Patient Engagement in Benefit-Risk Assessment throughout the Life Cycle of Medical Products
Dissemination Plan

Dissemination: The intentional, active process of identifying target audiences and tailoring communication strategies to increase awareness and understanding of evidence and to motivate its use in policy, practice, and individual choices.

- What information about the evidence will help people make decisions?
- In what ways can that information be provided?

Key factors:

1. **Identify audience with potential to adopt practices or influence the adoption of practices**
2. **Identify audience with potential to address gaps and challenges**
3. **Involve stakeholders from beginning in all aspects of the project**
4. **Identify the questions they have about the issue/topic**
5. **Identify audience values, motivation, and expectations**
6. **Determine the incentives for necessary adoption/change/response to call to action**
7. **Define goals for adoption of practices**
8. **Target messages to the audience and their needs and motivations**
9. **Identify relevant engagement vehicles**

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1. **Audiences with potential to adopt practice:**
   - Biopharmaceutical and medical device company personnel
   - Academic clinical researchers
   - Patient organizations
   - Regulators: policy makers, reviewers, decision-makers

   Audiences with potential to influence adoption of practices:
   - Biopharmaceutical and medical device companies C-level, VP, Director levels
   - Organizations visible to or influencing biopharma and medical device companies: DIA, PhRMA, BIO, ACRES, ISPOR, ISPE, The Center for Innovation in Regulatory Science (CIRS), Medical Device Innovation Consortium (MDIC), AdvaMed, National Pharmaceutical Council (NPC)
   - Patient organizations
   - Organizations visible to or influencing multiple stakeholders: FasterCures, National Health Council (NHC), NORD, International Association of Patient Organizations (IAPO), Clinical Trials Transformation Initiative (CTTI), Rare Disease Legislative Advocates (RDLA), Global Genes, Genetic Alliance
   - Regulators: policy makers
   - Legislators

2. **Audiences with potential to address gaps and challenges:**
   - All of influencers above

- Legislators
- DIA with its Communities and Scientific Working Groups

3. How can these audiences be involved in the project?

- Involved in planning of the conference/project:
  - Academia with expertise in area (Duke Clinical Research Institute, Johns Hopkins, University of Colorado)
  - Biopharmaceutical thought leaders in area (AstraZeneca, EMDSerono, GSK, Janssen, Lilly, Merck)
  - Patient Organizations (JDRF, PPMD, T1DExchange)
  - Stakeholder Influencers (FasterCures)
  - Health Canada
- Participated in program content (Speaking and conference participation):
  - CTTI, NHC
  - diaTribe/Close Concerns, LUNGevity, iConquerMS
  - Yale University School of Medicine, Northwestern University Feinberg School of Medicine, U of Maryland School of Pharmacy
  - FDA (Patient programs, Patient Focused Drug Development, Data and Statistical Review, OSE (Pharmacovigilance and Epidemiology))
- Participating in the program:
  - Patient Scholarships (PCORI and DIA-supported registrants)
  - FDA Reviewers (DIA supported five registrations)
- Additional stakeholders to be briefed on program and its key messages:
  - FDA (Patient programs, Patient Focused Drug Development, Data and Statistical Review, OSE (Pharmacovigilance and Epidemiology)
  - PhRMA, BIO, AdvaMed, ISPOR, ISPE, CIRS, NPC
  - IAPO, NORD, RDLA, Global Genes, Genetic Alliance
  - Multiple patient organizations and DIA Patient Engagement Community
  - DIA Communities – including Pharmacovigilance, Clinical Research, Study Endpoints, Preclinical Sciences, Pediatric, Regulatory Affairs, Medical Communications, Electronic Regulatory Submissions, Statistics
  - Legislator(s) associated with 21st Century Cures

4. Identify the questions they have about the issue/topic

- Pre-registration survey results
- Post-conference evaluation on questions still remaining
- Update Note on Findings – Key Areas of Knowledge Needs (identified among all stakeholders, both at large and registrants) :
  - Methods/approaches for engaging all appropriate patients/patient partners in the clinical research process, and particularly in benefit-risk assessment
  - Approaches to network building with other stakeholders (patients and researchers alike have these questions)
  - Fundamentals of benefit-risk assessment
  - The value/impact of patient perspective in the medical product life cycle
  - How to systematically incorporate data on patient perspectives into the medical product submission for consideration during review and approval

5. **Identify audience values, motivation, and expectations**
   - Pre-registration survey results

6. **Determine the incentives for necessary adoption/change/response to call to action**
   - CTTI report from CTTI-DIA survey on practices and barriers to patient engagement in clinical research (general, not specific to benefit-risk assessment)Stakeholder discussions in planning committee (and ongoing feedback)
   - Ongoing contacts and briefing discussions with key stakeholders and influencers
   - Pre-conference surveys:
     - At large survey – specific to benefit-risk practices
     - Attendee pre-conference survey
   - Pre-conference focus group with industry clinical researchers on patient engagement practices within their companies
   - Questions during work on visual model
     - What are the challenges with patient engagement in benefit-risk assessment?
     - What new information is needed to better engage patients, better gather & incorporate their input?
     - What changes in attitudes, culture, and systems are needed?
     - What resources are needed?
   - Conference evaluation to include questions specific to:
     - Audience learning and take-aways
     - Questions they still have; issues needing further discussion/learning opportunities
     - How likely they are to apply the learnings in their work
     - Barriers they anticipate in applying learnings
   - Post-conference work to refine messages based on learnings
     - Review Visual Model to confirm incorporation of all comments
     - Finish Visual Model in format clearly illustrates concepts, breaking them down into components that are applicable at specific (appropriate) stages

