Patient Engagement in Clinical Trials

Literature Review – Updated December 2020

A Report of the Science of Patient Input Working Group
of the Medical Device Innovation Consortium (MDIC)

March 17, 2021

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DISCLAIMER

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The general recommendations in this document:

- Do not imply FDA concurrence for specific applications
- Do not represent the opinion or policy of the FDA or of the companies represented
- Do not necessarily reflect the official policy or position of MDIC

ABOUT THE MEDICAL DEVICE INNOVATION CONSORTIUM

The Medical Device Innovation Consortium (MDIC) is the first public-private partnership created to advance the medical device regulatory process for patient benefit.

MDIC was formed in 2012 to bring the U.S. Food and Drug Administration (FDA) and industry together to share vital knowledge that can help bring safe, affordable, and effective devices to patients and providers more quickly. MDIC membership and participation is open to nonprofit, industry, and government organizations that are substantially involved in medical device research, development, treatment, or education; or in the promotion of public health; or that have expertise or interest in regulatory science.

MDIC has been designed to pursue several strategies that support its mission:

- Create a forum for collaboration and dialogue
- Make strategic investments in regulatory science, utilizing working groups to identify and prioritize key issues, and to request, evaluate, and implement project proposals
- Provide and enable implementation of tools from these projects that drive cost-effective innovation

The activities and outputs from MDIC are intended to:

- Ensure that innovative technology is readily available to U.S. patients
- Provide industry and government with methods and tools that may be used to expedite medical device development and the regulatory process
- Reduce the risk and expense of clinical research
- Reduce time and cost of medical device development

MDIC members provide guidance and leadership through collaboration to develop solutions for regulatory, scientific, health, and economic challenges within the medical device and diagnostic industry.

MDIC Science of Patient Input (SPI) Program

MDIC's Science of Patient Input (SPI) program provides a venue for continued collaboration to advance the science of patient input in regulatory decision-making, including advances in methodologies, and tactical considerations for integrating the patient's perspective in the design, clinical development, and regulatory review of innovative medical technologies.

OVERVIEW

This document provides an updated Literature Review for the Medical Device Innovation Consortium (MDIC) Science of Patient Input (SPI) Working Group for submission as a deliverable under the MDIC Broad Agency Agreement (BAA) contract.

This high-level review reflects a broad scan of publicly available information about relevant organizations, initiatives, materials, and publications.

While it is up to date as of December 2020, this summary is not exhaustive and should be viewed as a representative snapshot of activities and resources within the highly active and evolving field of patient engagement in clinical research. As new organizations, resources, and publications are being developed and launched continually, this work should be updated regularly to ensure its completeness and relevance over time.

1 ORGANIZATIONAL PATIENT ENGAGEMENT INITIATIVES (ALPHABETICAL ORDER)

Center for Information & Study on Clinical Research Participation (CISCRP)

Center for Information and Study on Clinical Research Participation is an independent, nonprofit organization dedicated to educating patients about clinical research.

Website: https://www.ciscrp.org/
Engagement Focus: Clinical Trial Design
Therapeutic Areas: Drugs, Biologics, Devices

Activities: CISCRP is an organization devoted to educating and informing stakeholders in clinical research, including patients, about clinical studies and what it means to be a clinical research participant. CISCRP organizes and facilitates patient panels and advisory boards to address clinical study issues, including patient perceptions on clinical trial design. The organization also conducted a global study on public and patient perceptions of clinical research.

Education Center provides resources for patients about participating in clinical research, including an interactive online community. https://www.ciscrp.org/education-center/

CISCRP joined with World Courier to conduct a "Patient Engagement in Clinical Trials" webinar on September 24, 2019. https://www.ciscrp.org/patient-engagement-in-clinical-trials/

Clinical Trials Transformation Initiative (CTTI)

CTTI is a public-private partnership to develop and drive adoption of practices that will increase the quality and efficiency of clinical trials.

