the design and conduct of clinical investigations

Gains

Although the ethical benefit of engaging patients in clinical phase has been demonstrated, there are also many pragmatic reasons that could feasibly improve clinical trial efficiency and outcomes. These gains include potential impact on clinical trial efficiency and impact, patient and industry perception, and even regulatory submissions:[35,36,37]

› Clinical Trial-Related

- Improved recruitment, accrual, and retention can lower trial costs, and reduce the length of time/ resources a trial takes to administer
- Fewer costly protocol amendments and resubmissions to ethical committees which delay study timeline
- Patient and family insights that can be leveraged for post-approval activities such as marketing and public communications regarding the product or communication with payers about product value

› Patient-Related

- More beneficial and impactful outcomes data from the patient and family perspective
- Patients and families experience increased satisfaction due to more effective products that address the metrics they care about
- More compelling regulatory submissions and products that are streamlined for the approval processes
- Patients and families are more educated due to their involvement

› Industry-Related

- Alignment between the industry and the patients it serves on the purpose and benefits of patient engagement, establishing patients and families as active stakeholders
- Uniformity in institutional standards for when and how patients and families should engage in clinical trials for medical devices, lending to increasing awareness and appreciation

35. Pains were drafted by Alira Health through primary and secondary research and internal expertise and were revised during the Think Tank with input by all Think Tank members to reach this list.
36. Patient Engagement in the Design and Conduct of Medical Device Clinical Studies. U.S. Food and Drug Administration. January 26, 2022. Accessed July 7, 2022. https://www.fda.gov/media/130917/download.
37. Maximizing Patient Input in the Design and Development of Medical Device Clinical Trials. Medical Device Innovation Consortium. April 5, 2021. Accessed July 5, 2022.

Value Proposition

The Think Tank concluded that successful patient engagement strategies, starting from the earliest clinical phases, can have a widespread impact on patients, clinical trials, and MedTech itself, resulting in a device or diagnostic that successfully addresses the unmet needs of patients and their caregivers/families.

The Think Tank articulated the value proposition for patient engagement in clinical phases as follows:

> *Engage patients during medical device clinical trials to design studies that reflect what they value and feel, facilitate their active participation, and address shortcomings of existing solutions, thereby enabling the collection of impactful data that resonates with patients and families, the execution of efficient trials, and the resolution of impactful unmet needs.*[38]"

Activities

As indicated in *Figure 1*, the Clinical Phase of lifecycle management is divided into four subphases: Patient and Caregiver Journey, Research Priorities, Research Design and Planning, and Research Conduct and Operations.

> Phase 0. Patient and Caregiver Journey

Clinical Activity #1: Identification and analysis of the patient journey and caregiver journey

Before starting clinical research, it is critical to first map and analyze the patient journey, as well as the caregiver/family journey (as applicable). In identifying the patient/caregiver journey, the trial sponsor can better understand when, where, and how to reach and engage the target patient population. Critical endpoints to evaluate include:

- How the patient flows through their care continuum (e.g., referral patterns, settings in scope, timing)
- Which stakeholders (including caregivers, healthcare providers, advocacy organizations, others to be identified) are involved in the patient's care and their respective roles/degree of involvement
- Emotional pathway when dealing with the disease
- What treatment options are currently available to the patient
- How and when the patient goes about obtaining their treatment
- Channels through which the patient is engaged/activated/educated, etc. regarding their disease state, treatment options, etc.
- Others, to be identified based on disease state, treatment type, etc.

For caregivers/families, it could be important to understand:

- The role of the caregiver/family in patient care and support
- How they interact with the patient and other clinical and non-clinical stakeholders
- How and when they play a role in clinical decision-making
- How caregivers/families receive education/support/activation in their role as caregivers and advocates

This research should be performed through primary sources including engagement activities with patients, caregivers/families, healthcare professionals, and advocacy organizations. Secondary sources such as medical societies and guidelines should also be consulted. The information gleaned from this research can also be useful in later pre- and post-commercialization stages.

38. This value proposition was workshopped over the course of the second Think Tank meeting to come to a conclusion that was satisfactory for all participants.

> Phase 1. Research Priorities

Clinical Activity #2: Identification and prioritization of patient/caregiver unmet needs

In the current state of patient engagement in MedTech, most research questions in the pursuit of device development are typically posed from the medical or regulatory perspective, with the intention of meeting clinical and regulatory expectations.[39] While these endpoints are undoubtedly important, by using this approach, MedTech devices and diagnostics are at risk of failing to address the true unmet needs from the patient and caregiver perspective. Failure to engage patients while setting the research agenda could result in the following:

- Treatments that **fail** to address true patient need could **continue** to be used due to lack of evidence
- Treatments that **successfully** address true patient need could **fail** to be used due to lack of evidence
- Treatments that **fail** to address true patient need could continue to be marketed, resulting in challenges with treatment adherence[40]

Organizations such as the Patient-Centered Outcomes Research Institute (PCORI), the Cochrane Centre, Core Outcomes Measures in Effectiveness Trials (COMET), Outcome Measures in Rheumatology Clinical Trials (OMERACT), and Clinical Trials Transformation Initiative (CTTI) are dedicated to establishing meaningful partnerships with patients, advocates, and caregivers in accelerating patient-centered outcomes research (see Resource Appendix).

To begin setting research priorities, it is first important to identify and prioritize patient/caregiver unmet needs. These may pertain to:

- Failure to fully understand their disease state and/or treatment options (due to lack of participation, access, or evidence)
- Perceived gaps in evidence on existing treatment options, their efficacy, their impact, etc. (e.g., in asthma, unaddressed uncertainties around long-term use of steroids is a principal shared concern)
- Failure of existing treatment/devices to address their most pertinent symptom(s)
- Others, to be identified

It is critical to engage patients and their caregivers directly to understand and then prioritize the persistent unmet needs they experience in their disease state. Patients and caregivers alone have the ability to explain the details and nuances surrounding the management of their disease state and the ways it could be more effective.

Clinical Activity #3: Setting the research agenda

Before moving on to clinical trial design, it is necessary to integrate patient/caregiver unmet needs and values into the research agenda. Unmet needs should be matched with research priorities to the extent they are feasible to address. Additionally, this can be done by defining patient relevance added-value endpoints and outcomes for the study. Defining patient needs and values before research design and planning can enable the researcher to ultimately collect data that is most important for the patient/caregiver, which could have a positive downstream impact on utilization and, potentially, regulatory review.

