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Defining and Demonstrating the Value of Patient Engagement in Medtech Research and Product Development

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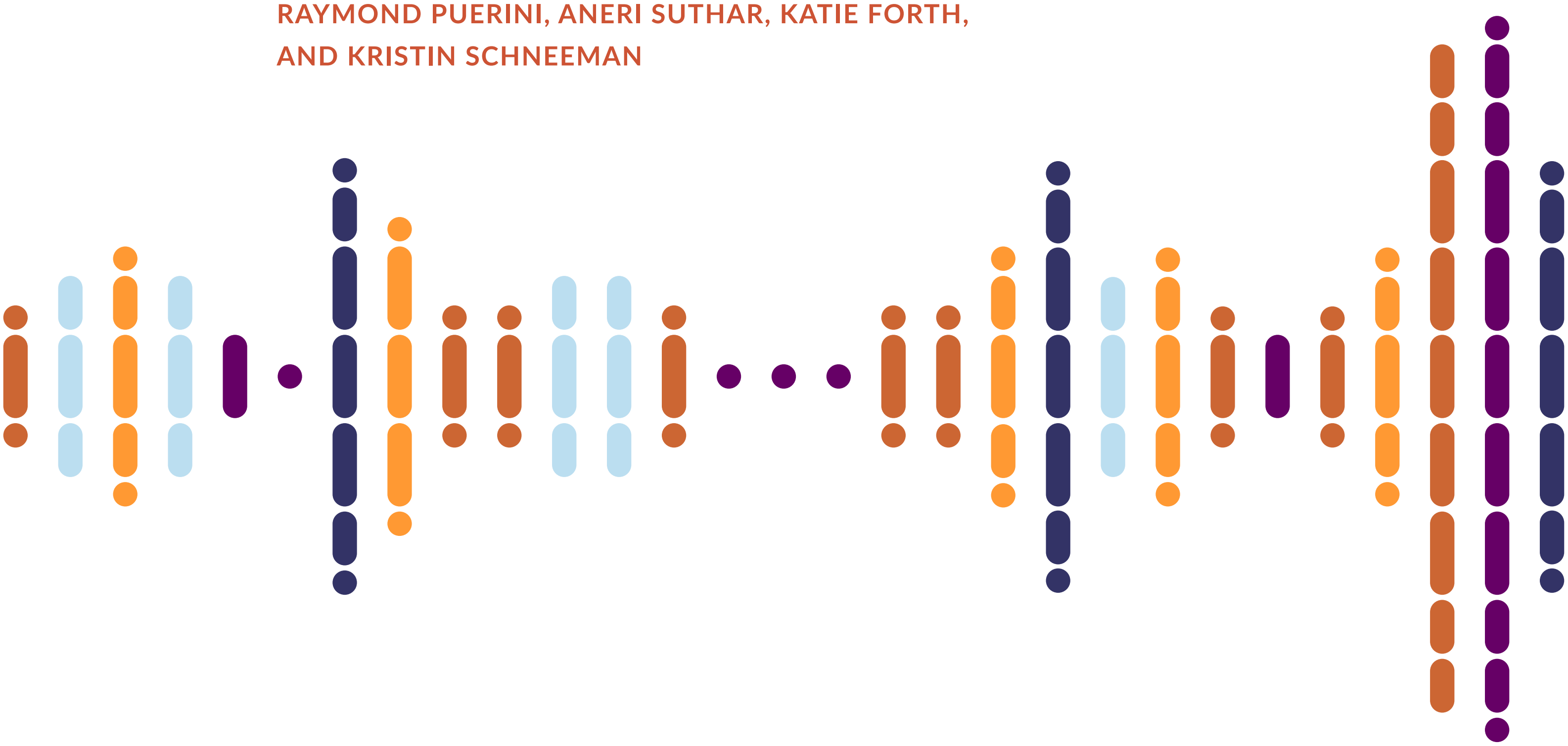

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The Milken Institute is a nonprofit, nonpartisan think tank focused on accelerating measurable progress on the path to a meaningful life. With a focus on financial, physical, mental, and environmental health, we bring together the best ideas and innovative 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.

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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.

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Executive Summary

FasterCures has consistently contributed to advancing patient engagement (PE) in biomedical research in the last 15 years by undertaking initiatives aimed at bringing together the voices of diverse stakeholders to assess gaps and solutions and by developing tools and resources that decision-makers can leverage. In 2022, FasterCures released a report on ***The Current Landscape of the Science of Patient Input***, which explored advances in PE in biomedical research and identified opportunities for addressing remaining challenges, including:

- establishing how patient information is used in decision-making by sponsors and regulators,
- building a precompetitive space for knowledge sharing, and
- investing in building capacity in the patient community.

FasterCures has most recently explored how these insights and recommendations apply to the context of medical device, diagnostic, and digital health (known collectively as "medtech") product development. The field of medtech is broad and diverse and has generated groundbreaking advancements in recent years. It encompasses minimally invasive surgical devices and diagnostic imaging tools used primarily in the health-care system, as well as patient-operated devices, such as remote monitoring devices and wearable technologies that monitor glucose, track vital statistics, and facilitate health and wellness. Across the diversity of medtech products, the approaches taken to PE in research and development (R&D) activities vary widely.

PE across biopharmaceutical R&D and medtech has evolved at different paces, with medtech generally seeing slower and less uniform adoption across the spectrum of products, likely due to a number of factors, including:

- significant heterogeneity of products that include both patient-facing and non-patient-facing devices, diagnostics, and digital health technologies;
- differences in authorization requirements and regulatory pathways;
- shorter development timelines and differences in early R&D pathways; and
- challenges identifying appropriate patient community partners.

CHALLENGES AND BARRIERS TO PATIENT ENGAGEMENT IN MEDTECH

A number of barriers contribute to low rates of PE in medtech product development. Many of these challenges are the same as for drug developers, but some have aspects unique to medtech:

- resource constraints and poorly understood return on investment,
- compliance concerns,
- lack of regulatory and reimbursement clarity,
- reaching appropriate patient populations,
- engaging early,
- adoption of tools and frameworks, and
- cultural inertia.

CAPTURING THE BENEFITS AND VALUE OF PATIENT ENGAGEMENT ACTIVITIES IN MEDTECH

Important benefits contribute to the value and return on investment of PE:

- increased commercial success;
- expanded product indication;
- enhanced investor relations;
- reduced time to market;
- more informed regulatory, health technology assessment, and coverage decisions;
- improved patient satisfaction and use of product;
- reduced R&D cost; and
- improved clinical trial recruitment efforts.