Website: https://www.ctti-clinicaltrials.org/
Engagement Focus: Clinical Trial Design
Therapeutic Areas: Drugs, Biologics, Devices

Activities: CTTI is engaged in multiple activities related to increasing patient engagement in the design and conduct of clinical trials. Recent activities include:

- **Framework for Calculating Value of Patient Engagement** developed by CTTI to estimate net present value of patient engagement in clinical trials. https://www.ctti-clinicaltrials.org/sites/www.ctti-clinicaltrials.org/files/new_ctti_resource_26feb2020_final.pdf
- CTTI cohosted a March 2019 workshop with FDA focused on **Enhancing the Incorporation of Patient Perspectives in Clinical Trials**. https://www.ctticlinicaltrials.org/news/ctti-and-fda-workshop-will-explore-how-best-include-patientperspectives-clinical-trials/

- **Clinical Trials Recruitment Planning** initiative led to publication of a proposed framework to enhance trial recruitment strategies, including the central recommendation of engaging the patient as an equal partner in trial planning. https://www.sciencedirect.com/science/article/pii/S155171441730753X?via%3Dihub/
- **Digital Health Trials** is an effort to encourage the widespread use of mobile technology in clinical trials. One of the main objectives of this work is the focus on novel endpoints that reflect measures that are meaningful to patients. https://www.ctti-clinicaltrials.org/programs/mobile-clinical-trials/
- **Patient Groups & Clinical Trials** is an effort to engage patient groups early and often in the clinical trial process. CTTI has published recommendations for successful collaborations between sponsors and patient groups working on clinical trials. https://journals.sagepub.com/doi/full/10.1177/2168479017720247/
- **Financial Value of Patient Engagement** is an effort to provide evidence of value from patient engagement to sponsors conducting clinical trials. This work also resulted in a recent publication. https://journals.sagepub.com/doi/full/10.1177/2168479017716715/
- **Effective Engagement with Patient Groups Around Clinical Trials** provides recommendations for trial sponsors (academic and industry) and patient groups to advance collaboration in this area. https://www.ctti-clinicaltrials.org/files/pgctrecs.pdf

CTTI's website has a large volume of information and tools for use by sponsors and patient groups interested in engaging patients in all aspects of the clinical trial process, regardless of therapeutic area.

DIA

DIA is a membership organization focused on innovation in life sciences and product development. It was formerly known as the Drug Information Agency, but more recently has expanded its scope of interest to all medical products.

Website: https://www.diaglobal.org/en/resources/areas-of-interest/patient-engagement/
Engagement Focus: General Patient Engagement
Therapeutic Areas: Drugs, Biologics, Devices

Activities: DIA has been focused on patient engagement initiatives since 2006, developing resources for the public and its members and focusing on related topics during its meetings.

DIA Patient Engagement Community is an online forum for DIA members, consisting of blog posts, discussion boards, event notifications and other resources. https://www.diaglobal.org/en/resources/areas-of-interest/patient-engagement

Study of Patient-Centric Initiatives:

- **Considerations Guide** is a practical resource for companies launching or advancing patient-centered initiatives that support healthcare product research and development. http://engage.diaglobal.org/PatientEngagementConsiderationsGuide.html?_ga=2.233867806.1209464920.1609281055-501829263.1609281055
- **Capturing the Value of Patient Engagement** presents key insights in the following areas: adoption of patient-centric initiatives, barriers to adoption, comparative analytics, and results of patient-centric initiatives.

http://engage.diaglobal.org/DIAPatientEngagementStudy.html?_ga=2.138076019.297563242.1550515532-714288693.1548869872/

DIA-Tufts Center for the Study of Drug Development (CSDD) Patient Engagement Research Project is funded by 17 pharmaceutical companies and contract research organizations. https://www.diaglobal.org/en/resources/press-releases/2016/10-31-patient-engagement-study/

FasterCures—A Center of the Milken Institute

FasterCures is a nonprofit think tank focused on cutting through roadblocks that slow medical progress by expanding the science of patient input, fostering policies to support biomedical innovation, and spurring cross-sector collaboration in research to get better outputs.

Website: https://milkeninstitute.org/centers/fastercures
Engagement Focus: General Patient Engagement
Therapeutic Areas: Drugs, Biologics, Devices

Activities: FasterCures develops and provides curation for resources for patient advocacy organizations and stakeholders in the medical research ecosystem through its "Engaging Patients in Research" program. https://milkeninstitute.org/programs/engaging-patients-in-research

- **Advancing Models of Patient Engagement: Patient Organizations as Research and Data Partners** (October 2020). https://milkeninstitute.org/reports/advancing-models-patient-engagement-patient-organizations-research-and-data-partners
- **Patient-Focused Drug Development Meetings: Smart Practices from Community Leaders** (September 2020). https://milkeninstitute.org/reports/patient-focused-drug-development-meetings-smart-practices-community-leaders
- **Nonprofits: A Growing Force in Drug Development** (May 2020) https://milkeninstitute.org/reports/nonprofits-growing-force-drug-development
- **Patient-Centric Initiatives: Focusing for Impact** (2019) https://milkeninstitute.org/reports/patient-centric-initiatives-focusing-impact

Health Technology Assessment International (HTAi)

HTAi is a membership organization focused on the understanding and use of health technology assessment to inform healthcare decision-making and efficient use of healthcare resources.