7. **Goals for adoption of practices/implementation of conference learnings:**
   - **Goal 1:** Medical product researchers will use the learnings resulting from this conference to engage patients and incorporate patient input data into all benefit-risk assessment decisions during the life cycle of the medical product
   - **Goal 2:** Patients/Caregivers/Patient Organizations will use the learnings from this conference to participate in and engage their communities in providing appropriate data for benefit-risk assessment and participation in the decision-making process
   - **Goal 3:** The conference learnings will contribute to the dialog informing the larger regulatory process on Patient Focused Drug Development, specifically the incorporation of data on patient benefit-risk balance perspectives into the medical product review and approval process, and the guidance provided to medical product researchers on collection and utilization of patient perspective data

8. **Target messages to audience and their needs and motivation:**
   - Learnings from the conference about:
     - Importance and impact of patient engagement in all key stages of benefit-risk assessment of medical products

- Approaches, methods, and timing for stakeholders to appropriately engage patients and utilize patient input in benefit-risk assessments
- Patient opportunities and approaches to providing appropriate input to benefit-risk assessment
- Operational, methodological, and regulatory challenges all stakeholders may experience in engaging patients and incorporating patient input into benefit-risk decision-making; approaches to overcoming these challenges
- Needs for improvement and new knowledge in engaging patients, collecting patient perspectives on benefits, risks, and balance in the context of patient needs
- Opportunities to work toward changes and improvements to assure better outcomes for patients

- Visual model will be refined to capture learnings and facilitate stakeholder view of the overall picture and elements of patient engagement in benefit-risk assessment of medical products
  - Visual model iterations tailored to specific stakeholder groups will be considered
- Update Note on Findings:
  - Pre-registrants industry/research desired help for better patient engagement in benefit-risk:
    - 94% say need more guidance on methodology
    - 71% say need information on establishing collaborations with patients
    - 65% are concerned with assuring representative patient input
    - 12% say need to obtain buy-in within organization
    - 18% have other needs, mainly FDA guidance, methods/approaches, support
  - At large industry/research percentages differ slightly:
    - 5% say need no additional help
    - 88% say need more guidance on methodology
    - 43% say need information on establishing collaborations with patients
    - 54% are concerned with assuring representative patient input
    - 18% need to obtain buy-in within their organization
    - 15% have other needs, mainly FDA guidance, methods/approaches, support
  - Pre-registrants patient groups:
    - 88% say need information on establishing collaboration with researchers
    - 75% say need more guidance on methodology
    - 63% say need more information on the medical product life cycle processes
    - 25% say need guidance on obtaining buy-in from the patient community
  - At large patient groups expressed a different pattern of needs:
    - 100% say need more guidance on methodology
    - 71% say need more information on the medical product life cycle processes
    - 48% say need information on establishing collaboration with researchers
    - 33% say need guidance on obtaining buy-in from the patient community
  - Conclusion: All groups seem to need more information on methodology for obtaining patient input on benefit-risk; the need to assure representative patient input is high among all groups, also. There is a fairly strong need among all stakeholders to better understand/approach collaboration with other stakeholders. Buy-in from the stakeholders' own organizations seems to be a less pressing need.

9. Identify relevant engagement vehicles (for dissemination of learnings):

- **Presentations and Conference Summary:** Emailing and online posting

- **Visual Model**
  - Refine with all input from conference discussions and feedback to reflect current state and desired future state of patient engagement in benefit-risk assessment
  - Share with conference participants to confirm that it represents the consensus of the group (all stakeholders)
  - Model to be produced in electronic and print formats
  - Distribute as attachment to other dissemination vehicles
  - Distribute as stand-alone visual piece for stakeholder learning, discussion, self-assessment, and planning tool
  - Distribute to professional associations of stakeholders (after peer-reviewed publication) as basis for call to action to address new knowledge and improvement needs
  - References and resources to be attached
  - Crowdsourcing for additional model feedback after model is posted
    - Refined model to be posted and distributed as a "living" model
    - Distribution through DIA and conference stakeholders and supporters (reference Item 3 above)
    - Comments and feedback from stakeholders at large to be funneled back to DIA and reviewed by a dissemination team
    - Early dissemination team includes stakeholder representatives from the program committee; ongoing, it will be coordinated by DIA and will always include representatives of all stakeholder groups
  - Visual Model to be periodically updated
- **Peer reviewed journal:**
  - Conference proceedings/White Paper – DIA Journal of Therapeutic Innovation and Regulatory Science (TIRS) referenceable, open access article subsequent to conference to reach industry and academic research audiences, regulators, patient organizations, related organizations
  - *Possible* special section on patient engagement in benefit-risk assessment:
    - Articles by stakeholders or stakeholder collaboration groups relating to specific content and learnings from the conference
- **Articles in multiple publications:**
  - For awareness raising and message distribution: Conference recap, highlights, learnings, featured patient organizations, featured speakers and messages, sources for more information
  - DIA Global Forum – print and electronic
  - Newsletters/publications of Program Committee, Speaker, and other partners' organizations
- **Blogs, podcasts, and social media** on conference highlights, learnings, featured patient organizations, featured speakers and messages, sources for more information
- **Briefing document and related materials as completed for presentation at conferences and meetings:**
  - Stakeholder organizations
  - Expansion to additional stakeholders who were beyond the scope of this conference, i.e. payers, clinician groups