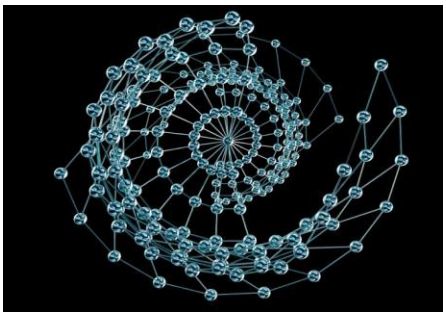
Forum on
DRUG DISCOVERY, DEVELOPMENT,
and TRANSLATION

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

May 9, 2018

National Academy of Sciences Building
Lecture Room
Washington, DC





The National Academies of
SCIENCES • ENGINEERING • MEDICINE

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

May 9, 2018

Meeting Materials

Workshop Materials

Forum Background Information

The National Academies of SCIENCES ENGINEERING MEDICINE

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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

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** – *Participants get lunch and return at 11:45 am for Session 2 (Working Lunch)*

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** – *Participants return to their seats at 2:15 p.m.*

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)***
    - 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?

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**

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PARTICIPANT LIST

Nina Ahluwalia, Genentech
Philip Alberti, Association of American Medical Colleges
Ronald Bartek, Friedreich's Ataxia Research Alliance
Lauren Bataille, The Michael J. Fox Foundation for Parkinson's Research
*__Marc Boutin,__ National Health Council
John Bridges, The Ohio State University
Nicholas Brooke, The Synergist
Tanisha Carino, FasterCures, Milken Institute
Kristin Carman, Patient Centered Outcomes Research Institute
Luther Clark, Merck
Donna Cryer, Global Liver Institute
Mark Dant, EveryLife Foundation for Rare Diseases
Hildy Dillon, Cancer Support Community
Andrea Ferris, LUNGevity Foundation
Rachael Fleurence, National Evaluation System for health Technology Coordinating Center
Kevin Fowler, Kidney Health Initiative
Danielle Friend, Biotechnology Innovation Organization
*__Kenneth Getz,__ Tufts Center for the Study of Drug Development
*__Cynthia Grossman,__ FasterCures
Lynn Hagger, AstraZeneca
Marni Hall, IQVIA
*__Mats Hansson,__ Uppsala University
Brett Hauber, RTI Health Solutions
*__Lynn Hudson,__ Critical Path Institute
Telba Irony, Center for Biologics Evaluation and Research, U.S. Food and Drug Administration
Laura Lee Johnson, Center for Drug Evaluation and Research, U.S. Food and Drug Administration
Patricia Jones, National Center for Advancing Translational Sciences, National Institutes of Health
Eva Katz, Janssen
Jennifer Liao, Evidation Health
Barbara Lopez Kunz, Drug Information Association
Codrin Lungu, National Institute of Neurological Disorders and Stroke, National Institutes of Health
Domitilla Masi, Avalere Health
Joey Mattingly, University of Maryland School of Pharmacy

* Planning Committee Member

Robert McBurney, Accelerated Cure Project for Multiple Sclerosis
*__Marilyn Metcalf,__ GlaxoSmithKline
Carol Meyer, Takeda Pharmaceuticals
Debra Michaels, Drug Information Association
Megan Moncur, Center for Biologics Evaluation and Research, U.S. Food and Drug Administration
*__Theresa Mullin,__ Center for Drug Evaluation and Research, U.S. Food and Drug Administration
Kathryn O'Callaghan, Center for Devices and Radiological Health, U.S. Food and Drug Administration
Anuj Patel, PatientsLikeMe
Bray Patrick-Lake, Duke Clinical Research Institute
Eleanor Perfetto, National Health Council
*__William Riley,__ Office of Behavioral and Social Sciences Research, National Institutes of Health
Andrew Robertson, Sanofi
Raj Sabharwal, AcademyHealth
*__Roslyn Schneider,__ Pfizer
*__Suzanne Schrandt,__ Arthritis Foundation
Karlin Schroeder, Parkinson's Foundation
Jessica Scott, GlaxoSmithKline
T.J. Sharpe, Starfish Harbor
Joy Simha, National Breast Cancer Coalition
Janine Simmons, National Institute of Mental Health, National Institutes of Health
*__Lana Skirboll,__ Sanofi
Jaye Bea Smalley, Celgene
Yanni Souroutzidis, Datavant
France Sowell, Pharmerit
Arthur Stone, University of Southern California
*__Pamela Tenaerts,__ Clinical Trials Transformation Initiative
Mary Tobin, Alliance for Clinical Research Excellence and Safety
Mark Trusheim, Co-Bio Consulting LLC
James Valentine, Hyman, Phelps, & McNamara, P.C.
*__John Wagner,__ Takeda Pharmaceuticals
Kevin Weinfurt, Duke University School of Medicine
*__Richard Willke,__ International Society For Pharmacoeconomics and Outcomes Research
Anthony Yanni, Sanofi

Staff

Carolyn Shore, Forum Director
Morgan Boname, Program Officer and Staff Lead
Rebecca English, Program Officer
Amanda Wagner Gee, Program Officer
Noam Keren, Associate Program Officer
Melvin Joppy, Senior Program Assistant

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PRESENTER BIOGRAPHIES

LAUREN BATAILLE, M.S., started at the Michael J. Fox Foundation for Parkinson's Research (MJFF) in August 2015. In her role as Associate Director of Research Partnerships, she manages MJFF's wearable projects as well as developing and implementing new clinical studies utilizing smart phone apps and watches. Prior to joining MJFF, Lauren was the Director of Programs at Basic Health International where she managed pilot programs and clinical trials focused on cervical cancer prevention in low-resource settings. Lauren also worked at the consulting firm Global Health Strategies where she helped leading foundations and nonprofits to plan and implement global advocacy and media campaigns. Lauren graduated with an M.S. in Neuroscience & Behavior from the University of Massachusetts at Amherst, and holds a B.A. in Neuropsychology from the University of Massachusetts's Commonwealth Honors College. During her free time, Lauren enjoys traveling, scuba diving, and listening to podcasts.

LYNN HAGGER, PH.D., is the Global Patient Engagement Director for Respiratory, Inflammation, Neuroscience and Autoimmune diseases in Global Medical Affairs at AstraZeneca Pharmaceuticals. Lynn is responsible for developing strategies to generate patient insights that will strengthen the global development of patient-centric products and services. She has a special interest in innovative digital solutions that will help patients better self-manage their conditions. Since 1998, Lynn has served as a successful member and leader of cross-functional teams in Medical Affairs, Marketing, and Clinical Operations, across a variety of therapeutic areas at AstraZeneca. Lynn holds a Ph.D. in Microbiology and Medical Genetics from the University of Toronto, Canada.

***MATS HANSSON, PH.D.,** is the director of the Centre for Research Ethics & Bioethics and has conducted extensive research in biomedical ethics as principal investigator in several multi-disciplinary research projects dealing with issues ranging from ethical, social and legal aspects of the implementation of genetic diagnosis in clinical practice and the use of human tissue materials in research, to clinical and medical ethics. Dr. Hansson is Professor of Biomedical Ethics, funded by Uppsala University and the Uppsala County Council together. He also works as a clinical consultant at Akademiska sjukhuset (Uppsala University Hospital). Dr. Hansson leads work packages on ethical, legal and social issues in several EU projects on biobank and registry research. He is co-coordinator of the Innovative Medicines Initiative PREFER project, the principal investigator

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in Mind the Risk, and one of the co-ordinators of BBMRI-ERIC's ELSI common service. He holds an undergraduate degree in biology (1974) and a doctoral degree of theology (1991).

BRETT HAUBER, PH.D., is a Senior Economist and the Vice President of Health Preference Assessment at RTI Health Solutions (RTI HS) and an Affiliate Associate Professor in the Department of Pharmacy at the University of Washington. He has more than 20 years of academic, research, and government experience in health and environmental economics. His primary area of specialization is in quantifying preferences for health interventions and health outcomes. He also has extensive experience in conducting benefit-risk analysis with patients and other health care decision makers. He has studied the theoretical and empirical relationships among various health utility measures. His most recent applied work has included the use of multiple stated preference techniques to elicit patient and physician benefit-risk preferences for treatments for conditions in numerous therapeutic areas. Dr. Hauber regularly teaches courses on conjoint analysis and discrete-choice experiments. He was a member of to the Patient-Centered Benefit-Risk Steering Committee of the Medical Device Innovation Consortium (MDIC) and was the principal investigator for developing the Catalog of Methods for Assessing Patient Preferences for Benefits and Harms of Medical Technologies for MDIC. He is currently a member of the scientific advisory board for the IMI-PREFER project and an advisor to a number of initiatives led by industry and patient-advocacy organizations to incorporate patient preferences in regulatory and reimbursement decision making in multiple disease areas. He was the chair of the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Conjoint Analysis–Statistical Analysis, Reporting, and Conclusions (CA-SARC) task force and was previously a member of the ISPOR task force that developed the ISPOR Checklist for Good Research Practices in Conjoint Analysis. Dr. Hauber's research has been published in numerous health outcomes and medical journals.

JENNIFER LIAO is Director of Business Development at Evidation Health. Prior to joining Evidation Health, Ms. Liao managed business development and partnerships for Rock Health, a full-service seed fund that supports startups working in digital health and one of the first investors of Evidation Health. She also previously researched amyotrophic lateral sclerosis (ALS) in Tom Maniatis's lab at Columbia University. Jennifer graduated from the University of Pennsylvania with a B.A. in Biological Basis of Behavior, with a minor in Healthcare Management.

***THERESA MULLIN, PH.D.,** is Associate Director for Strategic Initiatives in the Center for Drug Evaluation and Research (CDER) at the U.S. Food and Drug Administration (FDA). As CDER Associate Director for Strategic Initiatives, Dr. Mullin serves as the principal advisor and spokesperson on matters related to the development and implementation of key areas of program strategy including but may not be limited to CDER's international cooperation and harmonization strategy and decision science-based strategic initiatives including incorporation of the patient's experience and voice in drug development and benefit-risk assessment. As part of her work Dr. Mullin leads FDA Patient Focused Drug Development, and heads the FDA delegation to the International Council on Harmonization (ICH) where she also serves as Chair of the ICH Management Committee. Dr. Mullin previously served as Director of the CDER Office of Strategic Programs whose mission is to transform and modernize drug regulatory operations and informatics. Having led successful negotiations for the 2002, 2007 and 2012 cycles of reauthorization, Dr.

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Mullin recently led FDA negotiations for the 2017 FDA Reauthorization Act reauthorization of the Prescription Drug User Fee Act, providing more than $800 million per year in fee funding for new drug review. She has also led FDA negotiations for the 2017 FDARA reauthorization of the Biosimilar User Fee Act. Before joining CDER in September 2007, Dr. Mullin was Associate Commissioner for Planning in the FDA Office of Commissioner. Since joining FDA Dr. Mullin has received numerous awards including the Senior Executive Service Presidential Rank Award for Distinguished Service in 2011, and the Presidential Rank Award for Meritorious Service in 2006. She was also named a recipient of the 2017 FDLI Distinguished Service and Leadership Award. Before coming to FDA, Dr. Mullin was a Senior Manager with The Lewin Group, and prior to that, Principal Scientist at Decision Science Consortium. Dr. Mullin received her B.A., magna cum laude, in Economics from Boston College, and Ph.D. in Public Policy Analysis from Carnegie-Mellon University.

KATHRYN (KATIE) O'CALLAGHAN is Assistant Director for Strategic Programs at the Center for Devices and Radiological Health (CDRH) at the U.S. Food and Drug Administration (FDA). Ms. O'Callahan oversees a broad program portfolio at CDRH, with teams supporting a number of strategic partnership and regulatory science programs. Her focus is on directing the Center's 2016-2017 Strategic Priority of Partnering with Patients. This work aims to promote a culture of meaningful patient engagement between patients and CDRH's employees, and to increase use and transparency of patient input as evidence in regulatory decision-making. Execution impacts ~1700 employees and dozens of programs and policies across eight organizational departments. Ms. O'Callahan is a biomedical engineer by training and worked in MedTech industry prior to her 11+ years at FDA.

ANUJ PATEL, M.B.A., is currently the Director of Product for PatientsLikeMe, a digital biology and patient powered research network. He is focused on building the portfolio of products and services that advance drug development and improve the lives of patients with rare or chronic disease. Prior to working at PatientsLikeMe, Anuj led corporate development and new ventures for IQVIA specifically focusing in the digital health space. He received his M.B.A in Healthcare Management and Finance from Duke University and currently resides in New York.

JOY SIMHA is one of the three original Co-Founders of the Young Survival Coalition (YSC) and represents the YSC on the Board of Directors of The National Breast Cancer Coalition. Joy was an ad hoc reviewer for the Department of Defense (DOD) Peer Reviewed Breast Cancer Research Program (BCRP). She currently sits on the Integration Panel of the DOD BCRP and served as Chair of the Integration Panel in FY2015. In 2010, she was appointed to the Centers for Disease Control and Prevention (CDC's) Panel on Breast Cancer in Young Women. She has served as a Consumer Advocate for various Cochrane Coalition Systematic Reviews and as a Consumer Advocate for the Agency for Healthcare Research and Quality (AHRQ's) brochures and communications to the public. Ms. Simha has been a panel member to the Institute of Medicine Evidence Communication Innovation Collaborative. Ms. Simha comes from a background in Video and Media Production and enjoys writing. She currently resides in New Jersey with her husband and two children.

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ARTHUR STONE, PH.D., was trained as a clinical psychologist and is currently Professor of Psychology, Economics, and Public Policy, and Director of the USC Dornsife Center for Self-Report Science at the University of Southern California. He is also Emeritus Distinguished Professor of Psychiatry and Behavioral Science at Stony Brook University. Stone's early work was concerned with improving the measurement of life events and coping with the goal of understanding how events and coping impact our susceptibility to somatic illnesses. These studies led to an interest in psychobiology with a particular emphasis on how environmental events affect the immune system and the endocrine system. At the same time, he was researching how people self-report information about their psychological and symptom states. This led to the development of various kinds of daily diaries that measured end-of-day and within-day phenomena, which ultimately yielded a set of techniques known as Ecological Momentary Assessment. More recently, Stone has been involved with the development of alternative methods for capturing the ebb and flow of daily experience for large-scale surveys, including the development of the Day Reconstruction Method. Stone has used these new methods to explore a variety of phenomena including pain, fatigue, stress, and wellbeing. He is also been involved with the development of questionnaires for use in clinical trials, which has been supported by a consortium from the National Institutes of Health. Regarding health policy, Stone's work with the OECD, the US National Academy of Science, and international population surveys (SHARE, HRS) on subjective well-being has, in part, been focused on policy decisions implications of well-being data.

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PLANNING COMMITTEE BIOGRAPHIES

CO-CHAIRS

CYNTHIA (CYNDI) GROSSMAN, PH.D., is director, Science of Patient Input at *FasterCures*. Prior to joining *FasterCures*, Grossman was chief of the HIV Care Engagement and Secondary Prevention Program in the Division of AIDS Research at the National Institute of Mental Health. Her grant portfolio focused on research to improve the lives of people living with HIV/AIDS, including reducing the risk of onward transmission. She has spent her career encouraging research to address the unmet patient needs related to mental health, stigma, and other social determinants of health. She has also played a lead role in defining the social and behavioral scientific agenda for microbicides as HIV prevention as well as HIV cure related research. Grossman hold a bachelor's degree in psychology and biology from Earlham College, a doctoral degree in clinical psychology from the University of Vermont and completed her postdoctoral work at Brown University. She works at the Institute's Washington, DC office.

MARILYN METCALF, PH.D. I am the U.S. spokesperson for GlaxoSmithKline's (GSK's) Patients in Partnership team. We leverage the company's experience collaborating with patients to grow consistent best practices throughout the organization and beyond, working with patients as expert stakeholders in creating medicines. As a family member and care partner of people who have lived with life-threatening illnesses, I am committed to partnering with patients to enable clear understanding of our medicines and how they can meet people's health goals. I am a member of GSK's Global Safety Board and participate in a number of alliances including Patient Focused Medicine Development; Patients as Partners; FasterCures; National Health Council; National Academies of Sciences, Engineering, and Medicine; TransCelerate; PhRMA; Drug Information Association; and Council for International Organizations of Medical SciencesWorking Group XI. Previously I was Family Health International's project director of an NIH master contract for HIV vaccine research, primarily in lower- and middle-income countries. At the former GlaxoWellcome I studied the safety and efficacy, health economics, and quality of life effects of HIV, oncology, and respiratory therapies. In 2001, I moved with my family to the U.K. to rebuild GSK's international Decision Sciences team. After returning to the U.S., I went to Centocor to lead their R&D Portfolio Management team. I came back to GSK and formed our Benefit Risk Evaluation team, then led GSK's Pharmacovigilance Centre of Innovation. I began my current role in June 2017.

MEMBERS

MARC M. BOUTIN, J.D., is the Chief Executive Officer of the National Health Council. He has been a leading voice for greater patient involvement at every stage of the health care continuum, starting with the development of new drugs, to regulatory oversight of health care delivery, to shared decision-making at the point of care. Under his leadership, the National Health Council has convened a broad range of stakeholders to create and effectively implement pragmatic strategies and public policy that address diverse issues, such as enhancing patient engagement, advancing the development of new treatments, and developing a better health delivery system to meet the needs of people with chronic conditions. Boutin has a long history of board and committee service. Currently he serves as a member of the Patient-Centered Outcomes Research Institute (PCORI) Patient Engagement Advisory Panel, FasterCures Benefit-Risk Advisory Council, and the Medical Device Innovation Consortium (MDIC) Patient-Centered Benefit-Risk Steering Committee. Boutin has been actively involved in patient advocacy organization management, health advocacy, and both federal and state policy throughout his career. He is a founding member of the international Patient-Focused Medicine Development consortium and has served on the Governing Board of the International Alliance of Patients' Organizations as a member and treasurer. He is also a former member of the Partnership to Fight Chronic Disease Board of Directors, the Humana Cares Clinical Advisory Board, the eHealth Initiative Leadership Council, Community Health Charities Board of Directors, Healthcare Systems Research Collaboratory, and the North America Advisory Board to the Drug Information Association.

KENNETH (KEN) A. GETZ, M.B.A., is the Director of Sponsored Research Programs and an Associate Professor at the Tufts Center for the Study of Drug Development where he studies R&D management practices; pharmaceutical and biotechnology company operating models; and global investigative site, outsourcing, and study volunteer practices, trends and policies. Ken is also the chairman of CISCRP – a nonprofit organization that he founded to educate and raise public awareness of the clinical research enterprise — and the founder and owner of CenterWatch, a leading publisher in the clinical trials industry. A well-known speaker at conferences, symposia, universities and corporations, Ken has published extensively in peer-review journals, the trade press, and books. He holds a number of board appointments in the private and public sectors, is on the editorial boards of Contemporary Clinical Trials, Research Practitioner, the Drug Information Journal, Pharmaceutical Medicine and writes a column for Applied Clinical Trials that was a 2010 Neal Award finalist. Ken received an M.B.A. from the J.L. Kellogg Graduate School of Management at Northwestern University and a bachelor's degree, Phi Beta Kappa, from Brandeis University. Prior to founding CenterWatch, Ken worked for over seven years in management consulting where he assisted biopharmaceutical companies develop and implement business strategies to improve clinical development performance.

MATS HANSSON, PH.D., is the director of the Centre for Research Ethics & Bioethics and has conducted extensive research in biomedical ethics as principal investigator in several multi-disciplinary research projects dealing with issues ranging from ethical, social and legal aspects of the implementation of genetic diagnosis in clinical practice and the use of human tissue materials in research, to clinical and medical ethics. Dr. Hansson is Professor of Biomedical Ethics, funded by Uppsala University and the Uppsala County Council together. He also works as a clinical

consultant at Akademiska sjukhuset (Uppsala University Hospital). Dr. Hansson leads work packages on ethical, legal and social issues in several EU projects on biobank and registry research. He is co-coordinator of the Innovative Medicines Initiative PREFER project, the principal investigator in Mind the Risk, and one of the co-ordinators of BBMRI-ERIC's ELSI common service. He holds an undergraduate degree in biology (1974) and a doctoral degree of theology (1991).

Lynn D. Hudson, Ph.D., serves as the Chief Science Officer for the Critical Path Institute (C-Path) and the Executive Director of the International Neonatal Consortium (INC) and the Multiple Sclerosis Outcome Assessments Consortium (MSOAC). She started at C-Path by overseeing the first regulatory qualification of an imaging biomarker for patient enrichment in clinical trials for Alzheimer's disease. Recent collaborative efforts include launching INC and co-chairing the Scientific Advisory Committee of CFAST, the partnership between C-Path and CDISC for creating and maintaining therapeutic area data standards. Lynn graduated with a B.S. in Biochemistry from the University of Wisconsin and a Ph.D. in Genetics and Cell Biology from the University of Minnesota, and trained at Harvard Medical School and Brown University. As Chief of the Developmental Genetics Section at the National Institute of Neurological Disorders and Stroke, she conducted research to define the network of genes involved in neural development. She served as an officer for the American Society for Neurochemistry and the PMD Foundation, and as an advisor for a number of granting agencies, including NIH, NSF, and the National MS Society. At the National Institutes of Health, Lynn directed the Office of Science Policy Analysis from 2006-2011. Presently Lynn represents C-Path on the board of BIOSA, the Bioindustry Organization of Southern Arizona. She has a Research Professor appointment in the College of Medicine at the University of Arizona, and was honored by AZBIO as the 2015 Arizona Bioscience Leader of the Year.

Theresa Mullin, Ph.D., serves as Associate Director for Strategic Initiatives in the Center for Drug Evaluation and Research (CDER) at the U.S. Food and Drug Administration (FDA). She oversees areas of strategic interest to external stakeholders. She leads the Patient-Focused Drug Development initiative, which includes work related to the FDA Reauthorization Act and implementation of the 21st Century Cures Act. She also leads CDER's International Program. Dr. Mullin previously served as director of CDER's Office of Strategic Program for almost a decade. Under her leadership, the office became a critical part of CDER's sustained effort to modernize drug regulatory operations. Before joining CDER in 2007, Dr. Mullin was Assistant Commissioner for Planning in FDA's Office of the Commissioner. Dr. Mullin received her bachelor's degree, magna cum laude, in economics from Boston College, and she has a Ph.D. in public policy analysis from Carnegie-Mellon University. Dr. Mullin received the Senior Executive Service Presidential Rank Award for Meritorious in 2006 and for Distinguished Service in 2011.

William (Bill) T. Riley, Ph.D., was appointed Associate Director for Behavioral and Social Sciences Research, and Director of the Office of Behavioral and Social Sciences Research (OBSSR) at the National Institutes of Health (NIH) in August, 2015. Under his leadership, the OBSSR instituted its third and current strategic plan Download PDF (997 KB), which reflects key research challenges that the Office is uniquely positioned to address over the next five years, along with four foundational processes to enhance and support these scientific priorities as well as the OBSSR's broader mission. Since joining the NIH in 2005, he has served in extramural leadership positions at the National Institute of Mental Health (NIMH), the National Heart, Lung, and Blood Institute

(NHLBI), and the National Cancer Institute (NCI). He has contributed to several trans-NIH initiatives including serving as Chief Science Officer for the Patient-Reported Outcomes Measurement Information System (PROMIS) and as NIH Interim Deputy Director of the Precision Medicine Initiative (PMI, now called the All of Us Research Program). He has been the recipient of several NIH Director's Awards including recognition for his work on the PROMIS and PMI initiatives. Dr. Riley received his undergraduate degree in Psychology and Sociology from James Madison University, and his M.S. and Ph.D. in Clinical Psychology from Florida State University. He interned in Medical Psychology at Baylor College of Medicine. He has served on the faculty of the Medical College of Georgia and Virginia Commonwealth University. After 15 years in academic medical schools, he became Director of Research at PICS, Inc., a health behavior research and development firm. Dr. Riley holds an appointment as Professorial Lecturer in the School of Public Health at The George Washington University. Dr. Riley's research has contributed significantly to the behavioral and social sciences, particularly in the application of digital technologies to behavioral assessment and intervention. Among his over 130 publications, he published the first application of text messaging for smoking cessation, and a highly cited article on the limitations of current health behavior theories to mobile health (mHealth) interventions.

ROSLYN (ROZ) SCHNEIDER, M.D., joined Pfizer in 2006 and is the Global Patient Affairs Lead on Pfizer's Medical Leadership Team of the Chief Medical Office (CMO). In this newly created role, Dr. Schneider drives patient centricity and integration of the voice of the patient throughout the lifecycle of medicines and their development. Just prior to this she worked with the CMO on Medical Strategy and has held other leadership roles in Medical Affairs and Medicine Development at Pfizer. Dr. Schneider received her Bachelor of Science from the Sophie Davis School of Biomedical Education of the City College of New York, M.D. from Mount Sinai School of Medicine, and later her M.Sc. in Pharmaceutical Medicine from Hibernia College. Dr. Schneider is a retired Clinical Professor of Medicine of Albert Einstein College of Medicine, an Internist, Pulmonologist, Intensivist, and cared for patients at Beth Israel Medical Center, NY, for twenty years. She presented and published primarily in the areas of pulmonary complications of HIV infection, venous thromboembolic disease, medical ethics and medical education. Dr. Schneider was a Program Director for the Fellowship in Pulmonary and Critical Care, the Residency in Internal Medicine, and Assistant Chair of the Department of Medicine. She is a fellow of both the American College of Physicians and the American College of Chest Physicians where she chairs the Clinical Research Network. She also serves as Secretary on the Board of Trustees of the Physician Assistant Foundation, and is on the Advisory Council of the Keck Graduate Institute.

SUZANNE (SUZ) SCHRANDT, J.D., is Patient Engagement Director at the Arthritis Foundation. She was previously Deputy Director, Patient Engagement for the Patient-Centered Outcomes Research Institute, where she helped to launch key efforts including the Engagement Rubric. Ms. Schrandt's patient engagement focus stems from her own rheumatological diagnosis at age 14. Prior posts include roles in health and disability law and policy, genetics, and public health. Schrandt is chair of the International Society for Pharmacoeconomics and Outcomes Research Patient Roundtable and a member of the FDA's Patient Engagement Advisory Committee.