Some of these benefits are currently measured by some companies, and others would be important to formalize and translate into trackable metrics. There are available metrics to measure the value and impact of PE, though most of them have been developed in the context of the biopharmaceutical industry and have not been widely adopted in the medtech industry. Examples include:

- PFMD's Patient Engagement Metrics Selector
- PARADIGM's Patient Engagement Monitoring and Evaluation Framework
- FasterCures' Patient Perspective Value Framework
- Clinical Trials Transformation Initiative study of expected net present value

- ICON’s **Patient Involvement Value Dossier**
- A **collaborative study** conducted by DIA and the Tufts Center for the Study of Drug Development.

OPPORTUNITIES TO SPUR MORE PATIENT ENGAGEMENT IN MEDTECH

A number of factors create opportunities to encourage more regular and rigorous PE activities in the medtech industry:

- Evidence of the value of PE exists.
- Many resources exist to inform PE approaches.
- Regulators’ commitment to PE remains high.
- Demand for and rigor around the use of real-world data in R&D is on the rise.
- The whole ecosystem is prioritizing diversity and inclusion in research.
- Medtech has a culture of rapid-cycle innovation and user-centered design.
- There is an appetite for change.

RECOMMENDATIONS

Given the benefits of and current opportunities for enhancing PE in medtech R&D, we offer the following recommendations for action:

- Craft a framework to more clearly define and demonstrate the value and impact of PE for the diverse range of medtech product developers.
- Develop a toolkit that curates existing tools and resources, and creates new ones to fill identified gaps, that can be used by medtech companies seeking to engage patients in their R&D activities.
- Adapt resources created for drug development and human-centered design contexts.
- Build the capacity of the medtech ecosystem to support more PE.
- Request that regulators and payers provide more clarity and examples of the impact of PE on their decision-making.
- Address legal and compliance challenges.
- Identify and address misalignments in requirements and processes.

CONCLUSION

Patient engagement in medtech product development lags behind its adoption in drug development likely for reasons that are specific to the medtech context (or contexts, given the heterogeneity of types of products and approval pathways of medtech products). The smaller size of companies, shorter development timelines, and diffuse patient populations make imperative a clearer definition and demonstration of the value and impact of engaging patients across the development life cycle as a means to encourage more PE. Stronger signals from regulators, payers, and investors about their interest in using the outputs from PE activities in their decision-making, perhaps leading to a higher likelihood of favorable outcomes, would accelerate developers' investment in these activities.

Introduction

FasterCures has consistently contributed to advancing patient engagement (PE) in biomedical research in the last 15 years by undertaking initiatives aimed at bringing together the voices of diverse stakeholders to assess gaps and solutions and by developing tools and resources that decision-makers can leverage. In 2022, FasterCures released a report on ***The Current Landscape of the Science of Patient Input***, which explored advances in PE in biomedical research and identified opportunities for addressing remaining challenges. The report found evidence of progress as a result of regulatory leadership, new guidance documents, and the proliferation of tools and resources. The report also identified persistent challenges, including patient communities' capacity to serve as effective partners, poor understanding of the value of PE, underuse of tools and resources, a need for more widely available examples of best practices, and a lack of alignment across stakeholders. The report's recommendations for addressing these persistent barriers included establishing how patient information is used in decision-making by sponsors and regulators, building a precompetitive space for knowledge sharing, and investing in building capacity in the patient community.

FasterCures has most recently explored how these insights and recommendations apply to the context of medical device, diagnostic, and digital health (known collectively as "medtech") product development. As part of this assessment, we wanted to better understand the approaches medtech companies are using for PE, where they face barriers, and how they and their collaborators perceive and quantify the value of pursuing PE activities. Investigating these topics is ultimately aimed at incentivizing more holistic approaches to PE in medtech by better defining and demonstrating the value and return on investment (ROI) across the life cycle of medtech research and development (R&D) activities, and for a diverse range of medtech company sizes and product types.

This report summarizes our assessment of PE in the medtech space, including barriers, benefits, metrics for capturing the value of PE, and opportunities to expand the use of patients' perspectives in medtech R&D. It contains useful information for medtech product developers as well as others with whom they collaborate across the R&D continuum.

Overview of Patient Engagement in the Medtech Landscape

The field of medtech is broad and diverse and has made groundbreaking advancements in recent years. It encompasses minimally invasive surgical devices and diagnostic imaging tools used primarily in the health-care system, as well as patient-operated devices, such as remote monitoring devices and wearable technologies that monitor glucose, track vital statistics, and facilitate health and wellness. Across the diversity of medtech products, the approaches taken to PE in R&D activities vary widely.

WHAT WERE THE INPUTS TO THIS REPORT?

- **Desktop research:** Review of 71 sources from peer-reviewed journals, published research, grey literature, and online sources.
- **Key informant interviews:** 57 interviews to date with leaders involved in developing, funding, or using medical devices and digital technology, including product developers of varying sizes, patient organizations, nonprofit umbrella organizations, investors, value assessors, and health-care providers.
  - Targeted device companies developing Class III products that required clinical trials and are patient-facing (i.e., products that are directly used by patients), consumer-facing (i.e., products marketed directly to consumers), or implantable, therapeutic, or aesthetic devices.
  - Targeted digital health companies with FDA-approved products and patient-facing products as characterized in the Digital Therapeutics Alliance fact sheet, regardless of whether they underwent a clinical trial.
  - To ensure diversity of perspectives, we also targeted medtech companies across multiple product types, company sizes, and levels of market capitalization.

