

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

May 9, 2018 ▪ Washington, DC

### Workshop Background and Objectives

Converting traditionally anecdotal patient input into rigorous, credible evidence for use by a broad range of stakeholders could better align medical product research and development (R&D) and regulatory decision-making with patient experience of and preferences for disease management and treatment. Accordingly, many efforts have been launched to advance a **science of patient input**—the development and use of systematic approaches and tools to collect, analyze, and apply patient input to the medical product R&D lifecycle and regulatory decision-making processes. Despite significant progress made in highlighting the value and impact of patient input in medical product R&D, and momentum towards the development of a science of patient input, there is a critical need to examine gaps in the knowledge base and other barriers that are hindering the advancement of this field and explore a research agenda for addressing them.

The Forum on Drug Discovery, Development, and Translation at the National Academies of Sciences, Engineering, and Medicine is hosting a one-day, discussion-based workshop to examine gaps in knowledge and other barriers that are hindering the advancement of a science of patient input in medical product R&D, with consideration of downstream regulatory and post-market decision-making. Subject matter experts representing a range of disciplines will engage in discussions to:

- Examine the state of the science of patient input, including successes and limitations of current efforts.
- Explore gaps in the knowledge base and other barriers that impede progress.
- Discuss potential components of a research agenda for addressing gaps or barriers to realizing a science of patient input.

### Workshop Planning Committee

**Cynthia Grossman (co-chair)**, FasterCures  
**Marilyn Metcalf (co-chair)**, GlaxoSmithKline  
**Marc Boutin**, National Health Council  
**Kenneth Getz**, Tufts Center for the Study of Drug  
Development  
**Mats Hansson**, Uppsala University  
**Lynn Hudson**, Critical Path Institute  
**Theresa Mullin**, U.S. Food and Drug  
Administration

**William Riley**, National Institutes of Health  
**Roslyn Schneider**, Pfizer Inc.  
**Suzanne Schrandt**, Arthritis Foundation  
**Lana Skirboll**, Sanofi  
**Pamela Tenaerts**, Clinical Trials Transformation  
Initiative  
**John Wagner**, Takeda Pharmaceuticals  
**Richard Willke**, International Society for  
Pharmacoeconomics and Outcomes Research

## Agenda

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### 8:30 am Welcome and overview of the day

*Background and Goals of the Workshop*

**Marilyn Metcalf** (Workshop Co-Chair), Lead, Patient Engagement, GlaxoSmithKline

*State of the Science of Patient Input*

**Cynthia Grossman** (Workshop Co-Chair), Director, Science of Patient Input, FasterCures

*Format and Structure of the Day*

**Mark Trusheim** (Workshop Facilitator), President, Co-Bio Consulting

## Session 1: Understanding Patient Experience with Disease or Medical Condition

### 9:00 am Lightning presentations

- **Lauren Bataille**, Senior Associate Director of Research Partnerships, Michael J. Fox Foundation for Parkinson's Research
- **Jennifer Liao**, Director, Business Development and Digital Health Lead, Evidation Health
- **Theresa Mullin**, Associate Director for Strategic Initiatives, Center for Drug Evaluation and Research, U.S. Food and Drug Administration
- **Arthur Stone**, Director, Dornsife Center for Self-Reported Science, University of Southern California

### 9:45 am Small group tabletop exercise

*Participants will be seated in small group tables, with one participant at each table acting as scribe. Participants will have approximately 25 minutes to brainstorm and complete the discussion tool as a group, followed by approximately 20 minutes of voluntary report-outs.*

### 10:30 am Moderated plenary discussion

*Each of the following questions is intended to build on the prior, as one moves from left to right across the discussion tool.*

- In the context of the category of patient input identified, is additional research needed to a) determine the optimal points at which patient experience data should be solicited throughout medical product R&D and/or b) examine the impact of—and consequences of not—collecting and applying patient input? If yes, what research is needed? *(Column 1)*
- What types of methods (quantitative, qualitative, or mixed) and data sources can be used to gather patient experience data for the identified category? How well do the most appropriate methodological approaches align with the types of methods that are more likely to persuade stakeholders/decision makers? *(Column 2)*
- Taking into account the purpose, use, and impact of the input (Column 1), and considerations around the science to solicit and apply that input (Column 2), what gaps or barriers exist and should be addressed? *(Columns 3 and 4)*
- Is the gap/barrier identified researchable? If so, what research currently exists? What novel or additional research should take place? *(Column 5)*
  - Which stakeholder(s) would be best suited to advance the research?
  - What skillsets already exist in the workforce that could be leveraged?
  - What new competencies are needed? Who could/should provide this training and/or education?

