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Framing the Value of Patient Engagement for Medtech Product Developers

About Us

About the Milken Institute

The Milken Institute is a nonprofit, nonpartisan think tank focused on accelerating measurable progress on the path to a meaningful life. With a focus on financial, physical, mental, and environmental health, we bring together the best ideas and innovative resourcing to develop blueprints for tackling some of our most critical global issues through the lens of what's pressing now and what's coming next.

About FasterCures

FasterCures is working to build a system that is effective, efficient, and driven by a clear vision: patient needs above all else. We believe that transformative and lifesaving science should be fully realized and deliver better treatments to the people who need them.

Disclaimer

This work is supported by the U.S. Food and Drug Administration (FDA) Broad Agency Announcement (BAA) under contract #75F40124C00120. The contents represent the views of the author(s) and do not necessarily represent the official views of, and are not an endorsement by, the U.S. Food and Drug Administration (FDA)/Department of Health and Human Services (HHS) or the U.S. Government. The views, findings, and interpretations contained in this document do not constitute FDA guidance, position on this matter, or legally enforceable requirements.

Acknowledgments

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- **Nicholas Brooke**, Founder and Executive Director, The Synergist
- **Diane Clynes**, Executive Director, American Association of Kidney Patients
- **Andrew Fish**, President and CEO, Medical Device Innovation Consortium
- **Dominic Galante**, Chief Medical Officer, Access Experience Team, Market Access, Precision AQ
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Contents

Introduction: Why This Framework?

Context

- **Patient engagement (PE) is gaining traction in medtech**. Over the past decade, companies developing medtech products (e.g., devices, diagnostics, and digital health) have increasingly embraced the value of involving patients throughout the R&D process.
- **There is no one-size-fits-all approach.** Patient engagement in medtech varies widely due to the heterogeneity of products, rapid development timelines, resource constraints, and other factors.
- **When patients are left out, innovation falls short.** Skipping or minimizing patient input can lead to misaligned R&D, underperforming products, poor patient uptake, and even difficulty securing reimbursement.
- **Time is tight, and so are budgets.** In a fast-paced, resource-constrained environment, patient engagement can be deprioritized unless companies see a clear return on investment and a compelling case for its impact.

A Framework Organizes Information in a Way That Can Drive Action

This framework aims to support more robust patient engagement among medtech product developers by:

- Defining the benefits and value of patient engagement—and the risks of not doing it—for a broad range of medtech companies and stakeholders
- Identifying possible approaches to achieving those benefits
- Highlighting measures for evaluating the impact of patient engagement on core business needs

Future Plans

As part of our efforts, FasterCures is:

- Curating existing resources into a toolkit that can be readily utilized by a wide range of medtech companies seeking to engage patients in their R&D activities
- Identifying and developing needed complementary resources to support medtech patient engagement

About the Framework: Patient Engagement Through the Lens of Developers

Framing the Value of Patient Engagement for Medtech Product Developers is intended to capture the ways in which engaging patients and utilizing patient experience data can inform decisions that developers make across the total product life cycle (TPLC) and support the information and evidence they generate for a broad range of stakeholders.

The primary audience for this framework is medtech companies—particularly small- to-medium-sized companies that may lack resources to prioritize these activities—although it is also relevant for large companies. Other stakeholders that influence medtech developers' decision-making (e.g., patients, patient organizations, regulators, payers, providers, and investors) may also find this tool useful for understanding how to engage with medtech product developers around patient insights.

This framework was developed with input from a voluntary expert steering committee comprising leaders from medtech companies, patient organizations, and other key opinion leaders. It builds on insights shared in FasterCures' white paper, *Defining and Demonstrating the Value of Patient Engagement in Medtech Research and Product Development*.

The framework is organized around the stages of the TPLC, from ideation through post-approval surveillance and monitoring. At each stage, it identifies key information in several domains related to how **patient engagement** and **patient experience data** (PE/PED) can support companies' decision-making and success. (See definitions of these and other select terms in Appendix B). Those domains include:

- **Life cycle stage**
- **Key stakeholders** whose needs and requirements companies aim to address
- **Product developer decisions and information needs** at each stage of the TPLC
- **Insights and activities** from PE/PED that can inform those decisions
- **Sources and methods** for acquiring those insights
- **Metrics** to assess the impact of engagement
- **Relevant resources** (a curated list)

The framework is not meant to be prescriptive but to present a menu of options and opportunities for developers' consideration.

Framework Benefits

- Aligns patient engagement activities with key business needs and decisions, including regulatory evaluation and reimbursement strategies
- Facilitates justification for the time and cost of patient engagement activities for resource-constrained companies
- Lays the groundwork for evaluating the impact of patient engagement activities
- Provides examples of meaningful patient engagement
- Creates a common language around patient engagement internally and externally
- Serves as an educational tool for medtech R&D stakeholders

Framework Assumptions

1. **Patients are at the center—all patients.**
    - Though they are only identified as “key stakeholders” at the product adoption stage, the entire framework is oriented around patients’ central importance throughout the TPLC.
    - The term “patients” is meant to encompass patients *and* caregivers, whenever their role is relevant.
    - The term “patients” can also mean “consumers,” as in some instances, medtech products are intended to be preventive or can be used by individuals without a diagnosed condition.
    - It is important that developers seek input from a sample of the patient community that is representative, across all relevant domains, of the intended population.
2. **It is primarily relevant for patient-facing products.** However, an expansive definition of “patient-facing” is encouraged, as the development of most medical products would benefit from the inclusion of patient insights. Developers often consider providers the end user rather than patients.
3. **It is a linear representation of an iterative and cyclical process.** Insights and activities associated within the framework with a specific stage of the TPLC are best contemplated and initiated before that stage begins.

