The National Academies of SCIENCES ENGINEERING MEDICINE

Forum on DRUG DISCOVERY, DEVELOPMENT, and TRANSLATION

Advancing the Science of Patient Input in Medical Product R&D: Towards a Research Agenda – *A Workshop*

Discussion Tool

Background: This tool will be used during a tabletop exercise to inform the moderated discussion at the workshop. If time permits, we request that you review and consider prepopulating this table in advance of the event. Soliciting input from colleagues within your organization is encouraged. If you choose to prepopulate this tool, please bring it with you to the workshop. With your permission, staff will collect completed tables at the conclusion of the workshop. *Please do not return this tool to Forum staff in advance of the workshop.*

Instructions: Potential categories of patient input are provided under in the top-left corner of the tool for each session. Please choose up to **three** categories to populate the table (one per row). You may also choose to add your own categor(y)(ies). For each category, work across the table from left to right. A general example for each session is provided—we would encourage you to provide as much detail as you deem necessary in your own population of the tool.

A note on the workshop: At the workshop, participants will be seated in small group tables (approximately six to eight per table). In each session, we will devote 45 minutes to a tabletop exercise using this discussion tool. One participant at each table will be the designated scribe. Participants will have approximately 25 minutes to brainstorm and complete the tool as a group, followed by approximately 20 minutes of voluntary report-out from tables. These report-outs will serve as the basis for a 45-minute moderated discussion.

Contact Information

Name: _____

Organization: _____

Note: Your written responses to this tool are for use by Forum staff and members of the associated Science of Patient Input Collaborative only; they will not be publicly released. Staff may contact you for follow-up or clarification on responses. You may also choose to share and discuss your responses to this tool during public sessions of the workshop.

SESSION I: Understanding Patient Experience with Disease or Medical Condition

Categories of Patient Input • Natural history of disease/condition • Burden of living with disease/condition • Burden of managing disease/condition • Views on currently available treatment options and unmet medical need	**STAGE SETTING: Decision-Making Context and Data Needs**		**BRAINSTORMING: Identifying Gaps/Barriers**		**NEXT STEPS: Research Approach**
	Purpose/Use and Potential Impact	**Methods/Tools and Data Sources**	**Scientific/Technical**	**Non-Scientific/Technical**	**Target Audience/ Stakeholders for Future Research**
EXAMPLE: Burden of living with disease X	• *Inform preclinical research questions / study design (e.g., identification of a biological pathway or target)*	• *Methods: Qualitative; Natural language processing* • *Data source: Social media*	• *Accounting for heterogeneity of responses and response bias (high-reporters and low-reporters)* • *Defining quality standards and rigor for social media data*	• *Impact of cultural values on experience of disease*	• *Survey developers* • *Academic researchers*
Category 1:	•	• Method(s): • Data source(s):	•	•	•
Category 2:	•	• Method(s): • Data source(s):	•	•	•
Category 3:	•	• Method(s): • Data source(s):	•	•	•

SESSION II: Patient Perspectives and Preferences on Benefit–Risk

Categories of Patient Input • Minimum expectations of benefits • Tolerance for harms or risks • Acceptable tradeoffs of benefits and risks • Attitudes towards uncertainty	**STAGE SETTING: Decision-Making Context and Data Needs**		**BRAINSTORMING: Identifying Gaps/Barriers**		**NEXT STEPS: Research Approach**
	Purpose/Use and Potential Impact	**Methods/Tools and Data Sources**	**Scientific/Technical**	**Non-Scientific/Non-Technical**	**Target Audience/ Stakeholders for Future Research**
EXAMPLE: Minimum expectations of benefits for treatment Y	• *Research funding (e.g., R01 or nonprofit funder)*	• *Method: Quantitative* • *Data Source: Questionnaire*	• *Establishing credibility of questionnaire, including validation and/or qualification*	• *Guidance on level of governance (e.g., classification of market research versus human subject research)*	• *Public-private partnership* • *Policy-makers/Regulators*
Category 1:	•	• Method(s): • Data source(s):	•	•	•
Category 2:	•	• Method(s): • Data source(s):	•	•	•
Category 3:	•	• Method(s): • Data source(s):	•	•	•

SESSION III: Patient Input on Clinical Trial Development and Continuous Improvement

Categories of Patient Input • Protocol development • Recruitment, enrollment, and retention • Measuring trial participant experience	**STAGE SETTING: Decision-Making Context and Data Needs**		**BRAINSTORMING: Identifying Gaps/Barriers**		**NEXT STEPS: Research Approach**
	Purpose/Use and Potential Impact	**Methods/Tools and Data Sources**	**Scientific/Technical**	**Non-Scientific/Technical**	**Target Audience/ Stakeholders for Future Research**
EXAMPLE: Protocol development	• *Inform inclusion/exclusion criteria*	• *Method: Mixed-method* • *Data source: Survey*	• *Determining rigor of evidence needed for sponsor/internal decision-making versus potential use in regulatory-decision making*	• *Establishing partnerships/ engaging patients on protocol development committees*	• *Industry partners* • *Patient groups*
Category 1:	•	• Method(s): • Data source(s):	•	•	•
Category 2:	•	• Method(s): • Data source(s):	•	•	•
Category 3:	•	• Method(s): • Data source(s):	•	•	•