39. Sacristan. Patient involvement in clinical research. Patient Prefer Adherence. 2016;10:634.
40. Tallon, D, Chard, J, Dieppe, P. Relation between agendas of the research community and the research consumer. The Lancet. 2000; 355, 2037-2040.

> Phase 2. Research Design and Planning

Clinical Activity #4: Clinical trial design

A. Defining the target population: In selecting the trial population, it is critical to first recognize health inequities, both on a broad scale and specific to the disease or therapy area in scope. The selection of the target population must be representative of a disease state from a socioeconomic, racial, and ethnic perspective to understand potential differences in treatment effects and outcomes based on these factors. It may also be necessary to remove barriers to participation for disadvantaged groups through measures such as providing convenient locations to avoid long commutes to trial sites and appointments at times outside of the workday to avoid financial burden. Ensuring such practical considerations such as covering travel expenses, providing support for patients and families, offering mobility solutions, etc., are feasible measures to ensure the target population is selected in a patient-centric manner.

B. Involving patients and/or patient advocacy organizations: It is vital to involve patients and/or patient advocacy organizations when designing clinical trial protocols to adequately select relevant patient outcomes, especially QoL, PROs, PREMs, and PPI. Additionally, patient advocacy organizations can provide input to the trial steering committee and Data and Safety Monitoring Board (DSMB) in order to consider the patient perspective when defining inclusion and exclusion criteria, assessing the risk/benefit balance for the trial, and establishing protocol logistics and adherence measures. Ultimately, involving patient advocacy organizations can help achieve more relevant clinical trial results, while also enhancing patient access, safety, and retention.[41]

> Phase 3. Research Conduct and Operations

Clinical Activity #5: Informing patients and caregivers

It is vital that all patients and caregivers are provided proper information before, during, and after the clinical trial. Before the trial commences, patients and caregivers must receive all the information required to make an educated decision about whether to participate in the trial. The information presented must be comprehensive, relevant, and easy to interpret; this is often a challenge because informed consent documents and patient information sheets can be difficult to read and understand. Patients can even be involved in the process of designing the informed consent documents in order to make them more understandable.

Likewise, it would be beneficial for patients and caregivers to be involved in ethical research discussions to ensure clarity.[42] During a clinical trial, it is also important that proper lines of communication are established with patients and caregivers to ensure awareness concerning updates to safety information, protocols, or amendments.

Finally, after the clinical trial, patients and caregivers should be provided with the most significant results, associated implications, anticipated long-term effects, and how the results will be communicated broadly to relevant stakeholders. The transparent communication of aggregate results will facilitate patient satisfaction from knowing their contributions had an impact, regardless of the direction of the trial results.[43,44]

41. Sacristan. Patient involvement in clinical research. Patient Prefer Adherence. 2016;10:632.
42. Sacristan. Patient involvement in clinical research. Patient Prefer Adherence. 2016;10:634.
43. Shalowitz DI, Miller FG. Disclosing individual results of clinical research: implications of respect for participants. JAMA. 2005;294:737–740.
44. Sacristan. Patient involvement in clinical research. Patient Prefer Adherence. 2016;10:635.

Clinical Activity #6: Assessing patients' experiences

Understanding patients' experiences through methods like surveys is a necessary activity to perform during the clinical trial process in order to make positive adaptations to the clinical trial protocols, if necessary. Quite often, patients are only asked for their opinions at the end of the clinical trial, which limits the ability to implement real-time modifications to improve patient experience. This typically stems from the sponsoring organization's concern that collecting negative feedback may expose weaknesses in the trial.[45] However, improvements made throughout clinical trials tend to lead to greater patient adherence and limit potential dropouts, which are essential determinants for the validity of clinical trial results.[46]