Website: https://htai.org/
Engagement Focus: General Patient Engagement
Therapeutic Areas: Drugs, Biologics, Devices

Activities: The Patient and Citizen Involvement special interest group (PCIG) is focused on obtaining patient perspectives to improve health technology assessment. This group has generated multiple resources for its members relating to patient engagement in HTAi. https://htai.org/interest-groups/pcig/

- **HTAi Glossary for Consumers and Patients** is a beginner's guide to the words used in health technology assessment (HTAi). https://htai.org/wp-content/uploads/2018/02/HTAiPatientAndConsumerGlossaryOctober2009_01.pdf
- **For HTA Agencies and Policy Makers** includes multiple resources to support HTA submissions. https://htai.org/interest-groups/pcig/resources/for-hta-agencies-and-policymakers/
- **For Patient Groups and Individual Patients**, https://htai.org/interestgroups/pcig/resources/for-patients-and-patient-groups/
- **For Industry and Researchers** includes links to ethical guides and national codes of practice for patient engagement collaborations. https://htai.org/interestgroups/pcig/resources/for-industry-and-researchers/
- **PCIG Webinar (2017) to the International Network of Agencies for Health Technology Assessment (INAHTA)** https://www.dropbox.com/s/r6ieika37813w68/INAHTA%20Patient%20Involvement%20Webinar_2017.mp4?dl=0 /

Professional Society for Health Economic and Outcomes Research (ISPOR)

ISPOR is the professional society for health economic and outcomes research (HEOR). ISPOR has a strategic initiative focused on patient engagement in HEOR, including a Patient Representative Roundtable program, which convenes stakeholders with the goal of engaging patient representatives in the research and decision-making processes, and a Patient Centered Special Interest Group, which works to facilitate the involvement of patient representatives in all stages of research and decision-making to improve healthcare and its delivery and outcomes.

Website: https://www.ispor.org and https://www.ispor.org/strategic-initiatives/patient-initiatives
Engagement Focus: Outcomes Research
Therapeutic Areas: Drugs, Biologics, Devices

Medical Device Innovation Consortium (MDIC)

The Medical Device Innovation Consortium (MDIC) Science of Patient Input (SPI) initiative comprises several projects aimed at enhancing patient engagement in medical device clinical trials and product development efforts. This initiative, previously known as Patient Centered Benefit-Risk, relies on multistakeholder working groups and committees to evaluate the landscape, identify gaps, and generate resources on these topics for the medical device community.

Website: https://mdic.org/program/science-of-patient-input/
Engagement Focus: Patient Preference Assessment, Clinical Trial Design
Therapeutic Area: Devices

Activities: MDIC and its partners are collaborating to improve the field's ability to include patient perspectives in the development, premarket approval, and postmarket evaluation of medical devices. The vision of the MDIC Science of Patient Input Steering Committee is to provide a venue for continued collaboration to advance the art and science of patient engagement in regulatory science, including advances in methodologies and tactical considerations for integrating the patient's perspective and preferences in the design, clinical development, and regulatory review of innovative medical technologies. The MDIC SPI website includes multiple relevant resources:

- **MDIC's Patient Input in Clinical Trial Design project** focuses on three workstreams to develop: (1) methodologies to systematically identify outcomes that matter most to patients and establish these

outcomes as primary or secondary endpoints for clinical studies, (2) guidelines to integrate patient preferences into the statistical design of clinical trials, and (3) methodologies to maximize patient participation in clinical trials. https://mdic.org/project/patient-input-in-clinical-trial-design