### 11:15 am BREAK

## Session 2: Patient Perspectives and Preferences on Benefit–Risk

### 11:45 am Lightning presentations

- **Mats Hansson**, Professor and Director, Centre for Research Ethics & Bioethics, Uppsala University and Coordinator, Innovative Medicines Initiative PREFER
- **Brett Hauber**, Senior Economist and Vice President, Health Preference Assessment, RTI Health Solutions
- **Kathryn O’Callaghan**, Assistant Director, Office of Strategic Programs, Center for Devices and Radiological Health, U.S. Food and Drug Administration

### 12:30 pm Small group tabletop exercise

*Participants will be seated in small group tables, with one participant at each table acting as scribe. Participants will have approximately 25 minutes to brainstorm and complete the discussion tool as a group, followed by approximately 20 minutes of voluntary report-outs.*

### 1:15 pm Moderated plenary discussion

*Each of the following questions is intended to build on the prior, as one moves from left to right across the discussion tool.*

- In the context of the category of patient input identified, is additional research needed to a) determine the optimal points at which patient preference information should be solicited throughout medical product R&D and/or b) examine the impact of—and consequences of not—collecting and applying patient input? If yes, what research is needed? **(Column 1)**
- What types of methods (quantitative, qualitative, or mixed) and data sources can be used to gather patient preference information for the identified category? How well do the most appropriate methodological approaches align with the types of methods that are more likely to persuade stakeholders/decision makers? **(Column 2)**
- Taking into account the purpose, use, and impact of the input (Column 1), and considerations around the science to solicit and apply that input (Column 2), what gaps or barriers exist and should be addressed? **(Columns 3 and 4)**
- Is the gap/barrier identified researchable? If so, what research currently exists? What novel or additional research should take place? **(Column 5)**
  - Which stakeholder(s) would be best suited to advance the research?
  - What skillsets already exist in the workforce that could be leveraged?
  - What new competencies are needed? Who could/should provide this training and/or education?

### 2:00 pm BREAK

## Session 3: Patient Input on Clinical Trial Development and Continuous Improvement

### 2:15 pm Lightning presentations

- **Lynn Hagger**, Director, Patient Engagement, AstraZeneca
- **Anuj Patel**, Director, Commercial Offerings, PatientsLikeMe
- **Joy Simha**, Board Member, National Breast Cancer Coalition

### 3:00 pm Small group tabletop exercise

Participants will be seated in small group tables, with one participant at each table acting as scribe. Participants will have approximately 25 minutes to brainstorm and complete the discussion tool as a group, followed by approximately 20 minutes of voluntary report-outs.

### 3:45 pm Moderated plenary discussion

Each of the following questions is intended to build on the prior, as one moves from left to right across the discussion tool.

- In the context of the category of patient input identified, is additional research needed to a) determine the optimal points at which patient input should be solicited and applied throughout the clinical trial lifecycle and/or b) examine the impact of—and consequences of not—collecting and applying patient input? If yes, what research is needed? *(Column 1)*
- What types of methods (quantitative, qualitative, or mixed) and data sources can be used to gather patient input for the identified category? How well do the most appropriate methodological approaches align with the types of methods that are more likely to persuade stakeholders/decision makers? *(Column 2)*
- Taking into account the purpose, use, and impact of the input (Column 1), and considerations around the science to solicit and apply that input (Column 2), what gaps or barriers exist and should be addressed? *(Columns 3 and 4)*
- Is the gap/barrier identified researchable? If so, what research currently exists? What novel or additional research should take place? *(Column 5)*
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## Wrap-Up

### 4:30 pm Reflections and vision for the future

- **Tanisha Carino**, Science of Patient Input Collaborative Co-Chair, Executive Director, FasterCures

### 4:45 pm Concluding remarks and next steps

- **Cynthia Grossman**, Workshop Co-Chair, Director, Science of Patient Input, FasterCures
- **Marilyn Metcalf**, Workshop Co-Chair, Lead, Patient Engagement, GlaxoSmithKline

### 5:00 pm Adjourn