LANA SKIRBOLL, PH.D., M.S., is Vice President of Academic and Scientific Affairs at Sanofi, where she works on policy issues of importance to innovation. She formerly served as Director of Science Policy at the National Institutes of Health (NIH), where she was responsible for identifying policy issues relevant to the support and conduct of research, analyzing and recommending and creating new policies that advance the interest of the Agency. These included human subject protections, the privacy and confidentiality of research records, conflicts of interest, human embryo research, cloning and fetal tissue research, genetics, health, and society, dual use research, gene therapy and nanotechnology, comparative effectiveness research, personalized medicine, among others.Dr. Skirboll played a leadership role in NIH's organizational strategic planning and evaluation, where, for example, she developed NIH's efforts to measure and report on Agency performance. She also worked with the NIH Director, Elias Zerhouni, to design and implement the "Roadmap for Medical Research." She initiated the development of a new program on Return on Investment to explore NIH's impact on local economies and national competitiveness. She was responsible for developing and coordinating the NIH Public-Private partnership program, which leveraged NIH investments by working with industry. In addition, her team ran a small, but highly creative, science education program that worked nation-wide to develop materials for students and teachers on behalf of national science literacy and the pipeline of new young scientists, as well as creating opportunities with local students to learn about careers in science. As Acting Director of the NIH Division of Program Coordination, Planning, and Strategic Initiatives, she led national efforts to identify and address emerging scientific opportunities and rising public health challenges that cut across institutes, including the management of nearly 0.75 billion dollars in research funds. In addition, Dr. Skirboll was also responsible for NIH offices that coordinate research and activities related to research on AIDS, behavioral and social sciences, women's health, disease prevention, rare diseases, and dietary supplements. She also led NIH's burgeoning efforts to design and implement a program in Portfolio Analysis to inform Agency investments. Dr. Skirboll was trained in Pharmacology and Neuroscience. She completed her Ph.D. at Georgetown University Medical School, followed by post-doctoral work and research positions at Yale University, the Karolinska Institute (Sweden), and the National Institute of Mental Health. She is the author of more than 70 peer reviewed scientific publications.

PAMELA TENAERTS, M.D., M.B.A., is Executive Director of the Clinical Trials Transformation Initiative (CTTI). Dr. Tenaerts works closely with the CTTI Executive Committee to develop and implement strategies to accomplish CTTI's mission. She provides senior level oversight of the day-to- day operations of CTTI and orchestrates efforts to effectively engage all interested stakeholders to improve the conduct of clinical trials. She is a member of PCORI's CTAP expert post-award subcommittee and MIT's Collaborative Initiatives Clinical Trials Process Expert Advisory Board, a Member of the Advisory Council North America, DIA. With more than 20 years' experience in the conduct of clinical trials across a number of sectors, she practiced medicine in both the emergency department and private practice setting for several years before embarking on a career in research. Most recently Dr. Tenaerts oversaw European operations for CoAxia, a medical device company focused on cerebral ischemia. She received her MD from Catholic University of Leuven, Belgium, and a M.B.A. from the University of South Florida. She speaks five languages and has obtained Six Sigma Green Belt certification.

JOHN WAGNER, M.D., PH.D., received his M.D. from Stanford University School of Medicine and Ph.D. from the Johns Hopkins University School of Medicine. Postgraduate training included Internal Medicine Internship and Residency, as well as Molecular and Clinical Pharmacology Postdoctoral Fellowships at Stanford. He began his professional career in academic research on Cystic Fibrosis and has continued in the pharmaceutical industry, largely in the context of drug development as well as biomarkers. Currently, Dr. Wagner is Senior Vice President and Head, Clinical and Translational Sciences, Takeda Pharmaceutical International. He is also President, the American Society for Clinical Pharmacology and Therapeutics (ASCPT), a premier translational medicine and clinical pharmacology scientific association in the US. Dr. Wagner is also on the adjunct faculty at Harvard - Massachusetts Institute of Technology. Previously, he was Senior Consultant to the Institute of Medicine, Vice President and Head, Early Development Pipeline and Projects and Head, Global Project Management at Merck & Co., Inc., co-chair of Merck's early development governance committee, Vice President and Head, Clinical Pharmacology, at Merck & Co., Inc and Acting Modeling and Simulation Integrator, Strategically Integrated Modeling and Simulation. He is the past chair of the PhRMA Clinical Pharmacology Technical Group, past chair of the adiponectin work group for the Biomarkers Consortium, past committee member of the National Academies Institute of Medicine Committee on Qualification of Biomarkers and Surrogate Endpoints in Chronic Disease, and current full member of the National Academies Institute of Medicine National Cancer Policy Forum. Over 200 peer-reviewed publications detail work across a variety of therapeutic areas and disciplines.

RICHARD (DICK) J. WILLKE, PH.D., M.A., is Chief Science Officer of the International Society for Pharmacoeconomics and Outcomes Research (ISPOR). Dr. Willke has more than 25 years of experience in the life sciences arena and has specialized in outcomes research in a succession of group leadership roles with Pfizer and its legacy companies. At ISPOR, Dr. Willke is responsible for designing and implementing strategic initiatives related to scientific research and content priorities that will advance the Society's mission of promoting health economics and outcomes research excellence to improve decision making for health globally. Previously, Dr. Willke was Vice President, Outcomes & Evidence Cluster Lead at Pfizer for its Global Health & Value division. He has also served in a number of leadership roles with affiliated organizations, including the Chair of ISPOR Institutional Council (2010), ISPOR Board of Directors (2007-2009), and Chair of the PhRMA Health Outcomes Committee (2002-2004).Prior to joining industry, Dr. Willke served as Department Director in the Center of Health Policy Research at the American Medical Association and held research and teaching positions at The Ohio State University. Dr. Willke earned a Ph.D. and M.A. in economics from Johns Hopkins University. He has authored more than 80 scholarly publications that examine the science and methodologies of health economics and outcomes research.

National Academies of Sciences, Engineering, and 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

LANDSCAPE ANALYSIS

OVERVIEW

The purpose of this landscape analysis is to serve as background and reference for the National Academies of Sciences, Engineering, and Medicine's (National Academies') Forum on Drug Discovery, Development, and Translation's (Forum's) workshop, *Advancing the Science of Patient Input in Medical Product R&D – Towards a Research Agenda*, held on May 9, 2018.

This landscape analysis is a non-exhaustive list of initiatives and case studies that center or touch on the science of patient input. This analysis has been curated (with permission) from robust landscape analyses already completed by Patient Focused Medicines Development and FasterCures and supplemented by a stakeholder survey conducted in July 2017 and Forum staff research.

Forum staff have made every effort to ensure the content in this landscape analysis is updated. However, we urge you to visit the website listed for each initiative to find the most up-to-date information.

Please send any additions, updates, or revisions to drugforum@nas.edu.

DISCLAIMER: *This landscape analysis was prepared by staff on the Forum on Drug Discovery, Development, and Translation (Forum) at the National Academies of Sciences, Engineering, and Medicine (National Academies) for informational purposes only. It has not been reviewed and should not be cited or quoted, as the views expressed do not necessarily reflect the views of the National Academies or the Forum.*

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Alliance for Clinical Research Excellence and Safety (ACRES)
Patient Empowerment Initiative

Background

Many patient-related initiatives are currently underway across the enterprise. However, there *are still no universally accepted principles or definitions of patient involvement* in either research or healthcare (whether patient-empowerment, patient-centered, patient engagement, patient centricity, patient voice). This includes distinctions between patients as end users of product vs. patients as subjects in clinical trials vs. patients as customers of clinical care.

Similarly, there is *no clinical research or healthcare-wide mechanism for bringing the stakeholders together to work out how best to incorporate the perspectives, needs and voice of patients*, how best to involve patients in research, how to measure the impact of the empowerment and engagement of patients in research and care.

Finally, while the multiplicity of patient centricity efforts is heartening, *doing so without integrating these efforts into the larger biomedical and healthcare environment results in the siloed environment* so detrimental to progress.

ACRES Role in Patient Empowerment

ACRES, as a 'system of systems' or meta-systems-based organization, recognizes the need to integrate patient centricity efforts across the research and healthcare environment. Also, focusing on the patient alone, without examining the needs and priorities of the other stakeholders within the patient research and care eco-system, will make embedding patient needs permanently into research and care impossible.

Given ACRES history of building alliances—bringing together all of the stakeholders in the clinical research enterprise to address system needs—ACRES PEI is optimally positioned to create a "home" for comprehensive multi-stakeholder dialogue on these issues.

ACRES Patient Empowerment Efforts

ACRES efforts focus on:

- The comprehensive priorities and needs of patients as well as other stakeholders in clinical research and healthcare
- Defining the ideal principles regarding and role(s) for patients in both
- Development of implementable actions inherent in the patient and other stakeholder roles.
- Testing the feasibility of solutions proposed by various stakeholders.
- Facilitation of consensus with respect to implementable solutions.

Growing out of these discussions, ACRES PEI can serve as a mechanism for collecting, synthesizing and testing best practice methods of involving patients in clinical research.

Given ACRES' matrix approach to developing and connecting all elements of a clinical research system, thorny multi-stakeholder issues that heavily impact patient engagement, such as ethics, technology, or protocol design in research, can be recognized and discussed.

Source: *http://www.acresglobal.net/about-us/initiatives/patient-empowerment-initiative-pei*

Association of Clinical Research Organizations (ACRO)
Patient Centered Drug Development: Engage

About Initiative

ACRO are a step removed from patient engagement as the trade association. Their interaction is with members who have a strategy to go about this. ACRO is looking at standardization; focus at the FDA, and supporting policy; all of a lot of interest to its membership. At a higher level, the association is promoting the concept of patient focused drug development (PFDD). Through video and legislative efforts to look at how we can better

engage patients from the initial design of a clinical trial, recruitment of clinical trials and through to development. Issues our members are focused on. We are addressing public awareness and public affairs and it is a theme that runs through our work. We have a video series on PFDD. One area that has really emerged is using social media for patient recruitment. We have asked the FDA to provide guidance on that issue. On how electronic health records can be mined as a source for patient recruitment. Another area is the use of wearable technologies to make drug development easier and more convenient for patients to access. But there is a need for guidance and standardization.

Initiative Goals

Our main goal is to support the membership.

Problems Addressed by Initiative

Supporting its membership of Contract Research Organizations (CROs) in advocating safe, ethical, high-quality medical research so patients can benefit from the development of new treatments and therapies. This includes ensuring members have clear guidance around how they can use new technologies to make patient engagement as accessible, representative and convenient as possible for the patient.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-centered-drug-development-engage

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ALS Association
Patient-Focused Guidance for ALS Drug Development

Resources

Guidance Document (May 2, 2016) | Overview | Timeline | Infographic | Participant List | FAQs

Overview

The resources made possible by the ALS Ice Bucket Challenge have enabled the ALS community to join together to create the first patient-focused Guidance for ALS drug development that will be submitted to the FDA. The Guidance will serve as a roadmap to help industry navigate the drug development process and provide the FDA with an ALS community-centered view of how the Agency should approach therapies for ALS. The goals are to increase the efficiency, predictability and speed of the drug development process, including clinical trials, and lead to a more effective and earlier assessment of efficacy. This will speed access, reduce costs, help ensure resources are most effectively utilized and incentivize industry to enter the ALS market and develop new treatments for ALS.

Additional information about the guidance, including the current draft, is available in the links below. The Guidance Steering Committee encourages all stakeholders to learn more about the guidance project, review the draft ALS drug development Guidance and provide comments.

Source: http://www.alsa.org/advocacy/fda/

American Institutes for Research (AIR)
Center for Patient and Consumer Engagement

Resources

- Patient & Family Engagement Framework
- *Patient And Family Engagement: A Framework For Understanding The Elements And Developing Interventions And Policies* (Carman et al., Health Affairs, February 2013)
- Roadmap to Patient and Family Engagement in Healthcare Practice and Research

Overview

Active partnerships with patients and their families are an essential element of creating patient-centered health care. Engaging these key stakeholders, however, requires a firm understanding of the needs, issues, and strategies that ensure success. Health care researchers, clinicians, administrators, funders, and federal and state government agencies across the nation use AIR's cutting-edge research and tools, such as the Guide to Support Patient and Family Engagement in Hospital Quality and Safety and our Roadmap to Patient and Family Engagement in Healthcare Practice and Research, to effectively engage patients, caregivers, families, and health consumers to reduce costs, improve outcomes, and increase quality and safety.

AIR's framework offers a practical model for the varying levels of and contributors to engagement. This framework, along with a growing body of resources and ideas available at the Center for Patient & Consumer Engagement, guides the rapid evolution of patient-centered health care, prevention, and policy.

Source: http://www.air.org/topic/health/patient-and-consumer-engagement

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Amyloidosis Research Consortium (ARC)
Changing the Amyloidosis Drug Development Pathway: Guidance for the FDA

Resources

- Enhancing the Amyloidosis Drug Development Pathway: Guidance for More Efficient and Successful Programs (November 16, 2015 meeting agenda)
- What amyloidosis patients hope to accomplish by writing draft guidance (Dec 7, 2015)
- ARC, FDA continue exchanging ideas on amyloidosis (June 7, 2016)

Overview

Under the guidance of the U.S. Food & Drug Administration (FDA), the ARC is convening a public patient and expert forum to stimulate increased understanding of amyloidosis.

Building on ARC's successful scientific roundtable meeting in mid-September 2015, the policy forum is the second step in the framing of draft guidance on amyloidosis drug development to be submitted for review to the FDA. ARC will ensure that all discussions and related policies are not only grounded in science but also reflect innovative approaches that address patients' and families' experiences and needs.

Source: http://www.arci.org/fda-page/

AstraZeneca
Lupus Patient Protocol Simulation

About Initiative

The initiative was to simulate the first two visits, screening and first drug visit, of a lupus clinical study. It brought in a number of patients, at two different sites, to go through the simulated steps at the stage before protocol completion. As it was an infusion study, steps included sitting in the infusion chair, for example, but no invasive activities took place. This allowed the study to change based on patients' input.

Initiative Goals

The goals of the initiative were to understand how the patients feel about this kind of clinical trial and make improvements based on this insight.

Problems Addressed by Initiative

The aims of the initiative were to make our study simpler and easier for patients and the trial sites, and secondly, to aspire to make the recruitment of traditionally hard to recruit lupus studies easier.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/lupus-patient-protocol-simulation

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AstraZeneca
Patient Partnership Program

What is the Patient Partnership Program (PPP)?

The Patient Partnership Program is a global forum made up of a small group of members from AstraZeneca teams and patients/caregivers with personal or professional experience in a given disease area whose goal is to learn from each other and co-create patient centric medicines and solutions throughout drug development.

Who can participate in the Patient Partnership Program?

Although it will eventually cover many disease areas, the PPP currently includes eligible individuals living with moderate to severe asthma, moderate to severe lupus, ovarian cancer, or lung cancer, as well as current or past caregivers for ovarian cancer or lung cancer patients. PPP partners must also have personal or professional experience in such areas as medicine, scientific research, health marketing, patient advocacy/government affairs, health education, market access, drug safety and/or digital health. The program also requires advisors to be:

- 18 years or older
- Fluent in English (written and spoken)
- Resident of either Canada, Germany, United Kingdom, USA, Spain, Belgium, Italy, China, South Korea, Japan, or Australia.

What do Patient Partnership Program Partners do?

Based upon their area(s) of functional expertise and their personal health experiences, PPP partners will share their opinions, ideas, perspectives and insights with AZ teams about projects in a number of different areas such as clinical development, marketing or disease education. Meetings may take place via telephone, email, video conference and/or in-person.

Source: https://www.azpatientpartners.com/home.html

Bayer
Patient Insights and Engagement Team

About Initiative

Patient Insights and Engagement team at Bayer is a cross-functional group established to connect, exchange and collaborate on this topic across the company.

Initiative Goals

1. Build internal and external knowledge on patient engagement
2. Learn through pilots in research & development
3. Inspire colleagues by connecting what we do to real people
4. Create a platform to enable exchange and collaboration

Problems Addressed by Initiative

Helps to build a coordinated and concerted effort around this topic.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/pie-team

Biotechnology Innovation Organization (BIO) & Parent Project Muscular Dystrophy (PPMD)
Best Practices for the Development of Disease-Specific Patient Preference Studies

Resources

- Key Considerations for Developing and Integrating Patient Perspectives in Drug Development: Examination of the Duchenne Case Study (Report; June 2016)
- PPMD & BIO Release New Report on the Development of Patient Preference Studies (June 8, 2016)
- How BIO and PPMD plan to create a blueprint for patient preference studies (BioCentury; Dec 22, 2015)
- BIO and PPMD Launch Initiative to Share Best Practices on Patient Preference Studies (PPMD; Dec 9, 2015)

Overview

BIO and PPMD teamed up to share best practices for the development of disease-specific patient preference studies based off of the PPMD experience. 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.

Source: ***https://www.bio.org/key-considerations-developing-and-integrating-patient-perspectives-drug-development-examination***

Biotechnology Innovation Organization (BIO)
Lifecycle Approach to FDA's Structured Benefit-Risk Assessment (White Paper)

This White Paper was developed by the Structured Benefit-Risk Working Group of BIO. The paper identifies considerations for biopharmaceutical companies who choose to use the U.S. Food and Drug Administration's (FDA's) Structured Benefit-Risk Assessment Framework earlier and more broadly throughout a product's lifecycle as a mechanism to both solicit patient perspectives on areas of unmet medical need and assess patient preferences, and to align with FDA on key benefit-risk considerations. (June 17, 2015).

Source: ***https://www.bio.org/FDAwhitepaper***

British Medical Journal (BMJ)
Partnering with Patients

About Initiative

The BMJ launched an innovative strategy to promote patient partnership in 2014. It took this step because it sees partnering with patients, their families, caregivers and support communities, and the public as an ethical imperative, which is essential to improving the quality, safety, value, and sustainability of health systems. The strategy has seen the journal move to co-produce its content with patients and advancing international debate on how to embed meaningful partnership with patients in clinical practice, service delivery, research, education, and policy. The strategy was drawn up with and continues to be informed by a dedicated international patient advisory panel. The internal changes that The BMJ have introduced are making patient partnership integral to the way the journal works and thinks, as well as something we advocate for in healthcare. Steps taken include: asking authors of educational articles to co-produce their papers with patients; authors of research papers are required to document how they involved patients in setting the research question, the outcome measures, the design and implementation of the study, and the dissemination of its results; embedding patient review of papers alongside our standard peer review processes. To do this we have established a database of patients, patients advocates, and carers to comment on papers. We welcome readers help to build this further by extending this invitation to patients; appointing patients and patient advocates to our editorial board and a patient editor to bring the patients' perspective to discussions conducted by internal decision making committees; inviting blogs from patients and publishing a patient led series called *What your patient is thinking*.

Initiative Goals

Making sure that we have quality research and support doctors who are our main readers, to become better, more informed doctors. We aim to train doctors to become aware and applicable to patients' needs.

Problems Addressed by Initiative

Making medicines more relevant to those people who actually need it. Too many researchers and clinicians are working on medicines development with no actual relation or experience with the condition. This then leads to them looking and investigating the wrong thing, which actually does not apply to patients' needs and priorities. If researchers were to ask patients before designing the research, what it is they had concerns with and wanted the study to address, this would lead to more relevant outcomes from research studies.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development:
https://involvement-mapping.patientfocusedmedicine.org/initiatives/partnering-with-patients

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Community and Patient Preference Research (CaPPRe)
Patient Voice Initiative

About Initiative

Incorporating the patient perspective on the value of medicines into health technology assessments (HTA) is becoming increasingly more important as acknowledged by several prominent HTA agencies. NICE in the UK has employed several measures, including a Citizen's Council. Canada, particularly in oncology, has a formalized process through which patient input on drug reviews and feedback on recommendations is obtained to ensure patients' experiences (both good and bad) of living with cancer and undergoing treatment are routinely considered.

In Australia, there is a consumer representative on the PBAC and patients can provide written input during the assessment process, although the process of how PBAC consider and incorporate this information is not transparent. There is a need for a more formalized framework for eliciting meaningful patient input and a more transparent process for how that input is incorporated into the decision-making process.

In the current process health outcomes are traditionally measured using patient reported outcomes (e.g. quality of life measures) and incorporated into a benefit assessment to determine the value of a health intervention. Patient preference research extends beyond health outcomes (endpoints) measured in clinical trials to include all aspects of treatment that are important to the patient.

Patient preference research methods are interested in measuring the values (needs / views) of patients with a condition. The goal is to explore how patients perceive treatments (both current and new treatments) and understand what is most important to these patients when evaluating treatments. Patient preference studies commonly use trade-off technique (such as discrete choice experiments / conjoint analysis) which directly measure the relative value of specific components of a treatment (e.g. Oral administration vs. Injection).

The Patient Voice Initiative began in 2015 when a group of stakeholders from industry, academia and patient groups came together to discuss methodologies and approaches for eliciting the patient perspective on the value of medicines. Following these meetings, a conference was organized aimed at increasing patient engagement in HTA in Australia. Following the conference, a steering committee was formed to action items generated from the workshops.

The steering committee is currently made up of a patient advocate, CaPPRe, three pharmaceutical companies and a patient advocacy organization. The meetings have been sponsored by nine different pharmaceutical companies.

Methodology

There were three meetings in 2016. One in Sydney; the objective of the day was to discuss ways of improving patient involvement in HTA processes that are used in Australia by bodies such as the PBAC (Pharmaceutical

Benefits Advisory Committee), MSAC (Medical Services Advisory Committee) and PLAC (Prostheses List Advisory Committee) for the reimbursement of new drugs, devices, procedures and prostheses. This included discussion on specific elements of the existing system e.g. patient experience in PBAC hearings. There was a follow up meeting in Melbourne to make it easier for patients and patient groups to be involved and another in Canberra.

The first meeting was divided into 2 parts:

- Firstly, a download of information, including:
  - Top line review of HTA in Australia and PBS processes and the PBAC
  - Presentation on consumer engagement in PBAC processes
  - Direct experiences from a patient with multiple myeloma and a patient with Cystic Fibrosis
- Secondly, a workshop facilitated by two patients on patient views of the current system and ideas on how best to improve patient involvement in the HTA process for listing of new drugs on the PBS. The ideas generated were then compared to ideas generated at the earlier meeting in Sydney, which took place in February 2016.

Patient engagement/input is not only limited to the regulatory or reimbursement stage. This approach can be applied across the drug development life-cycle, including eliciting patient preferences from the earliest stages of drug development and clinical trials that will drive treatment enhancement and alignment with patient values.

Initiative Goals

An educated consumer voice will help signal to Government, clinicians and industry the importance of consumer preferences Bringing the two worlds together will help strengthen the PBS for Australia.

Benefits to different stakeholders include:

Patients

- Gives patients a meaningful and transparent voice
- Patient feedback (not all patients are the same): Provides patients with feedback about their own preferences and how they compare to other patients with their condition
- Preferences are dynamic: show how preferences develop / change over time with experience, knowledge and at different stages of the illness

Government

- Provides a formal platform to allow patient input to assist in regulatory and reimbursement decisions

Pharmaceutical companies

- Treatment alignment – measures how well treatments align with patient values
- R & D – discover what new aspects of treatment patients would value the most

Physician / Patient alignment

- Do physicians and patients have the same preferences? Preference information can be used to guide the conversation between the physician and the patient.

Problems Addressed by Initiative

Often, PBAC submissions are already done deals by the time that consumers are aware of them. The Department of Health also does not systematically seek out consumer views – currently it is just a website and a form. Up until now, the committee has been dominated by clinicians.

There is a need for a more formalized framework for eliciting meaningful patient input and a more transparent process for how that input is incorporated into the decision-making process.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-voice-initiative

Cancer Voices NSW
Consumer Involvement In Research

About Initiative

Cancer Voices' Consumer Involvement in Research Program has nominated many trained consumers to research projects within around 25 research organizations to date. Researchers can request a suitable nominee via an electronic request form on our website. This year 77 consumers were matched to 47 cancer research projects and committees.

Participants who undertake training can join our "matching" data base. If you look at our website you will see the form which we ask cancer researchers to complete, so we have all necessary details about their project and consumer needs. We pass this information to those on the data base who we think would make a good match – e.g. who have experiences of the studied cancer, its treatment or fit the demographic. It is then up to the consumer to decide if they would like to help this project team. If they accept, we then connect the consumer to the researcher. Their privacy, beyond giving contact details to requesting researchers, is assured.

Initiative Goals

This program is a national leader in this area. This is largely because trainees are equipped to provide an informed, balanced consumer view to researchers and research funders.

Problems Addressed by Initiative

Cancer Voices NSW itself is the voice of people affected by cancer in our state, and began the generic cancer consumer movement in Australia 16 years ago.
Early this century it became evident that a number of our members had a keen interest in research.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/consumers-in-research and http://www.cancervoices.org.au/consumer-involvement-in-research-program/

Community Catalyst
Meaningful Consumer Engagement: A Toolkit for Plans, Provider Groups and Communities

Overview

As state demonstrations to improve and integrate care for Medicare-Medicaid enrollees (also known as "dual eligibles") move forward, health plans and provider groups (here, referred to collectively by the term "delivery systems") must employ meaningful consumer engagement strategies. Federal guidance from the Centers for Medicare and Medicaid Services calls upon states to ensure the voices of older adults, persons with disabilities, and their caregivers are heard in the design, implementation, and oversight of the demonstrations. Their voices are vital because Medicare-Medicaid enrollees have complex medical and social needs, as well as personal preferences, that all members of the delivery system need to understand and respect in order to truly provide person-centered care. Community Catalyst believes that consumer engagement, done well, fosters an atmosphere of active, ongoing collaboration and conversation that will benefit consumers and their caregivers, health plans and provider groups, and ultimately transform the health care delivery system. To ensure meaningful consumer engagement occurs, Community Catalyst created a Toolkit for delivery systems to use as they implement effective strategies of engagement.

Source: http://www.communitycatalyst.org/resources/tools/meaningful-consumer-engagement

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Critical Path Institute (C-Path)
Patient-Reported Outcome (PRO) Consortium

Resources: Introduction | Past Workshops | Collaborators | PRO Consortium Team

Overview

The PRO Consortium was formed in late 2008 by C-Path in cooperation with the U.S. Food and Drug Administration's (FDA) Center for Drug Evaluation and Research and the pharmaceutical industry, and formally launched in March 2009. The mission of the PRO Consortium is to establish and maintain a collaborative framework with appropriate stakeholders for the qualification of PRO measures and other clinical outcome assessment (COA) tools that will be publicly available for use in clinical trials where COA-based endpoints are used to support product labeling claims. The PRO Consortium's membership is comprised of pharmaceutical companies along with C-Path as the managing member. Patients, clinicians, measurement consultants, and representatives from the FDA and National Institutes of Health (NIH) provide critical advice and assistance to the PRO Consortium's Coordinating Committee and working groups.