- MDIC's Patient Centered Benefit-Risk (PCBR) project launched in 2013 with the vision "to establish a credible framework for assessing patient preferences regarding the probable benefits and risks of a proposed medical device and for incorporating patient preference information into pre-market and post-market regulatory submissions and decisions." This project resulted in publication of "A Framework for Incorporating Information on Patient Preferences Regarding Benefit and Risk into Regulatory Assessments of New Medical Technology" (May 2015). https://mdic.org/resource/patient-centered-benefit-risk-pcbr-framework/
- MDIC's Patient Centered Outcomes Research Project engaged the Michael J. Fox Foundation for Parkinson's Research (MJFF), CDRH, Massachusetts Institute of Technology (MIT), and RTI Health Solutions to investigate how the preferences of patients with Parkinson's disease (PD) could be used to set statistical significance levels in clinical trial design. https://mdic.org/project/patient-centered-outcomes-research/
- MDIC's Heart Failure Project engaged six medical device companies supportive of learning more about patient preferences in the treatment of heart failure to develop and conduct a patient preference study aimed at understanding patient preferences for features relevant to various heart failure interventions including effectiveness outcomes. The primary study objective was to apply best-practice stated-preference methods to quantify heart-failure patients' willingness to accept therapeutic risks in exchange for improved effectiveness of devices used to manage heart failure. https://mdic.org/project/heart-failure-study/

National Cancer Institute (NCI)

The National Cancer Institute Office of Advocacy Relations links advocate stakeholders with NCI to ensure collective patient perspectives are included in NCI cancer research activities.

Website: https://www.cancer.gov/about-nci/organization/oar
Engagement Focus: Clinical Trial Design
Therapeutic Areas: Drugs, Biologics, Devices

Activities: NCI's Office of Advocacy Relations (OAR) is the primary resource at the organization for engaging with the cancer advocacy community. https://www.cancer.gov/aboutnci/organization/oar/research-advocacy/

A summary of OAR's engagement activities was presented at the FDA Patient Engagement Advisory Committee (PEAC) meeting in October 2017. Included in these activities are:

- Clinical Trials and Translational Research Advisory Committee (CTAC), an external multistakeholder oversight committee that advises NCI on ways to strengthen its intramural and external research enterprises, leveraging several ad hoc working groups that engage with patient advocates within various high-priority tumor types. https://www.cancer.gov/about-nci/organization/ccct/ctac/
- NCI's Council of Research Advocates (NCRA), the only federal advisory committee solely comprises advocate leaders at NCI. NCRA convenes around broad cancer research issues and provides the Director with advice and strategic insights from the community's perspective. https://www.cancer.gov/about-nci/organization/oar/ncra

- **NCI's Scientific Steering Committees and Task Forces**, which include patient advocates, guide NCI's clinical trial enterprise.

National Health Council (NHC)

National Health Council (NHC) is an advocacy organization whose mission is to provide a voice for patients with chronic diseases and disabilities as well as their caretakers. NHC is a membership organization with participation from advocacy groups, and industry, payer, and other medical product and healthcare stakeholders.

Website: https://www.nationalhealthcouncil.org/
Engagement Focus: General Patient Engagement
Therapeutic Areas: Drugs, Biologics

Activities: One of NHC's public policy areas is patient engagement. Though NHC has participation from all therapeutic areas, including devices, NHC's work in patient engagement in clinical research is limited to drugs and biologics. NHC desires to identify and implement best practices for engaging patients in the research, development, and regulatory review of new drugs. http://www.nationalhealthcouncil.org/public-policy/patient-engagement/

In collaboration with the Genetic Alliance, NHC has generated white papers on the topic of patient engagement in clinical research and is working to generate recommended language for use in FDA patient engagement guidance documents.

- **Engagement Toolbox** comprises resources developed by NHC to support patient engagement by medical product developers, including tools to determine appropriate compensation of patients and tools to support contracting (coming soon). https://nationalhealthcouncil.org/patient-engagement-compensation-and-contracting/
  - Fair-Market Value Calculator and Engagement Templates https://www.nationalhealthcouncil.org/resources/fair-market-value-calculator
- **Clinical Outcomes Assessment** is an area of current focus for NHC, which has convened several webinars on the topic. https://www.nationalhealthcouncil.org/publicpolicy/clinical-outcome-assessment/
- **Sponsor-Patient Interactions During Drug Development** was a multistakeholder 2017 meeting convened to discuss best practices for patient engagement. https://www.nationalhealthcouncil.org/wp-content/uploads/2019/12/Sponsor-Patient%20Interactions%20during%20Drug%20Development.pdf

Patient-Centered Outcomes Research Institute (PCORI)

PCORI was established to fund clinical-effectiveness research that can help patients and caregivers make better-informed healthcare decisions. PCORI also funds projects focused on research methods and capacity and works to influence research funded by others to be more useful to patients and other healthcare decision-makers.