Framework Overview

Patient Engagement Can Inform Key Decisions and Stakeholders Across the Total Product Life Cycle

Internal discovery/ R&D teams

Product design
Desired outcomes

Discovery/ Preclinical

Clinical/Testing

Internal R&D/Clinical affairs team
Contract research organizations

Protocol design
Measure selection
Recruitment/retention strategy

Evaluation/ Authorization

Regulators
Internal regulatory affairs team

Benefit-risk assessment
Risk mitigation
Labeling

HTAs, payers, internal market access team

Comparative effectiveness
Cost-effectiveness
Quality-of-life impacts
Value assessment

Coverage/ Payment

Adoption

Providers, patients
Internal medical affairs, marketing teams

Efficacy
Quality-of-life impacts
Safety profile
Ease of use
Cost-effectiveness

Post-Market Surveillance/ Monitoring

Regulators, health technology assessors (HTAs), payers
Internal compliance teams

Safety signals
Product improvements

Fundraising

Investors

Unmet needs
Customer demand
Target market
Competitive position

Key

🟪 **Life cycle stage**

🟧 **Key stakeholders**

🟦 **Product developer decisions and information assets**

The Impact of Engagement Across the Total Product Life Cycle

	Product Type	Purpose	Activity	Impact
Fundraising	**Digital telehealth, education, and support platform for women's health care (Source: Stakeholder interview)**	To develop a better understanding of the market and consumer needs	A digital health developer coordinated in-person and virtual peer sharing salons where women had a chance to engage with each other and share their experiences, concerns, and challenges with women's health	An investor who funded this company shared that understanding the voice of the patient is critical in helping a company demonstrate proof of concept, and she considers information that can be gleaned from patient engagement when making investment decisions.
Product Development: Discovery/ Preclinical	**SmartCurve Breast Stabilization System**	To develop a more comfortable, patient-friendly breast stabilization system for mammograms to improve patient experience	The SmartCurve System was specially designed to address patient-voiced pain during mammograms, as discovered through clinical trials and early research.	Studies showed that SmartCurve use resulted in a significant reduction in patient pain, from an average of 6.6 on a scale of 10 with the brand's legacy product to 3.4 of 10 with the new system.
Product Development: Clinical/ Testing	**Lilly CoDESIGN program**	To improve the feasibility and execution of trials across a company's portfolio	The program consistently brings together patients and others involved in clinical trial conduct to provide input on both the design and implementation of studies and to participate in simulations of trials and process improvements.	Clinical trial teams have changed trial materials, reduced the number of invasive procedures, etc., resulting in lower costs, less rework, and shared learnings for other trials.
Product Evaluation/ Authorization	**Maestro device**	To evaluate the trade-offs that patients would make among effectiveness, safety, and other attributes of weight-loss devices	RTI Health Solutions conducted a preference study to estimate patients' benefit-risk tolerance and trade-offs for weight-loss devices.	FDA acknowledges that this was the first time a patient-preference study impacted a new device approval.
Product Coverage/ Payment	**Power wheelchair seat elevation equipment**	To inform coverage on seat elevation systems as accessories to power wheelchairs	A coalition of patient organizations provided evidence to support coverage and generated significant public comment.	The payer expanded coverage.
Product Adoption	**Dexcom G5 Continuous Glucose Monitoring System and Animas Vibe System**	To improve a device to make it safer for young users	The company held discussions with patients and caregivers about their concerns around the safety of the pediatric population using an insulin pump.	The company developed additional risk mitigation strategies, including a lockout feature to improve safety for young people, increasing parents' comfort with their children's self-management of the condition.
Post-Approval Surveillance/ Monitoring	**NxStage's home hemodialysis device**	To capture whether patient preferences supported hemodialysis at home without the presence of a trained care partner	The sponsor worked with the Kidney Health Initiative to develop and conduct a patient-preference study to understand whether patients are willing to accept the risks associated with hemodialysis at home without a qualified care partner.	The FDA cited this survey in its decision to expand an indication for the first time, based in part on asking patients about their tolerance for risk.

Framework Domains: Mapping Patient Engagement Across the Total Product Life Cycle

- **Life Cycle Stages**
- **Key Stakeholders**
- **Product Developer Decisions and Information Needs**
- **Insights/Activities from PE/PED**
- **PE/PED Sources/Methods**
- **Metrics to Assess Impact of Engagement**
- **Relevant Resources**

Life Cycle Stages

Fundraising

Includes all possible points of fundraising opportunities, which can happen at multiple points in the life cycle, as well as types of funders (e.g., government, philanthropic, and private) and forms of capital

Product Development: Discovery/Preclinical

Includes discovery, ideation, invention, prototyping, modeling/simulation, early user experience testing, and early-stage digital product development activities (e.g., connectivity, user agreements)

Product Development: Clinical/Testing

Includes clinical trial design and conduct, outcome measures selection, recruitment, and later-stage digital product and user interface development activities (e.g., verification, validation, and usability testing)