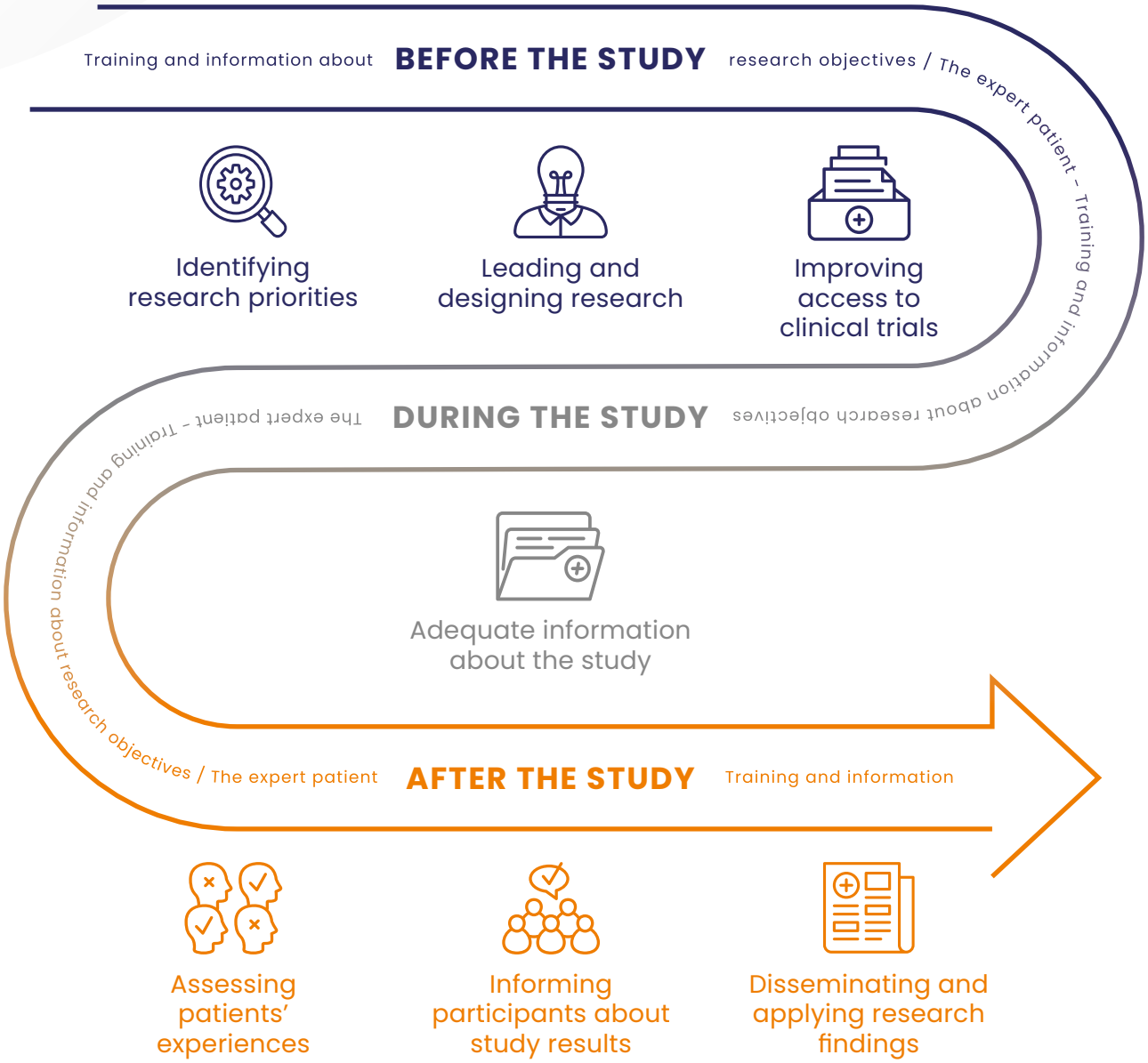


Figure 3. *How to engage patients in clinical research*[15]

45. Lee, J., et al. Patient engagement surveys in clinical trials: dos, don'ts and how they help. Applied Clinical Trials. 2018.
46. Tantoy, I.Y., et al. Patient satisfaction while enrolled in clinical trials: a literature review. Patient Experience Journal. 2021;8;125.

5. Patient Engagement in Regulatory and Market Access

Background and Key Intelligence Questions

Patient engagement in the regulatory process for medical devices and diagnostics has historically been dictated by the FDA, which has evolved its patient engagement guidance and initiatives since the mid-1990s. The evolution of the FDA's efforts to include patients in regulatory processes began with the appointment of patient representatives to advisory committees in 1993, continued with encouraging patients to report device errors in the mid-2000s, and moved to developing frameworks for incorporating PPI in decision-making in the mid-2010s. Today, the FDA engages patients through the Patient Engagement Collaborative and Patient Resources sections of the FDA website as well as through the Patient Engagement Advisory Committee, while publishing guidance for involving patients in clinical development and encouraging incorporation of patient-reported outcomes, experiences, and patient preferences into regulatory filings.[47,48,49]

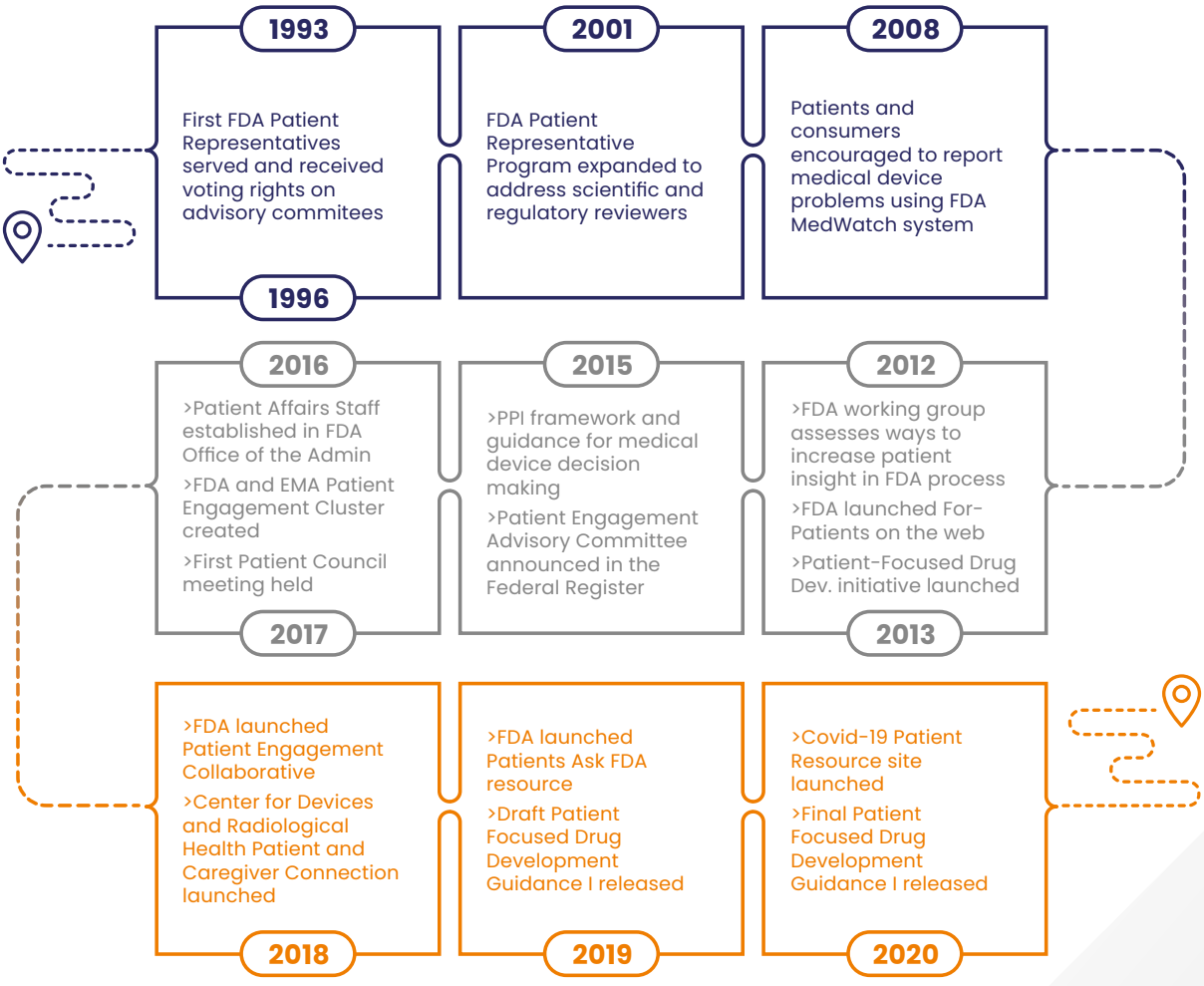


Figure 4. *Timeline of FDA Patient Engagement Initiatives*

47. Weldring, Theresa, and Sheree M.S. Smith. "Patient-Reported Outcomes (PROs) and Patient-Reported Outcome Measures (PROMs)." Health Services Insights 6 (August 4, 2013): 61–68.
48. CMS, "Patient Reported Outcome Measures," (2021).
49. FDA, "Evolution of Patient Engagement at the FDA".