Website: https://www.pcori.org/
Engagement Focus: Clinical Outcomes Development
Therapeutic Areas: Drugs, Biologics, Devices

Activities: PCORI has supported engagement of multiple stakeholders in clinical research through their "Engagement in Research PCORI" program. Some of these activities include:

PCORI Engagement Resources & Guidance Documents

- Engagement in Health Research Literature Explorer is a searchable list of publications on engagement in health research. This resource is sortable by article topic type, types of stakeholders engaged, and phase(s) of research in which engagement occurred, from identifying research questions to sharing study results. Articles are listed if they met PCORI's search and inclusion criteria. https://www.pcori.org/engagement/engagement-literature
- Engagement Plan Template provides guidance for methods of engaging stakeholder partners throughout each phase of a research study (Updated October 2020). https://www.pcori.org/sites/default/files/PCORI-Updated-Engagement-Plan-Template.pdf
- Engagement Rubric provides guidance, definitions, and principles for patient engagement in research (developed in 2014 and updated in 2016). https://www.pcori.org/sites/default/files/Engagement-Rubric.pdf
- Compensation Framework provides guidance for developing fair compensation levels for patients, caregivers, and organizations that participate in research. https://www.pcori.org/sites/default/files/PCORI-Compensation-Framework-for-EngagedResearch-Partners.pdf/
- PCORI Methodology Standards provide specific benchmarks for researchers across an array of aspects. There are four standards associated with Patient Centeredness, and standard PC-1 focuses specifically on engagement. https://www.pcori.org/researchresults/about-our-research/research-methodology/pcori-methodologystandards#Associated%20with%20Patient-Centeredness/
- Advisory Panel on Patient Engagement meets regularly to discuss key topics in the field of patient engagement. https://www.pcori.org/events/2019/advisory-panel-patient-engagement-fall-2019-meeting
- Engagement Tool and Resource Repository is a searchable database of engagement tools and resources used by PCORI awardees. https://www.pcori.org/engagement/engagement-resources/Engagement-Tool-Resource-Repository

PCORI Fact sheets

- Better Research Through Engagement https://www.pcori.org/sites/default/files/PCORI-Better-Research-ThroughEngagement.pdf
- Engagement Challenges, Strategies, and Resources https://www.pcori.org/sites/default/files/PCORI-Patient-Stakeholder-EngagementChallenges-Strategies-Resources-Handout-120517.pdf
- Roles of Patient and Stakeholder Partners in Patient-Centered Research https://www.pcori.org/sites/default/files/PCORI-Engagement-in-Research-Making-aDifference-Webinar-Info-Sheet-091917.pdf
- Research Done Differently https://www.pcori.org/sites/default/files/PCORI-Research-Done-Differently.pdf
- Initiating Partnerships for Patient-Centered Research https://www.pcori.org/sites/default/files/PCORI-Engagement-Strategies-for-Initiating-Research-Partnerships-Info-Sheet-71917.pdf

PCORI Webinars Archive

- Engagement Strategies, Challenges, and Resources https://www.pcori.org/events/2017/patient-and-stakeholder-engagement-researchengagement-challenges-strategies-and

- **Strategies for Initiating Research Partnerships** https://www.pcori.org/events/2017/patient-and-stakeholder-engagement-researchstrategies-initiating-research-partnerships
- **Engagement Challenges and Solutions Presentation**, PCORI Annual Meeting (2016) https://custom.cvent.com/90B233E1C037437BB4FE6D4881DF9A22/files/0fc56ffc17b445d5bae44c31360b3f73.pdf

PCORnet Engagement Assessment Report (PCORnet) deals with engagement of patients and stakeholders in research networks rather than individual research studies, but there may be common lessons and challenges about engagement.
https://www.pcori.org/engagement/engagement-resources/Engagement-Tool-Resource-Repository/engagement-assessment-project

TransCelerate BioPharma Inc.

TransCelerate BioPharma's mission is to collaborate across the global biopharmaceutical research and development community to identify, prioritize, design, and facilitate implementation of solutions designed to drive the efficient, effective, and high-quality delivery of new medicines. TransCelerate membership is available to biopharmaceutical research and development organizations that engage in innovative discovery, development, and manufacturing of new drugs. TransCelerate's growing portfolio of initiatives focuses on the shared vision of accelerating and enhancing the research and development of innovative new therapies. Their initiatives develop practical solutions to overcome inefficiencies in clinical research and are drawn from the combined expertise of members and industry collaborators.