Source: ***https://c-path.org/programs/pro/***

Clinical Trials Transformation Initiative (CTTI)
Patient Groups & Clinical Trials: Best Practices for Effective Engagement with Patient Groups around Clinical Trials

Resources

- Poster from ISPOR Meeting (May 23, 2016): Framework for Aligning Research Sponsors and Patient Groups on Methods for Engagement
- Patient Engagement in Neurological Clinical Trials Design: A Conference Summary (Clin Trans Sci, Dec 2015)
- Patient Engagement Practices in Clinical Research among Patient Groups, Industry, and Academia in the United States: A Survey (Oct 14, 2015, PLoS ONE)
- Webinar: Presenting CTTI Recommendations (Oct 7, 2015)
- Summary of CTTI Recommendations: Effective Engagement With Patient Groups Around Clinical Trials (Oct 7, 2015)
- CTTI Recommendations: Effective Engagement With Patient Groups Around Clinical Trials (Oct 7, 2015)
- Patient Groups and Clinical Trials Expert Meeting (Jan 21–22, 2015)

Overview

While patient groups have increasingly been recognized as equal partners in the clinical trials enterprise, there has been a lack of understanding about how patients and research sponsors can best work together to improve the medical product development process. CTTI's recommendations highlight important roles for patient groups throughout all stages of the process and provide best practices that can be applied by sponsors, patient groups, and other stakeholders to ensure the relationship is mutually beneficial.

To further support the benefits of involving patients in the medical product development process, CTTI published a financial model that can be used to estimate the value of patient engagement on key business drivers such as cost, risk, revenue, and time. It shows that patient engagement can have a considerable impact on the bottom line. Having a clear example of how to calculate a return on investment is expected to support the broader uptake of patient engagement.

Through this project, CTTI continues to explore ways to support effective engagement of patient groups, leading to higher quality, patient-focused, and efficient clinical trials.

Source: ***http://www.ctti-clinicaltrials.org/what-we-do/investigational-plan/patient-groups***

Drug Information Association (DIA)
DIA Insights: Patient Engagement

Overview

DIA believes patients should be at the epicenter of the drug development life cycle, and for more than 15 years has pioneered efforts to advocate for the patient voice and discuss future patient engagement efforts across the health care continuum.

Beginning with the first patient fellowship program at the European Annual Meeting more than ten years ago, DIA has led and supported various patient engagement initiatives including: the Clinical Trial Transformation Initiative (CTTI), European Patient Academy on Therapeutic Innovation (EUPATI), Patient-Centered Outcomes Research Institute (PCORI) sponsored patient-engagement workshop, and most recently, the DIA-Tufts Center for the Study of Drug Development (CSDD) patient-engagement research project.

Reflecting growing stakeholder agreement that patient-centricity is key to meeting patient needs and improving patient outcomes, industry, in particular, is looking at how to operationalize patient involvement. Despite an increase in the number and type of efforts, little published data exist to quantify the benefits of patient-centric initiatives in terms of outcomes and resource utilization. DIA recognized that more data is needed to demonstrate value and guide decision-making among biopharmaceutical companies and other industry stakeholders, and so it initiated a research project with Tufts CSDD.

As a result of these initiatives, DIA is uniquely positioned to ask and help answer important questions around this effort such as: What is meaningful patient engagement? How can patients, industry, regulatory, health care providers, and payers collaborate on innovative and affordable solutions? How can we quantify the benefits of patient-centricity in drug development? What are best practices in patient-centricity? What capacity is required for patients to be meaningfully involved?

DIA continues to examine the current and future impact of patient engagement efforts on patients' life goals, quality of life, and desired clinical outcomes. The knowledge shared provides actionable insights designed to increase the knowledge and understanding of issues central to the promotion of patient-centered health care, biomedical research, and therapeutic development.

Source: ***http://www.diaglobal.org/en/resources/how-we-think/patient-engagement***

Drug Information Association (DIA) & Clinical Trials Transformation Initiative (CTTI)
Patient Engagement Practices in Clinical Research Among Patient Groups, Industry, and Academia in the United States: A Survey

Overview

DIA and CTTI conducted a joint survey in 2014 with multiple stakeholders to:

- Assess types of relevant patient organizations by querying a representative sample across disease states to highlight distinctions among their missions, reach, infrastructures, governance models, and interest and engagement in clinical trials
- Identify current research sponsor and investigator practices for engaging with patient groups (PGs), and practices used by patient groups to engage with research sponsors and investigators around clinical trials
- Explore successes and failures to identify models of engagement with PGs that have led to more quality-driven and efficient trials
- Formulate recommendations and opportunities for implementation of best practices with PGs, academia, and industry that will lead to more efficient and successful clinical trials

The survey elicited feedback from 244 respondents and examined current practices and perceptions among the different stakeholders about the value of, and barriers to, successful patient group engagement in clinical trials. The raw data from the survey has been summarized and published:

Publication: Sophia K. Smith , Wendy Selig, Matthew Harker, Jamie N. Roberts, Sharon Hesterlee, David Leventhal, Richard Klein, Bray Patrick-Lake, and Amy P. Abernethy. Patient Engagement Practices in Clinical Research among Patient Groups, Industry, and Academia in the United States: A Survey. PLOS ONE. 2015.

Source: http://journals.plos.org/plosone/article?id=10.1371/journal.pone.0140232

Drug Information Association (DIA) & Patient-Centered Outcomes Research Institute (PCORI)
Visual Model of Patient Engagement in Benefit-Risk Assessment through the Medical Product Life Cycle

Resources

- Visual Model of Patient Engagement in Benefit-Risk Assessment (Infographic)
- Patient Engagement at a Tipping Point—The Need for Cultural Change Across Patient, Sponsor, and Regulator Stakeholders: Insights From the DIA Conference, ''Patient Engagement in Benefit Risk Assessment Throughout the Life Cycle of Medical Products". (September 2016)
- Conference Program
- Conference Short Summary
- Dissemination Plan

Overview

Benefit-risk assessments of medical products, the weighing of benefits against the risks for harm from using a product for treatment, are the foundation for making decisions about the product throughout its life cycle. For a medical product to truly meet the needs of the patient for whom it is intended, its benefits, risks, and uncertainties must be balanced in the context of the patient's perspective, requiring both technical assessments of the evidence base and stakeholders' value judgments about relative importance. Because patients are the beneficiaries of effective treatments and also bear the risks associated with those treatments, their perspectives and judgments about value and relative importance are at the heart of this process.

In many cases, patients or patient partners (patients, family, caregivers, advocates, and patient organizations) are not engaged effectively or at all in the benefit-risk assessment process, especially in the early development stages of medical products. This will change only with widespread awareness of the importance of patient engagement and with collaboration among all research stakeholders, including patients, to develop and adopt more effective engagement tools and processes.

The conference "Patient Engagement in Benefit-Risk Assessment Throughout the Life Cycle of Medical Products" was targeted to patient partners, industry and academic medical researchers, and regulators, and addressed the important challenge of how and when to best engage patient partners in benefit-risk assessment. The overall goals of the conference were to:

1. Raise awareness of these stakeholders of the importance of patient engagement in benefit-risk assessment throughout the life cycle of the medical product
2. Involve the stakeholders in sharing of existing approaches, identifying implementation challenges and gaps or needs for new information, and best practices
3. Identify recommended next steps for addressing the identified gaps to inform stakeholder actions, including current legislative and regulatory processes

The outputs from this project include the conference summary and briefing materials, including a visual model, that can be disseminated for education and awareness building, practice assessment, problem-solving, and system improvement, and used to inform the development of new knowledge, best practices, and guidelines to address gaps in this area. A manuscript on cultural transitions necessary to effect change among all stakeholders has been published.

Project collaborators include Program Committee representatives from Amgen; AstraZeneca; Duke Clinical Research Institute; Eli Lilly and Company; FasterCures; GlaxoSmithKline; Health Canada; Janssen Research & Development; JDRF; Johns Hopkins Bloomberg School of Public Health; Merck Research Laboratories; Parent Project Muscular Dystrophy (PPMD); T1D Exchange; and the University of Colorado/Colorado School of Public Health. Graphic facilitation was provided by Visual Ink, LLC.

Source: ***http://www.diaglobal.org/en/resources/tools-and-downloads/dia-pcori***

Drug Information Association (DIA) & Tufts Center for the Study of Drug Development (CSDD)
Study of Patient-Centric Initiatives in Drug Development

Resources

- Study Results
- DIA Releases Results of Patient Engagement in Drug Development Study (Nov 14, 2016)
- DIA's Considerations Guide to Implementing Patient-Centric Initiatives in Health Care Product Development

Overview

A priority theme for DIA stakeholders increasingly revolves around the integral role patients play in influencing treatments being developed to address unmet medical needs. While general consensus accepts that patient engagement is beneficial, significant barriers to patient-centricity exist that could potentially be overcome by quantifying the value of patient input and defining a process to guide organizations in the 'how to' of patient-centricity.

After committing to explore the question - How can we quantify the benefit of patient-centricity in drug development? - DIA selected DIA Fellow Dr. Ken Getz and the Tufts CSDD to partner with us to on the first phase of a research project that included working with group participants from 17 companies, including traditional pharmaceutical companies, small biotechnology firms, CROs, and other stakeholders.

The objectives of phase 1 of the study were to:

- Quantify the impact of patient-centric initiatives using Return on Engagement metrics looking at retrospective data and develop a metrics toolkit
- Collect real examples of measurable benefit to drug development from patient involvement
- Catalog patient-centric initiatives and assess adoption
- Characterize implementation and management models
- Identify and assess current guidances and frameworks on patient-centricity

Source: ***http://www.diaglobal.org/en/resources/tools-and-downloads/dia-tufts-csdd-study***

Envision Pharma Group
First Systematic Literature Review, Planned and Conducted with Patient Experts, on Patient Involvement in Preparing Clinical Trial Peer-Reviewed Publications or Results Summaries

About Initiative

Patient involvement is being encouraged throughout the development lifecycle of new medicines and devices. Many stakeholders (eg, patients, carers, regulators, payers, drug and device companies) have welcomed patient involvement as an important and fundamental change in the development lifecycle, and have promoted the potential benefits that meaningful, transparent, and ethical interactions with patients could bring. As with any change, however, research should be conducted to ensure the potential benefits and harms of patient involvement are understood, and that evidence-based best practices can be identified.

Compared with research on patient involvement in the clinical trial process, there appears to have been relatively limited research on patient involvement in peer-reviewed publication process. Publications can affect patient care and we have shown that patients are engaging with the peer-reviewed literature. Consistent with this interest from patients, medical journals are striving to facilitate greater patient involvement in the peer-reviewed publication ecosystem (eg, as authors, peer-reviewers, readers). The extent of published evidence on patient involvement in peer-reviewed publications, however, is not known.

In addition to sharing clinical trial results through the peer-reviewed publications, results can also be shared through clinical trial results summaries. The forthcoming regulatory requirement in Europe to provide plain language clinical trial results summaries has driven strong interest in this method of results sharing. The extent of published evidence on patient involvement in clinical trial results summaries, however, is not known.

The systematic literature review is directed toward audiences who want to know the size and quality of the evidence base that exists to guide patient involvement in peer-reviewed publications and clinical trial results summaries.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/

European Forum for Good Clinical Practice (EFGCP)
Patients' Road Map to Treatment

About Initiative

The European Forum for Good Clinical Practice (EFGCP) and The European Genetic Alliances' Network (EGAN) have created a common Working Party aiming to drive all elements in the clinical drug development process involving patients' interests.

Vision: To strengthen patients' possibilities to impact access to efficient and safe new treatments.

Mission: To support patient organizations' contribution to the European aim of faster development of efficient and safe new treatments by providing information, know-how, skills and funding for patients and patient organizations on clinical trials organization, risks and benefits. On areas of opportunities for patients' influence on the clinical development process, on optimization of communication between patients, physicians, pharmaceutical industry and regulatory authorities on treatments' benefits and risks. What are the Working Party activities?

Development of strategies and action plans to support the "Innovative Medicines Initiative" and other organizations' initiatives to improve the efficiency of drug development; Information to patients on clinical trials to improve patients' participation in clinical trials; Organization of workshops and seminars on relevant topics ; Support to EFGCP and EPPOSI workshops and conferences with topics, speakers and program chairs.

Preparation of grant application to the 7th Research Framework Program: TITLE and execution of the program in case the grant would we provided . Preparation of books, handbooks, articles and brochures in collaboration with different support partners like: "It is my life" "Report on EFGCP/EGAN Workshop at the EFGCP Annual Conference 2006". "Patients as partners in the drug development process" "Book on Biobanking" How is the Working Party organized? Two Co-Chairs, one from EGAN and one from EFGCP, are chairing the Working Party consisting of representatives from interested organizations like different patient organizations, industry associations, pharmaceutical companies, physician organizations, academic institutions, health authorities, ethics committees, etc. In face-to-face meetings, tele-conferences and per e-mail the different projects are discussed, designed and planned, priorities are set, roles and responsibilities distributed, the execution supervised and the final "product" discussed, agreed and disseminated. The representatives take the responsibility to discuss and comment the Working Party's activities and products internally in their organization.

Initiative Goals

The goal is to strengthen patients' possibilities to impact and be involved in clinical trials; as well as support patients' organizations' contribution to faster development of new medicines and treatments.

Problems Addressed by Initiative

The problem this initiative addresses is the lack of opportunities for patients' engagement, we try to ease and maximize patients access as equal partners in the process of medicines development. It also addresses the lack of communication between stakeholders and the lack of platforms for stakeholders to meet and discuss potential issues, solutions and share ideas.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/patients-road-map-to-treatment

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European Organization for the Research and Treatment of Cancer (EORTC)
Patient Representative Concept Review

About Initiative

In 2016, because we were very satisfied with the way in which our patient review programs were going, we began to pilot patient representative reviews of study concepts. We have now had seven concepts reviewed by patient representatives. This has been much more challenging as it has required patient representatives to have a much greater understanding of some of the processes involved. We did a survey to test how understandable the documents are. We also started this pilot in the UK as we knew that reviewers were more mature, well trained and had already proven their value. We have since received valuable reviews of all kinds of research concepts for clinical trials. This process is important for visibility of issues, it has already flagged patient opinion that we were not aware of internally. We are not trying to enlarge this program into other countries and engage other nations and cultures. Patient organizations want to be involved in discussions for concepts. We are in the process of an internal debrief to fine tune this process. In the near future we plan to move this to step three, to look at the protocols from the perspective of clinical trials being acceptable and convenient for patients - not a full protocol review, but those that are relevant and key to patient experience. This will come in the course of this year. We also plan to involve patients deeper in our organizational oversight and governance, this was decided at the beginning of 2016. EORTC is a stakeholder-run organization focusing on clinical trials in oncology; all different types of cancer, internationally. This approach brings challenges to patient engagement as it needs to build relationships with a number of different patient organizations across cancer types and internationally. These relationships are maintained through regular contact, speaking opportunities and newsletters that are sent to patient organizations for them to digest to their members. Patient organizations are also including in steering committees for some clinical trials, who represent a European voice, which can be difficult to find. We are increasing the number of strategic programs that have patient involvement, for example, a program called Spectre. We also provide patient information and educational tools and brochures to guide patient advocates through clinical trials, explaining randomization for example. All patient information sheets go through a review panel with patient representation. These exists in English, Dutch, French and Polish due to patient demand. Since 2012 we have also piloted patient involvement in clinical trial protocols at different levels. We began this in the UK as consumer involvement was much more developed than in some other countries - it was easier to find patients who have relevant experience. We also widened this to France. We involved patients in the design of patient information. We then asked patient representatives to review the final document once the protocol was designed and approved.

Initiative Goals

To increasingly involve the patient voice in clinical trial research concepts to give greater perspective.

Problems Addressed by Initiative

Helping to increase the visibility of issues and challenges from a patient representative perspective when designing clinical trial concepts. Increasing the role of patient engagement beyond patient information reviews.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-representative-concept-review

European Medicines Agency (EMA)
Framework of Interaction

About Initiative

Introduction

Patient involvement in EMA activities is well-established and, where appropriate, patients are involved systematically in many different areas of the Agency's work. This involvement contributes not only to increased transparency and trust in the regulatory processes but also ensures that in addition to the medical and scientific aspects of assessment, a real-life perspective of living with the disease is also considered throughout the medicines lifecycle.

Their involvement in every aspect of the medicines lifecycle is not only important in terms of numbers observed but also in the added-value of their contributions, the support they receive and the impact of their input on the regulatory processes. In 2015, patients and consumers were involved in a total of 743 occasions in EMA activities.

Framework of Interaction

The framework for interaction between the EMA and patients, consumers and their organizations outlines the basis for involving patients and consumers in Agency activities. It forms the backbone for the collaboration between the EMA and these stakeholder groups and initiated the creation of a permanent platform for liaison; the Patients and Consumers Working Party (PCWP).

The framework emphasises the importance of regular interactions with patients and consumers to:

1. access real-life experiences of diseases, their management and the current use of medicines
2. determine how best to communicate with these stakeholder groups and to support their role in the safe use of medicines and,
3. to enhance their understanding of the role of the medicines regulatory network in the EU.

The Framework places an emphasis on Participation, Consultation and Information to ensure the active engagement of patients and consumers and to further build transparency and trust.

- Participation: More focus will be placed on the preferences of patients and consumers on benefits and risks, which are key areas where their experience brings a unique element to the evaluation of a specific medicine. Various methods exist to capture these preferences and values and several options are currently being explored.
- Consultation: The framework also emphasises the importance of listening to and consulting patients and consumers and their organizations in the development of plans and policies. To do this and to facilitate and encourage the flow of communication between the Agency and these groups as well as to assist the cascade of information within these groups, communication tools must be optimised.
- Information: As patients and consumers are included in many activities at the EMA, it is important to enhance their understanding of EMA's role within the EU regulatory network regarding development, evaluation, monitoring and provision of information on medicines.

The framework relies on 5 critical elements:

- A network of European patients and consumers' organizations for consistent and targeted interactions with organizations with a diverse range of expertise and interests.
- A platform for dialogue and exchange: EMA Working Party with Patients and Consumers' organizations
- A pool of individual patients acting as experts in their disease and its management
- Interaction between the network of European patients and consumers and the EU Regulatory Network particularly in the area of dissemination of information
- Capacity-building focusing on training and raising awareness about the work and the mandate of the EMA as well as the EU regulatory system.

Initiative Goals

The framework allows the Agency to build transparency and trust with patients' and consumers' communities through their active engagement (participation-consultation- information). In order to achieve this goal the framework aims at meeting the following specific objectives:

1. Facilitate participation of patients and consumers in benefit/risk evaluation and related activities, to capture patients' values and preferences and obtain information on the current use of medicines and their therapeutic environment; all along the lifecycle of the medicines, from early development throughout evaluation and post-marketing surveillance;
2. Ensure that patients, consumers and their representative organizations are listened to and consulted and where appropriate involved in the development of EMA policies and plans;
3. Enhance patients and consumers' organizations understanding of the mandate and role of the Agency and the EU Regulatory Network within the context of the development, evaluation, monitoring and provision of information on medicines;
4. Optimize communication tools (content and delivery) to facilitate and encourage the cascade of information to the constituencies of patients and consumers' organizations (i.e. to reach out to individual patients and consumers) with the aim of supporting their role in the safe and rational use of medicines.

Problems Addressed by Initiative

The interaction with patients, consumers and their representatives are also affected by time, budget and availability constraints on both sides: organizations and Agency. Streamlining the interactions and focusing on areas where mutual benefit can be anticipated are two underlining principles to consider when implementing the framework.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: ***https://involvement-mapping.patientfocusedmedicine.org/initiatives/framework-of-interaction*** ***and*** ***https://www.eupati.eu/advocacy/consultation/***

European Medicines EMA (EMA)
Patient and Consumer Working Party

About Initiative

In 2006, the EMA established a permanent "Patients and Consumers Working Party" (PCWP), which is a dedicated platform for exchange of information with the EMA and its scientific committees on matters of direct and indirect interest to patients in relation to medicines.

The PCWP meets four times a year, including joint meetings with its counterpart, the Working Party with Healthcare Professionals' Organizations (HCPWP), where issues of common interest are discussed.

The working party is an important platform for exchange between the EMA and patients' and consumers' organizations. Discussions occur on a wide-range of topics that are of direct or indirect interest to patients in relation to medicinal products. Through it the EMA will inform and obtain feedback and contribution on various initiatives. It includes a balanced representation of the different types of patients and consumers (such as general organizations representing patients, consumers or civil society, and organizations representing diseases within the mandatory scope of the centralized procedure for marketing authorization, as well as organizations representing special populations not well represented in medicines development such as older people and women, etc).

A maximum of 20 patients/consumers' organizations will be members. Members of the PCWP are selected from the list of eligible patients and consumers' organizations. If several organizations in the same area are eligible, EMA may select only one/some of them, as appropriate. Representatives of EMA human scientific committees are also members of the working party (each nominates one representative). Management Board observers and the European Commission are also invited to participate. Members of the PCWP will be nominated for a term of 3 years, after which the membership can be renewed.

The working party is mandated to monitor the progress of the interaction between the EMA and patients and consumers and their representative organizations. It also provides a forum to further identify gaps and priorities in the overall interaction.

As of 2016, there were 20 members and 16 alternates or observers. The PCWP co-chair, Kaisa Immonen (EPF) is also a patient representative and the EMA co-chair is Isabelle Moulon (EMA).

The representative of the organization has the responsibility to liaise with their organization as necessary in order to provide the position of the organization on the topics to be addressed. It is also their responsibility to inform their organization about the activities of the group.

Membership of the PCWP implies a commitment to participate actively in the work of the working party and to attend the meetings of the working party regularly. After a patients'/consumers' organization has presented its apologies 3 consecutive times, the membership will be revoked, and the EMA would consider participation of another organization.

Members who would like to bring additional participants with relevant experience for a specific topic should notify the EMA secretariat in advance of the meeting. Participation will be subject to the agreement of the Chairpersons.

The PCWP members do not get involved in medicine specific evaluations – for that patients/consumers/carers participate as individuals after completing a DOI and confidentiality agreement.

Initiative Goals

To ensure that patients are involved systematically in many different areas of the EMA's work. This involvement contributes not only to increased transparency and trust in the regulatory processes but also ensures that in addition to the medical and scientific aspects of assessment, a real-life perspective of living with the disease is also considered throughout the medicines lifecycle.

Problems Addressed by Initiative

The working party is established to provide recommendations to the EMA and its Human Scientific Committees on all matters of direct or indirect interest to patients in relation to medicinal products. Through it the EMA will inform and obtain feedback and contribution on various initiatives.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: ***https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-and-consumer-working-party***

European Medicines EMA (EMA)
Patient Membership in EMA Management Board and Scientific Committees

About Initiative

Patients involved in the EMA Management Board and the Scientific Committees serve to represent patients' organizations. These members are appointed by the European Commission in consultation with the European Parliament on the basis of their expertise. All members are required to have signed a Declaration of Interest and Confidentiality form in relation to their activities in the EMA.

Management Board: The Management Board is the EMA's integral governance body and includes two members representing patients' organizations. This group has a general responsibility for budgetary and planning matters, the appointment of the Executive Director and the monitoring of the EMA's performance. Other members include one representative of each Member State, two representatives of the Commission, two representatives of the European Parliament, one representative of doctors' organizations and one representative of veterinarians' organizations. Members are appointed for a term of three years, which may be renewed. General meetings are held twice a year.

Scientific Committees: There are seven scientific committees for human medicines at the EMA and patients are full voting members of four of these. They carry out the EMA's scientific assessments. In this context they represent patients or patients' organizations. Activities performed by patients' representatives in these committees include orphan designation of medicinal products, assessment of paediatric investigation plans, classification of advanced therapies and assessment and monitoring of safety issues of medicines. For more information on patients specific role see http://www.ema.europa.eu/docs/en_GB/document_library/Other/2011/12/WC500119614.pdf (This is being updated)

The committees generally meet once per month at the EMA offices in London. Meetings usually last between two and four days. Between plenary meetings, committee members and EMA staff liaise to discuss matters related to ongoing assessments and outstanding issues. Where appropriate, a committee can invite a pharmaceutical company or other third party to present verbal evidence and answer any questions at a committee meeting, as set out in the committee's rules of procedure.

Initiative Goals

Patient involvement in EMA activities is now well-established and, where appropriate, patients are involved systematically in many different areas of the EMA's work. This involvement contributes not only to increased transparency and trust in the regulatory processes but also ensures that in addition to the medical and scientific aspects of assessment, a real-life perspective of living with the disease is also considered throughout the medicines lifecycle.

Problems Addressed by Initiative

Patient representation in management and regulatory decisions at a European level.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: ***https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-membership-in-ema-management-board-and-scientific-committees***

European Patients' Academy (EUPATI)

EUPATI Guidance for Patient Involvement

EUPATI has launched a public consultation in order to review its EUPATI guidances for patient involvement in the medicines research and development process. The guidances are provided as four distinct frameworks:

- patient involvement in industry-led R&D
- patient involvement in health technology assessment (HTA) bodies
- patient involvement in regulatory processes
- patient involvement in ethics committees

The frameworks have been developed in response to the increasing need to draw on the experience and specific knowledge of patients, and their day-to-day use of medicines, in order to improve medicines development and evaluation. The frameworks suggest approaches to allow structured interaction with patients, and thereby facilitate the exchange of information and constructive dialogue at national and European level where the views from users of medicines can and should be considered.

The four frameworks have already received feedback from a number of partners of the EUPATI consortium (including patient organizations), but further evaluation and feedback from patient organizations and other key stakeholders are essential in order to validate the guidance. The internal review resulted in some suggestions that require more discussion (applies namely to patient involvement with industry, but also with regulators). We specifically welcome further input on these by reviewers.