CMS and private payers, in contrast, have issued disparate policies and unclear expectations relative to the FDA for patient engagement and patient-generated data in market access considerations and HTAs. However, opportunities and potential benefits exist to include patient data and patient voices in coverage and payment decision-making. In the coverage and payment decision-making process, CMS will issue awareness about public hearings in which patients or patient representatives can share input on the medical device under evaluation. Patients can be brought before these public meetings by MedTech companies or can attend on their own volition. Companies tend to prioritize patients that are well-versed in concepts of patient engagement, and often approach patient advocacy groups to identify patients for testimony. Sharing of insights by patients in this format enables the inclusion of their perspectives, experiences, and preferences into coverage and payment decisions. Stakeholders indicate that MedTech industry participants have successfully pursued this channel to elevate patient voices and needs to payers during coverage and payment decisions.

To better understand the FDA's sentiment and expectations surrounding patient engagement in regulatory processes, and to clarify payer consideration of patient voice and patient-generated data, the following Key Intelligence Questions served as guides to structure this analysis:

1. What tangible impacts from FDA guidance on patient engagement have been seen by industry participants, policy makers, patient advocacy groups, patients, and others?

2. What value does the FDA place on patient outreach and community activation? (e.g., patient-driven efforts to convince FDA of product need, co-creation of a study protocol with patients)

3. To what extent do these initiatives speed regulatory submission and approval?

4. To what extent are patients or patient advocacy groups involved in HTAs performed by payers in the US?

5. What value/emphasis do payers place on patient preference studies and patient-generated data in coding, coverage, and payment decisions?

6. What impact do patient engagement initiatives have on payers' decision-making processes, both in terms of speed to decision and outcome?

Responses to these questions informed the identification of the pains and gains within the regulatory and market access phases, and were further leveraged to develop patient engagement activities that could address the associated pains and realize the asserted gains.

Pains

MedTech patient engagement in regulatory and market access phases is challenged by MedTech's limited understanding of the benefits of these activities and a misaligned perception of regulator and payer sentiment toward patient engagement. These specific challenges can be segmented based on their association to either regulatory or market access processes:[50,51,52]

> Regulatory-Related

- Lack of clarity for MedTech companies on the proven benefits of engaging patients during regulatory phase (i.e., lack of evidence of its impact on FDA submission timeline or outcome)
- Perception of high monetary and time cost and unproven ROI from generation of PROs, PREMs, and PPI during regulatory and market access phases
- Insufficiently understood FDA expectations towards evaluation of patient outcomes, experiences, and preferences in the regulatory phase
- Variability in submission expectations and requirements, depending on device classification (e.g., implantable vs. continuous monitoring devices)
- Additional potential for FDA rejection if patients engage negatively with the medical device or the associated changes to QoL, symptoms, or impacts on behavior
- FDA and MedTech bias toward the most well-informed and active patient advocates, preventing representation of all patient populations in regulatory evaluation and processes

> Market Access-Related

- Lack of payer guidance on the role of patient engagement during market access processes, including the extent to which patients can be involved in the HTA process
- Lack of understanding of the extent to which payers value patient engagement activities and RWE, including its potential to impact HTA and generate ROI through more favorable market access
- Fragmented payer landscape and payer lack of acceptance of patient engagement data in coverage and payment decisions, including PROs, PREMs, and PPI
- Poor visibility into the acceptance and value of patient engagement for private payers
- Variability in payers' expectations, acceptance, and utilization of PROs, PREMs, and PPI
- Rigidity of payer process, including rigidity in parameters measured, but not excluding those collected through patient engagement

Gains

Patient engagement activities in regulatory and market access processes for a medical device or diagnostic have clear ethical benefits to patients by elevating their experiences and preferences to regulator and payer stakeholders, in turn generating greater transparency and trust in these entities. For MedTech, these activities can generate many logical and pragmatic benefits that, though not easily quantifiable, are understood based on regulator and payer's increasing desire to receive PROs, PREMs, and PPI as part of submissions and filings. These benefits can be segmented based on their association to either regulatory or market access processes:[53,54,55]

50. Hurst. Stimulating Patient Engagement in Medical Device Development in Kidney Disease, 563.
51. Pains were drafted by Alira Health through primary and secondary research and internal expertise and were revised during the Think Tank with input by all Think Tank members to reach this list.
52. Weldring, Theresa, and Sheree M.S. Smith. "Patient-Reported Outcomes (PROs) and Patient-Reported Outcome Measures (PROMs)." Health Services Insights 6 (August 4, 2013): 61–68. https://doi.org/10.4137/HSI.S11093.
53. Hurst. Stimulating Patient Engagement in Medical Device Development in Kidney Disease, 563.
54. Pains were drafted by Alira Health through primary and secondary research and internal expertise and were revised during the Think Tank with input by all Think Tank members to reach this list.
55. Weldring, Theresa, and Sheree M.S. Smith. "Patient-Reported Outcomes (PROs) and Patient-Reported Outcome Measures (PROMs)." Health Services Insights 6 (August 4, 2013): 61–68. https://doi.org/10.4137/HSI.S11093.

> Regulatory-Related

- Further realization of the gains achieved in clinical phases, including more transparent medical device lifecycle management, potentially lower budget required for approval, shorter clinical trials, and increased chance of approval
- Potential for favorable review by the FDA resulting from provision by the MedTech company of PROs, PREMs, PPI, or patient-generated data indicating favorable reception among patients
- Expedited review by the FDA in response to patient engagement campaigns executed or underway by the MedTech company or in association with patient advocacy groups
- Increased patient trust in the regulatory process and consequently greater trust in the product's efficacy and safety, benefiting both the MedTech company's device and the credibility of the FDA
- Potential for more accurate and complete adverse events reporting when patients can directly engage with regulators, benefiting the safety of the medical device
- Overall improved patient sentiment that the MedTech company has valued their input, benefiting the perception of the MedTech company among patients and the broader public

> Market Access-Related

- Potential for market access advantages due to generation of patient engagement data such as PROs, PREMs, and PPI. Potential advantages include:
  - Establishing and sustaining competitive reimbursement
  - Convincing payers of efficacy and cost savings associated with the device
  - Opportunity to increase available data for payer measurements of patient QoL and burden
- Record of patient adherence for relevant devices may convince payers of superior value and improve the access outlook for the device, especially applicable for value-based care
- More compelling story for coverage if payers receive insights in the form of testimony, communication, and letters directly from patients, providers, family members, and others associated with the patient
- Greater patient impact directly on the market to achieve access to a medical device they desire or otherwise need to improve their QoL or lessen their burden

Value Proposition

The Think Tank concluded that successfully engaging patients during the regulatory and market access phases can improve approval, time to market, and ROI outlook for MedTech while expanding access for patients.