Website: https://www.transceleratebiopharmainc.com/
Engagement Focus: Clinical Trial Design, Patient Experience
Therapeutic Area: Drugs

Activities: TransCelerate leverages collaboration among multiple companies to improve clinical trial efficiency and advance drug development. The organization has many activities across multiple areas of focus, including its Patient Experience (PE) Initiative, which seeks to facilitate and accelerate the industry's progression toward a future where the patient experience is enhanced in clinical trials and patient burden is reduced, and its Patient Technology (PT) Initiative, which seeks to facilitate and accelerate the industry's progression toward a future where patients have access to innovative technologies that enhance the patient experience and reduce patient burden in clinical trials.

- **Patient Protocol Engagement Toolkit** for trial sponsors. https://www.transceleratebiopharmainc.com/ppet/planning-for-patient-engagement/
- **Patient Experience Initiative** includes multiple resources and solutions aimed at improving the patient experience in clinical research. https://www.transceleratebiopharmainc.com/initiatives/patient-experience/
- **Patient Technology Initiative** addressed barriers for patient participation in clinical trials. Published manuscript on accelerating adoption of patient-facing technologies. https://journals.sagepub.com/doi/10.1177/2168479018801566 and **Implementation Guide** http://www.transceleratebiopharmainc.com/wp-content/uploads/2018/12/PT-Implementation-Framework-v3.6-12.11.18-2.pdf
  https://www.transceleratebiopharmainc.com/initiatives/patient-technology/

2 FDA ACTIVITIES, GUIDANCE DOCUMENTS, AND RESOURCES

Patient Engagement Collaborative (PEC), a work group created with CTTI, comprises patient advocacy organizations convened to discuss patient engagement at the FDA.
https://www.fda.gov/forpatients/patientengagement/default.htm#collaborative

Patient Participation in Medical Product Discussions (FDASIA Section 1137) recognizes the value of patient input by facilitating increased involvement of patients earlier in the regulatory process for medical product review.
https://www.fda.gov/forpatients/patientengagement/default.htm#fdasia1137

Patient Engagement in the Design and Conduct of Medical Device Clinical Investigations. Draft Guidance September 2019.
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-engagement-design-and-conduct-medical-device-clinical-investigations

Patient Reported Outcomes – Clinical Outcomes Assessment Qualification
https://www.fda.gov/regulatory-information/search-fda-guidance-documents/requests-feedback-and-meetings-medical-device-submissions-q-submission-program

- **Guidance for Industry**
  https://www.fda.gov/downloads/drugs/guidances/ucm193282.pdf

- **Patient-Focused Dug Development (PFDD) CDER Guidance Series**

FDA is developing a series of four methodological patient-focused drug development (PFDD) guidance documents to address, in a stepwise manner, how stakeholders can collect and submit patient experience data and other relevant information from patients and caregivers for medical product development and regulatory decision-making.
This series of guidance documents is intended to facilitate the advancement and use of systematic approaches to collect and use robust and meaningful patient and caregiver input that can better inform medical product development and regulatory decision-making.
These guidances are part of FDA's PFDD efforts in accordance with the 21st Century Cures Act and The Food and Drug Administration Reauthorization Act of 2017 Title I.
https://www.fda.gov/drugs/developmentapprovalprocess/ucm610279.htm

- *Guidance 1*: Collecting Comprehensive and Representative Input
- *Guidance 2*: Methods to Identify What is Important to Patients
- *Guidance 3*: Selecting, Developing or Modifying Fit-for-Purpose Clinical Outcomes Assessments
- *Guidance 4*: Title forthcoming, will address topics related to COA-related endpoint development and interpretation, including topics related to instrument administration and meaningful within-patient score changes
  - **Plan for Guidance Development**
    https://www.fda.gov/drugs/developmentapprovalprocess/ucm610279.htm

- **Voice of the Patient Meetings**
  https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm347317.htm

Center for Devices and Radiological Health (CDRH) Patient Engagement
https://www.fda.gov/about-fda/center-devices-and-radiological-health/cdrh-patient-engagement