If you have significant expertise and knowledge in patient interaction with industry, HTA, ethics committees or regulatory bodies, please consider contributing to this review; please add your suggestions (in tracked changes) and comments directly on the documents after downloading them here:

1. Framework for patient involvement in regulatory processes (DOCX)
2. Framework for patient involvement in HTA (DOCX)
3. Framework for patient involvement in industry-led medicines R&D (DOCX)
4. Framework for patient involvement in ethics committees (DOCX)
5. Letter: EUPATI guidances: public consultation until September 15 (PDF)

Source: ***https://www.eupati.eu/advocacy/consultation/***

EveryLife Foundation

Incorporating the Patient Perspective in Rare Disease Drug Development (7th Annual Rare Disease Scientific Workshop)

Resources

- Patients As Critical Partners in Rare Disease Drug Development (Draft Framework)
- Patients as key partners in rare disease drug development (Workshop findings – Nat Rev Drug Disc; July 2016)

Overview

This framework was the result of the EveryLife Foundation's 2015 Rare Disease Scientific Workshop, which brought together leading stakeholders in the rare disease community to work towards meaningful incorporation of patient perspectives in the development of new treatments. Presentations and other materials from this workshop are available online.

In the article, the authors note that, "Rare disease patients and patient organizations are ready to play a larger role in drug development. It is now up to regulators and drug developers to fully engage patients and in doing so, improve the efficiency and effectiveness of development for the next generation of therapies for rare diseases."

Source: ***http://everylifefoundation.org/annual-rare-disease-scientific-workshop-7/***

FasterCures

Patients Count

- Study of Patient-Centric Initiatives in Drug DevelopmentScience of Patient Input Resources (Landscape analysis)
- Benefit-Risk Boot Camp – Session II: The Science of Eliciting Patient Preferences in Benefit-Risk
- Integrating Patient Perspective in to the Development of Value Frameworks (Mar 2016)
- Expanding the Science of Patient Input: Building Smarter Patient Registries (Nov 2015)
- From Anecdotal to Actionable: The Case for Patient Perspective Data (Nov 2015)

Through its Patients Count program, FasterCures aims to improve health by expanding opportunities for patients' perspectives to shape the processes by which new therapies are discovered, developed and delivered.

We do this by:

- expanding the capacity of academics, industry and patient organizations to build upon the science of patient input
- fostering patient-centric policies and practices that enable greater patient participation in decision-making
- advancing the dialogue on the benefits of patient-centricity across the medical product lifecycle

Source: ***http://www.fastercures.org/programs/patients-count/***

U.S. Food and Drug Administration (FDA)
21st Century Cures Act

Resources

- Plan for Issuance of Patient-Focused Drug Development Guidance Under 21st Century Cures Act Title III Section 3002 (May 2017)
- Proposed FDA Work Plan for 21st Century Cures Act Innovation Account Activities (June 9, 2017)
- FDA landing page for 21st Century Cures Act
- 21st Century Cures Act Deliverables by FDA
- Final Legislative Text (p. 131–137: TITLE III—DEVELOPMENT – Subtitle A—Patient-Focused Drug Development) and Summary of Title III – Subtitle A – Patient-Focused Drug Development

Overview

Sec. 3001. Patient Experience Data. Requires the FDA to include a statement regarding any patient experience data that was used at the time a drug is approved. Patient experience data includes data collected by any persons (including patients, family members, and caregivers of patients, patient advocacy organizations, disease research foundations, researchers, and drug manufacturers).

Sec. 3002. Patient-focused Drug Development Guidance. Requires the FDA to issue guidance regarding how to collect patient experience data. Such guidance documents shall address:

- Appropriate ways to collect data for use by the FDA for use in regulatory decisions;
- How patients wishing to propose draft guidance to FDA may submit such documents;
- How FDA will respond to patient experience data submissions to FDA;
- The format and content for patient experience data submissions to FDA; and
- How the FDA plans to use relevant patient experience data and related information when evaluating the risks and benefits of a drug.

Sec. 3003. Streamlining Patient Input. Exempts FDA from going through the Paperwork Reduction Act clearance process when requesting information from patients regarding their disease or treatments, allowing FDA to get more timely feedback from patients.

Sec. 3004. Report on Patient Experience Drug Development. Requires FDA to report on FDA's review of patient experience data and information on patient-focused drug development tools as part of approved drugs not later than June 1 of 2021, 2028, and 2031

Source:
https://www.fda.gov/RegulatoryInformation/LawsEnforcedbyFDA/SignificantAmendmentstotheFDCAct/21stCenturyCuresAct/default.htm

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U.S. Food and Drug Administration (FDA) – Office of the Commissioner
Patient Representative Program

Resources

- Role of the FDA Patient Representative
- Criteria for Becoming a FDA Patient Representative
- Conflict of Interest
- Frequently Asked Questions

Overview

The FDA Patient Representative Program is managed by the Office of Health and Constituent Affairs within the Office of the Commissioner. The Office of Health and Constituent Affairs-Patient Liaison Program coordinates the

recruitment, training, and retention for over 200 FDA Patient Representatives, who are patients or primary caregivers to patients. These FDA Patient Representatives are knowledgeable and experienced in over 300 diseases and conditions and participate on 47 FDA Advisory Committees and panels, and in review division meetings. These Patient Representatives provide direct input to inform the Agency's decision-making associated with medical products for drugs, biologics, and medical devices.

The Unique Voice of Our Patient Representatives

Unlike other Advisory Committee members, FDA's selection of patients serving involves identifying those with direct experience with the disease. Usually this means that a FDA Patient Representative is specific to the Advisory Committee meeting topic. Also, FDA Patient Representatives serve in review division meetings and FDA workshops. Requests for FDA Patient Representative involvement in FDA regulatory meetings continues to increase to actively implement FDASIA section 1137.

We are committed to making more opportunities for patients to participate in FDA decision-making. Our FDA Patient Representative Program brings the patient voice to the discussions about new and already approved drugs and devices and policy questions.

We recruit FDA Patient Representatives on an *as-needed* basis to:

- Help advise us on drugs, devices, and biologics that are currently being considered for approval
- Give us input earlier in the regulatory medical product development and review process.

Source: ***http://www.fda.gov/ForPatients/About/ucm412709.htm***

U.S. Food and Drug Administration (FDA) – Center for Drug Evaluation and Research (CDER)
Pilot Clinical Outcome Assessment Compendium

Overview

This pilot *Clinical Outcome Assessment (COA) Compendium* is part of FDA's efforts to foster patient-focused drug development. The *COA Compendium* is intended to facilitate communication and to provide clarity and transparency to drug developers and the research community by collating and summarizing clinical outcome assessment (COA)[2]information for many different diseases and conditions into a single resource. It can be used as a starting point when considering how certain clinical outcome assessments might be utilized in clinical trials and will likely be most informative in early drug development.

The *COA Compendium* is a table that

- Describes how certain *clinical outcome assessments* have been used in clinical trials to measure the patient's experience (such as disease-related symptoms) and to support labeling claims.
- Identifies clinical outcome assessments that have been qualified for potential use in multiple drug development programs under the COA type of the Drug Development Tool (DDT) Qualification Program of the Center for Drug Evaluation and Research (CDER).
- Recognizes ongoing qualification projects to encourage community collaboration in the development of clinical outcome assessments for unmet measurement needs.

FDA is seeking public comment and feedback about the pilot *COA Compendium* through establishment of a docket, as announced on January 13, 2016 in the *Federal Register*. Comments and recommendations received will be reviewed by the Agency as we consider developing future iterations of the *COA Compendium*.

Source:
https://www.fda.gov/Drugs/DevelopmentApprovalProcess/DevelopmentResources/ucm459231.htm

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U.S. Food and Drug Administration (FDA) – Center for Drug Evaluation and Research (CDER)

CDER Patient-Focused Drug Development

Resources

- Enhancing Benefit-Risk Assessment in Regulatory Decision-Making: Structured Benefit Risk Assessment and Patient-Focused Drug Development
- FDA-led PFDD Meetings for Fiscal Years 2013-2017
- Externally-Led PFDD Meetings
- "Voice of the Patient" Reports
- PDUFA V: Fiscal Years 2013 – 2017
- PDUFA VI: Fiscal Years 2018 – 2022
- PDUFA Reauthorization Performance Goals and Procedures Fiscal Years 2018 through 2022 ("Commitment Letter") (p. 27–29, Section J-1: Enhancing the Incorporation of the Patient's Voice in Drug Development and Decision-Making)

Overview

Patient-focused drug development (PFDD) is a systematic approach to help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation. As experts in what it is like to live with their condition, patients are uniquely positioned to inform the understanding of the therapeutic context for drug development and evaluation.

- The primary goal of patient-focused drug development is to better incorporate the patient's voice in drug development and evaluation, including but not limited to:
- Facilitating and advancing use of systematic approaches to collecting and utilizing robust and meaningful patient and caregiver input to more consistently inform drug development and regulatory decision-making
- Encouraging identification and use of approaches and best practices to facilitate patient enrollment and minimizing the burden of patient participation in clinical trials
- Enhancing understanding and appropriate use of methods to capture information on patient preferences and the potential acceptability of tradeoffs between treatment benefit and risk outcomes
- Identifying the information that is most important to patients related to treatment benefits, risks, and burden, and how to best communicate the information to support their decision making.

21st Century Cures Act and Prescription Drug User Fee Act (PDUFA) VI

FDA will develop a series of guidance on the collection of patient experience data, and the use of such data and related information in drug development. FDA will develop these guidances over a period of five years to implement provisions of the 21st Century Cures Act and to fulfill commitments under the sixth authorization of the PDUFA.

PDUFA V Patient-Focused Drug Development Initiative

Ensuring the safety, effectiveness and quality of human drugs is an increasingly complicated regulatory task, requiring FDA's expert consideration of a multitude of complex factors. Over the past several years, FDA has developed an enhanced structured approach to benefit-risk assessment in regulatory decision-making for human drug and biologic products.

The Benefit-Risk Assessment Framework was developed through extensive review and analysis of previous and ongoing regulatory decisions. PDUFA V commitments include further development and implementation of the Framework into FDA's human drug and biologic review process. Section 905 of the FDA Safety and Innovation Act also requires FDA to implement a structured benefit-risk framework in the new drug approval process.

In PDUFA V, FDA also committed to a new initiative called PFDD with the goal of obtaining the patient perspective on certain disease areas during the five year period of PDUFA V. Assessment of a product's benefits and risks involves an analysis of the severity of the condition treated and the current treatment options available for the given

disease. This information is a critical aspect of FDA's decision-making as it establishes the context in which the regulatory decision is made. FDA believes that drug development and FDA's review process could benefit from a more systematic and expansive approach to obtaining the patient perspective on disease severity and current available options in a therapeutic area.

Source: https://www.fda.gov/Drugs/DevelopmentApprovalProcess/ucm579400.htm

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U.S. Food and Drug Administration (FDA) – Center for Devices and Radiological Health (CDRH)

Patient Preference Initiative (PPI)

Resources

- Final Guidance: Patient Preference Information – Voluntary Submission, Review in PMAs, HDE Applications, and De Novo Requests, and Inclusion in Decision Summaries and Device Labeling (August 24, 2016)
- Patient Engagement Advisory Committee

Overview

CDRH recognizes that while scientists, clinicians, device developers, and regulators play critical roles in understanding and communicating the benefits and risks of medical devices, only patients live with their medical conditions and make choices regarding their personal care. They provide a unique voice and unique perspective.

The goal of PPI is to develop a systematic way of eliciting, measuring, and incorporating patient preference information, where appropriate, into the medical device Total Product Life Cycle. Ultimately, the objective is to drive more patient-centric innovation, evaluation, and delivery to U.S. patients. As part of this initiative, CDRH seeks to advance the science of measuring patient preferences by developing guidance for industry and other stakeholders on how to assess patient valuations of benefit and risk related to relevant device types and specific illnesses and conditions.

CDRH gets patient preference information from patient groups, industry, and others who conduct studies, in device submissions, and from listening to patients' input through advisory panel meetings, public-private partnerships, public workshops and public comments submitted to FDA. As the medical device community conducts more patient preference studies, we will gain a better understanding of the tradeoffs that patients are willing to make in order to have access to new devices.

Source: http://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDRH/CDRHPatientEngagement/ucm462830.htm

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U.S. Food and Drug Administration (FDA) – Oncology Center of Excellence (OCE)

Patient-Focused Drug Development

Overview

The OCE PFDD program fosters collaboration between FDA Centers and external stakeholders involved in patient outcomes research in cancer populations. The program focuses on three key areas:

- Actively engaging with patients and advocacy groups.
- Fostering research into measurement of the patient experience.
- Generating science-based recommendations for regulatory policy.

The overarching goal is to identify rigorous methods to assess the patient experience that will complement existing survival and tumor information to better inform a cancer therapy's effect on the patient. Cancer patients experience

disease symptoms and symptomatic treatment side effects that can impact their ability to function and other aspects of their health-related quality of life.

Research Focus: PanPROE: Pancreatic Cancer Patient Reported Outcomes Using the Electronic Medical Record

Newly diagnosed pancreatic cancer patients are now being enrolled in a prospective natural history study initiated under a research collaborative agreement between the FDA's Office of Hematology and Oncology Products and the Division of Research of The Permanente Medical Group.

The study will investigate the use of patient-reported outcome (PRO) measurement of physical function in pancreatic cancer patients who undergo treatment and follow up for their illness. Investigators hope to better understand the electronic capture of PRO in a large integrated healthcare system (Kaiser Permanente Northern California), and learn more about measurement of the patient's perception of physical functioning throughout their pancreatic cancer treatment journey.

The study is done in collaboration with the patient-focused drug development program of the FDA Oncology Center of Excellence, which is exploring measurement of the patient experience in both the clinical trial and real-world setting to inform the risks and benefits of cancer therapies.

Source:
https://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/OCE/ucm544143.htm

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U.S. Food and Drug Administration (FDA) & the European Medicines Agency (EMA)
EMA/FDA Cluster on Patient Engagement

Resources

- Terms of reference for the EMA/FDA cluster on patient engagement
- EMA and FDA reinforce collaboration on patient engagement (EMA, 6/22/2016)

Overview

The FDA and EMA have created a new workgroup on patient engagement called the FDA/EMA Patient Engagement Cluster.

The FDA/EMA Patient Engagement cluster joins a series of currently existing EMA/FDA clusters. The cluster allows FDA and EMA to share best practices involving patients along drug and biologic regulatory lifecycles. Information that is discussed is covered by confidentiality agreements signed by the FDA and EMA.

The clusters will focus on specific topic areas where the FDA and EMA can benefit from a greater exchange of information and strengthen collaboration. These clusters discuss issues related to:

- biosimilars
- medicines to treat cancer
- orphan medicines
- medicines for children
- pharmacovigilance
- among other topics

The new cluster work group will meet up to four times per year by telephone. This increased interaction will help each agency:

- Learn how their respective patients are engaged and involved in the work performed.
- Develop common goals of expanding future engagement activities with patients.

The FDA/EMA Patient Engagement cluster workgroup will discuss:

- Ways for finding patients that appropriately speak for their community.
- Ways to ensure that patients involved in agency processes directly voice the concerns of their community.
- Ways to train selected patients and advocates to effectively participate in agency activities.
- Strategies for reporting the significant impact of patient involvement.

The launch of this cluster is the latest step in FDA and EMA's broadened approach to advance and strengthen international collaboration.

Source: *http://www.fda.gov/ForPatients/PatientEngagement/ucm507907.htm*

Genentech
Product Development Patient Insights

About Initiative

We work with select Roche clinical trial teams to collect, interpret, and help incorporate patient needs and preferences into their development strategies and clinical trial protocols

Initiative Goals

Improve the design and execution of our late-stage clinical trials to make them better reflect the patient voice

Problems Addressed by Initiative

Clinical trials are not always designed with the patient at the center, which can negatively impact recruitment, retention, compliance, and patient satisfaction

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: *https://involvement-mapping.patientfocusedmedicine.org/initiatives/product-development-patient-insights*

Genetic Alliance
Platform for Engaging Everyone Responsibly (PEER)

Overview

The Platform for Engaging Everyone Responsibly (PEER) creates a highly engaging, intuitive, consumer-centric, privacy-assured, and—importantly—customizable portal and service.

PEER enables participants and their caregivers to share clinical information and biological specimens within an environment that provides the look and feel of familiar, trusted communities, under access-permission rules defined by the participants themselves. PEER provides data-entry, data-query, and privacy-management services that are accessed through standard application programming interfaces (APIs).

PEER is customizeable to provide the look and feel of trusted communities.

One of the key elements of PEER is the use of trusted, community-based guides who provide step-by-step, participant-centric introductions about PEER's use and accessibility, including how to share health information and set reasonable privacy controls that are consistent with individuals' preferences and values.

For more information about customizing PEER, please visit our White Label project page.

Source: *http://www.geneticalliance.org/programs/biotrust/peer*

GlaxoSmithKline

Direct Patient Insight on Lupus With a Focus on Cutaneous Aspects

About Initiative

In October 2015, two medical doctors and one scientist from GSK interviewed 5 female patients diagnosed with systemic lupus with cutaneous manifestations, or diagnosed with cutaneous lupus with skin symptoms only - four patients interviewed in Cambridge in the UK, and one patient interviewed over Skype.

Initiative Goals

The objective was to hear patients' views on their disease and on research because GSK is planning clinical trials of an investigational medicinal product in patients with cutaneous lupus.

Problems Addressed by Initiative

Patients described a long history of the disease, and their general symptoms that impact on daily life, for example they become tired very soon, they have painful joints, cold feet and fingers, prolonged mouth ulcers, and they can feel isolated or have depression as they cannot always go outside or to work. With respect to the skin symptoms, the patients consistently reported that exposure to sunlight provokes or aggravates symptoms. This significantly limits outside activities, and they must put on sunscreen even several times a day. Skin lesions are itchy, can be thick, occur anywhere on the body including on the head or face, which can lead to social isolation. The patients indicated that they need several different treatments, all the time.

The information that patients shared helped the researchers to progress with designing a clinical trial, which is planned to start during 2016.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: *https://involvement-mapping.patientfocusedmedicine.org/initiatives/direct-patient-insight-on-lupus-with-a-focus-on-cutaneous-aspects*

GlaxoSmithKline

Patient Feedback on a Draft Plain Language Summary of Clinical Trial Results

About Initiative

Plain language summaries (PLSs) will be required for all interventional studies (Phase 1 to Phase 4) with a study site in the EU. Patients were asked to review a draft plain language summary from a completed Phase 3 study approximately 1 week in advance of a follow-up discussion. Individual telephone interviews with the patients were conducted by GSK staff members. Six patients were interviewed; two were EUPATI trainees. None of the patients had the condition that was evaluated in the study.

The patients provided valuable feedback about the wording, structure, and content of the plain language summary.

Every patient brought a different perspective, skill, expertise and level of knowledge. Each raised interesting questions which provoked follow on in-depth discussion both within the interview but also with the GSK team, particularly around the question "what happens after plain language summaries are released – what happens next for the patient who took part/the medicine/the research?" As a result, in addition to receiving similar points in each interview around reporting the key study finding, we also obtained a wide range of suggestions for overall improvement of the plain language summary document. Beyond this from insights shared, we were able to consider how patients may seek and retain the information provided by GSK.

Initiative Goals

Creating a PLS of clinical trials with patient feedback.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-feedback-on-a-draft-plain-language-summary-pls-of-clinical-trial-results

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Healthcare Quality Improvement Partnership (HQIP)
Patient and Public Involvement Strategy

About Initiative

As an independent organization, HQIP works in partnership with patients and healthcare professionals to improve practice through quality improvement.

We have developed and published a Patient and Public Involvement (PPI) Strategy focusing on how HQIP will increase and improve patient and public involvement internally and how we will support our stakeholders to effectively involve patients in national and local clinical audit activities.

The National Involvement Partnership (hosted by the National Survivor User Network) has developed the 4PI framework for involvement. This framework enables organizations to build standards for good practice.

HQIP has adapted the five headings to explain our approach to PPI:

1. Principles
    - Representation: Participating patients will be broadly representative of the relevant affected population. Consultations will be carried out through organizations such as National Voices to ensure broader representation on generic issues.
    - Inclusivity: HQIP will provide sufficient resources to overcome barriers such as issues of access or communication.
    - Root and branch: Patients will be involved as early as possible in a process / activity and continue to be involved throughout. Patients will be involved in all areas of HQIP.
    - Transparency: Those involved will be able to see and understand how decisions are made and Information on audit data and consultant outcomes will be published in clear and understandable formats.
    - Clarity of purpose: The nature and scope of involvement will be clearly defined prior to involvement. It will be clear how publications can be used to inform patients about the quality of services available.
    - Cost Effectiveness: Involvement must add value and be cost effective.
    - Feedback: The outcomes of PPI activities will be fed back to participants. Feedback on our products will be used to review and improve our publications.
2. Purpose

    HQIP aim to further improve the way we involve, engage and inform patients. We will involve patients in our activities and decision making processes in order to gain a more rounded perspective of how our outputs can be utilised to improve patient outcomes. We also aim to enable others to increase and improve their PPI in quality improvement initiatives and to empower patients themselves to become involved in national and local clinical audit activities. We will engage with specific patient groups and experts by experience on specialised projects and more broadly for generalist areas.
3. Presence

    HQIP is led by a consortium comprising of National Voices, the Royal College of Nursing and the Academy of Medical Royal Colleges. Our Chair is a member of National Voices. We have a designated lead for PPI who works across the organization and an active service user network (SUN).
    - We will work with National Voices on strategic and operational levels to reach a larger audience of patients.

- We will ensure published information designed to help patients become more engaged in decisions about their own treatment and care is available, accessible and clearly presented.
- We will continue to involve a diversity of service users at different levels and stages of our activities throughout HQIP and encourage and enable our delivery partners to do the same.
- We will provide information and training to commissioners, healthcare provider organizations, clinicians and patients on methods of involvement and engagement in quality improvement initiatives.
- When involving patients on specific projects we will carry out analysis of the population under consideration to ensure that the involvement activity reflects that population – and to ensure that people particularly affected by the service or issues under consideration are actively approached for inclusion.
- We will put monitoring procedures in place to measure the number and diversity of patients at all levels of involvement / engagement
- Different methods of patient involvement will be utilized to enable patients to be involved in ways that provide the best outcome for them and HQIP.

4. Process

   Information will be made available through a number of channels to ensure people are made aware of opportunities for involvement and the different ways in which they can be involved. The PPI lead will disseminate information but opportunities will also be highlighted using the e-bulletin, National Voices e-bulletin and CHAIN.

   Where appropriate, recruitment processes will be fair and transparent and job descriptions clearly laid out whether the roles are paid or unpaid.

   Throughout our activities and consultations communication will be clear and regular; jargon and acronyms will be avoided or (where necessary) explained; written documents will be sent out well in advance of meetings; feedback about the results or outcome of an activity will be provided.

   Information, guidance and training for commissioners, healthcare providers, clinicians and patients will be designed and consulted upon in line with The Information Standard criteria.
   Training needs assessment will be carried out and training made available where required for patients involved in particular activities.

   Staff development will be given to raise awareness of the value of PPI and practical training will be provided to key members of staff such as the communications team and the PPI lead as identified in their personal development plans.

5. Impact – by 2017 the HQIP Service User Network will have written an Impact Analysis report that will examine the impact of the SUN on HQIP activities from 2014-2016. This will be the first stage of ongoing Impact work.

   Patient and public involvement must be used to add value to a decision or activity. Indicators will be developed to measure the impact of increased PPI throughout HQIP. KPIs will be developed that demonstrate:

   - Whether the level of patient involvement/ engagement has increased (flow)
   - Were the intended outcomes achieved (quality)
   - What actual difference did involving patients make and was the outcome improved (impact)

Initiative Goals

The word involvement does not simply mean informing, but increasingly partnership working and, ultimately, patient led activity. Patients and the public will be involved in the structures and processes of HQIP's work i.e. through mechanisms such as governance, priority setting, training and education, identification of the need for innovation and assessment of technologies. The framework enables organizations to build standards for good practice.

Problems Addressed by Initiative

Clinical audit is a quality improvement cycle that involves measurement of the effectiveness of healthcare against agreed and proved standards for high quality and takes action to bring practice in line with these standards so as to improve the quality of care and health outcomes for patients.

Patients will be involved in this process by being provided with the forum to contribute to HQIP projects and discuss their first hand user experiences thereby ensuring that all patient concerns and issues are fully represented. In addition we will also support and advise our stakeholders in delivering effective and successful PPI in their local and national quality improvement projects.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-and-public-involvement-strategy

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Innovative Medicines Initiative (IMI)
PREFER – Patient Preferences

Overview

PREFER will establish recommendations to support development of guidelines for industry, Regulatory Authorities and HTA bodies on how and when to include patient perspectives on benefits and risks of medicinal products.

Over the next five years, we will run patient preference studies in both academic and industry settings. Our experience will provide a better understanding of what will be a recommended best-practice approach to patient-preference studies. We will also show how patient preference studies can give valuable information to support decision making for regulators and HTA bodies.

PREFER is divided into four 'work packages'. The methodology work package looks at the concerns stakeholders have around the use of patient-preference studies. Based on what they find, they will make recommendations about what methodologies to use in case studies that the case study work package will design and carry out. After evaluating the case studies, Finally, the recommendations work package will take over and draft recommendations based on the work. The management work package will make sure this work is done on time.

Source: https://www.imi-prefer.eu/

Innovative Medicines Initiative (IMI)
PARADIGM – Patients Active in Research and Dialogues for an Improved Generation of Medicines

Overview

PARADIGM is a public-private partnership and is co-lead by the European Patients' Forum and EFPIA. PARADIGM's mission is to provide a unique framework that enables structured, effective, meaningful, ethical, innovative, and sustainable patient engagement (PE) and demonstrates the 'return on the engagement' for all players. The objective is to develop much needed processes and tools for three key decision-making points: research priority setting, design of clinical trials and early dialogue. Building on advances at international level, PARADIGM will integrate the needs, perspectives and expectations of all actors (including vulnerable populations) involved and will also produce a set of metrics to measure the impact of patient engagement.

Project objectives

Needs and expectations

Strengthen the understanding of stakeholders' needs and expectations for engagement (including underrepresented and vulnerable populations);

Sustainability roadmap

Develop an inventive and workable sustainability roadmap to optimise patient engagement in key decision-making points across medicines' R&D;

Maximum synergies

Ensure maximum synergies with other initiatives focusing on the patient's voice in the life cycle of medicines, like Patient Focused Medicines Development (PFMD) or the European Patient Academy on Therapeutic Innovation (EUPATI).

Agreed metrics

Develop agreed patient engagement metrics to increase evidence demonstrating the impact of patient engagement practices;

Systems-readiness

Strengthen systems-readiness towards patient engagement across the diverse range of stakeholders that develop, regulate and assess medicines;

Source: *https://imi-paradigm.eu/*

International Academy of Health Preference Research (IAHPR)
Health Preference Study and Technology Registry (HSPTR)

Overview

The HSPTR has been launched. Registering on HPSTR.org can take as little as five minutes. Without a doubt, this has been the largest collaborative endeavor that IAHPR has yet undertaken. Along with the IAHPR members, the Academy thanks Chris Carswell, Bennett Levitan, Ernest H. Law, Winter Maxwell Thayer, and Max Masnick for their assistance with beta testing. Like our meetings and other collaborative initiatives, HPSTR will serve as an enduring contribution to the field.