The Think Tank articulated the value proposition for regulatory and market access phases as follows:

> *Engage patients during medical device regulatory and market access processes by incorporating the patient voice to elevate patient needs to the attention of the FDA, payers, and other policy makers. Utilize patient-generated data and insights to optimize regulatory approval, time to market, ROI, market access, coverage, and payment determinants.*[56]»

56. This value proposition was workshopped over the course of the third Think Tank meeting to come to a conclusion that was satisfactory for all participants.

Activities

> Phase 4. Evaluation and Dissemination

Regulatory Activity #1: Assessment of patients' challenges and opportunities provided by the MedTech solution that are worth consideration from regulators

The FDA has increasingly issued guidance reflecting its desire to evaluate and consider patient voice in regulatory submissions. Patient experiences and preference information increasingly contributes to FDA submission evaluation, particularly when the information reflects a significant challenge addressed or opportunity created by the product under evaluation. Consequently, it is critical to understand patient challenges that may be addressed by the product seeking approval, and learn how the product could address these challenges directly from patients, caregivers, healthcare providers, and patient advocacy groups. Collective engagement before regulatory submission can result in better representation of patient needs, improved sentiment by the FDA, and potentially favorable review of the regulatory submission.

Regulatory Access Activity #2: Involvement in the conformity assessment (with/without notified body)

Conformity assessment is essential to MedTech regulatory submissions, promoting patient safety by demonstrating that specified requirements of a device or diagnostic are fulfilled.[57] Thorough conformity assessment can include sampling and testing, inspection, supplier's declaration of conformity, certification, and management system assessment. Though not required by regulatory bodies, involving patients in conformity assessments offers the opportunity to improve visibility for patients into regulatory submissions, which over time can improve patient trust in the product's efficacy and safety, benefiting both the MedTech company's device and the credibility of the FDA.

> Phase 5. Health Technology Assessment

Market Access Activity #3: Assessment of PROs, PREs and PPI and PPI

Critical in early stages of HTA and pharmacovigilance for medical devices and diagnostics is the consideration by MedTech of PPI and analysis of PROs and PREMs. According to CMS, PROs can be identified by three key domains: i) health-related QoL (including functional status), ii) symptoms and symptom burden, and iii) health behaviors.[58] Thoughtful selection of PRO measures capable of evaluating PROs across these domains can bolster regulatory submissions while increasing trust among patients in the device and the regulatory process. Similarly, PPI accounts for perspectives on benefits and risks associated with a medical device or diagnostic, reflecting patients' desires. Collectively, PROs, PREMs, and PPI can improve chances for favorable or expedited review by the FDA resulting from positive outcomes and reception among patients.

Market Access Activity #4: Design a RWE strategy, including QoL and PROs (data collection, logistics, evaluation, follow-up, and amendments, etc.)

PROs, PREMs, and PPI remain relevant after regulatory approval has been granted. Continued efforts to capture patient-generated data as well as QoL metrics can support efforts to establish and sustain competitive reimbursement, convince payers of efficacy and cost savings associated with the device or diagnostic, and increase available data for payer measurements of patient QoL and burden. However, realizing a successful RWE strategy requires consistent engagement not only with patients, but with caregivers, healthcare providers, and patient advocacy partners to ensure the patient voice is fully represented. Similarly, RWE strategies for collection of QoL and patient-generated data should coincide with the pre- and post- commercialization activities discussed later in the playbook.

57. Center for Devices and Radiological Health. "Standards and Conformity Assessment Program." FDA. FDA, November 30, 2021.
58. CMS, "Patient Reported Outcome Measures," (2021).

Market Access Activity #5: Engagement of patients and elevation of patient voices to CMS public hearings

Finally, the ability to engage patients with CMS during public hearings pertaining to medical device and diagnostic evaluations presents a straightforward yet effective way to elevate patient voices directly to public payer decision makers. During the coverage and payment decision-making process, CMS will issue awareness about these public hearings to extend the opportunity to medical device and diagnostic sponsors to have patient experiences and preferences considered in the process. Sharing of insights by patients in this format enables the inclusion of their perspectives, experiences, and preferences into coverage and payment decision-making. Increased utilization of this opportunity to amplify patient voices by MedTech could help realize the benefits of patient engagement during these phases of lifecycle management.

6. Patient Engagement in Pre- and Post-Commercialization

Background and Key Intelligence Questions

Similar to the previous phases in the lifecycle management of medical devices and diagnostics, patient engagement initiatives within the commercialization phases lag across the MedTech industry. For instance, many MedTech companies rely solely on one or two communication channels to reach patients or have inadequate access to patient data, thereby limiting a patient's awareness to seek medical attention and the possibility of diagnosis, treatment, or QoL improvement.[59]

These communication shortcomings in the commercialization phase were further illuminated by the COVID-19 pandemic as patients postponed visits to healthcare providers or delayed treatment. In a 2020 survey conducted by Johnson & Johnson, more than two-thirds (68%) of Americans indicated that they or another person in their household delayed or cancelled healthcare services during the COVID-19 pandemic.[60]

As such, the two dimensions of focus for patient engagement initiatives during the commercialization phases are:

1. Presentation rate: Degree of patient appearance at the corresponding specialist's office

2. Likelihood of diagnosis, treatment, or quality of life (QoL) improvement: Probability of a patient being diagnosed, treated, or having QoL improvements from a medical device or diagnostic

The following Key Intelligence Questions served as guides to structure the analysis for the commercialization phases:

1. What obstacles exist that limit patient presentation rate and likelihood of diagnosis, treatment, or QoL improvement for patients?

2. Which activities should be considered to improve patient presentation rate and likelihood of diagnosis, treatment, or QoL improvement for patients?