Product Evaluation/Authorization

Includes clinical study reporting, regulatory submission and review, benefit-risk determination, risk mitigation, product labeling development, and regulatory authorization decision

Product Coverage/Payment

Includes health technology and value assessments, comparative and cost-effectiveness analyses, public- and private-payer coverage decisions, and development of real-world evidence strategies

Product Adoption

Includes product commercialization, launch, and marketing activities, provider purchasing, provider and patient adoption, education, and use

Post-Approval Surveillance/Monitoring

Includes adverse event reporting and monitoring, post-marketing study design and conduct, patient access and coverage support, the pursuit of additional indications/label expansions, and continuous product improvements

Key Stakeholders

Medtech product developers are encouraged to coordinate and collaborate with and serve the needs of a set of key stakeholders along the development pathway, including external entities as well as internal teams. Relevant key stakeholders for medtech companies include the following:

- Patients and caregivers and patient organizations
- Investors (e.g., government, philanthropic, and private)
- Internal discovery/R&D teams
- Internal R&D/clinical affairs teams
- Contract research organizations
- Regulators (FDA and other global medical device regulators)
- Internal regulatory affairs teams
- Health technology assessors
- Payers (public and private)
- Internal market access teams
- Providers (e.g., clinicians, hospital and health system administrators)
- Internal medical affairs teams
- Internal marketing teams
- Internal compliance teams

Product Developer Decisions and Information Needs

Across the medtech TPLC, key questions and evidence needs drive product developers' decision-making, and patient engagement input can reduce business risks and improve the likelihood of successful product authorization, adoption, and reimbursement. These key questions and evidence needs include:

- Unmet needs and customer demand
- Target market
- Competitive position
- Product design
- Desired outcomes
- Protocol design
- Measure selection
- Recruitment and retention strategy
- Benefit-risk assessment
- Risk mitigation
- Labeling
- Comparative effectiveness
- Quality-of-life impacts
- Value assessment
- Effectiveness
- Safety profile
- Ease of use
- Cost-effectiveness
- Product improvements
- Label expansions

Insights/Activities from Patient Engagement/ Patient Experience Data

Involving patients in R&D activities and utilizing data to better understand patient needs, experiences, and preferences can help product developers effectively and efficiently navigate key decisions and generate needed evidence. There are many types of information and insights that companies need and use that can be uniquely informed by patients, their input, and their data. Developers do not need to act on all of these, but specific information and insights include the following:

Disease and Treatment Landscape

Burden of disease

Unmet needs

Experience with current treatments

Real-world data and evidence of patient outcomes

Population Insights

Patient population characteristics

Intended populations

Expanded populations and indications for product

Market and Access Considerations

Market size

Competitive landscape

Sources of business risk

Reimbursement potential

Market access

Patient Preferences and Outcomes

Patients' desired outcomes

Treatment preferences

Desired product features

Meaningful change in function, symptoms, and quality of life

Meaningful endpoints

Satisfaction with product

Product Use and Adherence

Patients' adherence challenges

Product's ease of use and adherence

Product fit, adoption, and acceptability

Product enhancements and improvements

Clinical Trial Design and Participation

Protocol feasibility

Trial participation perceived burdens

Recruitment and retention assistance

Input on informed consent and trial materials

Need for additional supports

Strategies for representativeness

Safety and Risk

Benefit-risk trade-offs (ongoing)

Ethical considerations

Insights on safety profile

Potential design risks and errors

Communication and Engagement

Inform product labeling

Inform advisory committees

Input on communications and marketing

Sources/Methods for Patient Engagement/Patient Experience Data

Product developers can use a wide range of approaches to acquire information about patient needs, experiences, preferences, and insights, as well as to engage patients in their activities across the TPLC. These approaches vary widely in level of effort and value of output. Again, developers need not utilize all of these, but options include the following:

Direct Patient and Caregiver Input

Patient advisory boards

Patient advocacy organizations

Interviews, focus groups, and surveys

Journey mapping

Public comment and testimony

Advisory committee participation (e.g., Data Safety Monitoring Boards)

Patient-Generated Data and Patient-Led Insights

Human-centered design methodologies

Patient-generated health data

Patient-led registries

Patient Focused Drug Development (PFDD) *Voice of the Patient* reports

Patient-developed solutions

Patient-centered outcomes research

Patient-driven research agendas and investments

Patient-sensitive design controls

Studies and Assessments

Patient burden of disease studies

Patient preference information (PPI) and studies

Patient benefit-risk assessments

Health economics and outcomes research

Clinical outcome assessments (patient-reported outcomes [PROs], clinician-reported outcomes [ClinROs], and observer-reported outcomes [ObsROs])

Patient-reported experience measures (PREMs)

Real-World and Market Data

Real-world data and evidence

Natural history data

Adverse event data

Claims data

Market access data

Consumer data (e.g., health and other)

Customer satisfaction data

Social media and online listening

Metrics to Assess the Impact of Engagement

Demonstrating and measuring the potential and observable impact of patient engagement on R&D activities across the TPLC are likely central to determining whether companies invest in it. It can be challenging but critical. Metrics generally assess either experiences or outcomes, which typically vary in the speed and ease with which they can be measured.