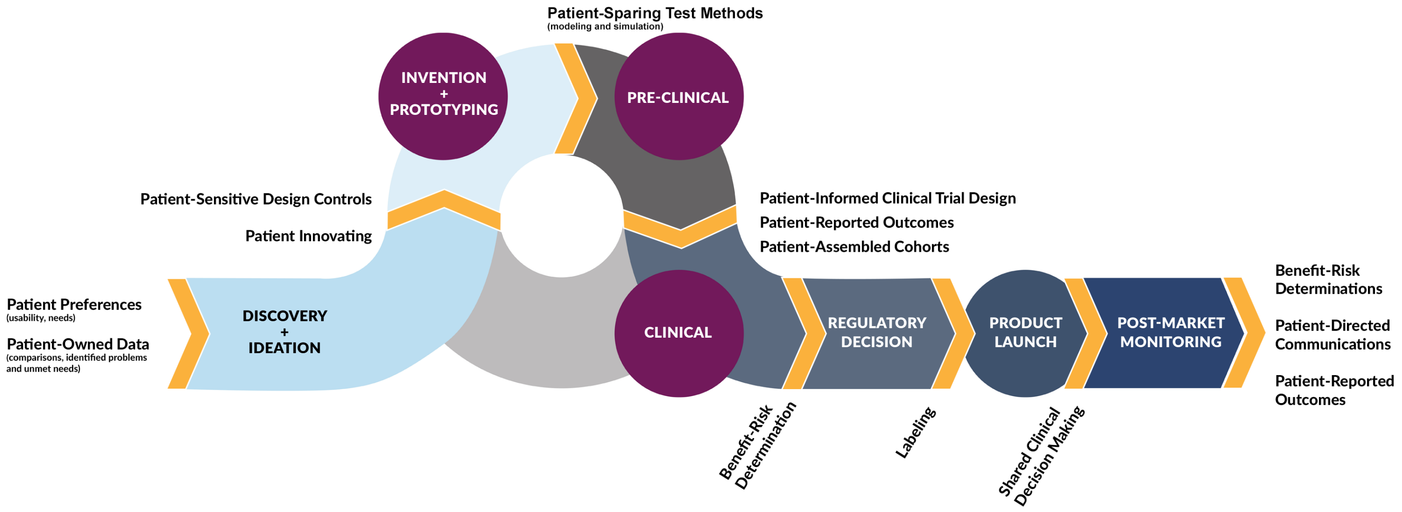
PE in medtech has evolved over the years, largely thanks to federal commitments to encourage more PE in R&D described in past and current iterations of the Medical Device User Fee Amendments (MDUFA), the 21st Century Cures Act, and priorities set by the Center for Devices and Radiological Health (CDRH) at the US Food and Drug Administration (FDA). These collective efforts led to the creation of new CDRH programs and guidance documents dedicated to the expansion and support of PE in medtech R&D activities, including the **CDRH Patient Engagement Advisory Committee**, **guidance on *Patient Engagement in the Design and Conduct of Medical Device Clinical Studies***, and multiple guidance documents and case examples related to use of **patient preference information** and **clinical outcome assessments** in medical device decision-making. In 2023, through the current implementation of MDUFA V, CDRH rolled out the **Total Product Life Cycle Advisory Program (TAP)** to provide more thorough and frequent pre-market interactions to support developer decision-making, enhance collaborative activities (including PE), and align evidence generation expectations.

In addition to FDA-developed PE resources, there has been a proliferation of resources developed by a range of stakeholders, including medtech developers, the **Patient-Centered Outcomes Research Institute** (PCORI), nonprofits and umbrella groups including **FasterCures,** the **Medical Device Innovation Consortium** (MDIC), **AdvaMed, Patient-Focused Medicines Development (PFMD)**, the **National Health Council**, **DIA**, the **Digital Medicine Society (DiME)**, and others. Many of the resources were developed with the biopharmaceutical context in mind, though some are medtech-specific and others are relevant regardless of the product type.

PE in medtech is at a less advanced stage relative to the biopharmaceutical sector. While there is increased awareness and interest in PE compared to a decade ago, its perceived relevance may vary based on the type of technology, the nature of the clinical issue that it addresses, its mode of use, its end user, and the perceived value and benefit of doing PE. In a recent unpublished **survey** of medtech companies by AliraHealth, 60 percent of respondents understood the importance of PE but just 15 percent had implemented some form of PE activities. However, there is interest among medtech companies, including small- and mid-sized organizations, in improving their PE practices, as evidenced by, for instance, a new initiative by AdvaMed to educate manufacturers about PE and foster an atmosphere of sharing the benefits and best practices.

Along the total product life cycle (TPLC), medtech companies can engage patients from the very early stages of product ideation all the way to post-approval and marketing activities. Figure 1 below, from CDRH, describes the various ways in which patient input may be applied across the medical device R&D continuum. Patients can have a role in helping to set pipeline and fundraising priorities; advise on product design, prototyping, informed consent, clinical trial design, and outcome measures; inform regulatory, value, and coverage decisions; and provide input on product education and communication initiatives.

Figure 1: Patient Input in Medical Device Total Product Life Cycle



Source: Adapted from 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 by the Food and Drug Administration (2016)

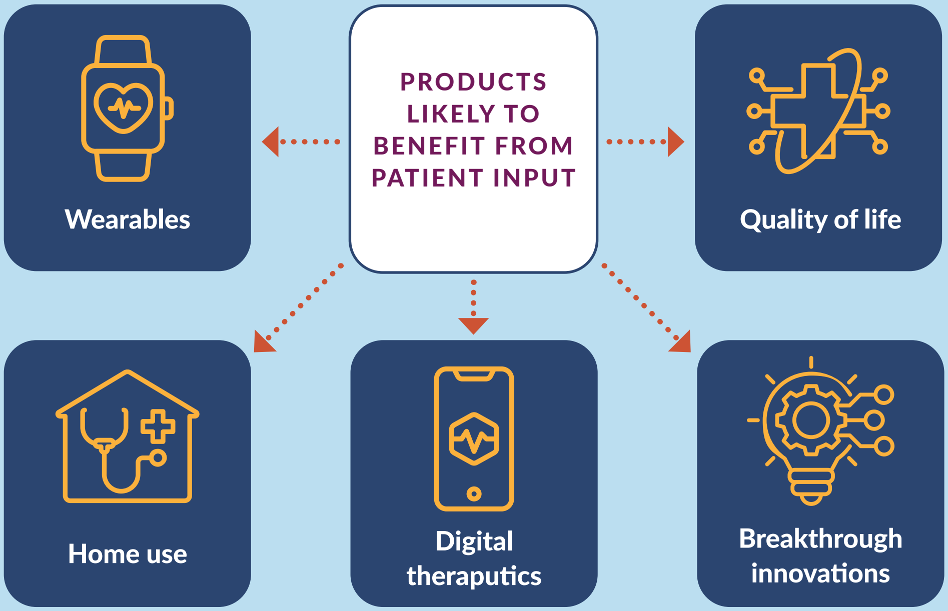
Whether and how patient input is actually sought varies considerably. A 2018 MDIC **survey** found that 50 percent of companies never sought feedback from patients when finalizing a study protocol, and 63 percent never sought feedback from patients on operational strategy after protocols were finalized. The vast majority of companies that did engage patients did not engage them consistently for every study protocol. In the AliraHealth **survey** mentioned previously, responding medtech companies indicated varying levels of PE across the life cycle, including in the research design and planning phases, regulatory activities, health technology assessment and pharmacovigilance, and pre- and post-launch activities; patients were reportedly most frequently engaged in the research planning and design and regulatory phases. Some level of PE was evidenced at all stages of R&D, suggesting that there is an opportunity to demonstrate the value of taking a more comprehensive approach to PE across the TPLC rather than discrete steps.