3. What is the appropriate method to identify suitable activities?

4. How should a MedTech company develop a patient engagement activity?

59. Bhatnagar, Sudhanshu, Shepley, Tanya. How to bridge patient engagement capability gaps for MedTech commercial success. ZS. January 11, 2021. Accessed July 20, 2022. https://www.zs.com/insights/how-to-bridge-patient-engagement-capability-gaps-for-MedTech-commercial-success.
60. Johnson & Johnson Medical Devices Companies Elective Surgery Survey. Accessed July 20, 2022.

Pains

As discussed above, impediments to successful patient engagement in the commercialization phase of lifecycle management can be categorized within the two dimensions previously stated: 1. Presentation rate, and 2. Likelihood of diagnosis, treatment, or QoL improvement.

> Presentation Rate

- Nonhomogeneous population including cultural/ethnic backgrounds and timing of disease onset

  Depending upon the disease, the characteristics of the patient population (e.g., cultural and ethnic backgrounds, timing of disease onset) may differ which can present challenges for patients interacting with healthcare providers.
- Limited healthcare system capabilities to reduce time to diagnose/clear referral process

  Contingent upon type of healthcare system and/or health insurance coverage, patients may require referrals in order to see a healthcare provider which can delay the visit or deter a patient from seeing a specialist.
- Lack of understanding by the patient and healthcare provider

  A patient may not feel inclined or comfortable seeing a healthcare provider due to the physical and/or emotional response to the symptoms they are experiencing, while healthcare providers may have unconscious bias and not fully acknowledge all the social determinants of health.
- Lack of insurance coverage and/or affordable access to care

  Whether or not a patient has insurance coverage could restrict a patient from appearing at a healthcare provider's office. Likewise, a patient may have coverage, but the cost of care is not affordable, thereby limiting a patient's ability to see a specialist.

> Likelihood of Diagnosis, Treatment, or Quality of Life Improvement

- Deficiency of centers for excellence

  The availability of centers for excellence within a patient's region becomes important when it comes to receiving the highest quality of care. Medical professionals can ensure that the device or diagnostic is being used correctly and address any areas of concern.
- Lack of device or diagnostic effectiveness

  A concern during the post-commercialization phases is reduced device or diagnostic effectiveness, therefore limiting the benefits and likelihood that a patient will be diagnosed or treated successfully within the given therapeutic area.
- Potential side effects

  Unsuspected side effects for patients can reduce the likelihood of physician recommendation. It can also impact patient adherence which negatively influences related outcomes and the likelihood of treatment success.
- Ease of use difficulties

  Issues surrounding ease of use when patients self-administer and/or use the medical technology can impact success and benefits of the given treatment. As a result, patient experience can be hampered, and in some instances, put the patient's health at risk.

Gains

Identifying appropriate activities and gradually executing upon those activities with the appropriate structure will enable MedTech players to overcome the identified pain points within the commercialization phases, thereby generating the following gains:

1. Higher presentation rates
2. Higher likelihood of diagnosis, treatment, or QoL improvement

Value Proposition

The Think Tank concluded that successful patient engagement initiatives during the commercialization phases of the product lifecycle management can have a widespread impact on patients and the commercial success of medical devices and diagnostics as a result of higher presentation rates and higher likelihood of diagnosis, treatment, or QoL improvement.

The value proposition for patient engagement in the commercialization phases is expressed as follows:

> *Engage patients during the commercialization phases through mobilization efforts, education initiatives, support systems, and partnerships with patient advocacy organizations, thereby improving patient presentation rates at healthcare providers and enhancing the likelihood of diagnosis, treatment, and quality of life improvement*[61]"

Activities

In order to overcome the recognized pains for presentation rate and likelihood of diagnosis, treatment, and QoL improvement, appropriate patient engagement activities must be identified and implemented for both dimensions.

The Patient Engagement Think Tank developed a matrix ("Commercialization Matrix") that can be leveraged to identify the suitable activities for each dimension based upon the positioning of the target disease type of a medical device or diagnostic. Refer to the matrix in *Figure 5*.