Although the IAHPR feels proud of its accomplishments over the last three years, it is important to recognize that HPSTR will require consistent support so that it can best serve our mission: "to improve decisions about health and healthcare throughout the world by developing, promoting, and supporting health preference research with the widest possible applicability." Over the next three years, the IAHPR plans:

1. to register every published health preference study and technology (old and new);
2. to pursue the endorsements of journals, sponsors, regulatory agencies, and other organizations involved in health preference research; and
3. to build and disseminate further resources from this platform, including a certification program, a survey database, and a series of educational resources (e.g., textbook, webinars, and workshops).

Source: *https://hpstr.org/landing*

International Children's Advisory Network (iCAN)
International Children's Advisory Network (iCAN)

About Initiative

iCAN officially launched in June, 2015 with its Launch and Research Summit, which was hosted in Washington, D.C., USA. The event brought together 130 children, parents and team leaders from young person's advisory groups in the United States, Canada, United Kingdom, Spain, France and Australia to learn from one another and engage with industry, regulatory, and government leaders in the areas of children's health, research and

innovation. This event marked the first major meeting of the iCAN network and showcased all of the accomplishments and hard work achieved by both our founding and rising teams.

Since its launch, the organization has grown to consist of 19 teams globally, spanning 8 countries on 3 continents. iCAN's rapid growth and success was showcased at the second-annual 2016 iCAN Research and Advocacy Summit, which was held in Barcelona, Spain and held a myriad of interactive sessions, workshops and expert panels. The objective of iCAN and the Summit is to educate and empower our youth to improve pediatric health, medicine, research and innovation by sharing children's voices in an impactful way. This event provides our youth with an invaluable opportunity to learn from one another and network with professionals from across the globe, while allowing the scientific community to engage with children and learn about the value and the significant importance of the influence of children on research, medicine, and innovation.

iCAN aims to continue to expand the network and opportunities for our youth, as well as increase the success of the Summit each year. Our 2017 Research and Advocacy Summit will be held July 10-14, 2017 in Orlando, Florida, and will help us further our mission.

Initiative Goals

Our goal is to include children in the future of pediatric research and medicine so that they can be empowered to make impactful changes and decisions surrounding the medical treatment of children across the globe. We collaborate with industry leaders and provide input to clinical study designs so that they better suit the needs of children.

Problems Addressed by Initiative

At this time, there is a very low level of youth involvement in the pediatric research process in terms of study designs and documentation. iCAN works with researchers and industry representatives to provide input from a young person's perspective so that the study designs better suit the needs of children and their families. We work to help redesign assent documents and overall design of research studies through surveys and feedback templates, as well as conduct some survey-based research of our own, designed, conducted and analyzed by the youth members in our network.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: ***https://involvement-mapping.patientfocusedmedicine.org/initiatives/international-children-s-advisory-network-ican***

International Society for Pharmacoeconomics and Outcomes Research (ISPOR)
Patient Initiatives

About Initiative

Through its worldwide membership and stakeholder groups, ISPOR has access to the ideas, skills, and experiences that are generating the evidence today to inform the health care decisions of tomorrow. As a result, ISPOR is uniquely positioned to offer world class health economics and outcomes research education through its global meetings, short courses, training programs, online tools, and scientific publications. ISPOR works with its members and thought leaders to create important tools and resources that support its mission. We recognize the best way to achieve this goal is to involve all stakeholders, including patients.

ISPOR has been committed to engaging patients as a key constituency since 2012 when the Society first invited patient representatives to participate in the Health Technology Assessment (HTA) Roundtable held during their 17th Annual International Meeting. The Society launched its Patient Representatives Roundtables initiative at its 2013 Annual European Congress. Since then, ISPOR has held annual Patient Representatives Roundtables in North America and Europe. The Society hosted its first Patient Roundtable in Latin America in September 2017 and is planning its first for Asia-Pacific in 2018. The Society introduced a patient membership category in 2015 that includes special member benefits, such as discounted membership and conference registration rates and travel grants. ISPOR also has established a Patient Centered Special Interest Group that has an active working group on Patient Engagement in Research.

Initiative Goals

The goal of ISPOR Patient Initiatives is to engage patient representatives in research on health outcomes, to develop the science of patient involvement in research, and to advance the value and impact of patients as partners in research through multi-stakeholder engagement.

- Patient Council: Serves as an advisory group to the ISPOR Board of directors and addresses recommendations for engaging patients to support ISPOR's Patient Initiatives activities.
- Patient Representatives Roundtables: Provides a platform for patient representatives and other key stakeholders to discuss issues and challenges of patient involvement in the health care research and decision making processes.
- Patient Centered Special Interest Group: Facilitates the involvement of patient representatives in all stages of research and decision making to improve health care, its delivery, and outcomes. This group aims to define patient engagement; patient centered; and related terms in the context of health care and outcomes research.

Source: ***https://www.ispor.org/about-ispor.asp***

Leukemia & Lymphoma Society (LLS)
Patient Preference Study in Acute Myeloid Leukemia

Overview

LLS is currently conducting a survey focused on understanding what patients diagnosed with acute myeloid leukemia (AML) and their caregivers want from their treatments.

Why?
The study's purpose is to guide drug development for AML based on what really matters to patients.
The results of LLS's survey will be shared with the FDA, drug companies, academic researchers and the broader AML community.

How?
LLS is partnering with a team of researchers at Johns Hopkins University to develop, administer, analyze and disseminate this survey.
LLS engaged a large community of AML patients and caregivers who participated in an advisory committee to guide the development of this survey. LLS and the advisory committee also engaged the FDA in the process.

Source: ***https://www.lls.org/lls-us.../acute-myeloid-leukemia-patients-patient-preference-study***

Medical Device Innovation Consortium (MDIC)
Patient Centered Benefit-Risk Assessment

Resources

- Project report: A Framework for Incorporating Information on Patient Preferences Regarding Benefit and Risk into Regulatory Assessments of New Medical Technology

Overview

MDIC and its partners are collaborating to improve our ability to include patient perspectives in the development, pre-market approval, and post-market evaluation of medical devices. First, we are trying to find scientifically valid ways to reliably assess patient views on the potential risks and benefits of specific devices. Second, we aim to establish a credible framework for incorporating that information into device development and benefit-risk assessments. We will share our findings with the FDA, which could then choose to use them in future guidance documents and regulatory decisions.

Results, included in project report:

- A framework for incorporating information on those preferences into benefit-risk assessments of new medical technology.
- A catalog of methods that can be used to assess patient preferences about the benefits and risks of a medical technology.
- An analysis of gaps in current methods for assessing patient preferences.
- An agenda for further research.

Source: http://mdic.org/pcbr/

Medicines and Healthcare Products Regulatory Agency (MHRA)
Patient Group Consultative Forum

About Initiative

The Patient Group Consultative Forum brings patients into the MHRA to discuss pertinent issues and provide patient insight to inform the organization's operations. Currently around 80 members and growing, the PGCF follows an informal format, which allows it to be flexible to the MHRA's needs. A clear role description manages patient expectations and sets clear guidelines for achieving outcomes.

Initiative Goals

To establish the patient voice within the agency and helping to recognise patient experiences and views of equal weighting to health care professionals. Giving patients a space where they can formally influence the way in which the MHRA operates and makes decisions.

Problems Addressed by Initiative

To address a growing Government and internal expectation that agencies such as the MHRA should have increasing access to the patient voice and opinion in medicines and services. MHRA is now also holding its board sessions in public and the forum has provided patient representative attendees.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-group-consultative-forum-pgcf

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Medicines Development for Global Health (MDGH)
Moxidectin for the Treatment of Scabies

About Initiative

The program hopes to develop a new treatment of scabies and intends to engage and involve the community at the earliest possible stage.

Prior to commencement of the clinical trials the program, MDGH will have discussions with communities who have endemic scabies - in Australia, Aboriginal communities are disproportionately affected and so the program hopes to involve these communities in the design of the clinical studies.

The program is in the early stages of planning but anticipates to have regular group meetings with patients where the plans of the program can be discussed and implementation questions addressed.

Initiative Goals

The ultimate goal is to deliver a medicine that people will use.

There are many stakeholders involved in the development of a new medicine - prescribers, Governments, payers, patients and scientists, all of whom have a key role to play. It is important to have everyone involved and have a broad consultation so that the drugs will be impactful.

Problems Addressed by Initiative

Firstly an assessment of the treatment paradigm for scabies was conducted, and it was noted that the current treatment options are not user friendly. Patients and the people who they are in close contact with apply a cream to their entire body and leave this on overnight. This is not only difficult to achieve but in resource poor settings, this ever more challenging. Ideally it is hoped that a single dose oral treatment can be developed.

Patient involvement at the very early stages of drug development is key as only they can provide the context of use. Open discussion with communities is critical to success.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: ***https://involvement-mapping.patientfocusedmedicine.org/initiatives/moxidectin-for-the-treatment-of-scabies***

Merck Sharp & Dohme (MSD)
Patient Input Forums

Initiative Goals

MSD engages with physician facilitators based in the U.S. who in turn identify patient(s) who are willing to share their experiences with their illness, including their overall treatment experience and systems of care and respond to questions. The primary objective of the PIF is to expose MSD's workforce to "real world" patients who suffer from diseases in MSD's priority therapeutic areas. The input provided by the patients will provide meaningful insight for MSD U.S. Headquarters-based employees as they work to optimize, develop, and launch innovative products and services that save and improve lives around the world. Specific meeting objectives are determined based on each disease area.

Problems Addressed by Initiative

- Patient journey
- Patient decision-making
- Patient perspective on benefit:risk, use of medications (MSD's and others),
- The interface among patient, caregiver, drug-maker, pharmacy and others along the healthcare continuum

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: ***https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-input-forums***

Michael J. Fox Foundation for Parkinson's Research (MJFF)
Fox Insight

Study Purpose

Biomedical research is entering a new era of "patient power" with seismic opportunity for people living with disease to partner with scientists to influence research design and shape patient-relevant outcomes. Technology now allows thousands to contribute vast volumes of data on their lived experience of disease: from symptoms and quality of life to treatment satisfaction and research participation preferences. This data is collectively known as patient-reported outcomes, or PROs, and holds the power to redefine therapeutic priorities, influence funding streams and optimize trial design.

For example, regulators (such as the Food and Drug Administration) and payers (Medicare/Medicaid/insurers) traditionally have relied on biological markers of disease to make decisions about approving and reimbursing new

therapies. Today they increasingly rely on PROs to fully understand health care and the potential impact of new treatments in real-world clinical practice.

The Michael J. Fox Foundation has established Fox Insight, a digital platform and dynamic online clinical study, to build a large, diverse cohort of people with Parkinson's and age-matched control volunteers. The study seeks to enroll tens of thousands of diverse participants, which would make it the largest and most representative Parkinson's research study to date. A large, diverse cohort will lead to more accurate, generalizable and powerful PROs.

It's important to note: While patient-reported outcomes supplement biological measures of disease and therapeutic impact, they are not a replacement. Fox Insight complements traditional, in-person research studies with scale and accessibility, overcoming traditional barriers to research participation such as geography and mobility limitations. Curated, de-identified (without names or email addresses, for example) Fox Insight data will be made available to researchers worldwide in real time. Access to the Fox Insight cohort and its data can drastically reduce research timelines, advancing new therapies faster.

Fox Insight launched in beta in March 2015. More than 5,000 volunteers (80 percent with Parkinson's diagnosis) contributed data that helped optimize the study experience and utility of data for researchers before the study's formal launch in April 2017.

Please participate by sharing your health-related experiences.

Study Visits

Fox Insight compiles health and disease information entered when participants first join the study and every 90 days thereafter. Email prompts and your Fox Insight dashboard will tell you when it's time for your next study visit: a series of online questionnaires that may ask about symptoms, experiences performing daily tasks, medical and family history, or other topics related to your health. All Fox Insight activities adhere to regulatory/institutional review board standards for a clinical study.

It's important you keep coming back to complete study visits. Seeing health patterns and trends over time from thousands of individuals – and capturing recent experiences -- will help capture accurate and powerful PROs.

Related Research

In addition to completing regular study visits, you may be offered ways to provide additional data. These may include surveys to answer specific questions about the lived experience of Parkinson's or sub-studies leveraging remote data collection mechanisms such as using mobile technologies (smartwatches and smartphones) for the passive capture of real-time, objective data on daily life with PD. In addition, Fox Insight is linked to the MJFF online smart-matching tool Fox Trial Finder, helping users find other clinical studies (in-person and online) that may also be a good match for them based on medical history and geography.

We're always looking for ways to grow the reach and impact of your participation in Fox Insight, so keep an eye on your email and on the Related Research page for new opportunities to contribute.

Community, Research Findings and Tools

By participating in Fox Insight, you're part of a growing community of people with Parkinson's, their loved ones and Parkinson's researchers. On the Community Page, view a snapshot of the aggregate data from all Fox Insight users (e.g., most bothersome and frequent symptoms, family neurological history). The Research Publications page shows how scientists are using Fox Insight data to integrate PROs in research and accelerate scientific discovery. The Tools page has resources to help prepare for upcoming visits with a physician such as an appointment reminder and physician report.

Source: *https://foxinsight.michaeljfox.org/about*

MyHealthTeams
Treating Patients Like Consumers

About Initiative

We have over 500,000 members across the 17 different chronic condition social networks we've launched. Three years ago, we began partnering with biopharmaceutical companies to do three things (1) treat patients like consumers and seek to understand the entire patient journey from pre-diagnosis to post-treatement, (2) Ask them their opinions and thoughts on clinical trial outcomes, schedules, wording, and logistics and use it to inform protocol design BEFORE the protocol is finalized and (3) directly reach out to them and invite them to participate in clinical research that is relevant to them (because none of their doctors are telling them about trials.

Initiative Goals

Empower our members to have a voice in the drug development process in a way that isn't taxing.

Problems Addressed by Initiative

Engaging consumers with chronic conditions in all stages of drug development.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: ***https://involvement-mapping.patientfocusedmedicine.org/initiatives/treating-patients-like-consumers***

National Breast Cancer Coalition
Project LEAD

Overview

Housed within the Center for NBCC Advocacy Training, Project LEAD® is the National Breast Cancer Coalition's premier science training program for activists that has created a revolution in the world of breast cancer research and public policy. The courses prepare graduates to engage in the wide range of local and national forums where breast cancer decisions are made. Project LEAD® graduates bring an educated consumer perspective and critical thinking skills to the important issues and controversies in breast cancer.

As a result of NBCC's work, scientists, government agencies and private industry have changed the way they design and implement breast cancer research and programs. NBCC has created a model for consumer influence marked by transparency, innovation and a peer relationship among scientists, researchers, policymakers and consumers nationwide.

Source: ***http://www.breastcancerdeadline2020.org/get-involved/training/project-lead/***

National Health Council (NHC)
Patient Engagement

Resources

- White paper: Patient Perspectives on Disease Impact and Treatment Options: A Stratification Tool (May 2014)
- Implementation Manual: How to Operationalize the National Health Council's Patient Information Tool

Overview

The NHC has undertaken an initiative to address current barriers to patient engagement through a multi-stakeholder approach by building a consensus-based conceptual framework for patient engagement and agreement on best practices in research and development in drug development.

The NHC also developed an information collection tool to help patient advocacy organizations systematically capture and organize patient concerns and comments about the benefits and risks of treatment options. The tool is designed to ensure that the FDA captures the comprehensive information it needs from patients, family caregivers, and patient advocates, and better engage patients in its work.

Source: ***http://www.nationalhealthcouncil.org/public-policy/patient-engagement***

National Health Council (NHC) & Genetic Alliance
Integrating the Patient into the Drug Development Process: Developing FDA Guidance

Resources

- White paper: *Integrating the Patient into the Drug Development Process: Developing FDA Guidance* (May 2016)

Overview

Many stakeholders in the health care community have expressed a need for the U.S. Food and Drug Administration (FDA or the Agency) to provide guidance to encourage product sponsors (e.g., biopharmaceutical companies) to engage patients throughout the drug research and development lifecycle, illustrate how this can be achieved, and ensure the information collected is useful to the regulatory review process. Patients, researchers, and product sponsors alike welcome formal guidance from the FDA on how patients can be appropriately and meaningfully engaged.

To move this effort forward, the National Health Council (NHC) and Genetic Alliance convened a December 9, 2015, meeting with 36 multi-stakeholder participants. The objective of the meeting was to inform the scope and contents of a proposed FDA guidance document intended to guide industry, patient organizations, and other stakeholders in collecting input and information from patients to help inform drug research, development, regulatory review, and post-marketing activities, spanning the entire product lifecycle.

Source: ***http://www.nationalhealthcouncil.org/Developing-FDA-Guidance***

National Health Council (NHC) & Genetic Alliance
Advancing Meaningful Patient Engagement in Research, Development, and Review of Drugs

Resources

- White paper: *Dialogue/Advancing Meaningful Patient Engagement in Research, Development, and Review of Drugs* (Sep 22, 2015)
- Press release: Meaningful Patient Engagement is More than an App or Big Data (Sep 22, 2015

Overview

The National Health Council and Genetic Alliance convened a multi-stakeholder group of key health care thought leaders to help develop action steps for the integration of the patient perspective into drug research, development, and approval. The Dialogue event was attended by representatives from patient organizations, the federal government, academia, and industry.

Source: ***http://www.nationalhealthcouncil.org/meaningful-patient-engagement***

National Institutes of Health (NIH) – National Cancer Institute (NCI)

Patient-Reported Outcomes Version of the Common Terminology Criteria for Adverse Events (PRO-CTCAE)

What is PRO-CTCAE?

- PRO-CTCAE is a patient-reported outcome measure developed to evaluate symptomatic toxicity in patients on cancer clinical trials.
- It was designed to be used as a companion to the Common Terminology Criteria for Adverse Events (CTCAE), the standard lexicon for adverse event reporting in cancer trials.
- PRO-CTCAE includes an item library (PDF, 179 KB) of 124 items representing 78 symptomatic toxicities drawn from the CTCAE.
- PRO-CTCAE provides a systematic yet flexible tool for descriptive reporting of symptomatic treatment side effects in cancer clinical trials.

How Do I Use PRO-CTCAE?

- PRO-CTCAE is publicly available for all to use in their clinical trials and we encourage and facilitate this use.
- PRO-CTCAE should be used and reported in conjunction with the CTCAE reports gathered by clinicians. It provides additional information that is complementary to existing safety and tolerability assessments reported by clinicians using the CTCAE.
- Choice of PRO-CTCAE items is dependent upon anticipated adverse events based upon previous preclinical data and regimen-specific information.
- Timing of assessments:
  - Recall period for PRO-CTCAE is the past 7 days.
  - Consider weekly assessment for key periods in the trial (eg. first two cycles in an early phase trial) or at other key clinical assessment timeframes based upon knowledge of the anticipated toxicity of the regimen.
- Once you have determined which symptomatic adverse events you wish to collect in your trial and at which timepoints of measurement, you can use our Form Builder to build a study-specific custom form.
- PRO-CTCAE responses are scored from 0 to 4, and there are as yet no standardized scoring rules for how to combine attributes into a single score or how best to analyse PRO-CTCAE data longitudinally.
  - PRO-CTCAE scores for each attribute (frequency, severity and/or interference) should be presented descriptively (e.g. summary statistics or graphical presentations).
  - CTCAE grades for the corresponding time period should be presented in conjunction with PRO-CTCAE scores.

National Institutes of Health (NIH) – National Center for Advancing Translational Sciences (NCATS)

NCATS Toolkit for Patient-Focused Therapy Development

Overview

NCATS is dedicated to engaging the patient community throughout the translational science process. The NCATS Toolkit for Patient-Focused Therapy Development (Toolkit) was created to provide a collection of online resources that can help patient groups advance through the process of therapy development and provide them with the tools they need to advance medical research.

Launched in September 2017, the Toolkit includes resources that have been developed primarily for the rare diseases community to facilitate therapeutics research and development. Since early 2016, NCATS has worked with a diverse group of partners in the rare diseases community to conduct an extensive landscape analysis of

available tools. These resources were defined, characterized and organized in a centralized portal that can be helpful to all patient groups regardless of how far along in the research and development process they might be.

Tools include:

- How to establish a patient registry;
- How to drive patient-focused discovery and pre-clinical research and development;
- How to work with NIH and the Food and Drug Administration; and
- How to conduct post-market surveillance.

Source: ***https://ncats.nih.gov/toolkit***

National Institute for Health Research (NIHR) – Medicine for Children Research Network (MCRN)

Generation R

About Initiative

Planning for the Generation-R meeting started in October 2012. The meeting was planned to 'Showcase how children, young people and families have improved the design, development and delivery of paediatric research' 'Improve the success of studies in partnership with children, young people and parents. A planning group made up of representatives from each regional young person's group alongside five parent representatives and a core team of MCRN staff was created. The group was responsible for ensuring young people's and parents' perspectives were core: setting the agenda, inviting the speakers and their topics, planning the activities, sessions, format and meals/refreshments, contributing to the publicity and communications.

Objectives:

Three core objectives were prioritised for the event:

- Demonstrate how children, young people and parents support/improve the design and feasibility of studies
- Choosing the right patient-reported outcome measures
- Showing how bringing patients and researchers together can improve research and educate both parties.

Initiative Goals

The main goal is to provide a forum for the involvement of young people that enables them to engage and ensures the involvement of young people in clinical research.

Problems Addressed by Initiative

The biggest problem it addresses is access to young people's views on research in general; it also addresses the issue of making sure young peoples' views are listened to, as we often find that parents tend to speak for them. It helps young people to have their say on clinical research.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: ***https://involvement-mapping.patientfocusedmedicine.org/initiatives/generation-r***

National Institute for Health Research (NIHR) – Medicine for Children Research Network (MCRN)

Patient Research Ambassadors Initiative

About Initiative

The Patient Research Ambassador Initiative led by the NIHR'S MCRN is to help raise awareness and open up research opportunities and choices for patients in local NHS care organizations. The initiative provides tools, guidance and information needed for NHS Trusts, NHS Organizations and GP surgeries to develop these roles to

help optimize patient experience in respect to health research in their organization. The Patient Research Ambassador Initiative has a vision of a patient-centered research culture as part of NHS organizations across England. Patients using NHS services should have greater access and better information about clinical research happening locally so they have the knowledge required to make informed choices about their care options. Patient Research Ambassadors are a great way of ensuring this happens and help the Trust achieve a more patient-centered research culture." Patient Research Ambassadors are patients, caregivers or lay people with a passion for clinical research. They want to help improve the way other patients are informed about research so that they have more opportunities and choices about participating in research studies as part of their NHS care. Maidstone and Tunbridge Wells NHS Trust is one of the first Trusts in England to have a Patient Research Ambassador volunteering for them. Hazel Everest, Research and Clinical Audit Manager at the Trust explains why these roles are beneficial to them: "The Patient Research Ambassador role is key to encouraging and supporting patient recruitment to trials and key to ensuring patients' research experience is a positive one. Patients enjoy and trust the support of a dedicated research volunteer through every stage of their research journey. This role is extremely valuable to the Trust as we strive to drive a research culture and ensure that all research opportunities for patients are exposed.

Initiative Goals

The main goal is about making research more visible within NHS organizations and making sure patients have the best NHS care which encompasses clinical research into the care package. We aim to support research ambassadors to conduct their role patient ambassador's role effectively in their community; we do this in various ways, such as highlighting training opportunities for them, for example online training on healthcare discovery and research. Our initiative is open to everyone and we now have roughly 5,000 people registered. Our goal is to help our ambassadors learn as much as possible from this role. We provide them with routes to finding better information.

Problems Addressed by Initiative

The main issue this initiative addresses is about raising the profile of clinical research and ensuring that clinical research is seen as part of the patients care package. We are working in collaboration with the NHS to introduce research as part of the patients care package by raising awareness of clinical research in the NHS.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: *https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-research-ambassadors-initiative*

Parkinson's Foundation
Parkinson's Advocates in Research

Overview

Despite promising research, there is neither a cure for Parkinson's nor are there medications to reverse its course. But there can be. We can bring about better treatments at a faster pace by ensuring that people with Parkinson's and care partners are primary partners in research alongside scientists, industry and government. As a signature program of the Parkinson's Disease Foundation (PDF), Parkinson's Advocates in Research (PAIR) is making this partnership a reality. We are bringing together the people who live with Parkinson's and the people who are developing new treatments.

How PAIR Works

- Through in-person trainings and an online course, the PAIR program provides people touched by Parkinson's with the knowledge and skills needed to pair up with scientists and health professionals.
- By collaborating with research institutions, the PAIR program facilitates partnerships between Research Advocates and professionals at the frontlines of research

Source: *http://www.pdf.org/pair*

Parkinson's UK
Patient and Public Involvement Program (PPIP)

About Initiative

This program was created to offer hands on support and advice for people affected by Parkinson's and researchers to work together to prioritize, design, manage and disseminate Parkinson's research. The program provides face-to-face training and resources for both people affected by Parkinson's and researchers to help prepare them to work together. Through the program Parkinson's UK have provided funding and facilitation for the initial face to face meeting as well as providing ongoing support for further collaboration between patients and researchers.

Both the training for people affected by Parkinson's and the training for researchers focus on the importance of the role of patients in shaping research as well as practical examples of how to work together. The training for people affected by Parkinson's also includes information about the research process, the pipeline to new treatments and ethical issues in research.

Initiative Goals

The goal is to ensure that people affected by Parkinson's are working in partnership with researchers in a meaningful way to make research more relevant, more likely to succeed and ensure that the benefits of the research are felt by the people who need it most, faster.

Problems Addressed by Initiative

It is now widely acknowledged that health research that is done in partnership with patients is more relevant and higher quality. However, researchers feel they need more support to work with patients in this way and patients can sometimes lack the confidence to know that their knowledge and experience is key to successful research. Parkinson's UK's PPIP helps overcome these barriers and ensure that both patients and researchers get the most out of their collaboration.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: *https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-and-public-involvement-programme-ppip*

Parkinson's UK
Research Support Network

About Initiative

The Research Support Network has a membership of 2300 people. Over 80% of the members are Parkinson's patients, and the remaining 20% includes carers, family members, health care professionals and researchers.