As in the pharmaceutical sector, there continue to be discrepancies in terminology and perceptions of PE among medtech developers, providers, patients, regulators, and payers, indicating a need for better alignment as to what types of activities are best suited for what purposes. For example, developers may characterize their PE efforts as “customer discovery,” “market analysis,” or “user and human factors testing,” which provide important information for developers but may not be considered meaningful or actionable PE by some decision-makers. For the purposes of our project and conversations, we adopted the ISPOR Patient-Centered Special Interest Group’s **definition** of PE: **“The active, meaningful, and collaborative interaction between patients and researchers across all stages of the research process, where**

research decision-making is guided by patients' contributions as partners, recognizing their specific experiences, values, and expertise."

In our key stakeholder interviews, participants shared perceptions about specific medical device, diagnostic, and digital health products that they believed would most benefit from patient input. These included products aimed at improving patients' quality of life (e.g., cochlear implants), as distinct from "lifesaving" products (e.g., pacemakers); products that are visible and/or worn on a patient's person; home-use or over-the-counter products; digital therapeutics; and products with breakthrough innovation potential (see Figure 2).

Figure 2: Products Likely to Benefit from Patient Input



Source: Milken Institute (2024)

However, it is important to note that these may be product types for which seeking patient input seems more readily achievable, a more obvious choice, or more justifiable with internal stakeholders. Principles of PE, however, require viewing people with lived experience of a condition as both key stakeholders in the R&D process at every stage and as key end users. These principles would require considering what insights they can offer that are relevant to the specific context, what they think would benefit the process, and how they can provide value to other stakeholders, including payers, providers, and the broader patient population.

PE across biopharma and medtech has evolved at different paces, with medtech generally seeing a slower and less uniform adoption across the spectrum of products. These differences are likely due to a number of factors, including:

- **Significant heterogeneity of products that include both patient-facing and non-patient-facing devices, diagnostics, and digital health technologies.** In some cases, providers rather than patients are targeted for engagement based on the uses of a product. In addition, there may be less direct-to-consumer marketing or product name recognition in medtech, which may have an impact on the perceived benefits of engaging the patient community.

- **Differences in authorization requirements and regulatory pathways.** Many devices and digital health products do not require clinical trials for authorization, or the FDA does not exercise its enforcement discretion over them. In the biopharma space, there is also a **requirement** that the FDA complete a Patient Experience Data (PED) table that allows the public to see the types of PED that it considered when it reviewed a new drug application, according to the Food and Drug Law Institute. In contrast, FDA is not required to complete a PED table for device reviews and thus medtech developers may not be as incentivized to submit PED in their submissions. (To be clear, PED does not always equate to "patient engagement." It often consists of patient-reported outcomes (PROs), which are not necessarily patient-centric or the result of engagement with patient communities.)

- **Shorter development timelines and differences in early R&D pathways.** Some medtech products originate in clinical or specialty settings or with engineers or app developers. In some cases, patients may not be considered until after a working prototype is available or until after a product is on the market.

- **Challenges identifying appropriate patient community partners.** Medtech products may serve populations without specific foundations or communities organized around addressing their needs (e.g., joint replacement devices) or address a range of conditions. Biopharma companies often develop products aimed at addressing specific conditions that may be more likely to have patient organizations in place to address the needs of those with the disease. Patient organizations may also perceive the role of drugs and devices differently in addressing their conditions of interest.

Challenges and Barriers to Patient Engagement in Medtech

Our research and interviews revealed a number of barriers that contribute to low rates of PE in medtech product development. Many of these challenges are the same as for drug developers, but some have aspects unique to medtech.

1. **Resource constraints and poorly understood return on investment.** PE requires staff, time, budgets, processes/procedures, and training, making evidence of its benefits imperative, whether those are seen in greater success in recruitment, higher likelihood of regulatory approval, more favorable treatment by payers, or a more competitive standing in the market. Medtech companies as a class tend to have shorter development timelines and less available capital, making justification of the value of PE perhaps even more important than in the drug development industry. These resource constraints are particularly limiting for smaller companies that operate with fewer resources.

2. **Compliance concerns.** PE presents challenges related to privacy, legal liability, and human subjects protection that require resources to address. Medtech companies may have some unique issues in this regard, especially those that are directly patient-facing and/or involve direct data collection from patients. Diverse regulatory approaches to devices and digital health products may create an additional lack of clarity that results in a lower level of risk tolerance where compliance is concerned.

3. **Regulatory and reimbursement clarity.** While regulators have been one of the important stakeholders promoting greater PE in product development, developers still often cite as an impediment a lack of certainty as to whether and how PE practices and patient experience, preference, and outcome data will be considered in regulatory decision-making. Despite—or perhaps in part because of—multiple guidances across the FDA, differences exist in the approach among the various centers at the FDA, and some have called for more consistent recommendations. There is also limited insight by product developers into how public and private payers accept and factor in patient perspectives, outcomes, and metrics into their decisions.

4. **Reaching appropriate patient populations.** Medical product developers of all types can be challenged to identify and engage the right patients and patient organizations with whom to partner for the type of insight needed. Medtech companies can be especially challenged as there is frequently not a ready-made patient community or patient foundation organized around the technologies they are developing.

   In recent years, the recognized need to make studies more representative of the specific populations needing and using medical products has added to the complexity. Even patient advocacy organizations, while tremendously helpful in connecting medtech companies with patients, are not always connected with a representative community of patients. Reaching out exclusively to organized patient communities can risk excluding the perspectives of those who may not be as well-connected. Finally, patients from communities that have been historically underrepresented or mistreated by the medical establishment sometimes hesitate to engage with companies or participate in trials, highlighting the importance of developing trust and centering health equity in PE.

5. **Engaging early.** Medical product developers have had to undergo a culture shift from one that views patients as consumers to whom they market products at the end of the process to one that treats them as key stakeholders who should be consulted at the earliest stages of research (as exemplified by this **resource** from Boehringer Ingelheim and Savvy Cooperative). Medtech developers have some special challenges in this regard, as they may be more accustomed to thinking about engaging "end users" to test their products relatively late in the development cycle, at which point improvements can only be made in future iterations or, in some cases, may no longer be economically feasible.

6. **Adoption of tools and frameworks.** As previously noted, there has been a proliferation of tools and resources to enable PE in recent years, but we have found persistent underuse of those frameworks, guidances, and metrics. Most are not specific to the context of the medtech industry, though there are some adapted for medtech from sources such as **MDIC**, **PFMD**, and **AliraHealth**. Additionally, existing tools and frameworks are not necessarily standardized, with different stakeholders using different terminology surrounding PE, making them difficult to navigate.