CDRH Patient Preference Initiative

- Patient Preference in Medical Device Decision-Making https://www.fda.gov/about-fda/cdrh-patient-engagement/patient-preference-information-ppi-medical-device-decision-making
- Patient Preference-Sensitive Areas: Using Patient Preference Information in Medical Device Evaluation https://www.fda.gov/about-fda/cdrh-patient-engagement/patient-preference-sensitive-areas-using-patient-preference-information-medical-device-evaluation
- Patient Perspective Workshop (2013) http://wayback.archiveit.org/7993/20170112084903/http://www.fda.gov/MedicalDevices/NewsEvents/Worksho psConferences/ucm361864.htm

CDRH Patient Engagement Advisory Committee (PEAC) provides advice to the Commissioner or designee on complex issues relating to medical devices, the regulation of devices, and their use by patients. The Committee may consider topics such as Agency guidance and policies, clinical trial or registry design, patient preference study design, benefit-risk determinations, device labeling, unmet clinical needs, available alternatives, patient-reported outcomes and device-related quality of life or health status issues, and other patient-related topics.
https://www.fda.gov/advisorycommittees/committeesmeetingmaterials/patientengagementadvisorycommittee/default.htm
https://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDRH/CDRHPatientEngagement/ucm462829.htm

- 2020 Meeting Materials: https://www.fda.gov/about-fda/cdrh-patient-engagement/cdrh-patient-engagement-advisory-committee
- 2019 Meeting Materials: https://www.fda.gov/advisory-committees/patient-engagement-advisory-committee/september-10-2019-patient-engagement-advisory-committee-meeting-announcement-09102019-09102019
- 2018 Meeting Materials: https://www.fda.gov/AdvisoryCommittees/CommitteesMeetingMaterials/PatientEngagementAdvisoryCommittee/ucm621264.htm
- 2017 Meeting Materials: https://www.fda.gov/AdvisoryCommittees/CommitteesMeetingMaterials/PatientEngagementAdvisoryCommittee/ucm578522.htm

MyStudies App, launched by Center for Drug Evaluation and Research (CDER) to facilitate the input of real-world data directly by patients, can be linked to electronic health data supporting traditional clinical trials, pragmatic trials, observational studies, and registries. https://www.fda.gov/Drugs/ScienceResearch/ucm624785.htm

3 UNITED STATES-BASED CONFERENCES AND MEETINGS (REVERSE CHRONOLOGICAL ORDER)

A selection of meetings that occurred or are planned with a specific focus on patient engagement in clinical trials. Materials produced by these meetings are or will be available at the conference websites. Note, it may be necessary to navigate the websites if the desired content is not on the home page.

- MDIC Patient Engagement Forum (November 2020) https://mdic.org/event/patient-engagement-forum-virtual/
- ISPOR-FDA Virtual Summit. Using Patient-Preference Information in Medical Device Regulatory Decisions: Benefit-Risk and Beyond (September 2020) https://www.ispor.org/conferences-education/conferences/past-conferences/ispor-fda-summit-2020
- NHC Science of Patient Engagement Symposium (September 2020) https://nationalhealthcouncil.org/2020-science-of-patient-engagement-symposium/
- CISCRP Patient Engagement in Clinical Trials Webinar (September 2019) https://www.ciscrp.org/patient-engagement-in-clinical-trials/
- National Academies of Science (NAS). Advancing the Science of Patient Input in Medical Product R&D Workshop (July 2019) https://www.nationalacademies.org/event/09-19-2017/advancing-the-science-of-patient-input-in-medical-product-r-d-towards-a-research-agenda-a-workshop
- CTTI-FDA Patient Engagement Workshop (March 2019) https://www.ctti-clinicaltrials.org/news/recording-now-available-ctti-and-fda-hold-workshop-incorporating-patient-perspectives-clinical
- CDRH Patient Engagement Advisory Committee (PEAC) Meeting (November 2018) https://www.fda.gov/AdvisoryCommittees/CommitteesMeetingMaterials/PatientEngagementAdvisoryCommittee/ucm621264.htm
- DIA Measuring Impact in Patient Centered Drug Development Conference (October 2018) https://www.diaglobal.org/en/conference-listing/meetings/2018/10/measuring-impact-inpatient-centered-drug-development
- MDIC Patient Centered Clinical Trials Workshop (May 2018) https://mdic.org/project/may-18-2018-patient-centered-clinical-trial-design-workshop/
- Patients as Partners, U.S. (Annual Conference). Conference focuses on involvement of patients throughout the lifecycle of medical products. https://theconferenceforum.org/conferences/patients-as-partners/overview/
- CDRH Patient Engagement Advisory Committee (PEAC) Meeting (October 2017). Inaugural PEAC meeting focused on engagement of patients in clinical trials. https://www.fda.gov/advisorycommittees/calendar/ucm568511.htm
- University of Maryland M-CERSI Conference on Patient Focused Drug Development (March 2015) https://www.pharmacy.umaryland.edu/centers/cersievents/patient-focuseddrug-development/