Strategic Alignment and Insight Utilization

Whether product addresses demonstrated need

Changes in understanding of patient needs

Changes in research priorities and goals

Number and type of planned actions based on insights

Study Design and Operational Efficiency

Number and representativeness of participants

Recruitment, accrual and retention rates, speed, and expenditures

Number of protocol revisions

Prototype development, revision, and costs

Reduced time and costs of development

Reduced risks to development program

Regulatory Success and Influence

Changes in regulators' understanding of patients' needs

Improved quality of regulatory review process (e.g., reducing patient experience–related queries)

Increased number of approved products that include patient experience data

Patient-centric product and labeling changes

Payer and Market Access

Success in convincing payers of effectiveness and cost savings

Data available for payer measurements of patient quality of life and burden

Changes in HTA and payer awareness and knowledge of patient needs

Payers' acceptance and utilization of PPIs, PROs, and PREMs

Product Use and Real-World Impact

Product adoption by patients

Product adherence by patients

Improvement in outcomes and quality of life

Patient satisfaction with product

Patient trust in effectiveness and safety

Changes in providers' awareness and knowledge of patients' needs

Participant Experience and Communication

Study participant experience, satisfaction, and perceived burden

Patient understanding and accessibility of information materials and informed consent

Organizational Impact

Employee satisfaction

Organizational health survey data

Source: The source for many of these metrics is Patient Focused Medicines Development's Metrics Selector

Relevant Resources

Each section includes links to several tools, FDA guidance, publications, trainings, and other information to support planning, conducting, and evaluating patient engagement activities. A more extensive toolkit will be produced in conjunction with this framework; however, these are some foundational or particularly relevant resources in the medtech context including:

- FDA guidance and information on conducting patient preference studies and the use of patient preference information
- Patient-centered benefit-risk frameworks
- Playbooks for patient engagement in medtech
- Practical tools for implementing patient engagement activities

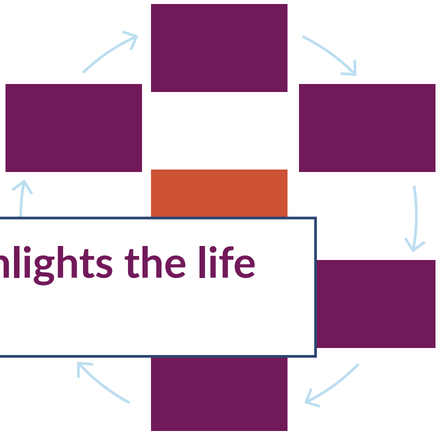
Note: These resources do not necessarily represent the official views of, and are not endorsed by FDA/HHS, or the U.S. Government, with the exception that FDA regulations and final guidance documents express the current thinking of FDA.

Framework Contents: Patient Engagement Across the Total Product Life Cycle

How to Read the Framework

Life Cycle Stage

Key Stakeholders:



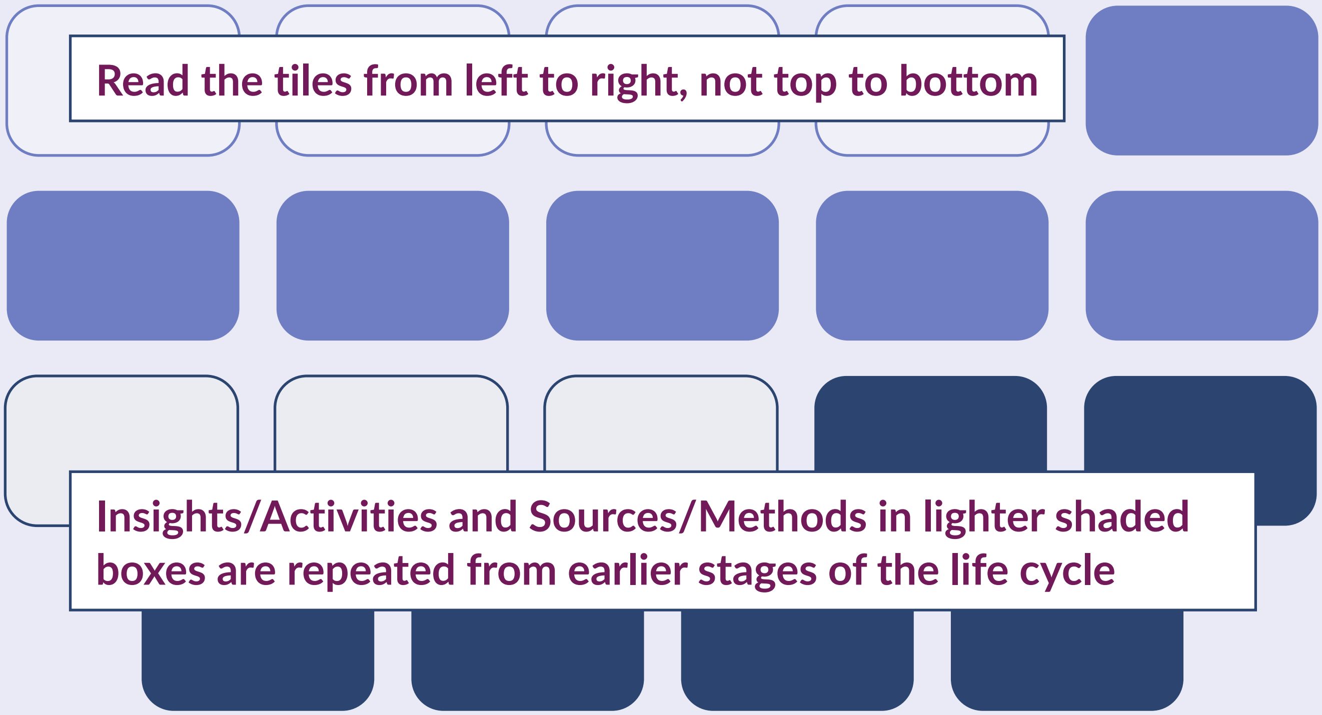
Wayfinder highlights the life cycle stage