7. **Cultural inertia.** Many medtech developers are used to operating in a certain way, and absent external regulatory pressures or convincing data about the financial ROI of PE, it can be difficult for companies to justify the investment. Additional incentives (both ones that reward engaging patients and penalize/discourage not doing so) will be needed to shift the cultural norm in the industry.

PE IN PRACTICE: EDWARDS LIFESCIENCES

The goals of the patient engagement work at Edwards Lifesciences are to better understand unmet needs along the patient journey, elicit the patient perspective, and improve the patient experience by supporting advocacy efforts through patient outreach. By elevating the patient voice and highlighting what matters most to patients, Edwards strives to remove barriers to care. Edwards does this through a variety of activities and engagements, which include convening patient listening sessions with employees, conducting patient preference research, supporting patient advocacy groups, and collaborating with external partners to advance patient engagement within the medical technology industry.

Edwards believes that when patients share their experiences, they inspire others to speak up and support each other. Edwards views patient advocacy groups as a vital stakeholder with the aim to understand and improve care, and the company supports such organizations through grants, sponsorships and charitable contributions. Edwards has set global **guiding principles** to ensure collaboration with patient organizations is productive and ethical.

As an example of Edwards' patient engagement initiatives, the company holds annual **Patient Experience** events, which were hosted in 11 countries in 2023. The company views the Patient Experience events as an important piece of how it fosters a patient-focused culture. During the events, the company welcomes patients and their care partners to Edwards to create and strengthen impactful connections between patients and employees. From symptoms and diagnosis through treatment and recovery, these touchpoints provide their teams with important insights into the patient journey to understand what patients are experiencing.

Another example of Edwards' work in patient engagement is the insights the company gathers by conducting patient preference research. Through these preference surveys, the company aims to better understand the patient perspective and preferences at each step of the patient journey. Edwards strives to then incorporate the feedback gathered into each stage of the product development process. By intentionally capturing patient input, Edwards endeavors to design innovations that address patient needs and improve quality of life. These efforts align with the company's focus on addressing the unmet needs of structural heart patients.

Capturing the Benefits and Value of Patient Engagement Activities in MedTech

Our research and interviews identified important benefits that contribute to the value and ROI of PE. Some of these are metrics that companies can and do evaluate, and others would be important to formalize and translate into trackable metrics.

- **Greater commercial success.** This can be defined as a product's market adoption and adherence, and its market share in cases where direct competition exists. PE in earlier design phases may help ensure the product is user-friendly, comfortable, functional, and aligns with needs and preferences of patients. It also allows companies to tailor their marketing approach and outreach.

- **Product indication expansion.** Real-world evidence and other insights derived from PE can contribute to product indication expansion by demonstrating growth points, effectiveness, and benefits of a product in broader or different patient populations. Regulators have worked with manufacturers to conduct PE efforts, such as patient preference studies to determine the appetite for expanded uses. For example, FDA cleared an expanded indication for a home hemodialysis machine in August 2018, citing the sponsor's patient-preference study about kidney patients' risk tolerance in their decision.[1]

- **Enhanced investor relations.** Though some manufacturers felt that PE information has no impact on investor decisions, others believed that patient-generated insights help to bolster the qualitative presentation of the technology and speak to how the product is addressing unmet needs. Even if investors do not ask directly about PE activities in their up-front due diligence, some indicated that there is an increasing expectation that developers are engaging patients in the design and execution of R&D programs, if only to ensure accrual to trials and uptake of marketed products. Being able to attribute adherence rates and other measures to PE, or even claim that they are heavily informed by patient experiences, helps companies convey confidence to investors that those results are reproducible.

- **Reduced time to market.** Manufacturers identified that an important reason to prioritize engaging patients would be reduced development timelines and regulatory evaluation. Engaging patients in product and trial design has been shown by DIA to help reduce costly protocol revisions; enable faster planning, approval, and enrollment; and help target endpoints that matter to patients.

- **Informing regulatory, health technology assessment, and coverage decisions.** Collecting information on patients' risk tolerance is valuable input for regulatory decision-making and the use of patient preference information in regulatory decisions is sometimes considered, though it may not be used in all cases. CDRH highlights on its website numerous examples of how both patient preferences and clinical outcome assessments (including patient-reported outcomes) have been used to inform regulatory decisions, with 52 percent of authorizations including PROs in clinical studies as of 2024.

  Informing payer decision-making has also been identified as an important benefit of PE. A 2022 MDIC report confirmed that payers are interested in a representative patient perspective, yet it is still not a significant component of coverage determinations. Both the MDIC report and our stakeholder interviews noted, however, that payers and organizations that conduct health technology assessments (HTAs) are most likely to consider the results of PE when there are multiple technologies with similar safety, effectiveness, and cost in the market. In these cases, evidence that products can effectively improve adherence, quality of life, and additional health outcomes that are of interest to patients can positively affect payer/HTA decisions. Payers may also perceive patient preference information to be relevant for coverage decisions based on methods used to gather the patient preference information, including their objectivity, perceived accuracy to define risks, and generalizability to the broader population, disease severity, risk of complications, costs, burden of disease, unmet medical needs, disparities between what clinicians consider important versus what patients do, and whether barriers to patient acceptance are understood.

- **Improved patient satisfaction and use of product.** Some product developers, especially those in the digital health space, indicated that they track patient satisfaction and use of their tools. For products that have either a therapeutic or monitoring component, they may also track disease progression over time or adherence to a drug or therapeutic intervention. Positive patient experience can translate to improved adherence, which in turn can translate to ongoing use and reimbursement. Greater adoption and adherence to a product is a clear competitive advantage and can decrease medical costs by reducing the occurrence of adverse events. Satisfied users are also likely to be "net promoters," recommending products via word of mouth or social media, demonstrating the benefit from a marketing and branding standpoint.

- **R&D cost reduction.** Some manufacturers indicated that they are strategically looking at how PE in the R&D process could allow them to avoid unnecessary costs or help reduce costs associated with product redesign, protocol amendments, etc. Interviewees pointed to the positive impact of PE on cost avoidance during both the product ideation phase, as well as all aspects of the development and clinical trial design and operation phases.

- **Improved clinical trial recruitment efforts.** A primary motivator of PE has long been improving recruitment to clinical trials, and there is ample evidence now that effective early engagement with patient communities can result in faster recruitment and higher retention rates. A **CTTI report** found that patient-centric study efforts can result in many positive impacts, including improved recruitment, retention, and reduced clinical trial cycle time, among others. Engaging patients in product development and clinical trials can both improve clinical trial outcomes and involve a more representative patient population.