The purpose of the network is to connect people affected by Parkinson's across the UK to Parkinson's research news, events and opportunities to get involved. This includes both opportunities to participate in research as well as to work in partnership with researchers to shape vital Parkinson's research.

Initiative Goals

The goals of the network are to ensure that people affected by Parkinson's can access information and opportunities about Parkinson's research, to grow the number of people affected by Parkinson's participating in Parkinson's research and to bring the voice of people effected by Parkinson's to the center of Parkinson's research.

Problems Addressed by Initiative

The Research Support Network was created in 2010 as the result of a group of people affected by Parkinson's who felt that they wanted more of a 'say' in Parkinson's research. This group worked with Parkinson's UK staff members to create the Research Support Network and continue to work together today to grow and develop the network.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/research-support-network

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Parkinson's UK
Unmet Needs in Parkinson's Research

About Initiative

In 2014, Parkinson's UK led a Priority Setting Partnership to identify the areas of research that would have the greatest impact on the management of Parkinson's from the perspective of people with Parkinson's, their family and friends, caregivers and relevant healthcare professionals.
The Parkinson's UK Priority Setting Partnership asked 1,000 participants which areas of research they considered a priority for the management of Parkinson's.

The top 10 priority areas that people affected by Parkinson's and clinicians outlined were:

4. What treatments are helpful for reducing balance problems and falls in people with Parkinson's?
5. What approaches are helpful for reducing stress and anxiety in people with Parkinson's?
6. What treatments are helpful for reducing dyskinesias (involuntary movements, which are a side effect of some medications) in people with Parkinson's?
7. Is it possible to identify different types of Parkinson's, e.g., tremor dominant? And can we develop treatments to address these different types?
8. What best treats dementia in people with Parkinson's?
9. What best treats mild cognitive problems such as memory loss, lack of concentration, indecision and slowed thinking in people with Parkinson's?
10. What is the best method of monitoring a person with Parkinson's response to treatments?
11. What is helpful for improving the quality of sleep in people with Parkinson's?
12. What helps improve the dexterity (fine motor skills or co-ordination of small muscle movements) of people with Parkinson's so they can do up buttons, use computers, phones, remote controls etc?
13. What treatments are helpful in reducing urinary problems (urgency, irritable bladder, incontinence) in people with Parkinson's?

The top 10 is now used to inform, guide and drive future Parkinson's research, and to help researchers focus on the most important issues in improving everyday life for people affected by the condition.

Initiative Goals

To find out what questions in the day to day care and management of Parkinson's are most important to people affected by Parkinson's and clinicians.

Problems Addressed by Initiative

Beyond the search for a cure this Priority Setting Partnership highlights the research areas that would have the greatest potential impact on quality of life with Parkinson's and management of the condition as outlined by people affected by Parkinson's. These priority areas will help direct research efforts in improving everyday life with Parkinson's.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/unmet-needs-in-parkinson-s-research

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Patient Focused Medicines Development (PFMD)
SYNaPsE: Patient Engagement Mapping

Resources

- Collaborative Patient Engagement: Mapping the Global Landscape (White Paper)

The Challenge

Although initiatives that seek to involve and engage patients have increasingly become a priority across healthcare, there are no globally accepted guiding principles around patient involvement and engagement that identify and integrate good practices. As a consequence, current patient engagement is sporadic, fragmented and unstructured with no clearly defined framework or agreed process. Without such a framework, the ability of patient engagement activities to meet agreed and desired objectives will be compromised.

The Goal

PFMD's overarching goal is to work with patients and other stakeholders to co-create and drive implementation of an integrated, efficient, measurable and robust meta-framework to deliver a consistent approach to patient involvement.

Why develop an online collection tool

This online collection tool has been developed to gather data where there may be no documentation publicly available. A key feature of the tool is that information about each initiative will be entered by those directly involved – rather than relying on desk research alone – providing a greater opportunity to understand patient engagement efforts, including their successes and limitations. The tool captures quality data using pre-defined standards to allow for consistency in depiction and to ensure that the information collected across various initiatives is credible, consistent, and up to date.

The online collection tool in the framework development context

Medicines are developed to improve the lives of patients. Serving patients in the best way possible requires a deep understanding of their medical conditions, needs and priorities. This can be gained only through direct, sustained and constructive interactions with 'patients' - a definition that includes those with the medical condition and their family or careers. Health stakeholders agree that broadening patient engagement is key to improving drug development and providing solutions that achieve both clinical and patient-desired outcomes. As a result, there is an encouraging and increasing number of patient engagement (PE) initiatives that aim to integrate the patient voice in medicines development specifically, and in the healthcare arena generally.

However, current PE is sporadic, fragmented and unstructured with no clearly defined framework or agreed process. Without such a framework, the ability of PE activities to meet agreed and desired objectives will be compromised. What is needed is a consistent approach to PE, through development and implementation of an efficient, measurable and reliable meta-framework that involves patients as partners and is accepted and used by relevant stakeholders.

Despite the substantial increase in PE initiatives, there is currently no efficient mechanism for accessing information on what PE activities are ongoing or planned and to identify challenges encountered and lessons learned. Therefore, an essential first step in development of a meta-framework is to identify and 'map' existing initiatives and frameworks, allowing a 360-stakeholder view of the PE landscape. This will provide a platform for identifying gaps and synergies from different stakeholder perspectives and allow those committed to effective PE to learn from good practice by actively sharing experience and to connect. It also means not always having to start from scratch each time but instead there is a growth in expertise and knowledge that can be incrementally build upon.

Methodology

Preliminary Mapping

PFMD has deployed various methods to conduct a landscape assessment and obtain needed information on current PE initiatives. These are: a preliminary mapping of known initiatives; an extensive literature search; and

interviews with stakeholders across healthcare who have partnered with patients and/or provided guidance to partnering with patients. The methodology used in preliminary mapping of initiatives involved a search of initiatives underway in both formal and non-formal publications, journal articles, conference hearings, and word of mouth. The preliminary mapping identified a need to validate data with various organizations given the limited publications on this topic, the inconsistencies that may be presented in anecdotal presentations, and the wide spectrum of how organizations categorize "partnership with patients".

Online Collection Tool

Much information on PE is not routinely published but instead is shared in meetings and discussions, mandating a need for a pro-active collection process. The online collection tool has been developed to gather data where there may be no documentation publicly available. The online collection tool captures quality data using pre-defined standards to allow for consistency in depiction and will also help to ensure that the information collected across various initiatives is credible, consistent, and up to date.

Collecting and understanding initiatives by talking directly with those involved and requesting their input (rather than relying on desk research alone) provides a greater opportunity to understand efforts underway, including their successes and limitations. In addition, it allows identification of potential tools to measure the impact of patient engagement. Good practices of the individual efforts underway will ultimately be integrated into our global meta-framework, if applicable. Finally, this allows a connection or contact with all initiatives, allowing further collaboration from the meta-framework point of view but also the creation of a global network of PE for further practical PE between stakeholders.

Source: *https://involvement-mapping.patientfocusedmedicine.org/*

PatientsLikeMe
Clinical Trial Access and Protocol Optimization

About Initiative

Industry tends not to consult patients in the protocol development process. Many will look at what regulators have previously accepted into a trial, or translate lessons learnt from animal models into human trials. However, even at this phase, industry is starting to make decisions that will affect who will take part in the study - without consulting them. Trials would never be designed without consulting a statistician, but many don't know how to involve patients and wonder why clinical trial recruitment is hard or why drop out is high. We will support that process by taking clinical trial protocols and boil them down to what a patient might worry about. There are clearly parts in clinical trials that are inconvenient, not fun; every clinical trial will have a deal breaker. It could be lumbar punctures, you have to drive to Phoenix, stay over in hospital, no money is provided to pay for a caregiver's hotel, you will be reimbursed 90 days later, we will cut a piece out of you, you need to come off all meds a month before. These might be medicines that must be delivered carefully to a strict schedule. You have 100% control in pre-clinical animal research work, but it's a different matter when you come to human trials and people don't always behave as intended. The number of procedures in clinical trials has gone up 6% year on year. We take aspects of the trial design – feed them into a system and turn them into a concise series of questions to get quantitative and qualitative fields. We will show these to patients to determine which are the deal breakers to participating in the trial and to whom – which subsets of people are ruled out. We look at statistically meaningful set of patients. We also use conjoint analysis and pick out parts that are salient. Using a mathematical model and simulator we can determine, this is the percentage change in interest that this protocol would have. We can try to help pharma to make better decisions. In terms of ROI, we are looking at whether our feedback matches what the regulators come back and say on current projects. Protocol deviation can have, conservatively, half a million dollars in direct costs. Having that information earlier, or anything that can be done to increase recruitment, decrease attrition or provide missing data - can only be good.

Initiative Goals

To reduce burden on patients, increase clinical trial retention, reduce attrition, avoid protocol amends, increase the likelihood of regulatory approval and improve the reputation of companies that seek to incorporate patient input.

Problems Addressed by Initiative

Taking part in a clinical trial is burdensome. There can be missing data, low retention and recruitment. To make the process less burdensome, more relevant and reflect what is important to patients.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/clinical-trial-access-and-protocol-optimisation

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PatientsLikeMe
TrialMark: The Patient Experience Measurement Standard

About Initiative

TrialMark clinical trial services are pioneering the measurement of patient experience in clinical trials through the Patient Trial Experience Scale. Through an industry coalition led by PatientsLikeMe, and with industry leaders such as AstraZeneca and Takeda, this scale will be presented to patients at study start, mid-point and closeout, simultaneously reflecting ongoing patient feedback while generating data useful for improving future trial design and execution.

Initiative Goals

TrialMark aspires that all trials are measured accurately from the patient perspective using a single, openly available, universal tool, and that this data is pooled and disseminated in responsible ways to assist the entire industry to create better trials for patients. To achieve this goal, TrialMark has a three part plan:

1. Development of the Patient Trial Experience Scale, a common, universally applied measure for patient experience in trials that is open-source and free to use; design will be driven using robust psychometric principals and heavy patient engagement
2. Infrastructure to pool the patient experience data from multiple sponsor companies into a common, de-identified database
3. Analysis and tools to help benchmark experiences and derive actionable ideas and best practices for trial design

Problems Addressed by Initiative

Think of every retail transaction you've ever conducted recently: the Uber ride you took, the meal you ate at a mall, the product you purchased on Amazon. Each one of those benign transactions was the cumulation of a closely scrutinized process, in which an enormous amount of data is collected, KPIs are generated, and organizations benchmark and cross-compare against each other to make the experience as pleasurable for you, the customer.

Now think of the clinical trial process for a patient. Taking part in a clinical trial is a big ask; it can be like a part-time job and can often be an enormous physical and psychological burden. Given the stakes, there is an enormous gap in measuring and studying the patient's experience in this process. Without a method of measuring a patient's experience in a clinical trial, there is no clear way of understanding the drivers of patient interest and developing actionable insights to improve their experience. This measurement gap is what TrialMark intends to bridge.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/trialmark-the-patient-experience-measurement-standard

PatientsLikeMe
Patient Reported Outcomes Development

About Initiative

We have done a number of projects to develop Patient Reported Outcome Measures (PROMs) that are relevant to specific conditions and that help support the development of successful drugs and treatments. This is a big area. We often measure things that don't matter to people. The outcome measure for dementia is a mini mental state exam. You are asked what day it is, who the prime minister is. If someone you know has dementia, and you create a drug, how much do care if they know who the PM is? Why don't we build the clinical trial to test if they remember who you are? This is hard. People's memories fade in and out from time to time; and there are other factors; maybe they don't have close family, it is more variable. The mini mental state gives consistency, but it doesn't necessarily matter to anyone. For example, a person might go from not knowing what day or year it is, to getting the correct day but 50 years out. It might move them statistically significantly by one point. A drug might get approval on the basis that it makes a difference on this scale. But in the real world, people are less controlled, more challenging, can be generally sicker patients. We are then disappointed that the drugs don't work.

We look at patient or care giver reported surveys; the impact of symptoms on daily living, work and productivity. To what extent are you able to do usual activities, what are the side effects, how is your mood, are you productive, able to work? Yes these measures might be fuzzier than a blood pressure reading, but it is what matters to people. Really efficacious drugs have an impact on that type of stuff. We have developed PROs in around 20 different conditions. For example, a new pain scale for MS, ALS, diabetes, suicide ideation, Micosis Fungoides (MF). MF is lymphoma of the skin, which was measured using psoriasis measures. This was not appropriate. We developed a measure with patients. We looked at issues, fielded it to patients, whether it performed well or not. Does it hold together mathematically? Is it helpful in understanding a patient's management of that condition. Having a measurement of a condition can give more perception of control and help understand where a patient is at that moment in time, and what might have helped in the past.

Initiative Goals

Rapid development of measures acceptable to the U.S. Food and Drug Administration (FDA) and other regulatory bodies. To provide guidance on other PRO development that will allow developers to correctly identify the benefits and downsides medicines might have on those taking part in studies.

Problems Addressed by Initiative

To rapidly develop measures that matter to patients to use as outcomes and endpoints in trials. To help physicians and patients to measure their progress with their condition.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: ***https://involvement-mapping.patientfocusedmedicine.org/initiatives/pro-development***

Patient Centered Outcomes Research Institute (PCORI)
Patient Engagement Program

Resources

- Engagement Rubric for Applicants (updated June 6, 2014)
- Conceptual and practical foundations of patient engagement in research at the patient-centered outcomes research institute (Quality of Life Research; May 2015)
- Financial compensation of patients, caregivers, and patient/caregiver organizations engaged in PCORI-funded research as engaged research partners (June 10, 2015)
- Patient Ambassador Program
- PCORI Evaluation Framework 2.0
- What We Mean by Engagement: Engagement in Research

Overview

Since our establishment, PCORI has been committed to meaningful involvement by patients, caregivers, clinicians, and other healthcare stakeholders in all our activities, as well as throughout the research we fund. Bringing together all healthcare stakeholders—with patients at the center—to help set research priorities and evaluate applications is our formula for ensuring we fund and conduct the most relevant research possible.

We believe that including patients and other stakeholders in the research process, from topic selection through dissemination and implementation of results, will lead to trustworthy and usable information likely to be taken up in practice.

We have three engagement goals:

- Build a patient-centered outcomes research, or PCOR, community
- Engage the PCOR community in research
- Promote dissemination and implementation of PCOR research findings

Although staff throughout PCORI are committed to authentic involvement of patients and other stakeholders, our Engagement program focuses specifically on such engagement. The program reaches out to patients and other stakeholders with events and workshops, trainings, funding opportunities, and an Ambassador program, as well as helping stakeholders participate in research topic generation and selection, review of research funding applications, and dissemination of research findings. We also guide researchers on ways to include patients and other stakeholders in their projects.

Pfizer
Community Conversations

About Initiative

In the first of Pfizer Rare Diseases Community Conversations" series, this session focused on personal stories of those living with Friedrich's Ataxia (a rare neuro-muscular disorder). Patients, caregivers and scientist from a research advocacy organization engaged in a conversation with the research teams at a Pfizer research site. Patients demonstrated aspects of living with their illness through simulation techniques in addition to the dialogue.

Initiative Goals

To provide an interactive (non-confidential) platform for exchange and dialogue between Pfizer scientists, patients, advocates, clinicians/researchers, and caregivers, with a focus on a rare disease.
Obtain insights on the diagnostic journey, disease experience (burden), caregiver experience, basic science and clinical understanding in an illness where research was at the pre-discovery stage.

Problems Addressed by Initiative

Patient involvement in medicine development may inform research programs at the earliest stages.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development:
https://involvement-mapping.patientfocusedmedicine.org/initiatives/community-conversations

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Pfizer
Input on Clinical Trial Protocol

About Initiative

A few patient advocacy groups in the specific disease area were invited separately to look at a draft of the clinical trial protocol. The advocacy groups provided salient comments about feasibility, recruitment, retention, outreach, some of which was incorporated before the protocol was finalized.

Initiative Goals

The purpose was to have a targeted discussion to advance the clinical plan that would integrate, where possible, the insights from people with life experience with this illness.

Problems Addressed by Initiative

Clinical trials in a rare disease with high unmet medical need

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/input-on-clinical-trial-protocol

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Pfizer
Patient Involvement in Pfizer's External Bioethics Advisory Panel

About Initiative

Meetings of the Bioethics Advisory Panel cover topics such as ethical considerations and patients' rights in conducting clinical trials in developing areas; the role of accreditation in positioning research sites to conduct clinical trial; and how informed consent should be structured in an environment of broader clinical data sharing and access, including the use of biological data and material in research. As our aim is to advance patient centricity more systematically in everything we do at Pfizer, in 2015 we added a patient expert to serve as a standing member so that a representative patient view would be included in consideration of all topics brought to the panel.

Initiative Goals

Include a patient view in consideration of all topics brought to the external bioethics advisory panel.

Problems Addressed by Initiative

Pfizer's External Bioethics Advisory Panel is a small group of global ethics experts convened to provide insights on emerging medical, scientific and ethical issues globally, to help inform the company's clinical research planning and policies and ensure that the clinical trials Pfizer sponsors are conducted according to the highest ethical standards. The lens of a patient expert may provide a more inclusive perspective on these issues.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-involvement-in-pfizer-s-external-bioethics-advisory-panel

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Pfizer
Patient Member of External Review Panel for Independent Grants for Learning & Change

About Initiative

Pfizer employs a Request for Proposal (RFP) model for grants and includes External Review Panels whose charge is to review, evaluate and ultimately approve or deny submitted Letters of Intent and proposals. Panel members may also consult on the development of RFP's.
Membership of the External Review Panels consists of professionals from the healthcare community with advanced degrees and expertise in a particular clinical area, or expertise in education, professional development, quality improvement and/or Public Health Administration. In 2016 Pfizer began including patient experts on these panels and aims to have patient representation on each of these panels.

Initiative Goals

Include patient representation in the review, approval and consultation on grants submitted and to provide insights on the review process.

Problems Addressed by Initiative

The mission of Pfizer's office of Independent Grants for Learning & Change (IGLC) is to partner with the global healthcare community to improve patient outcomes in areas of mutual interest though support of measurable learning and change strategies. The review process and decisions could potentially be enhanced with the involvement of patient experts.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-member-of-external-review-panel-for-independent-grants-for-learning-change

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Pfizer
Protocol Review

About Initiative

Pfizer advocacy colleagues asked a few patient advocacy groups to recommend patient members who potentially could review a clinical trial protocol. The patient groups then connected those patients with Pfizer colleagues who held phone call with patients to explain the process. With Confidential Disclosure Agreements in place, virtual meetings were held- the first to describe the protocol which was then sent to the patients via email, and the second with patients and Pfizer clinicians to discuss collated comments. Feedback was also provided from the team to the advocates about what was learned and after further review, where possible, about how and why their comments may or may not be incorporated in the protocol.

Initiative Goals

To gain feedback on patient reported outcome (PRO) instruments and study feasibility, particularly recruitment and retention.

Problems Addressed by Initiative

Protocol feasibility for participants.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/protocol-review

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Pharmerit
Conducting Rigorous Qualitative Research with and for Patients to Provide Novel Patient-Centered Perspectives and Insights on Diseases And Treatments

Initiative Goals

The goal of this initiative was to scientifically collect and report this unique perspective that the patients (and caregivers) offer to describe essential evidence in the drug development process and HTA decisions. We gathered key outcomes such as the impact of the disease on day-to-day life, limitations of existing treatments, expectations from new technology and the most meaningful outcomes for patients and their caregivers. Benefits and unwanted effects experienced with the new technology, the balance between unwanted effects and benefits, and the importance of these effects for the patient / caregiver were also elicited.

Problems Addressed by Initiative

Our initiative addresses two main issues: 1/the lack of patient/caregiver perspective in the drug development and HTA decision-making processes; and 2/the lack of rigorous and scientific methods to obtain and disseminate patient perspective.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/

Roche
Co-creation Workshop

About Initiative

Patients, HCPs and study coordinators convened to deliver personal and clinical insights related to an in-development clinical study protocol.

Initiative Goals

The goal was to improve retention and recruitment for a specific trial, by asking patient advisors to provide feedback regarding barriers related to the study protocol. Over the course of a full day session, the team generated solutions intended to eliminate potential barriers. The co-created solutions the group developed are informing critical changes to overall study design and execution that will help lead to better recruitment and retention strategies for patients and research sites

Problems Addressed by Initiative

Incorporating patient perspective in study protocol design

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/co-creation-workshop

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Roche
On-line virtual advisory board for patient input into protocol design

About Initiative

Integrating the patient perspective in our trials is important. In this on-line virtual advisory board, we brought together patients/parents from around the world to gain their input on specific trial related aspects.

Initiative Goals

To ensure that the patient perspective was integrated into our trial program, also attempting to address recruitment and retention.

Problems Addressed by Initiative

1. Optimizing the protocol from a patient perspective
2. Addressing recruitment and retention in our program

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/on-line-virtual-advisory-board-for-patient-input-into-protocol-design

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Roche
Patient Advisor Group

About Initiative

A group of three breast cancer patient advocates were selected to advise a study team over a set period of time. The study team met with the advocates to obtain input on their study protocol design and worked continuously with individual advocates as additional questions came up during the course of protocol design.

Initiative Goals

The goal was to seek patient advocate advice on key components of the protocol design, and ensure that the advocate group was available to study teams as advisors for ongoing conversations.

Problems Addressed by Initiative

Incorporating patient perspective in study protocol design

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-advisor-group

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Roche
Patient and Caregiver Interviews

About Initiative

A group of 30+ patients, caregivers and nurses were interviewed by phone regarding their experience and attitude towards clinical trials, and barriers to participation and retention.

Initiative Goals

The purpose of these interviews was to understand the clinical trial experience from the perspective of Alzheimer's patients and caregivers in order to improve patient experience and bolster trial retention.

Problems Addressed by Initiative

Incorporating patient perspective in improving study operations

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-and-caregiver-interviews

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Sanofi
Patient Engagement Portal

About Initiative

The Patient Engagement Portal was established in 2014. The goal, identified through internal interviews and surveys, was to improve the process of patient engagement across the organization by facilitating communication between the right areas of the business. Anyone in Sanofi who wants to have a meaningful patient engagement can request that through a web-based system. It connects them to internal advocacy experts and ensures they are engaged in the process. There are built-in rules that determine what stage of the process, what questions need to be answered and it will connect them to the right patient or patient group. It achieves two things: 1) more access to patient engagement and 2) utilizes the right teams with the right expertise. Patients need to be meaningfully engaged and interactions must be bi-directionally beneficial. We should now start to see medicines more directed toward patient needs, gaps in care and practicing clinicians views.

Initiative Goals

The goal is to achieve two things: 1) more access to patient engagement and 2) utilizes the right teams with the right expertise. Patients need to be meaningfully engaged and interactions must be bi-directionally beneficial. We should now start to see medicines more directed toward patient needs, gaps in care and practicing clinicians views.

Problems Addressed by Initiative

How do we make patient engagement part of the culture and accessible to the organization so the process is in place to meaningfully engage?

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-engagement-portal-1

Sanofi
Medical Intelligence and Patient Perspectives Group

About Initiative

"The Medical Intelligence and Patient Perspectives Group was established to evaluate disease areas of interest and provide teams with a deeper understanding of patient unmet need and the potential medical value of new interventions. Specifically, it engages patients to understand their perspectives regarding standard of care and to identify where they feel gaps exist. This is alongside the views of practicing healthcare providers to understand more fully what characteristics of a new agent will be required to be have a successful impact on the standard of care. Through a multi-pronged analysis, it provides teams with feedback that can change the direction of their work, and assists in developing assets with characteristics needed for successful entry into the treatment continuum. We 'front load' this analysis into the very early stages of research and development.

Initiative Goals

The main goal is to make patient engagement actionable and meaningful across the medicine development lifecycle and to involve patients from the very early stages of R&D.

Problems Addressed by Initiative

To operationalize patient engagement so it is not just information accumulation alone. To ensure there is a reproducible and sustainable method of engagement as well as a "vehicle" to make actionable the information in order to have meaningful impact on how new medicines are developed.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/medical-intelligence-and-patient-perspectives-group

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Takeda
R&D Patient Engagement Strategy and Key Priorities

About Initiative

Implementing a Patient Engagement strategy to change the way we work in R&D by anabling and empowering R&D project teams to incorporate the patient into the development of our medicines and therapies.

Initiative Goals

Our R&D strategy defined key priorities to be implemented in all phases of R&D:

1. Research
2. Clinical Development
3. Culture
4. External partnerships

Problems Addressed by Initiative

Takeda R&D will realize its shift in focus moving from a traditional R&D approach of developing medicines FOR patients to developing medicines WITH patients. Our goal is to *partner* with patients throughout the drug life cycle

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/r-d-patient-engagement-strategy-and-key-priorities

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Transcelerate
Patient Engagement and Experience

Rationale

Currently, there is no method to measure the patient experience in clinical trials, and Sponsor companies do not have available to them any examples or models for translating patient engagement into actionable insights that could help improve clinical trial design and trial execution ultimately to achieve a better patient experience. The Patient Experience Initiative is in the process of developing tools for clinical teams to engage patients in the study design and execution stages of clinical trials and increase the patient centricity of study programs. Ultimately, these tools will enable greater patient engagement and partnership with sponsors to design and execute clinical protocols that create better patient experiences in clinical trials.

Benefits

Benefits for Patients:

- Increased engagement through better communication and feedback processes
- Increased understanding of the value in participating in clinical trials
- Potential increase in the sense of altruism due to the confidence of knowing that their participation and feedback in trials may improve future study volunteers' experiences
- Potential decrease in the burden of participating in clinical trials

Benefits for Sponsors, Sites, and Investigators:

- Potential improvement in patient recruitment, retention, and adherence within clinical trials
- Potential reduction in long term costs through more effective patient engagement

Available Assets

The following assets will be delivered by this Initiative:

- Patient Protocol Engagement Toolkit (PPET): Toolkit for sponsor study teams to facilitate discussions with patient advisory group participants through the capture and incorporation of patient insights to optimize clinical protocols during the study design phase.
- Patient Experience Questionnaire Toolkit (PEQ): Toolkit for sponsor study teams to gain clinical trial participants' feedback on their trial experience during the study conduct phase.

Source: Synapse for Patient Engagement by Patient Focused Medicines Development: https://involvement-mapping.patientfocusedmedicine.org/initiatives/patient-engagement-and-experience

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Unitio
Community Application for Research and Engagement™ (CARE) Platform

Overview

Unitio's CARE platform is a proprietary real-world patient engagement platform designed to enable peer-to-peer support for people touched by disease while providing them with the opportunity to offer insights and engage in research opportunities.