Key Decision Points:

Questions/ Information Needs:

Product developers’ key decisions are translated into key questions and information needs

Relevant Patient Engagement Insights and Activities:



Read the tiles from left to right, not top to bottom

Patient Engagement Sources and Methods:

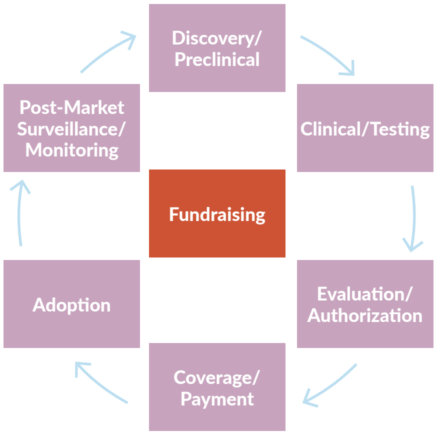
Insights/Activities and Sources/Methods in lighter shaded boxes are repeated from earlier stages of the life cycle

Relevant Metrics:

Relevant Resources:

Resources appear as numbered links; Appendix A at the end lists all

Fundraising



Key Stakeholders: Investors (Government, Philanthropic, and Private)

Key Decision Points:	Questions/ Information Needs:
Unmet needs and customer demand	What unmet needs do patients have? What are their priorities? Is there a demand for this product?
Target market	What is the size and composition of the intended patient population?
Competitive position	What products are patients currently using, and what alternatives do they have?

Relevant Patient Engagement Insights and Activities:

- Patients’ unmet needs
- Patients’ burden of disease
- Patients’ desired outcomes
- Intended patient populations
- Market size
- Competitive landscape
- Experience with current treatments
- Source of business risk
- Reimbursement potential
- Characteristics of patient population

Patient Engagement Sources and Methods:

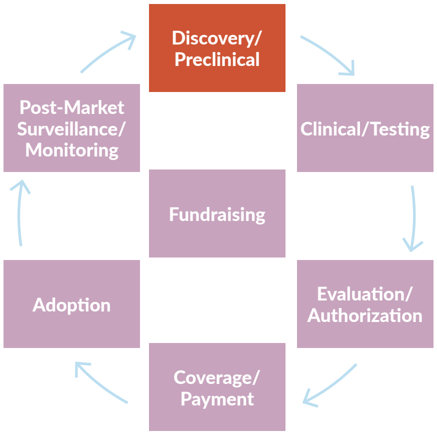
- Patient preference information and studies
- Natural history data
- Patient-led registries
- Journey mapping
- Interviews, focus groups, and surveys
- Social media/ online listening
- Patient-driven research agendas and investments
- Patient advocacy organizations
- Patient advisory boards

Relevant Metrics:

- Product addresses demonstrated need
- Reduced risks to development program
- Reduced time and costs of development

Relevant Resources: 1, 2

Product Development: Discovery/Preclinical



Key Stakeholders: Internal Discovery and R&D Teams

Key Decision Points:

Questions/ Information Needs:

Product design

What are patients' preferences and needs?

Desired outcomes

What are the most important outcomes that patients would like to have addressed?

Relevant Patient Engagement Insights and Activities:

- Patients' unmet needs
- Intended patient population
- Patients' burden of disease
- Experience with current treatments
- Sources of business risk
- Treatment preference
- Desired product features
- Product fit, adoption, and accountability
- Benefit-risk trade-offs
- Potential design risks and errors

Patient Engagement Sources and Methods:

- Patient advisory boards
- Interviews, focus groups, and surveys
- Patient preference information and studies
- Patient advocacy organizations
- Journey mapping
- Patient benefit-risk assessments
- Patient-sensitive design controls
- Patient-developed solutions
- Human-centered design methodologies

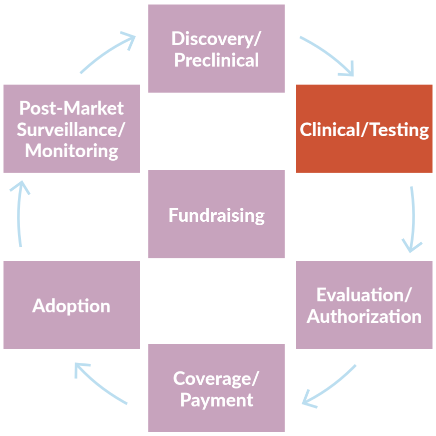
Relevant Metrics:

- Changes in understanding of patient needs
- Changes in research priorities and goals
- Number and types of planned actions based on insights
- Number and representativeness of participants
- Employee satisfaction and organizational health survey data

Relevant Resources: 3, 4, 5

Product Development: Clinical/Testing

Key Stakeholders: Internal Discovery and R&D Teams, Contract Research Organizations


Discovery/ Preclinical
Clinical/Testing
Evaluation/ Authorization
Coverage/ Payment
Adoption
Post-Market Surveillance/ Monitoring
Fundraising

Key Decision Points:	**Protocol design**	**Measure selection**	**Recruitment and retention strategy**
Questions/ Information Needs:	Is the study designed in a way to maximize patient participation and adherence?	Is the study measuring outcomes that matter to patients?	Will we be able to quickly and efficiently recruit a representative cohort of participants and retain them?