PE IN PRACTICE: AMERICAN ASSOCIATION OF KIDNEY PATIENTS

The **American Association of Kidney Patients (AAKP)**, founded in 1969, has long understood the value of bringing patients to the table as partners to codevelop devices and clinical trial approaches. To help bring patients to the table with device manufacturers, AAKP developed tools and resources for kidney patients who are interested in learning about FDA regulatory processes and the intricacies of working with industry.

AAKP developed a **Center for Patient Engagement and Advocacy** to serve as a hub for government, academia, scientists, and industry to engage kidney patient “experts.” This center, along with AAKP’s **Center for Patient Research and Education**, service requests for clinical trial design and awareness, recruitment, patient advisory boards, technical evaluation panels, focus groups, and surveys to increase the use of unique patient insights across the entire product development life cycle as well as within regulatory and payer decisions.

AAKP, as well as many other allied patient organizations, have long been trusted voices within their respective patient communities and, as a result, have been able to gain traction and high engagement through their awareness and education campaigns. The organization provides key insights to Congress and federal agencies and has developed a robust survey mechanism to provide real-time feedback to the FDA and the Centers for Medicare and Medicaid Services (CMS) on the impact of regulatory and payment decisions on kidney patients. AAKP also organizes patient advisory boards to review amendments or changes to clinical trial protocols, identify site locations to ensure diverse ethnic and geographic representation among participants, and evaluate informed consent documents and patient materials to ensure clear and appropriate messaging. AAKP has found that device manufacturers that lean into PE across the product life cycle see the value and ROI in these interactions, such as improved product development and enhanced trial experience, including increased retention of enrollees and more easily meeting diversity marks. These outputs have led to PE being incorporated as a standard of excellence among some of their new and long-term industry partners.

“We encourage companies to start with the patient and end with the patient. Ultimately, innovation and development are for the benefit of the end user, so it is important that end users are part of the entire process.”[2]

Metrics and Measures for Medtech Patient Engagement Impact

There are available metrics to measure the value and impact of PE, though most of them have been developed in the context of the biopharmaceutical industry:

- PFMD's **Patient Engagement Metrics Selector** helps companies identify relevant metrics to evaluate and monitor the outcomes and impacts of their PE efforts. It consists of 87 metrics, including patients' willingness to participate in research studies, emergence of new research questions, and money spent. PFMD is currently working to adapt these metrics for the medtech industry.

- PARADIGM's **Patient Engagement Monitoring and Evaluation Framework** is intended to help companies identify and tailor the metrics relevant to their specific objectives and purpose. It consists of a list of 105 metrics categorized into input metrics, activity and process metrics, learning and change metrics, and impact metrics.

- The **Clinical Trials Transformation Initiative** has taken a more direct approach to quantifying the benefits of PE by using risk-adjusted financial models to evaluate the financial impact of PE. This study assesses the impact of PE on a combined value measure of revenue, costs, time, risk, and intangibles within a company's portfolio (i.e., the expected net present value, or ENPV). The authors conclude that the reduction in costs and increases to ENPV significantly exceed the initial required investment in PE activities, potentially by several hundred times the investments in engagement.

- FasterCures also developed the **Patient Perspective Value Framework** to provide metrics and criteria to help assess the value of multiple health-care options from the patient's perspective. These metrics could have cross-over applicability when thinking about patient engagement in the regulatory, coverage, and post-approval contexts.

- ICON's **Patient Involvement Value Dossier** provides an overview of the evidence that is available to demonstrate the value/ROI of PE in clinical trial development and provides case examples of how PE positively affects cost savings and trial design and efficiency.

- A collaborative study conducted by DIA and the Tufts Center for the Study of Drug Development assessed the impact of patient-centric initiatives in biomedical research. As part of this effort, they looked at over 120 case examples and identified a range of different metrics applicable to long-term drug development portfolio considerations, internal and external reach, study volunteer feedback, and trial performance. Their analysis also assessed the ease and frequency of metric collection, and reported impact of PE related to study quality and speed as well as impact on the patient.

Despite the availability of these resources, most medtech companies we spoke with did not have specific metrics established to demonstrate the value and ROI of PE to their operations. Overall, those interviewed believed that medtech companies are putting in the effort to capture end-user perspectives as part of their development processes, though few standardized and widely used metrics were used for capturing the value of those efforts.

The concept of ROI often implies a financial return, though the benefits of PE cannot always be captured or expressed in those terms. Barry Liden, former vice president for patient engagement at Edwards Lifesciences, has reframed the ROI of PE as “impact of engagement.”[3] In Liden’s view, the main purposes for measuring the impact of engagement are to learn, improve, and evaluate, and metrics can generally be oriented toward either 1) patient, provider, and employee experiences related to their participation in R&D activities, which are generally easier and faster to measure, or 2) outcomes, which are often more challenging and require a longer time to measure.

Examples of measures and metrics to assess activities and outcomes related to PE across the TPLC of medtech development include:[4]

Pre-Clinical/Discovery

- New research questions/areas identified
- Relevance of new studies/products aligned with patient needs
- Number of prototype revisions

Clinical Development

- Identification of meaningful trial endpoints
- Number of trial revisions
- Metrics related to diverse and representative patient recruitment and retention
- Time to enrollment and study completion

Regulatory/Payer Authorizations/Approvals

- Use of patient preference and/or patient-reported data
- Number and type of comments and feedback received from regulators and payers

Post-Approval

- Patient satisfaction with and access to new products
- Safe and effective use measures
- Market share and product financial performance
- Product usage, compliance, and retention

In determining the metrics to evaluate the impact of PE, the key steps are identifying the end users of the information and determining which metrics are most important from their perspectives.[5] End users could include company leadership, business-unit leadership, different teams within the organization (R&D, regulatory, marketing, etc.), and external collaborators (e.g., academic researchers, patient representatives, clinicians, regulators, payers, investors, etc.). The measurement of activities, experience, and outcomes associated with PE activities can help to demonstrate the many benefits listed previously in this report and can help to justify the investment of resources, time, and effort by all parties.

PE IN PRACTICE: TIDEPOOL

Tidepool is an unusual digital health product developer—a nonprofit organization founded to make diabetes data more accessible, actionable, and meaningful for patients, caregivers, and providers. Tidepool's existing ecosystem of tools has supported over 650,000 patients. One of Tidepool's products, Tidepool Loop, originated as a patient-led initiative and is the first fully interoperable automated insulin dosing app cleared by FDA. Tidepool forged a unique path to bring forward technology that prioritizes patients' preferences, data interoperability, and the use of real-world evidence to unlock patient-led innovation. The company engages patients and caregivers for feedback, starting with early prototypes.