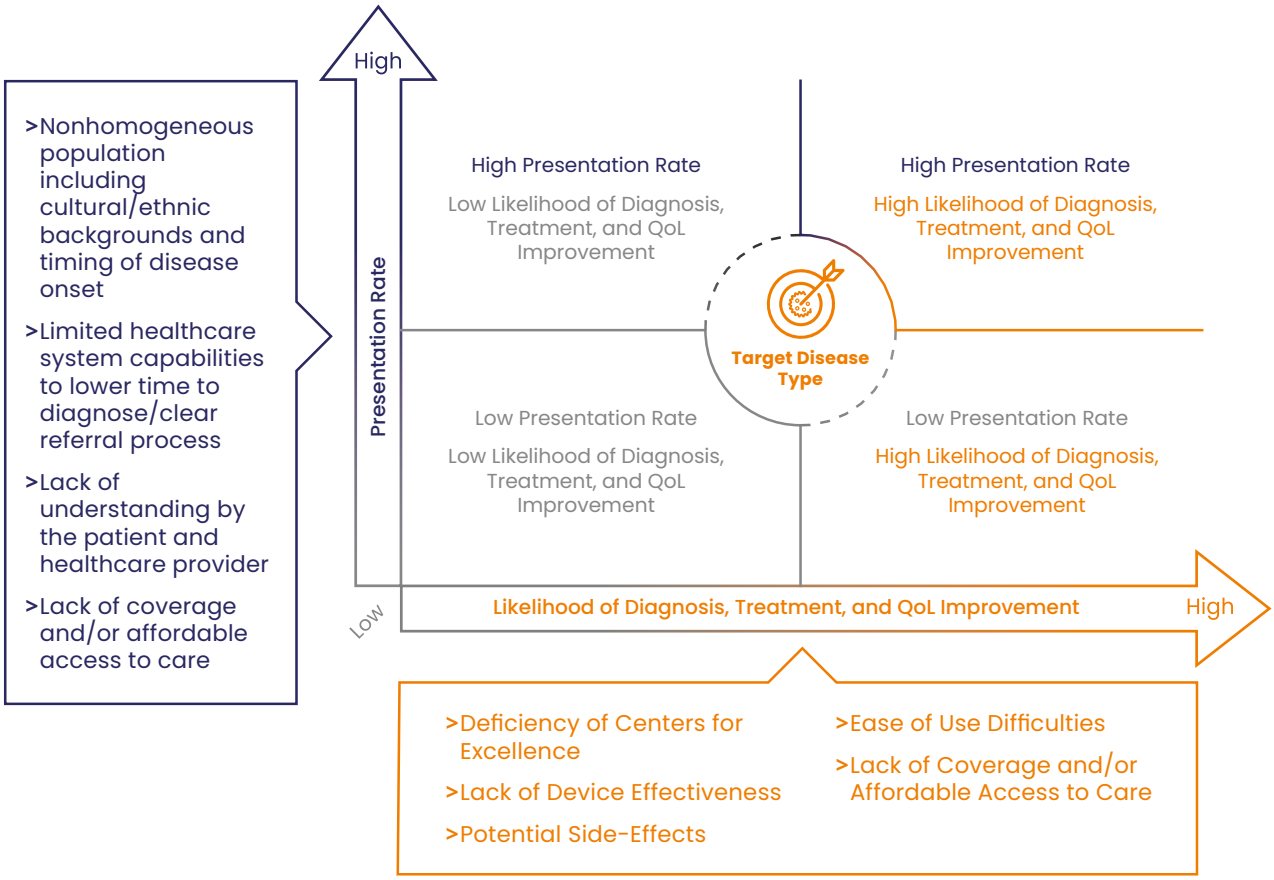


Figure 5. *Matrix for identifying patient engagement activities in the commercialization phases*

61. This value proposition was workshopped with members of the Think Tank to come to a conclusion that was satisfactory for all participants.

In order to identify these activities, the following four steps are provided:

1. Determine the current magnitude of the presentation rate and likelihood of diagnosis, treatment, or QoL improvement (i.e., low or high) for the target disease type.

2. Based on the magnitude for each dimension, classify the quadrant location of the target disease type. For example, a disease with a low presentation rate and a high likelihood of diagnosis, treatment, or QoL improvement would be positioned in the bottom right corner of the matrix.

3. The initial activities targeted for each dimension will be based upon the quadrant the target disease is located. As demonstrated in *Figure 6*, a disease with a very low presentation rate would commence with Activity 1 while a disease with a more moderate presentation rate would initiate with Activity 2.

4. As activities for each dimension are progressively executed, the positioning of the target disease type will move up each spectrum over time, thereby achieving a high presentation rate and high likelihood of diagnosis, treatment, or QoL improvement.

Presentation Rate Activities

1. Gain insights on the condition.

- Perform market research with patients to better understand the condition from the patient's perspective and the reasons for a low presentation rate

2. Mobilize the patients towards the healthcare providers.

- Create awareness campaigns
- Develop websites, social media, blogs, bursts, etc. providing information for patients, including etiology, diagnosis, specialists, and support groups

3. Help healthcare providers and patients manage the disease.

- Develop a range of courses to help healthcare professionals and patients stay up to date on disease management
- Create interactive sessions for patients with leading experts

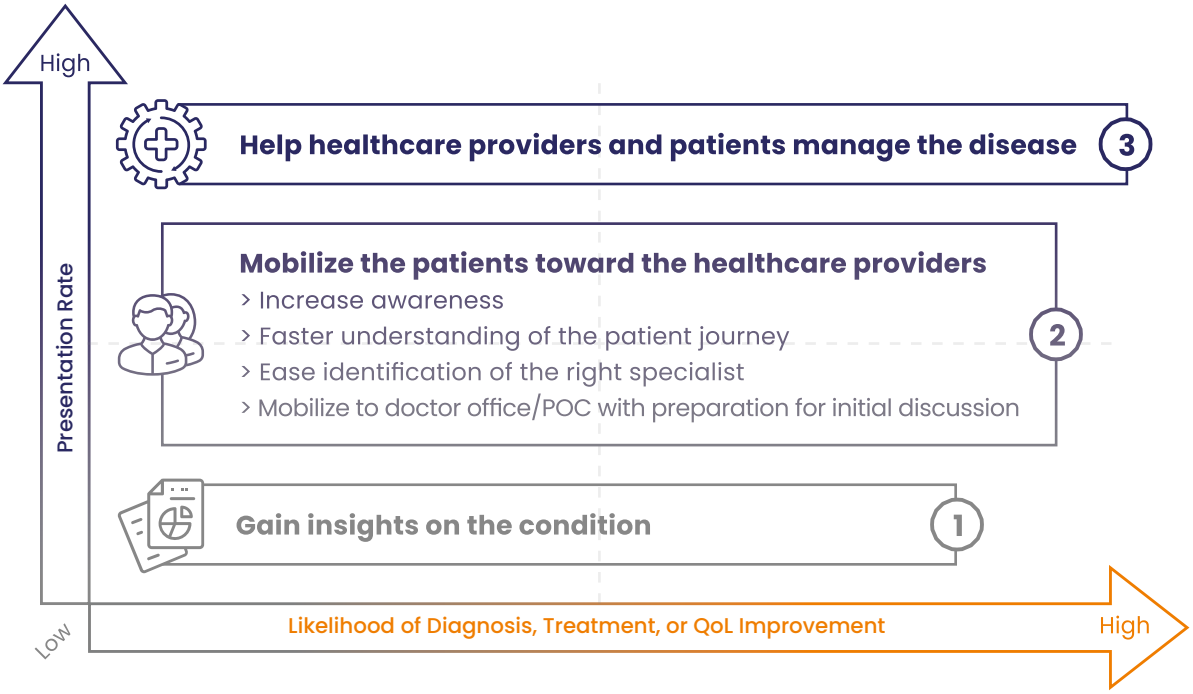


Figure 6. *Activities within the matrix focusing on the presentation rate dimension*

Likelihood of Diagnosis, Treatment or QoL Improvement Activities

1. Collaborate for research and development (R&D) priority setting.

- Integrate patient's foundation into R&D to better understand underlying causes of a disease
- This activity is primarily performed in the early stages of device development but has a residual effect on overcoming pains in the commercial phases

2. Educate patients about expectations toward diagnosis, treatment, and QoL improvement.

- Formulate education programs through community-based organizations that provide clinical education and leadership resources for healthcare professionals and patients that are culturally appropriate

3. Support patient access.

- Assist patients in their financial options and provide associated access support services, explain coverage plans, etc.