Relevant Patient Engagement Insights and Activities:

- Benefit-risk trade-offs
- Patients' desired outcomes
- Meaningful endpoints
- Meaningful change in function, symptoms, and quality of life
- Protocol feasibility
- Trial participation burdens
- Input on informed consent and trial materials
- Ethical considerations
- Recruitment and retention
- Strategies for representativeness

Patient Engagement Sources and Methods:

- Patient preference information and studies
- Interviews, focus groups, and surveys
- Patient advisory boards
- Patient advocacy organizations
- Advisory committee participation (e.g., Data Safety Monitoring Board)

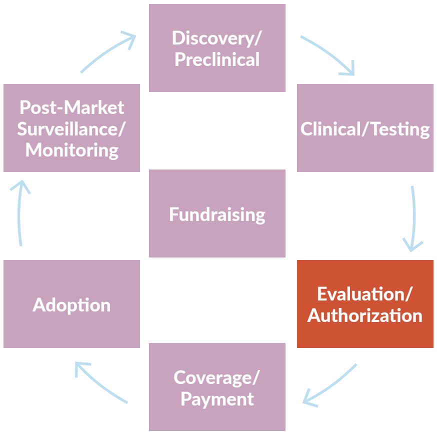
Relevant Metrics:

- Recruitment, accrual, and retention rates, speed, and expenditures
- Number of protocol revisions
- Number and types of planned actions based on insights
- Study participant experience, satisfaction, and perceived burden
- Patient understanding and accessibility of information materials and informed consent

Relevant Resources: 6, 7, 8

Product Evaluation/Authorization

Key Stakeholders: Regulators, Internal Regulatory Affairs Teams



Key Decision Points:

Questions/ Information Needs:

Benefit-risk assessment

Do the potential benefits of the product outweight the potential risks, as informed by the patients' perspective?

Risk mitigation

What are the product use risks and how can they be mitigated?

Labeling

What claims can be made on the product label regarding its use, risks, and benefits?

Relevant Patient Engagement Insights and Activities:

- Intended patient populations
- Patients' burden of disease
- Experience with current treatments
- Patients' unmet needs
- Patients' desired outcomes
- Treatment preferences
- Benefit-risk trade-offs
- Inform advisory committee
- Inform product labeling

Patient Engagement Sources and Methods:

- Natural history data
- Patient preference information/ studies
- Journey mapping
- Clinical outcome assessments (e.g., PROs, ClinROS, and ObsROs)
- Patient-reported experience measures (PREMs)
- Patient burden of disease studies
- PFDD *Voice of the Patient* reports
- Advisory committee participation
- Public comment and testimony

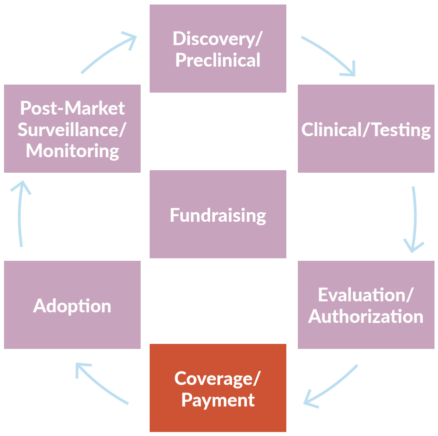
Relevant Metrics:

- Changes in regulators' understanding of patient needs
- Improved quality of regulatory review process (e.g., reducing patient experience-related queries)
- Increased number of approved products that include patient experience data
- Patient-centric product and labeling changes

Relevant Resources: 9, 10, 11, 12

Product Coverage/Payment

Key Stakeholders: HTAs, Payers (Public and Private), Internal Market Access Teams



Key Decision Points:	**Questions/ Information Needs:**
Comparative effectiveness	How does the product's effectiveness compare to that of other available products?
Cost-effectiveness	How does the product cost compare to that of other available products?
Quality-of-life impacts	How does use of the product impact patients' quality of life?
Value assessment	How does product use impact the total cost of care and patients' productivity?

Relevant Patient Engagement Insights and Activities:

- Patients' burden of disease
- Meaningful change in function, symptoms, quality of life
- Experience with current treatments
- Patients' unmet needs
- Patients' desired outcomes
- Patients' adherence challenges

Patient Engagement Sources and Methods:

- Patient preference information and studies
- Natural history data
- Patient burden of disease studies
- Clinical outcome assessments (e.g., PROs, ClinROS, and ObsROs)
- Patient-reported experience measures (PREMs)
- Public comment and testimony
- Health economics and outcomes research
- Patient-centered outcomes research
- Real-world data and evidence