> **"I invite you to find people who have the deepest understanding of both the needs and the solution. Learn from what they are building. Incorporate them as equal partners in the design phase and support this co-production."[6]**
>
> *—Kelly Watson, Vice President, Product and User Experience, Tidepool*

Tidepool employs several approaches to place the patients and users at the core of the innovation journey. The first is creating a scalable mechanism to engage patients by building a user research community registry of over 4,000 prospective participants. In this initiative, Tidepool engages with patient advocacy organizations to grow the community and asks existing product users to opt in and participate in research for new products.

The second approach is developing a decentralized, iterative user research and usability testing model. Due to the pandemic, Tidepool had to shift its human factors studies to remote settings and adopted software tools and study protocols that enabled research and design teams to interact with geographically diverse participants in their natural and intended-use environments, recording interactions with devices from multiple perspectives. They continue to use those processes to engage with patients. The third approach is building cross-functional cultures of PE, wherein the organization continually considers how PE fits into the structure, at what level patients should be included as stakeholders, and ensures that engagement communication flows between teams, affecting the products they produce.

Although Tidepool has not developed official metrics for valuing PE, the value of patient insight is deeply ingrained in its mission. It sees incredible potential to design products with patients and end users so that medical devices and apps can allow people with chronic conditions to do more of what they love.

Opportunities to Spur More Patient Engagement in Medtech

Based on our scan of the environment, a number of factors create opportunities to encourage more regular and rigorous PE activities in the medtech industry.

- **Evidence of the value of PE exists.** The field has matured to the point where it is possible to see quantitative and qualitative evidence of the benefits of PE (as well as the risks of not engaging patients early and often), including improved recruitment of research participants, reduced R&D costs and time to market, influence on regulatory and coverage decisions, and improved patient adherence. A number of tools and resources to guide the measurement of the impact of PE are available.

- **Many resources exist to inform PE approaches.** As we have indicated, there are copious tools and resources available, mostly developed for the context of drug development, that can be leveraged for medtech's purposes. Resources available through **PFMD's Synapse platform**, **MDIC**, **PCORI**, **FDA**, **DiMe**, and other organizations are available, if underused.

- **Regulators' commitment to PE remains high.** **FDA's MDUFA V commitments** are renewing and refreshing CDRH's focus on PE, including staff training on patient science and engagement to ensure consistent evaluations, development of education modules for industry on PE topics, and development of case examples. Its newly launched **TAP program** has the potential to identify and encourage promising practices for PE in medtech and to provide additional clarity around regulatory expectations and use of PE data and outcomes for regulatory decision-making. CMS has a new mandate to consider patient experience data in its implementation of Medicare drug price negotiations, which creates an opportunity for a more consistent approach to engaging patients in its decision-making.[7]

- **Demand for and rigor around the use of real-world data in R&D is on the rise.**[8] Patient experience data and patient-reported data are key sources of evidence that increasingly inform product development and support regulatory and reimbursement evaluations.

- **The whole ecosystem prioritizes diversity and inclusion in research.** The drive for more representative research, **including draft guidance introduced by FDA**, has created a new imperative for effective strategies that can engage patients in communities previously under-represented. The DiME Society's **Framework for Inclusive Development** of digital health products provides a roadmap for patient-centric product development more broadly.

- **Medtech has a culture of rapid-cycle innovation and user-centered design.** Iterative and human-centered design are traditional strengths of engineers and digital health product designers, creating an opportunity to link those approaches to a more patient-engaged approach across the TPLC. These are strengths that could be informative to a broad range of medtech companies as well as biopharmaceutical product developers.

- **There is an appetite for change.** Medtech trade associations see increasing interest from companies in building their capacity for effective PE. There is also increasing interest in patient-led innovation of the kind represented by Tidepool, and to which digital health particularly lends itself.

PE IN PRACTICE: SYNCHRON

Capturing and incorporating the patient voice into the end product has been critical for **Synchron**, a company developing the first endovascular, minimally invasive brain-computer interface. This device will allow people suffering from severe paralysis to engage with the world and communicate. Synchron sees people with paralysis as teammates, users of their systems, and stakeholders. It is constantly finding ways to engage the community and collect their preferences and feedback, to ensure that the company is designing a device that they will want and use and that is filling patients' needs. Given that its device is surgically implanted, Synchron engages people with paralysis to ensure the surgery is a risk that patients are willing to endure for the potential benefits.

Synchron started its PE efforts by speaking with prospective users and understanding their biggest unmet needs. It was very important for Synchron to seek insight from individuals with lived experience and build with the community, not for them. Following the needs-finding research, Synchron aimed to understand the community's public perception of the technology.

Many of its potential patients have lost the ability to communicate verbally and are in long-term care facilities, so identifying ways to capture perspectives from varying patient populations and their caregivers was critical and often involved patients using accessibility devices. To identify patients, the company worked with patient advocacy groups, spoke with physicians who provide care to target patient populations, and conducted direct outreach across the nation. Synchron incorporated the patient preference data it collected into study designs and protocols.

Synchron measures the value of PE in a number of ways. An important feedback metric it collects is patients' willingness to recommend its devices to others. As many of its patients are on Medicaid due to their disability status, Synchron has also begun coverage conversations with CMS and aims to advocate for patients by leveraging its PE data in that process. The company is engaging CMS early to learn whether it has sufficient evidence to show the benefits and value of its product.

Synchron also sees value in bringing its patient preference and feedback data into conversations with investors, as it demonstrates how the company is fulfilling a need in the market and that patients will want the device. Centering patients in its research and development activities allows Synchron to demonstrate to stakeholders the device's ability to improve patients' functional independence and quality of life according to their needs and preferences.

Recommendations

Given the benefits of and current opportunities for enhancing PE in medtech R&D, we offer the following recommendations for action:

- **Craft a framework to more clearly define and demonstrate the value and impact of PE for the diverse range of medtech product developers.** Such a framework could also identify possible approaches to achieving those benefits and propose core metrics for evaluating the impact of PE activities. Such a tool could ensure a common language around PE for companies internally and externally and provide the groundwork for developing and evaluating the return on PE activities.

  By having access to such a framework, medical product developers may feel better equipped to identify and justify PE strategies that would be implementable within their organizations. A framework should be adaptable for use by different types of medtech product developers (device, diagnostics, and digital health), with a particular focus on small and medium-sized companies with limited resources. It should seek to identify the characteristics of medtech product developers that might drive different approaches to conduct PE or assess its impact.