4. Design relational adherence programs.

- Develop support systems, including educational tools and resources, for patients and caregivers during treatment to better manage barriers to adherence

Building an Activity

Once the proper type of patient engagement activity has been identified, corresponding to the Commercialization Matrix, the activity must be constructed considering the following components:

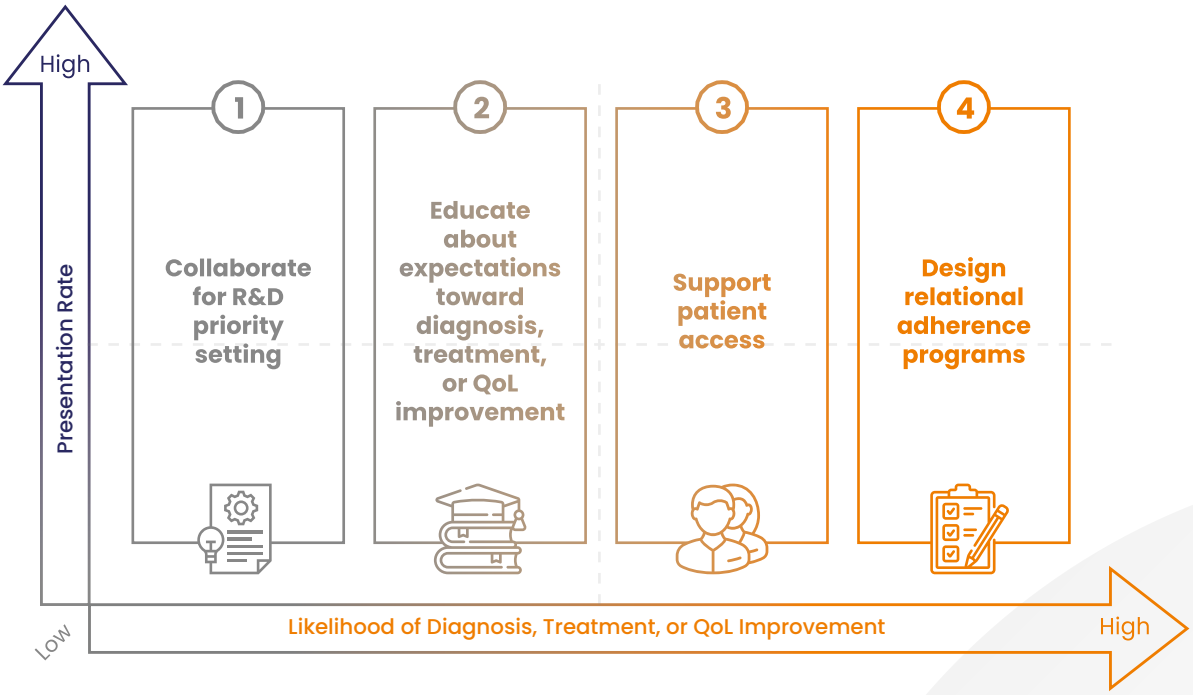


Figure 7. *Activities within the matrix focusing on the likelihood of diagnosis, treatment, or QoL dimension*

1. Know your patient.

- Define the patient segmentation and personas[62] including but not limited to age, sex, hobbies, needs, beliefs, culture, messages, support systems, occupation, income, geography, insurance coverage, stigmas, etc.

62. Personas refers to the profiles or characteristics of a typical patient.

2. Set up objectives.

Describe the patient segments intended to be reached by the activity and understand the aimed impact of the activity on the patient persona

3. Define the involved healthcare professionals and caregivers.

Identify the role healthcare professionals and caregivers will play in the patient's experience with the device or diagnostic and how they should be engaged

4. Identify how to reach your persona.

Understand the pathways, content creation, and collaboration (e.g., patient advocacy groups, media) to be leveraged in order to reach the targeted patient segments

5. Create and measure key performance indicators.

Performance indicators are necessary to realize the level of success of the initiative as defined by the activities' objectives

Example key performance indicators include:
> "Did the presentation rate of patients increase?"
> "Is the likelihood of treatment for patients higher?"
> "Is the activity increasing awareness or education of the disease?"
> "Is the activity increasing access to the device or diagnostic?"

Conclusion

MedTech should accelerate adoption of patient engagement efforts in medical device and diagnostic lifecycle management by leveraging the contents of this playbook. Patients, as well as caregivers, providers, and patient advocacy partners, are uniquely positioned to contribute to medical device and diagnostic development, yet many of the benefits of these contributions remain unrealized.

We assert that properly executed patient engagement strategies can lead to improved service of patient needs, improved outcomes, and maximized market access. For these reasons, patient engagement must be elevated to the attention of top MedTech management. It is now incumbent upon MedTech to appreciate the benefits of patient engagement and strive to improve industry-wide expertise and infrastructure, and to standardize sustainable patient-centric strategies.

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Resource Appendix

- **Clinical Trials Transformative Initiative (CTTI)** **ctti-clinicaltrials.org**
  A non-profit site that offers solutions, studies and recommendations on updating procedures to drive quality and efficiencies in clinical trials.

- **Cochrane Collaboration (COCHRANE)** **cochrane.org**
  The Cochrane Collaboration is a global nonprofit in 190 countries where patients, researchers, carers, and health professionals work together to gather and summarize the best evidence from research to help patients and families make informed choices about treatment.

- **Core Outcome Measures in Effectiveness Trials (COMET)** **comet-initiative.org**
  COMET aims to collate and stimulate relevant resources, both applied and methodological, to facilitate exchange of ideas and information, and to foster methodological research in this area. COMET offers a checklist for patients and public research participants along with research leaders in developing a Core Outcome Set research protocol.

- **Outcome Measures in Rheumatology Clinical Trials (OMERACT)** **omeract.org**
  OMERACT is an international collaboration of stakeholders interested in rheumatology outcome measurement. Since 2002, OMERACT has embraced Patient Research Partners (PRPs) as necessary and integral partners in research.

- **Patient-Centered Outcomes Research Institute (PCORI)** **pcori.org**
  PCORI is an independent, nonprofit research organization that seeks to empower patients and others with actionable information about their health and healthcare choices by funding comparative clinical effectiveness research (CER), which compares two or more medical treatments, services, or health practices to help patients and other stakeholders make better informed decisions.

- **Patient Engagement Think Tank** **youtu.be/vZut4_6DaBI**
  Patient Engagement Think Tank invited presentation to MDIC Patient Forum, "MedTech Patient Engagement in Early Clinical Phases" on September 12, 2022 with Amye Leong, Stephanie Raffey, Annabel de Maria, and Bill Schultz.

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