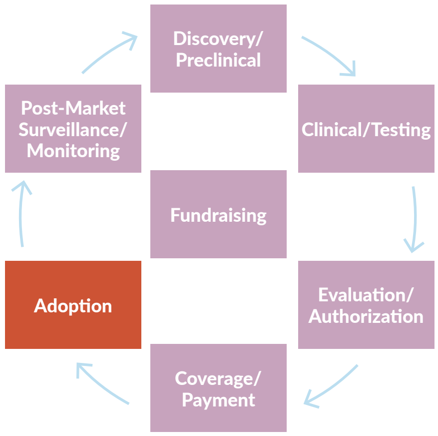
Relevant Metrics:

- Success in convincing payers of effectiveness and cost savings
- Data available for payer measurements of patient quality of life and burden
- Changes in HTA and payer awareness and knowledge of patient needs
- Payers' understanding, expectations, acceptance, and utilization of PPI, PROs, and PREMs

Relevant Resources: 13, 14, 15

Product Adoption

Key Stakeholders: Providers, Patients, Internal Medical Affairs and Marketing Teams


Discovery/ Preclinical

Clinical/Testing

Evaluation/ Authorization

Coverage/ Payment

Adoption

Post-Market Surveillance/ Monitoring

Fundraising

Key Decision Points:	**Effectiveness**	**Quality of life**	**Safety profile**	**Ease of use**	**Cost-effectiveness**
Questions/ Information Needs:	How well does the product work for its intended use?	How does the product impact patients' quality of life?	How safe is the product to use?	How easy is the product to use?	Is the product likely to be covered by insurance? What will the patients' out-of-pocket costs be?

Relevant Patient Engagement Insights and Activities:

- Desired outcomes
- Treatment preferences
- Meaningful change in function, symptoms, quality of life
- Patients' burden of disease
- Benefit-risk trade-offs
- Product's ease of use, adherence
- Satisfaction with product
- Input on communication and marketing

Patient Engagement Sources and Methods:

- Patient preference information/ studies
- Natural history data
- Patient burden of disease studies
- Clinical outcome assessments (e.g., PROs, ClinROS, and ObsROs)
- Patient-reported experience measures (PREMs)
- Interviews, focus groups, surveys
- Patient advisory boards
- Customer satisfaction data

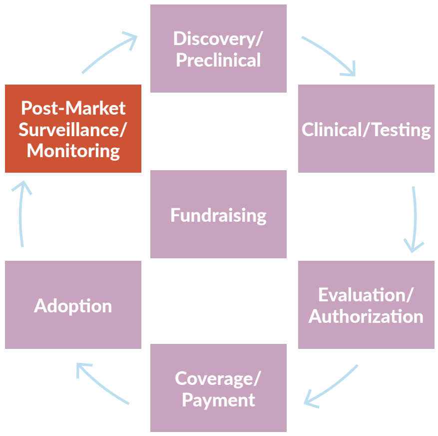
Relevant Metrics:

- Product adoption by patients
- Product adherence by patients
- Improvements in outcomes, quality of life
- Changes in providers' awareness and knowledge of patient needs

Relevant Resources: 16, 17

Post-Approval Surveillance/Monitoring

Key Stakeholders: Regulators, HTAs, Payers (Coverage, Policy and Utilization Management Teams), Internal Compliance Teams



Key Decision Points:	**Safety signals**	**Product improvements**	**Label expansions**
Questions/ Information Needs:	What adverse events and other potential safety concerns have arisen with product use?	What improvements might be made to the product based on real-world usage?	Is there evidence the product could be used by more patients or for other indications?

Relevant Patient Engagement Insights and Activities:

- Benefit-risk trade-offs
- Meaningful change in function, symptoms, quality of life
- Need for additional support
- Product enhancements/ improvements
- Expanded populations/ indications for product
- Insights on safety profile

Patient Engagement Sources and Methods:

- Patient preference information and studies
- Real-world data/ evidence
- Claims data
- Consumer data (health and other)
- Adverse event data
- Market access data

Relevant Metrics:

- Improvements in outcomes and quality of life
- Patient-centric product labeling changes
- Patient trust in effectiveness and safety
- Patient satisfaction with product

Relevant Resources: 18, 19

Appendix A: Relevant Resources

Fundraising

1. Revolutionizing Healthcare: Igniting Change Through Patient Engagement, Gilde Healthcare, 2024
2. Voice of the Patient Reports, FDA

Product Development: Discovery/Preclinical

3. Human-Centered Design Toolkit for Kidney Failure, Kidney Health Initiative, 2022
4. Device Development Blueprint: Guidance for Early and Systematic Patient Involvement, Boehringer Ingelheim and Savvy Cooperative, 2023
5. Patient-Centered Benefit-Risk Framework, Medical Device Innovation Consortium, 2015

Product Development: Clinical/Testing

6. Patient Engagement in the Design and Conduct of Medical Device Clinical Studies, FDA, 2022
7. Expansion of Expected Net Present Value Framework for Evaluating Patient Engagement Methods, Clinical Trials Transformation Initiative, 2020
8. Maximizing Patient Input in the Design and Development of Medical Device Clinical Trials, MDIC, 2022

Product Evaluation/Authorization

9. Patient Preference Information (PPI) in Medical Device Decision Making, FDA, 2024
10. Patient Engagement in the Design and Conduct of Medical Device Clinical Studies, FDA, 2022
11. Factors to Consider Regarding Benefit Risk in Medical Device Product Availability, Compliance, and Enforcement Decisions, FDA, 2016
12. Total Product Life Cycle Advisory Program (TAP), FDA, 2025