- **Develop a toolkit that curates existing tools and resources, and creates new ones to fill gaps, that can be used by medtech companies seeking to engage patients in their R&D activities.** While many tools and resources exist already, the field would benefit from the curation of an action-oriented collection of resources that can guide users, particularly from small-to-mid-sized companies, to develop and implement PE strategies and overcome known challenges and barriers. A toolkit would catalog the tools and resources (such as guides, templates, webinars, case studies, trainings, and checklists) that are publicly available to support medtech companies in developing, executing, and measuring PE strategies.

- **Adapt resources created for drug development and human-centered design contexts.** While there are meaningful differences between medtech and biopharmaceutical product development that affect their PE practices, there are opportunities to adapt many of the lessons learned and practices derived from the experiences and approaches in biopharma. This includes templates, frameworks, resources, and metrics for: 1) establishing, conducting, and measuring the impact of PE programs, 2) soliciting leadership approval of resources for PE activities, 3) integrating a patient-centric approach into internal business functions, 4) garnering the trust and the participation of the patient community, and 5) leveraging insights into how PE is used to inform R&D decisions. Similarly, principles and methods from the discipline of human-centered design could be more explicitly adapted and applied to PE in medtech product development.

- **Build the capacity of the medtech ecosystem to support more PE.** Medtech trade associations and regulators are offering training and forums for precompetitive knowledge sharing that could be expanded upon; the pharma industry has conferences exclusively devoted to partnering with patient communities that could be inclusive of or entirely replicated in medtech. Patient organizations themselves need more information about medtech product development, how it differs from drug development, and how they can support it effectively. Efforts should be made to forge more connections between medtech companies and patient groups.

- **Request that regulators and payers provide more clarity and examples of the impact of PE on their decision-making.** Evidence and examples exist to demonstrate that regulators and payers have used information derived from PE activities to inform their decisions. However, many stakeholders with whom we spoke indicated that there is not sufficient clarity on how patient input and perspectives are consistently integrated into regulatory and reimbursement decisions. This lack of clarity has hindered and disincentivized more medtech companies from actively pursuing PE activities. There may also be opportunities to pursue demonstration projects that could show the results of regulators' guidances in action.

- **Address legal and compliance challenges.** Specific legal provisions and common compliance challenges should be identified and strategies pursued to overcome them. Lingering concerns related to compliance with **anti-kickback legislation** and uncertainty around allowable remuneration procedures have prevented some medtech companies from pursuing patient engagement, according to AdvaMed. Additional guidance and clarification for smaller companies with limited legal resources should be created to navigate many of the common potential legal/compliance challenges with patient engagement (i.e., human subjects protection, Institutional Review Board review, informed consent, adverse event reporting, etc.).

- **Identify and address misalignments in requirements and processes.** Opportunities should be identified to synchronize approaches to PE in decision-making within and among regulatory bodies. Better alignment of the types of PE information that is valuable to regulators, payers, and investors would create additional incentives for medtech product developers. More in-depth examination of how the benefits of PE vary and align across medtech stakeholders could help determine where additional opportunities to pursue PE exist.

Conclusion

Based on the findings from the research and interviews conducted by FasterCures, PE in medtech product development lags behind its adoption in drug development likely for reasons that are specific to the medtech context (or contexts, given the heterogeneity of types of products and approval pathways of medtech products). The smaller size of companies, shorter development timelines, and diffuse patient populations make imperative a clearer definition and demonstration of the value and impact of engaging patients across the development life cycle as a means to encourage more PE. Stronger signals from regulators, payers, and investors about their interest in using the outputs from PE activities in their decision-making, perhaps leading to a higher likelihood of favorable outcomes, would accelerate developers' investment in these activities.

FasterCures will continue work in PE in medtech product development by bringing thought leaders together to further explore current challenges and discuss solutions, and by developing tools and resources for the field. We hope that the release of this report will fuel additional activities in the medtech ecosystem to address some of the barriers, opportunities, and recommendations identified. Our ultimate aim is the creation of an R&D environment in which medtech developers and their collaborators have a better understanding of the impact of patient engagement, are better equipped to bring patients into their R&D processes, and ultimately develop products that are more closely aligned to the needs and preferences of their end users.

Endnotes

1. Christina Howerton, “Patient-Focused Medical Product Case Examples,” National Health Council, April 1, 2020,

2. Diana Clynes, “Defining the Value of Patient Engagement for Medical Device and Digital Health Innovators Part 2: Considerations and Actions to Drive Progress,” presentation at Milken Institute FasterCures workshop, January 19, 2024.

3. Barry Liden, “Defining the Value of Patient Engagement for Medical Device and Digital Health Innovators Part 2: Considerations and Actions to Drive Progress,” presentation at Milken Institute FasterCures workshop, January 19, 2024.

4. Heather Pin and Beth Brooks, “Establishing KPIs and Metrics That Reflect Value and Impact Patient Engagement across R&D,” presentation at the Patients as Partners Conference, Philadelphia, March 20, 2024, https://patientsaspartnersconference.com/conference/patients-as-partners/agenda?page=1.

5. Barry Liden, “Defining the Value of Patient Engagement for Medical Device and Digital Health Innovators Part 2: Considerations and Actions to Drive Progress”; Heather Pin and Beth Brooks, “Establishing KPIs and Metrics That Reflect Value and Impact of Patient Engagement across R&D.”

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7. Meena Seshamani, Review of *Medicare Drug Price Negotiation Program: Revised Guidance, Implementation of Sections 1191–1198 of the Social Security Act for Initial Price Applicability Year 2026* (Center for Medicare, Department of Health and Human Services, June 30, 2023), **https://www.cms.gov/files/document/revised-medicare-drug-price-negotiation-program-guidance-june-2023.pdf.**

8. *How Can Patient Preference Information Be Used in Payer Coverage Decisions and Health Technology Assessment?* (MDIC, December 8, 2022), https://mdic.org/resource/hepv-ppi-for-payers-and-htas/.; “Patient Preference Information (PPI) in Medical Device Decision Making,” Center for Devices and Radiological Health, US Food and Drug Administration, July 3, 2023, **https://www.fda.gov/about-fda/division-patient-centered-development/patient-preference-information-ppi-medical-device-decision-making**; *2024 CDRH Innovation Report* (Center for Diseases and Radiological Health, US Food and Drug Administration, April 17, 2024), **https://www.fda.gov/media/177865/download?attachment.**

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