Track 5 | Patient Engagement

This track addresses meaningful patient engagement in medical product development, from early product development, and approval, through maintenance phases. It focuses on important questions for all stakeholders, including:

- How do we meaningfully engage patients and incorporate their voices into decision-making throughout the medical product lifecycle?
- How do we become truly patient- (and people-) centric in our approach?
- How do we operationalize patient-centric approaches in our day-to-day work?
- How can we measure the effectiveness of our efforts, both for patient outcomes and to meet the needs of other stakeholders such as industry and regulatory decision-makers?
- What have we learned that can be used to drive more meaningful patient engagement?
- How do stakeholders best work together to leverage their collective power and expertise to promote meaningful involvement of patients?

DIA recommends this track and associated sessions to professionals involved in patient affairs, patient advocacy, patient groups, patient support services, medical affairs (including CMOs and MSLs (Medical Science Liaison)), clinical trial design and optimization, clinical research and operations, regulatory affairs, regulatory agency, corporate and government affairs, safety and pharmacovigilance, outcomes research, epidemiology, and Health Technology Assessment.

Included Topic Areas

Meaningful patient engagement (PE), patient-centered drug development, patient centricity, fostering patient-centric culture, PE approaches, best practices for PE, building collaborative relationships with patients and patient groups, engaging with diverse patient populations, partnering with patients, science of PE, operationalizing PE, PE metrics, PE tools and resources, patient advocacy, lessons learned in PE, PE outcomes. Topics related to bioethical issues are also welcome and may be considered for a special track in the meeting.

Priority Topics

1. **Getting Strategic: Purposeful Patient Engagement Begins with the End in Mind**

a. Beyond box-checking: Whether it is an externally led Patient Focused Drug Development (PFDD) meeting or a Patient Advisory Board, how is your best-practice patient engagement positioned as one element of a bigger strategy to elicit and integrate patient perspectives in small and large trials to improve patient outcomes?

b. Fit for purpose: Designing and scaling a patient engagement activity based on the desired goals. What considerations have been most impactful to build initiative with the end in mind? For example: type of format (e.g., advisory board/focus group, standing council, survey, etc.); duration of engagement; number of patients/perspectives; involvement of advocacy groups in design, implementation, and recruitment.

c. Making the whole more than the sum of its parts: What learnings can you share from starting patient engagement early as a cross-functional initiative, so it informs activities spanning from pre-clinical research to market access (and/or the many steps in between)? Whose partnership did you seek and secure along the way? What challenges and successes have you experienced?

d. Defining meaningful endpoints: How and when is patient input collected to define meaningful endpoints? Which metrics are important to patients vs. industry vs. researchers vs. other stakeholders? Are patient-defined endpoints in line with priorities of other stakeholders? What endpoints are mutually beneficial to measure? How does the publication of recent guidances, such as for earlier regulatory interactions and COA development, change the thinking behind patient research and endpoint selection?

e. Diving into data: Patient Experience Data can be collected in many ways, and FDA is beginning to share its approach to review of these data. Has this impacted the ability to include patients more systematically in design and development? Does early alignment on domains of interest, data standards, and data sharing/contribution agreements help to decrease participant burden and increase the utility and flow of data for various purposes? What other lessons have you learned?

2. **Context and Contours: Illuminating Patient Engagement in Different Settings and Disease Areas**

a. Prevalence: How does the fact that a condition is rare or prevalent in the population affect patient engagement? Which factors drive patient engagement in rare diseases despite smaller numbers, and how can these be leveraged for other indications?

b. Special populations: How can feedback from children and adolescents be gathered? How have Young Person Advisory Groups, parents, and educational establishments been utilized?

c. Patient career engagement: Collaborating from the early stages of medicine development

d. Geography: What are some of the practical, pragmatic, and ethical considerations of limiting or expanding patient engagement beyond borders, from gaining a site-specific focus to getting a global set of viewpoints?

e. Data and digitalization: How are decentralized trials [?] patient engagement and vice versa? What is patient acceptance of DCTs (Decentralized Clinical Trials)?

f. Timing: Whether a condition is chronic or acute may affect the available or optimal "window" for engaging patients. The question driving patient engagement may also be specific to a point in time, such as developing PROs (Patient Reported Outcome) for early-stage disease vs. end-stage disease. Share examples and perspectives on how to assess timing as a consideration for patient engagement.

g. Diversity: How do patient engagement activities support clinical trial diversity goals? What insights are gained from engaging patients from underrepresented groups on clinical trial design, development of study communications, site-selection strategies, etc., and how do these activities impact enrolling a diverse cohort? What are organic hurdles that can be easily eliminated to bridge barriers that can inadvertently lead to underrepresentation? What are best practices for reaching and engaging more inclusively, especially for engagement activities that seek to understand disparities in practice, outcomes, or access that may occur by gender identity, race, ethnicity, socioeconomic status, health literacy level, etc.?

3. **Scaling Patient Engagement: Moving Across Therapeutic Areas, and More!**

a. Structure and staffing: What are your lessons learned about where in the company responsibility for patient engagement is centered and how that function is staffed? Is it centralized or diffused throughout the company? Is it shepherded by one individual per therapeutic area or at a particular stage of development? What type of professional experience best positions someone for success in these roles? How are you building capacity in your organization for increasingly meaningful patient engagement? How do you transition crucial relationships and learnings when necessary? How are you measuring success in your structure?

b. Training: How has your enterprise educated staff about this growing expectation for patient perspectives to inform medical product development? How can program and support staff, especially legal and compliance functions, better understand and foster optimal patient engagement activities? Do you have written standards to guide new initiatives? For patient advocacy patients and advocates to prepare them for these new opportunities to share their perspectives?

c. Demonstrating return on engagement: How are you and your collaborators tracking and measuring the outputs and outcomes of patient engagement to demonstrate its impact and value? Can benefits of engagement practices at various stages of the lifecycle be assessed? Ideas for assessing immediate and long-term benefits to the community, the program, and the sponsor are welcome.

d. Continuous feedback: What approaches are most effective to share results with patients who participated in trial design and development or in work at other stages of the lifecycle?

4. **"Yes, We Can!" Busting Misconceptions About Patient Engagement and Patient-Focused Medical Product Development**

a. Policy, regulations, and guidance: Have you participated in activities to expand knowledge about regulators' expectations for patient engagement practices, or apply existing laws that encourage patient-centered practices (i.e., 21st Century Cures Act) to aid in changing culture or practice at your institution or another? What ideas do you have for building on existing guidelines to help foster adoption and overcome resistance?

b. Precompetitive multistakeholder resources: Have you developed as part of a regional or global initiative to help de-risk patient engagement? How have these resources been used and what impact did their use have? Publications and case studies involving multiple partners are welcomed.

c. Patient organizations: How have you been able to initiate or lead collaborations with other stakeholders? What tips and lessons learned are applicable to other organizations? Where is the best place to start?

d. Managing conflicts of interest: More and deeper engagement between sponsors and patient organizations can (and has) raised concerns about influence and independence. How is your organization helping define appropriate boundaries and put this conversation in the new context of patient-focused medical product development? What are ways to ensure that collaboration does not have unintended consequences for either party?

5. **De-risking R&D: How has patient engagement been used as a strategic and systematic tool to de-risk research and development? How have partnerships with the patient community enabled improved research and development outcomes that benefit both patients and the industry? How are regulators part of this picture? What initiatives have been used to clarify that a product in development serves an unmet need?**