It is a secure, turnkey platform that enables a community to connect, share and participate in research. CARE provides a strong technology foundation and customizable tools that enable organizations with a disease- or

health condition-specific focus a way to gather, analyze and disseminate important patient-reported health data to advance research. In addition to participating in real-time research projects and studies, the platform brings together patients and caregivers in an engaging way to support, empower, and educate one another.

Source: http://www.transceleratebiopharmainc.com/initiatives/patient-experience/

University of Maryland – Center for Excellence in Regulatory Science and Innovation
Assessing Meaningful Patient Engagement in Drug Development: A Definition, Framework, and Rubric

Overview

A movement to include the patient voice in health care research and decision making is underway. In light of broad stakeholder interest in patient-focused drug development (PFDD), a range of stakeholders are considering approaches to increase the scope of PFDD and enhancing patient engagement. On March 9, 2015, the University of Maryland Center of Excellence in Regulatory Science and Innovation (M-CERSI), with the support of many partner organizations, held the "M-CERSI Conference on Patient Focused Drug Development." The objective was to allow stakeholders from patient groups, the US Food and Drug Administration (FDA), the biopharmaceutical industry, payer, and other organizations to voice their views on, activities in, and aspirations for PFDD. During the day-long program, participants discussed the challenges to successful PFDD including regulatory challenges, the patient and patient advocate role, the emerging payer role, along with future directions and opportunities for collaboration. This document summarizes the outputs of the conference including a suggested definition, rubric, and framework for PFDD.

Source:
http://www.pharmacy.umaryland.edu/media/SOP/wwwpharmacyumarylandedu/centers/cersievents/pfdd/mcersi-pfdd-framework-rubric.pdf

Forum Background Information

ABOUT THE FORUM

Forum on
DRUG DISCOVERY, DEVELOPMENT,
and TRANSLATION

The Forum on Drug Discovery, Development, and Translation (Forum) of the National Academies of Sciences, Engineering, and Medicine (National Academies) was created in 2005 by the National Academies' Board on Health Sciences Policy to provide a unique platform for dialogue and collaboration among thought leaders and stakeholders in government, academia, industry, foundations, and patient advocacy with an interest in improving the system of drug discovery, development, and translation. The Forum brings together leaders from private sector sponsors of biomedical and clinical research, federal agencies sponsoring and regulating biomedical and clinical research, the academic community, and patients, and in doing so serves to educate the policy community about issues where science and policy intersect. The Forum convenes several times each year to identify, discuss, and act on key problems and strategies in the discovery, development, and translation of drugs. To supplement the perspectives and expertise of its members, the Forum also holds public workshops to engage a wide range of experts, members of the public, and the policy community. The Forum also fosters collaborations among its members and constituencies. The activities of the Forum are determined by its members, focusing on the major themes outlined below.

INNOVATION & THE DRUG DEVELOPMENT ENTERPRISE

Despite exciting scientific advances, the pathway from basic science to new therapeutics faces challenges on many fronts. New paradigms for discovering and developing drugs are being sought to bridge the ever-widening gap between scientific discoveries and translation of those discoveries into life-changing medications. There is also increasing recognition of the need for new models and methods for drug development and translational science, and "precompetitive collaborations" and other partnerships, including public–private partnerships, are proliferating. The Forum offers a venue to discuss effective collaboration in the drug discovery and development enterprise and also hosts discussions that could help chart a course through the turbulent forces of disruptive innovation in the drug discovery and development "ecosystem."

SCIENCE ACROSS THE DRUG DEVELOPMENT LIFECYCLE

Key gaps remain in our knowledge about science, technology, and methods needed to support drug discovery and development. Recent rapid advances in innovative drug development science present opportunity for revolutionary developments of new scientific techniques, therapeutic products, and applications. The Forum provides a venue to focus ongoing attention and visibility to these important drug development needs and facilitates exploration of new approaches across the drug development lifecycle. The Forum has held workshops that have contributed to the defining and establishment of regulatory science and have helped inform aspects of drug regulatory evaluation.

CLINICAL TRIALS & CLINICAL PRODUCT DEVELOPMENT

Clinical research is the critical link between bench and bedside in developing new therapeutics. Significant infrastructural, cultural, and regulatory impediments challenge efforts to integrate clinical trials into the health care delivery system. Collaborative, cross-sector approaches can help articulate and address these key challenges and foster systemic responses. The Forum has convened a multiyear initiative to examine the state of clinical trials in the United States, identify areas of strength and weakness in our current clinical trial enterprise, and consider transformative strategies for enhancing the ways in which clinical trials are organized and conducted. In addition to sponsoring multiple symposia and workshops, under this initiative, the Forum is fostering innovative, collaborative efforts to facilitate needed change in areas such as improvement of clinical trial site performance.

INFRASTRUCTURE & WORKFORCE FOR DRUG DISCOVERY, DEVELOPMENT, AND TRANSLATION

Considerable opportunities remain for enhancement and improvement of the infrastructure that supports the drug development enterprise. That infrastructure, which includes the organizational structure, framework, systems, and resources that facilitate the conduct of biomedical science for drug development, faces significant challenges. The science of drug discovery and development, and its translation into clinical practice, is cross-cutting and multidisciplinary. Career paths can be opaque or lack incentives such as recognition, career advancement, or financial security. The Forum has considered workforce needs as foundational to the advancement of drug discovery, development, and translation. It has convened workshops examining these issues, including consideration of strategies for developing a discipline of innovative regulatory science through the development of a robust workforce. The Forum will also host an initiative that will address needs for a workforce across the translational science lifecycle.

Forum on Drug Discovery, Development, and Translation

Russ Altman (Co-Chair)
Stanford University

Robert Califf (Co-Chair)
Duke University and Verily Life Sciences

Christopher Austin
National Center for Advancing Translational Sciences, NIH

Linda Brady
National Institute of Mental Health, NIH

Tanisha Carino
FasterCures, a Center of the Milken Institute

Allison McElvaine
American Diabetes Association

Lori Dodd
National Institute of Allergy and Infectious Diseases, NIH

James Doroshow
National Cancer Institute, NIH

Jeffrey Drazen
New England Journal of Medicine

Steven Galson
Amgen Inc.

Carlos Garner
Eli Lilly and Company

Julie Gerberding
Merck & Co., Inc.

Lynn Hudson
Critical Path Institute

S. Claiborne (Clay) Johnston
University of Texas, Austin

Gregory Keenan
AstraZeneca

Rusty Kelley
Burroughs Wellcome Fund

Katharine Knobil
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Freda Lewis-Hall
Pfizer Inc.

Ross McKinney
Association of American Medical Colleges

Bernard Munos
InnoThink Center for Research in Biomedical Innovation

Michael Severino
AbbVie, Inc.

Rachel Sherman
Office of the Commissioner, U.S. FDA

Ellen Sigal
Friends of Cancer Research

Lana Skirboll
Sanofi

Brian Strom
Rutgers, The State University of New Jersey

Amir Tamiz
National Institute of Neurological Disorders and Stroke, NIH

Pamela Tenaerts
Clinical Trials Transformation Initiative, Duke University

John Wagner
Takeda Pharmaceuticals

Joanne Waldstreicher
Johnson & Johnson

Carrie Wolinetz
Office of Science Policy, NIH

Janet Woodcock
Center for Drug Evaluation and Research, U.S. FDA

Project Staff

Carolyn Shore, Ph.D.
Forum Director
cshore@nas.edu
202-334-2669

Rebecca English, M.P.H.
Program Officer

Amanda Wagner Gee, M.S.
Program Officer

Morgan Boname, M.S.
Program Officer

Melvin Joppy
Senior Program Assistant

For more information, please visit:

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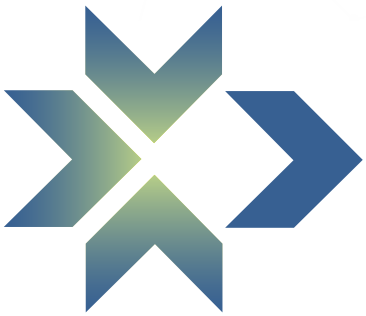


Forum on DRUG DISCOVERY, DEVELOPMENT, and TRANSLATION

Activities, Products, Impact

ABOUT THE FORUM

The Forum on Drug Discovery, Development, and Translation (the Forum) of the National Academies of Sciences, Engineering, and Medicine (the National Academies) was created in 2005 by the National Academies Board on Health Sciences Policy. The Forum provides a unique platform for dialogue and collaboration among thought leaders and stakeholders in government, academia, industry, foundations, and disease and patient advocacy with an interest in improving the system of drug discovery, development, and translation, educating the policy community about issues where biomedical science and policy intersect.

Members of the Forum convene several times each year to identify, discuss, and act on key problems and strategies in the discovery, development, and translation of drugs. To supplement the perspectives and expertise of its members, the Forum holds public workshops to focus substantial public attention on critical areas of drug discovery and development. Proceedings of these meetings are disseminated to the public. In addition, the Forum commissions or fosters the development of papers to explore scientific and policy issues on selected topics. The Forum also fosters collaborations among its members and constituencies.

The overarching theme underpinning all of the Forum's activities, workshops, action collaboratives, and publications is to understand and improve the system of drug discovery, development, and translation in a rapidly changing scientific and regulatory environment, in order to improve the public's health.

THEMATIC PRIORITIES

- Innovation & the Enterprise
- Science Across the Lifecycle
- Clinical Trials & Product Development
- Infrastructure & Workforce

ACTIVITIES

- Forum Meetings
- Workshops
- Action Collaboratives

PRODUCTS

- Workshop Proceedings
- Discussion Papers & Commentaries
- Independent Journal Articles

STRATEGIES

- Facilitate visibility & continuous discussion
- Foster collaboration
- Explore innovative & transformative approaches
- Educate & elevate understanding of issues
- Clarify & expand areas of agreement
- Set the stage for policy action

MEMBERS

- Federal Research & Regulatory Agencies
- Biopharmaceutical Companies
- Academic Experts
- Patient- & Disease-Focused Organizations
- Foundations & Nonprofit Funders
- Consortia & Associations

THEMATIC PRIORITIES

Innovation and Reform of the Drug Discovery and Development Enterprise

Despite exciting scientific advances, the pathway from basic science to new therapeutics faces challenges on many fronts. Innovative paradigms for discovering and developing drugs are being sought to bridge the ever-widening gap between scientific discoveries and translation of those discoveries into life-changing medications. There is also increasing recognition of the need for new models and methods to advance drug discovery, development, and translational science. To address these needs, "precompetitive collaborations" and other partnerships, including public–private partnerships, are proliferating. **The Forum offers a venue to discuss effective collaborative opportunities across the drug discovery and development enterprise and hosts discussions that could help chart a course through the turbulent forces of disruptive innovation in the drug discovery and development "enterprise."**

Science Across the Drug Discovery and Development Lifecycle

Key gaps remain in our knowledge about the science, technology, and methods needed to support drug discovery and development. Recent rapid advances in the science of drug discovery and development present opportunity for revolutionary developments of new scientific techniques, as well as therapeutic products and applications. **The Forum provides a venue to focus ongoing attention and visibility on these important needs and facilitates exploration of new approaches across the lifecycle of drug discovery and development. The Forum has held workshops that have contributed to the defining, establishment, and refinement of regulatory science and have helped inform aspects of drug regulatory evaluation.**

Clinical Trials and Clinical Product Development

Clinical research is a critical link between bench and bedside in developing new therapeutics. Significant infrastructural, cultural, and regulatory impediments challenge efforts to integrate clinical trials into the health care delivery system. Collaborative, cross-sector approaches can help articulate and address these key challenges and foster systemic responses. **In addition to sponsoring symposia and workshops to examine the state of clinical trials in the United States, the Forum fosters innovative, collaborative efforts to facilitate needed change in areas such as improvement of clinical trial site performance and sharing of clinical trial data.**

Infrastructure and Workforce for Drug Discovery, Development, and Translation

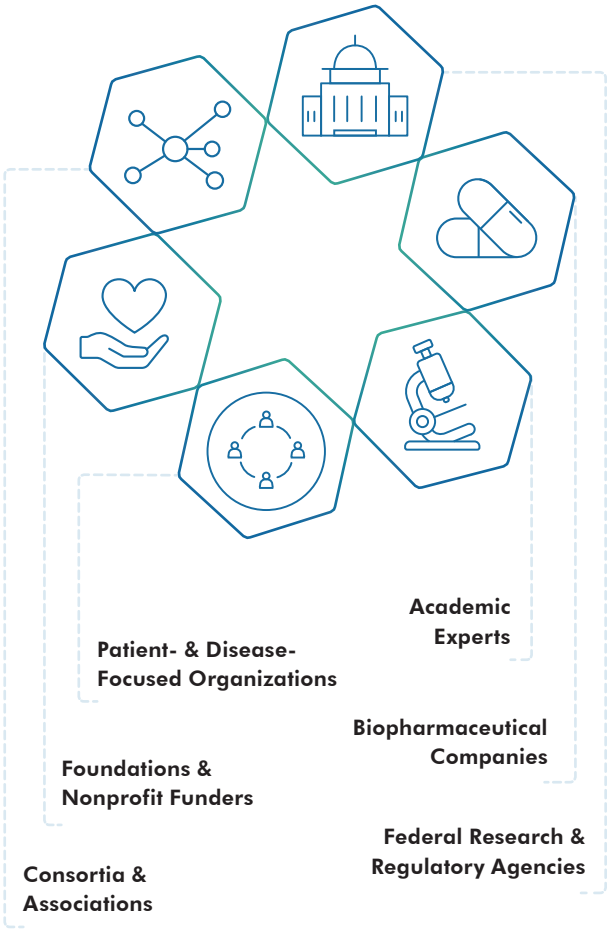
Considerable opportunities remain for enhancement and improvement of the infrastructure that supports the drug discovery and development enterprise. That infrastructure, which includes the organizational structure, framework, systems, and resources that facilitate the conduct of biomedical science for drug development, faces significant challenges. The science of drug discovery and development, and its translation into clinical practice, is cross-cutting and multidisciplinary. Career paths can be opaque or lack incentives such as recognition, career advancement, or financial security. **The Forum has considered workforce needs as foundational to the advancement of drug discovery, development, and translation. It has convened workshops examining these issues, including consideration of strategies for developing a discipline of innovative regulatory science through the development of a robust workforce.**

STRATEGIES

- Facilitate visibility and continuous discussion on important issues
- Foster collaboration on topics where there is synergy among potential partners
- Explore innovative and transformative approaches for resolving problem areas
- Educate and elevate understanding of issues
- Clarify and expand areas of agreement
- Set the stage for future policy action

Topics addressed by the Forum span a broad range of issues in drug discovery, development, policy, and practice. In providing a venue for independent, systematic discussions of these issues, the Forum employs the above strategies.

MEMBERS



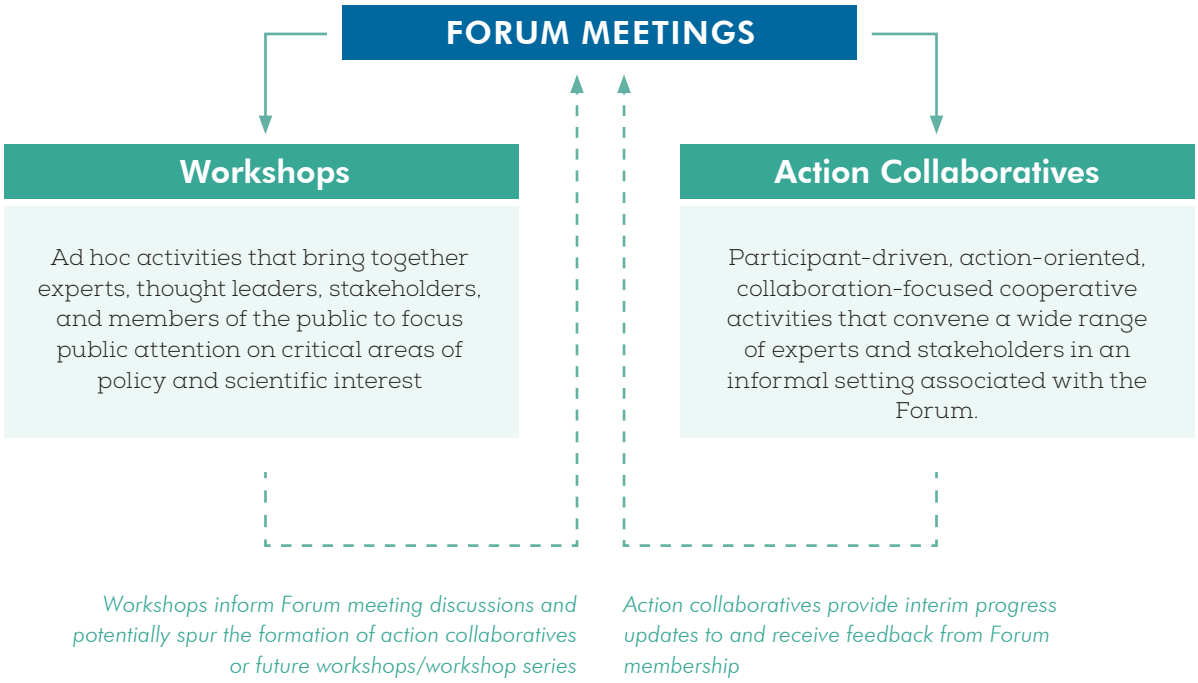
The Forum has approximately 30-35 members, identified for their professional and expert perspective as well as their technical and/or public policy credentials. Expertise includes basic and clinical biomedical research, therapeutics development, health administration and policy, and patient/disease advocacy. Additional experts are invited to participate in particular Forum discussions and other activities on an ad hoc basis.

ACTIVITIES

Members of the Forum convene several times each year to identify, discuss, and act on key problems and strategies in the discovery, development, and translation of drugs. To supplement the perspectives and expertise of its members, the Forum holds public workshops to focus substantial public attention on critical areas of scientific and policy interest. The Forum also fosters action-oriented collaboratives that engage a wide range of experts, the policy community, and members of the public in an informal, neutral setting.

Forum Meetings

Building on priorities identified by Forum members during the course of Forum meetings, the Forum membership envisions future workshops and collaborative activities.

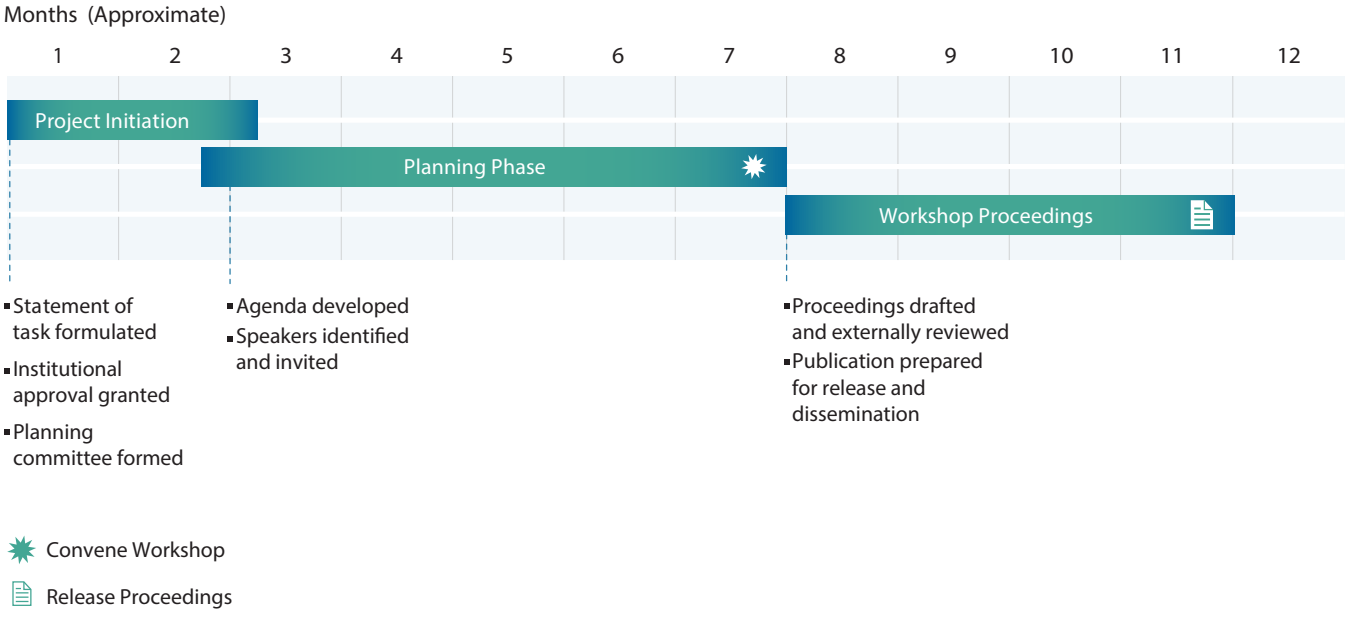


Workshops

Activity Goals
Public workshops convene experts, thought leaders, stakeholders, and members of the public from across the drug R&D and regulatory ecosystem to report on the current knowledge in an area, highlight and elucidate problems, focus substantial public attention on critical areas of scientific and policy interest, or discuss the progress of policy change following the release of a National Academies consensus study report.

Process
Workshops are organized by planning committees, which may include Forum members. Over the course of five to six months, the planning committee convenes to scope and plan the workshop (see the figure below). Forum staff facilitate convening the planning committee and promoting the workshop to relevant stakeholders and the public. Workshops typically result in the publication of a workshop proceedings (find out more under "Products").

Outcomes
Workshop proceedings are published to communicate the information and dialogue presented at the workshop to a wider audience. Workshops may result in the formation of action collaboratives, follow-on discussion papers or commentaries, a workshop series, or spin-off consensus studies.



Action Collaboratives

Activity Goals

The Forum fosters action collaboratives to engage participants with similar interests and responsibilities in cooperative activities that analyze in-depth high-priority issues and advance identified goals of the Forum and progress on recommendations highlighted in previous National Academies consensus reports. Collaboratives are participant-driven, action-oriented activities that foster collaboration and information sharing among Forum members and external thought leaders and stakeholders. Collaboratives may:

- identify issues of common interest and marshal needed leadership, expertise, and resources;
- highlight potential paths forward through, e.g., individually authored (or small group) literature summaries or discussion papers or through the convening of technical discussions;
- engage in cooperative development of tools needed for progress;
- incubate and pilot-test novel approaches; and
- develop proposals for formal workshops and studies for consideration by the National Academies.

Each action collaborative is an ad hoc activity associated with the Forum at the National Academies. The work it produces does not necessarily represent the views of any one organization, the Forum, or the National Academies and has not been subjected to the review procedures of, nor is it a report or product of, the National Academies.

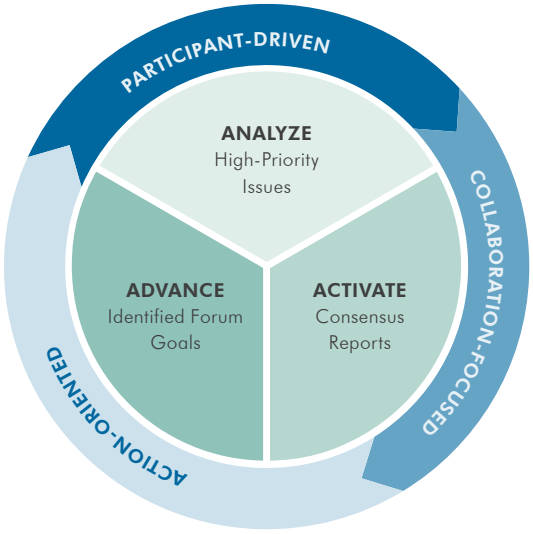
Process

Collaboratives meet under the auspices of the Forum. They vary in structure and content to meet the specified needs and challenges identified by their participants. Collaborative participants and their colleagues often provide in-kind support (such as human resources) to further the Collaborative's goals, with support and facilitation by Forum staff. The timeline and frequency with which Collaboratives meet is determined by its participants; however, many Collaboratives meet for an average of 1.5–2 years, hold regular teleconferences, and meet in person several times.

Impact/Reach

Collaboratives offer considerable flexibility for their participants to pursue a wide range of activities and products and the opportunity to have an impact on the field. Products are ascribed to the Collaborative participants and are not products of the National Academies, and endorsement and use of the Collaborative's work is at the discretion of its participants' individual organizations. Examples of impact/reach might include:

- advancing progress on recommendations highlighted in previous National Academies reports;
- developing papers authored by individual participants in the Collaborative;
- submitting journal articles to independent publications;
- spurring formation of public–private partnerships; and
- envisioning a charge for a planning committee to convene a workshop or a consensus study.



PRODUCTS

Workshop Proceedings

Workshops often result in a Proceedings of a Workshop (or Proceedings of a Workshop–in Brief). A workshop proceedings is written as a direct account of the workshop discussions and dialogue by National Academies staff and/or hired consultants, serving as rapporteurs. Members of the Forum and of the planning committee may not serve as rapporteurs. A workshop proceedings is reviewed by a diverse range of experts, which may include Forum members.

Perspectives: Discussion Papers and Commentaries

Authored discussion papers and commentaries (collectively termed Perspectives) can disseminate information or further elucidate topics covered in Forum discussions, workshops, or action collaboratives. Perspectives are distributed by the National Academy of Medicine (NAM) and represent the views of the authors (not necessarily of the authors' organizations, the NAM, or the National Academies). These papers are not subject to the review procedures of the National Academies. All discussion papers and commentaries are designed to be shared publicly.

Independent Journal Articles and Other Publications

The Forum may commission or foster the development of papers to undertake new research, as well as synthesize existing literature and explore scientific and policy issues on selected topics, through the publication of journal articles in independent publications or consultant-developed documents.

IMPACT

Foster Relationships and Collaborations

By bringing together a diverse group of stakeholders several times a year, the Forum fosters new professional relationships, facilitates cross-sector collaborations, and enables professional development and networking.

Spark Ideas and Actions

The Forum encourages the open sharing of diverse perspectives and the interchange of ideas among multidisciplinary stakeholders. Through these interactions and connections, participants in Forum activities often generate novel ideas and approaches to overcome existing challenges, spurring progress and inspiring action.

Inform Policies and Programs

The discussions and products arising from Forum activities can inform policy and legislation; programmatic planning, direction, and budgets; educational initiatives, such as curricula and training programs; and activities such as demonstration projects.