Product Coverage/Payment

13. ICER Patient Portal, Institute for Clinical and Economic Review, 2026
14. HTAi Interest Group for Patient and Citizen Involvement, Health Technology Assessment International, 2026
15. Use of Patient Preference Studies in HTA Decision Making: A NICE Perspective, Patient, 2020

Product Adoption

16. A Playbook for Patient Engagement in Medtech in the US, Alira Health, 2023
17. A Roadmap for Increasing the Usefulness and Impact of Patient-Preference Studies in Decision Making in Health, *Value in Health*, 2023

Post-Approval Surveillance/Monitoring

18. Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices, FDA, 2025
19. A Framework for Direct Patient Input (DPI) in Real World Data and Evidence Generation, MDIC, 2025

Note: These Resources do not necessarily represent the official views of, and are not endorsed by FDA/HHS, or the U.S. Government, with the exception that FDA regulations and final guidance documents express the current thinking of FDA.

Appendix B: Select Definitions: Patient Engagement Insights and Activities

- **Benefit-risk trade-offs:** making an informed judgment as to whether the benefits—with their uncertainties—of product use outweigh the risks, with their uncertainties and approaches to managing said risks.
- **Ethical considerations:** encompass issues such as social and clinical value, scientific validity, fair subject selection, favorable risk-benefit ratio, independent review, informed consent, and respect for potential and enrolled subjects.
- **Patient engagement:** refers to the intentional, meaningful interactions with patients that provide opportunities for mutual learning and effective collaborations. These partnerships with patients help advance the development and evaluation of innovative medical products and monitor the performance of marketed devices.
- **Patient-experience data:** includes data that 1) are collected by any persons (including patients, family members and caregivers of patients, patient advocacy organizations, disease research foundations, researchers, and product manufacturers), and 2) are intended to provide information about patients' experiences with a disease or condition, including: A) the impact (including physical and psychosocial impacts) of such disease or condition, or a related therapy on patients' lives, and B) patient preferences with respect to treatment of such disease or condition.
- **Sources of business risk:** patient insights and engagement can help alleviate sources of business risk for medtech companies, including technical and engineering, clinical and biological, regulatory and compliance, and market and commercial risks. (Milken Institute/FasterCures definition)

Note: Definitions in Appendix B are provided for purposes of this framework. Many definitions are available in FDA's Patient-Focused Drug Development Glossary.

Appendix C: Select Definitions: Patient Engagement Sources and Methods

- **Consumer data:** information that businesses collect about their customers to understand their preferences, behaviors, and purchasing patterns, which helps them tailor their marketing strategies, personalize their offerings, and improve overall customer experience. They can include demographic data, behavioral data, interaction data, attitudinal data, and non-HIPAA-protected health data.
- **Human-centered design methodologies:** an approach to interactive systems that aims to make them usable and useful by focusing on users' needs and requirements and by applying human factors and ergonomics as well as usability knowledge and techniques. This approach enhances effectiveness and efficiency, improves human well-being, user satisfaction, accessibility, and sustainability, and counteracts possible adverse effects of use on human health, safety, and performance.
- **Natural history data:** information collected about the natural history of a disease in the absence of an intervention, from the disease's onset until either its resolution or the individual's death.
- **Patient-centered outcomes research:** helps patients and their caregivers communicate and make informed health-care decisions, allowing their voices to be heard in assessing the value of health-care options and answering patient-centered questions.
- **Patient-developed solutions:** useful and innovative solutions developed by patients to cope with their health disorders, ranging from simple products for everyday use to unknown therapies and high-tech solutions.
- **Patient-driven research agendas and investments:** research agendas and investment portfolios crafted by patient communities that prioritize the perspectives and needs of patients in shaping research questions, methods, and outcomes.
- **Patient-generated health data (PGHD):** data created, recorded, or gathered by or from patients to help address a health concern. A distinguishing feature of PGHD is that patients, not providers, are the ones responsible for capturing and sharing these data.
- **Patient preference information (PPI):** assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions. The methods for generating PPI may be qualitative, quantitative, or mixed methods.
- **Patient-reported experience measures (PREMs):** PREMs focus on patients' experiences with health-care services and assess a wide range of patient experiences, including communication with clinicians and staff, courtesy and respect, access to care, and care coordination.

- **Patient-reported outcomes (PROs):** provide information on the patient's health condition as directly reported by the patient, without outside interpretation from anyone. These outcomes are assessed using PRO instruments such as questionnaires, numeric rating scales, or diaries.
- **Patient-sensitive design controls:** an interrelated set of practices and procedures incorporated into the design and development process to reduce risk and harm. Patient-sensitive design controls ensure that devices meet user needs, intended uses, and specified requirements.
- **PFDD *Voice of the Patient* reports:** an initiative to more systematically obtain the patient perspective on specific diseases and their currently available treatments. PFDD meetings are unique among FDA public meetings, with a format designed to engage patients and elicit their perspectives on two topic areas: (1) the most significant symptoms of their condition and the impact of the condition on daily life and (2) their current approaches to treatment. *Voice of the Patient* reports summarize the insights coming out of condition-specific PFDD meetings.
- **Real-world data/real-world evidence (RWD/RWE):** RWD are the data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources. RWE is the clinical evidence regarding a medical product's use and potential benefits or risks derived from the analysis of RWD.

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