- **CTTI Patient Groups and Clinical Trials Expert Meeting (January 2015).** Meeting focused on successes, barriers, and the value of patient engagement in clinical trials. https://www.ctti-clinicaltrials.org/briefing-room/meetings/patient-groups-and-clinical-trials-expert-meeting

4 ENGAGEMENT RESOURCE COMPENDIA (ALPHABETICAL ORDER)

There are multiple organizations that provide assemblies of information on patient engagement. Each of these "engagement resource compendia" includes large volumes of information:

Alliance for Clinical Research Excellence and Safety (ACRES)
http://www.acresglobal.net/
Patient Empowerment Initiative (PEI)
Resource: Collects, synthesizes, and tests best practice methods for engagement in clinical research. Integrate patient centricity efforts across the research and healthcare environment.
Therapeutic Areas: Drugs, Biologics, Devices
Focus: General Patient Engagement

Biotechnology Innovation Organization (BIO)
https://www.bio.org/sites/default/files/legacy/bioorg/docs/BIO_PPMD_Paper_2016.pdf
Key Considerations for Developing and Integrating Patient Perspectives in Drug Development: Examination of the Duchenne Case Study
Resource: Shares best practices for the development of disease-specific patient preference studies based on the joint initiative between Parent Project Muscular Dystrophy (PPMD) and BIO. The paper outlines key considerations to help guide stakeholders on the development of patient preference studies and the multitude of ways they can be used, including to help inform the drug development and regulatory processes.
Therapeutic Areas: Drugs, Biologics
Focus: Benefit Risk, Patient Preferences

MDIC
https://mdic.org/resource/patient-centered-benefit-risk-pcbr-framework/
Patient Centered Benefit-Risk (PBCR) Framework
Resource: Provides background on the concepts of benefit-risk and patient preference, discusses the potential value of including patient benefit-risk in a regulatory submission and when in the product lifecycle such information might be collected, outlines factors to consider when selecting a patient preference method, and discusses considerations regarding the use of patient preference information in the regulatory process.
Therapeutic Areas: Devices
Focus: Benefit-Risk, Patient Preferences

Patient Focused Medicines Development
www.patientfocusedmedicine.org
SYNAPSE: Synergizing Patient Engagement
Resource: A registry of patient engagement initiatives across the globe; includes a search engine to identify specific patient engagement efforts
Therapeutic Areas: Drugs, Biologics, Devices
Focus: General Patient Engagement

PCORI
https://www.pcori.org/literature/engagement-literature/
Engagement in Health Research Literature Explorer
Resource: Searchable database of publications about engagement in health research, not limited to PCORI studies
Therapeutic Areas: Drugs, Biologics, Devices
Focus: General Patient Engagement, Technology

TransCelerate
https://www.transceleratebiopharmainc.com/
Patient Experience Initiative: Enables greater patient engagement and partnership between patients and sponsors to design and execute clinical protocols that create better patient experiences in clinical trials
Patient Technology Initiative: Addresses the barriers to the use of patient-facing technologies (PT) in clinical trials to benefit individual patients and the broader patient community, as well as sponsors, sites, and vendors

The Research Acceleration and Innovation Network (TRAIN)
https://milkeninstitute.org/centers/fastercures/train
Resource: A collection of tools, publications, webinars, and events related to patient engagement, including patient input to clinical research
Therapeutic Areas: Drugs, Biologics, Devices
Focus: General Patient Engagement

5 SELECTED REFERENCES

This curated list contains selected references identified through a high-level review of published literature, literature database searches, and citations in other documents. The list is divided into categories of literature pertaining to development of clinical outcomes, clinical trial design, and general patient engagement. These categories are not mutually exclusive; they are intended to provide some guidance as to which articles cover the two specific topic areas of interest. New publications on the topic of patient engagement in research are being disseminated frequently, and this list should be updated regularly.

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