Shape the Field

The dialogue and ideas generated during the convening activities of the Forum and captured in related products reach varied, multidisciplinary audiences interested in health and medicine. The Forum helps to advance and shape the field by framing issues, shining a light on important topics, and fostering the cultivation of new leaders.

The National Academies of
SCIENCES • ENGINEERING • MEDICINE

ROUNDTABLES AND FORUMS

IN THE HEALTH AND MEDICINE DIVISION

nationalacademies.org/HMD

ROUNDTABLES AND FORUMS IN THE HEALTH AND MEDICINE DIVISION:

ADVANCING THE DISCUSSION AND FOSTERING DIALOGUE ON CRITICAL HEALTH TOPICS

The National Academies of | SCIENCES ENGINEERING MEDICINE

The National Academies of Sciences, Engineering, and Medicine ("the National Academies") provide independent, objective analysis and advice to the nation, and conduct other activities to solve complex problems and inform public policy decisions. The National Academies also encourage education and research, recognize outstanding contributions to knowledge, and increase public understanding in matters of science, engineering, and medicine.

The Health and Medicine Division (HMD) is a program unit of the National Academies. The aim of HMD is to help those in government and the private sector make informed health policy decisions by providing evidence upon which they can rely. HMD advises the nation through consensus committees but also provides opportunities for open dialogue on complex and diverse topics through roundtables and forums.

Representatives from government, private businesses, academia, and other stakeholder groups gather regularly on neutral ground in order to identify and discuss contemporary issues of mutual interest and concern. Roundtables and forums cover a range of topics, including health care at the local and global levels, health literacy, health equity, health professional education, obesity solutions, violence prevention, and medical and public health preparedness.

Contact HMD:

HMD-NASEM@nas.edu | (202) 334-2352

nationalacademies.org/HMD

ROUNDTABLES AND FORUMS

Roundtables and forums create communal environments to foster dialogue across sectors and institutions. Although roundtables and forums do not produce solutions themselves, they illuminate issues that need to be resolved, and opportunities for further work often developed from their meetings, workshops. and publications. For example, the activities of a roundtable or forum may result in the establishment of separate consensus study committee.

Unlike a consensus committee, which publishes a report with conclusions and recommendations, a roundtable or forum may not issue work with such advice.

ROUNDTABLE AND FORUM MEMBER SELECTION

Usually, roundtable and forum members are selected based on each individual's expertise, but other considerations may be a factor. Since roundtables and forums do not give advice, their membership is not restricted with regard to financial or other types of bias and conflicts of interest.

The membership of a roundtable or forum is approved by the HMD Executive Office and appointed by the chair of the National Academies for three years (or a shorter duration, depending on the activity). Government officials from sponsoring agencies are appointed on an ex officio basis upon the recommendation of their agencies, and the length of their service will match the length of their term in office. Nongovernmental membership appointments to the roundtable or forum may also be considered ex officio if they are by virtue of the office in a professional society, corporation, or other independent organization—particularly if the sponsoring organization chooses the person and office to be on the roundtable or forum.

ROUNDTABLE AND FORUM ACTIVITIES

Roundtables and forums host a number of activities such as discussion meetings, workshops, and symposia. Within the scope of their approved topic, roundtables and forums are self-governing in that, for example, they decide their own agendas for meetings. A chair, who presides at the meetings, is nominated by HMD and appointed by the chair of the National Academies, just as the members are.

Because they do not give advice, roundtables, forums, and their activities are not subject to Section 15 of the Federal Advisory Committee Act, an act that guarantees independence from government interests and necessitates disclosure of all reference materials to the public.

However, roundtable and forum meetings and workshops are announced on the HMD website in advance and are open to the public, except in two cases: if the meeting includes only members and is dedicated to administrative matters, or if the meeting will discuss issues described in U.S. Code Title 5 Section 552(b). Under this law, closed meetings may be held if the discussion delves into such topics as security, privacy, or legal matters.

Roundtables and forums often use authored background papers or workshops to help inform their discussions. These follow the same rules of public access as above. Workshops are organized by planning committees, which may include roundtable or forum members. A roundtable or forum member may also serve as a speaker at a workshop.

PLANNING COMMITTEES

Planning committees develop workshop agendas for roundtables and forums and are not subject to the same rules and limitations placed on study committees. However, all planning committee members must complete bias and conflict of interest forms, which ask about affiliations and opinions, and they must also participate in bias and conflict of interest discussions.

Potential sources of bias usually relate to individuals holding positions that arise from the close identification or association with a particular point of view.

Most, if not all, planning committee members will have some level of intellectual bias in relation to a particular topic, but those biases should be declared. An ideal planning committee will represent a balance of positions. In the face of evidence, an ideal member of a planning committee will be able to engage in dialogue with others and consider adopting a new point of view.

INNOVATION COLLABORATIVES

Roundtables and forums may establish innovation collaboratives—also called action collaboratives— to engage participants with similar interests and responsibilities in cooperative activities to advance aspects of each roundtable or forum's statement of task. These ad hoc convening activities foster information sharing and collaboration toward roundtable and forum aims as well as evaluation on progress on findings and recommendations highlighted in prior National Academies reports.

PUBLICATIONS

If a roundtable or forum holds a workshop, this workshop may result in a Proceedings of a Workshop or a Proceedings of a Workshop—in Brief, published by the National Academies Press (NAP), the publishing arm of the National Academies. Workshop proceedings are typically authored by some combination of HMD staff and hired consultants, serving as rapporteurs.

Like consensus committee reports, workshop proceedings are reviewed by an independent panel of experts, which may include roundtable and forum members. The Proceedings may not be transmitted to a sponsor or released to the public until review has been completed to the satisfaction of the Report Review Committee of the National Academies and the HMD Executive Office.

Other types of publications may develop from roundtables and forums. Independent, cooperative projects between sponsors and members, spin-off studies, and individually authored papers are some of the most common projects that grow out of roundtable and forum discussions. For instance, discussion papers and commentaries (collectively termed Perspectives) are individually

authored with the goal of further elucidating topics covered in roundtable or forum discussions. Small groups of roundtable or forum members, or individual members, may author a discussion paper or commentary to offer a particular perspective on a topic. Though distributed by the National Academy of Medicine (NAM), the views in the discussion papers and commentaries represent only those of the authors, not necessarily of the authors' organizations, the NAM, or the National Academies. These papers are not subject to the review procedures of the National Academies. All discussion papers and commentaries are designed to be shared publicly.

ROLE OF HMD STAFF

Each roundtable and forum is assisted in its work by a team of highly qualified staff members. Staff assist with research contributing to meetings and workshops, and they may act as the authors of a workshop proceedings. As with any HMD activity, staff may not insert their personal opinions into the publication. Overall, HMD staff is responsible for ensuring that the institutional procedures are followed and that the roundtable or forum stays within its budget.

COMMUNICATIONS

Although roundtables and forums do not issue advice or recommendations, it is important to emphasize communications to stimulate further discussion, attract workshop attendees, hold successful workshops, issue informative workshop proceedings, and inform a broader readership.

To help with these goals, HMD and the National Academies have a number of offices focused on communications support:

The HMD Office of Communications is responsible for HMD's report production functions as well as communications strategies and activities. One of its primary objectives is to communicate effectively the substantive messages of HMD activities and publications to its key audiences.

The Office of Congressional and Government Affairs is responsible for dissemination and outreach to congressional members and staffs. This may include congressional briefings and testimonies.

The Office of News and Public Information (ONPI) is the liaison between the National Academies and the news media and general public. ONPI should be informed of substantive conversations with the news media, especially if there is a problem.

The NAP website (nap.edu) makes all National Academies publications available online. All publications are free in PDF format to the public. As volunteers, roundtable and forum members receive a 25 percent discount on all books purchased from the NAP.

OUR ROUNDTABLES AND FORUMS

FOOD FORUM

Sylvia Rowe, Chair
Heather Cook, Director

Established in 1993, the Food Forum convenes scientists, administrators, and policymakers from academia, government, industry, and public sectors on an ongoing basis to discuss problems and issues related to food, food safety, and regulation. The forum provides a mechanism for these diverse groups to explore possible approaches for addressing food and food safety problems and issues surrounding the often complex interactions among industry, academia, regulatory agencies, and consumers.

FORUM ON AGING, DISABILITY, AND INDEPENDENCE

Terry Fulmer and Fernando Torres-Gil, Co-Chairs
Tracy Lustig, Director

The Forum on Aging, Disability, and Independence fosters dialogue and addresses issues of interest and concern related to aging and disability. This includes aging and the related disabling conditions that can occur, as well as aging with an existing disability. The forum seeks to promote bridging of the research, policy, and practice interests of the aging and disability communities to accelerate the transfer of research to practice and identify levers that will effect change for the benefit of all. Of particular concern is promoting healthy aging, independence, and community living for older adults and people with disabilities. This is a joint activity of HMD and the Division of Behavioral and Social Sciences and Education.

FORUM ON DRUG DISCOVERY, DEVELOPMENT, AND TRANSLATION

Russ B. Altman and Robert Califf, Co-Chairs
Carolyn Shore, Director

The Forum on Drug Discovery, Development, and Translation was created in 2005 by the Board on Health Sciences Policy to provide a unique platform for dialogue and collaboration among thought leaders and stakeholders in government, academia, industry, foundations, and patient advocacy with an interest in improving the system of drug discovery, development, and translation. The forum brings together leaders from private sector sponsors of biomedical and clinical research, federal agencies sponsoring and regulating biomedical and clinical research, the academic community, and patients. The forum has identified four core components of translational science across this continuum that serve as thematic pillars to frame the forum's focus areas and activities: (1) Innovation and the Drug Development Enterprise; (2) Science Across the Drug Development Lifecycle (Basic, Translational, and Regulatory Sciences); (3) Clinical Trials and Clinical Product Development; and (4) Infrastructure and Workforce for Drug Discovery, Development, and Translation.

FORUM ON GLOBAL VIOLENCE PREVENTION

Sheldon Greenberg, Chair
Julie Pavlin, Acting Director

The Forum on Global Violence Prevention was established in July 2010 to explore cross-cutting topics related to the prevention of child and elder abuse, sexual and intimate partner violence, youth and collective violence, and self-directed violence. The forum has a global scope, with a special focus on low- and middle- income countries. The forum highlights bidirectional learning opportunities and emphasizes an evidence-based, public health prevention approach. It works to reduce violence worldwide by promoting research on both protective and risk factors. The forum's aim is to facilitate dialogue and exchange by bringing together experts from all areas of violence prevention, including behavioral scientists, policy makers, criminal justice and public safety professionals, health and social services providers, economists, legal experts, journalists, philanthropists, faith-based organizations, and corporate social responsibility officers, among others. This is a joint activity of HMD and the Division of Behavioral and Social Sciences and Education.

FORUM ON MEDICAL AND PUBLIC HEALTH PREPAREDNESS FOR DISASTERS AND EMERGENCIES

Dan Hanfling and Suzet McKinney, Co-Chairs
Andrew Pope, Acting Director

The Forum on Medical and Public Health Preparedness for Disasters and Emergencies was established in September 2007 and provides a neutral venue for broad-ranging discussions that serve to facilitate coordination and cooperation among public and private stakeholders and enhance the nation's medical and public health preparedness for, response to, and recovery from disasters and other emergencies. The forum also serves as a a catalyst for collaboration among voluntary public–private partners; raises attention and visibility to important preparedness, response, and recovery issues; explores new approaches for identifying and resolving challenges; sets the stage for future policy action; and elevates the understanding of medical and public health preparedness among the broader research, public policy, and practice communities.

FORUM ON MICROBIAL THREATS

Peter Daszak, Chair;
Kent E. Kester and Mary E. Wilson, Vice Chairs
Ceci Mundaca-Shah, Director

The Forum on Microbial Threats was created in 1996 at the request of the U.S. Centers for Disease Control and Prevention and the National Institutes of Health to provide a structured opportunity for discussion and scrutiny of critical, and possibly contentious, scientific and policy issues related to infectious disease research and the prevention, detection, surveillance, and responses to emerging and reemerging threats in humans, plants, and animals as well as the microbiome in health and disease. The forum brings together leaders from government agencies, industry, academia, nonprofit and philanthropic organizations, facilitating cross-sector dialogue and collaboration through public debate and private consultation, to stimulate original thinking about the most pressing issues across the spectrum of microbial threats.

FORUM ON NEUROSCIENCE AND NERVOUS SYSTEM DISORDERS

Steven E. Hyman, Chair; Story Landis, Vice Chair
Clare Stroud, Director

The Forum on Neuroscience and Nervous System Disorders was established in 2006 to provide a venue for building partnerships, addressing challenges, and highlighting emerging issues related to brain disorders, which are common, major causes of premature mortality, and, in aggregate, the largest cause of disability worldwide. The Forum's meetings bring together leaders from government, industry, academia, disease advocacy organizations, and other interested parties to examine significant—and sometimes contentious—issues concerning scientific opportunities, priority setting, and policies related to research on neuroscience and brain disorders; the development, regulation, and use of interventions for the nervous system; and related ethical, legal, and social implications.

FORUM ON PROMOTING CHILDREN'S COGNITIVE, AFFECTIVE, AND BEHAVIORAL HEALTH

William R. Beardslee and C. Hendricks Brown, Co-Chairs
Wendy Keenan, Director

Cognitive, affective, and behavioral disorders incur high psychosocial and economic costs for the young people who experience them, their families, and the communities in which they live, study, and will work. The Forum on Promoting Children's Cognitive, Affective, and Behavioral Health aims to inform a forward-looking agenda for building a stronger research and practice base around the development and implementation of programs, practices, and policies to promote the health and well-being of all children, including those with disabilities. Forum members engage in dialogue and foster partnerships to connect the prevention, treatment, and implementation sciences with the places where children are seen and cared for, including health care settings, schools, social service and child welfare agencies, and the juvenile justice system. This is a joint activity of HMD and the Division of Behavioral and Social Sciences and Education.

FORUM ON PUBLIC–PRIVATE PARTNERSHIPS FOR GLOBAL HEALTH AND SAFETY

Jo Ivey Boufford and Clarion E. Johnson, Co-Chairs
Rachel M. Taylor, Director

The Forum on Public–Private Partnerships for Global Health and Safety is reflective of the growing role of the private sector in contributing to diverse global health initiatives. The forum seeks to foster a collaborative community of multisectoral health and safety leaders from multinational companies, governments, foundations, humanitarian and professional organizations, academia, and civil society to leverage the strengths of varying sectors and multiple disciplines to yield benefits for global health and safety. Partnerships among these stakeholders can foster dialogue, utilize innovation and process efficiencies, and expand public and private action to synergistically advance humanitarian, international development, and global health interests. The forum emphasizes opportunities in low- and middle-income countries to contribute to interventions and research in the areas of occupational and environmental health, community public health, and health systems strengthening.

FORUM ON REGENERATIVE MEDICINE

Jay P. Siegel and Sharon Terry, Co-Chairs
Sarah Beachy, Director

The Forum on Regenerative Medicine provides a convening mechanism for interested parties from academia, industry, government, patient/provider organizations, regulators, foundations, and others to discuss difficult issues in a neutral setting. The overall goal is to engage in dialogue that addresses the challenges facing the application of, and the opportunities for, regenerative medicine to improve health through the development of effective new therapies. The forum identifies potential barriers to scientific and therapeutic advances and discusses opportunities to facilitate more effective partnerships among key stakeholders. The forum examines the impact of current policies on the discovery, development, and translation of regenerative medicine therapies and addresses the unique challenges of identifying, validating, and bringing regenerative medicine applications to market. Ethical, legal, and social issues posed by regenerative medicine advances are also explored.

GLOBAL FORUM ON INNOVATION IN HEALTH PROFESSIONAL EDUCATION

Caswell Evans and Deborah Powell, Co-Chairs
Patricia Cuff, Director

The Global Forum on Innovation in Health Professional Education brings together stakeholders from multiple nations and professions to network, discuss, and illuminate issues within health professional education. Currently, there are over 55 appointed members to the Forum who are academic experts and health professionals representing 18 different disciplines from 8 developed and developing countries. Of these members, 46 are sponsors. Members of the forum gather twice a year to attend forum-sponsored events that address critical issues within the education to practice continuum. Topics for these activities have included discussions on financing health professional education; addressing the social determinants of health; and ensuring a mentally and physically stable health workforce.

NATIONAL CANCER POLICY FORUM

Edward Benz, Jr., Chair
Sharyl Nass, Director

The National Cancer Policy Forum serves as a trusted venue in which experts can work collaboratively to identify emerging high-priority policy issues in cancer research and care and to examine those issues through convening activities that promote discussion about opportunities for action. The forum provides a continual focus within the National Academies on cancer, addressing issues in science, clinical medicine, public health, and public policy that are relevant to the goal of reducing the cancer burden, through prevention and by improving the care and outcomes for those diagnosed with cancer. Forum activities inform stakeholders about critical policy issues through published proceedings and often inform consensus committee studies. The forum has members with a broad range of expertise in cancer, including patient advocates; clinicians; and basic, translational, and clinical scientists. Members represent patients, federal agencies, academia, professional organizations, nonprofits, and industry.

ROUNDTABLE ON ENVIRONMENTAL HEALTH SCIENCES, RESEARCH, AND MEDICINE

Frank Loy, Chair; Lynn R. Goldman, Vice Chair
Kathleen Stratton, Director

The Roundtable on Environmental Health Sciences, Research, and Medicine was organized in 1988 to provide a mechanism for parties interested in environmental health from the academic, industrial, and federal research perspectives to meet and discuss sensitive and difficult environmental health issues of mutual interest in a neutral setting. Since its inception, the roundtable has addressed current and emerging issues in environmental health through discussions related to the state of the science, research gaps, and policy implications. The roundtable has moved toward an increasingly global perspective in its discussions on the UN Sustainable Development Goals, the relationship between trade and health, and corporate social responsibility in environmental health. The roundtable is currently focused on issues of domestic and international importance, such as climate change, sustainable drinking water, transportation–related energy use, and environmental health decision making.

ROUNDTABLE ON GENOMICS AND PRECISION HEALTH

Geoffrey Ginsburg and Sharon Terry, Co-Chairs
Sarah Beachy, Director

The Roundtable on Genomics and Precision Health provides both a mechanism and a venue for interested parties from government, academia, industry, and other stakeholder groups to discuss global issues of mutual interest and concern regarding the translation of genomic research findings for medicine and health in a neutral setting. The purpose of the roundtable is to foster dialogue across sectors, as well as to illuminate andd scrutinize critical scientific and policy issues in which roundtable engagement will help further the field. The roundtable explores strategies for improving health through the translation of genomics and genetics research findings into medicine, public health, education, and policy. Current areas of emphasis include the development of targeted therapeutics and diagnostics; clinical implementation of genomic medicine; health information technology and digital health; use of genomic information for health care decision making; next generation sequencing; use of genomic information and data science to generate knowledge for clinical practice and research; education; and ethical, legal, and social issues.

ROUNDTABLE ON HEALTH LITERACY

Bernard M. Rosof, Chair
Lyla M. Hernandez, Director

The Roundtable on Health Literacy envisions a society in which the demands of the health and health care systems respect and align with people's skills, abilities, and values. The mission of the roundtable is to inform, inspire, and activate a wide variety of stakeholders to support the development, implementation, and sharing of evidence-based health literacy practices and policies, with the goal of improving the health and well-being of all people. In order to accomplish its mission, the roundtable brings together leaders from academia, industry, government, foundations and associations, and patient and consumer groups to meet in a neutral setting in order to discuss complex issues regarding health literacy research, practice, and strategies for promoting health literacy through mechanisms and partnerships in both the public and the private sectors.

ROUNDTABLE ON OBESITY SOLUTIONS

William Purcell, III, Chair
Leslie Sim, Director

The Roundtable on Obesity Solutions engages leadership from multiple sectors to solve the obesity crisis. Many sectors have recognized the need for action, and a number of groups have formed across the country to tackle specific aspects of the epidemic. Nonetheless, a significant gap exists between what we have learned about obesity solutions and the implementation of those solutions. Through meetings, public workshops, background papers, and innovation collaboratives, the roundtable provides a trusted venue for accelerating the discussion, development, and implementation of multisectoral collaborations and policy, as well as environmental and behavioral initiatives, that will reduce the prevalence and adverse consequences of obesity and eliminate obesity-related health disparities.

ROUNDTABLE ON POPULATION HEALTH IMPROVEMENT

George J. Isham and Sanne Magnan, Co-Chairs
Alina Baciu, Director

The Roundtable on Population Health Improvement brings together multiple sectors and disciplines to broaden the national conversation about the factors that shape our health and to support cross-sector relationships and engagement to transform the conditions for health across US communities. By hosting workshops, spurring individually-authored papers, and organizing action collaboratives, the roundtable engages members and outside experts, practitioners, and stakeholders around models, best practices, and other evidence about actions that will contribute to building a strong, healthful, and productive society that cultivates human capital and equal opportunity. The roundtable has explored issues ranging from novel and emerging financing mechanisms for population health to the power and potential of communities; from collaboration between public health and health care to the interface between the education and health sectors.

ROUNDTABLE ON THE PROMOTION OF HEALTH EQUITY

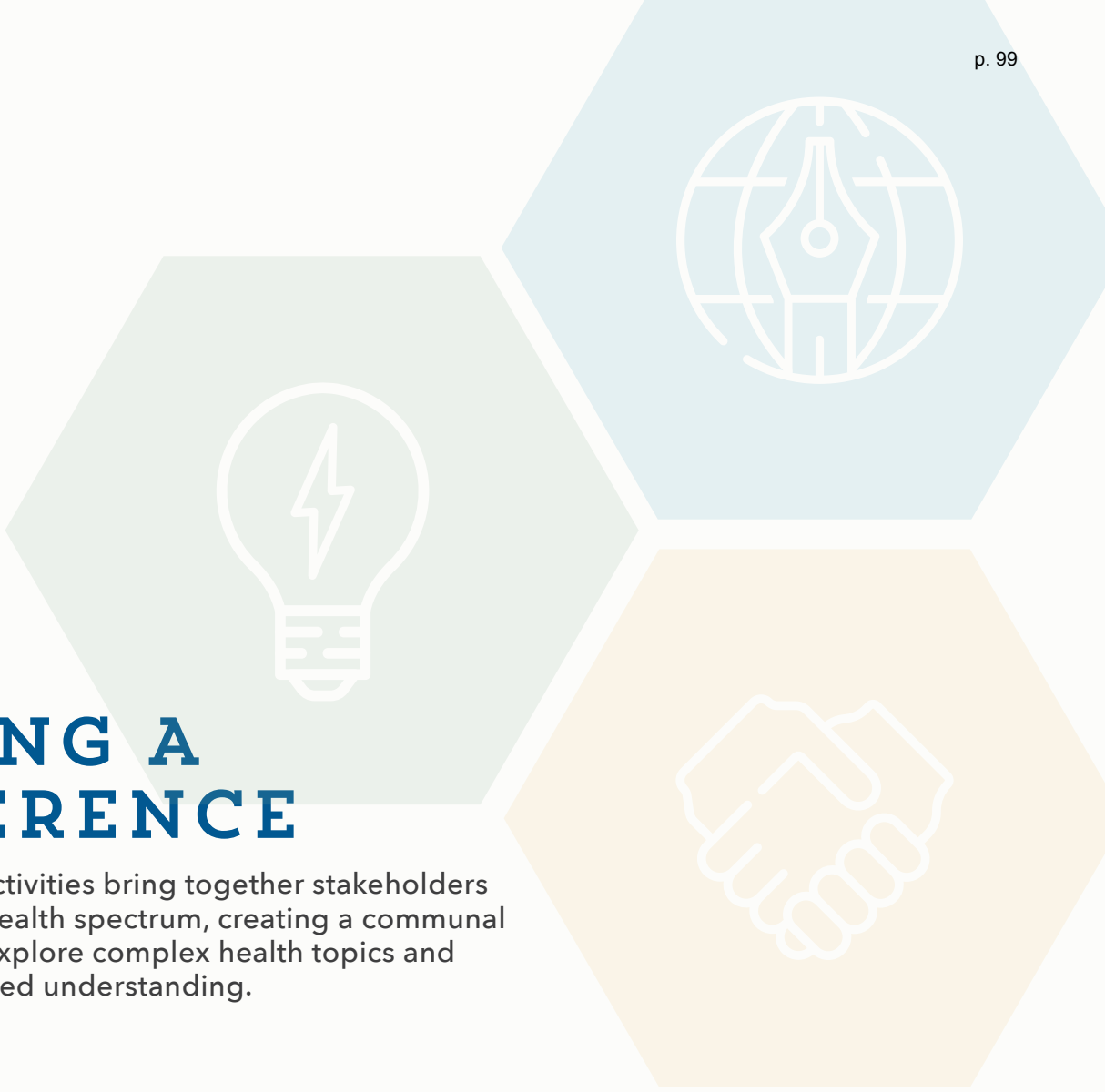
Antonia M. Villarruel, Chair
Karen Anderson, Director

The Roundtable on the Promotion of Health Equity serves as the conveners of the nation's experts in health disparities and health equity, with the goal of raising awareness and driving change. The roundtable promotes health equity and the elimination of health disparities by: (1) advancing the visibility and understanding of inequities in health and health care among racial and ethnic subpopulations; (2) amplifying research, policy, and community centered programs; and (3) catalyzing the emergence of new leaders, partners, and stakeholders

ROUNDTABLE ON QUALITY CARE FOR PEOPLE WITH SERIOUS ILLNESS

Leonard D. Schaeffer, Chair; James A. Tulsky, Vice Chair
Laurie Graig, Director

The Roundtable on Quality Care for People with Serious Illness, which launched in mid-2016, works to foster an ongoing dialogue about critical policy and research issues to accelerate and sustain progress in care for people of all ages with serious illness. Inspired by previous work at the National Academies, including the 2014 Institute of Medicine report *Dying in America: Improving Quality and Honoring Individual Preferences Near the End of Life*, the roundtable convenes key stakeholders to focus on five priority areas: (1) delivery of person-centered, family-oriented care; (2) communication and advance care planning; (3) professional education and development; (4) policies and payment systems; and (5) public education and engagement. Roundtable membership includes patient advocates, health care professional organizations, health care providers and insurers, foundations, federal agencies, researchers, and others interested in the topic.



MAKING A DIFFERENCE

Our convening activities bring together stakeholders from across the health spectrum, creating a communal environment to explore complex health topics and work toward shared understanding.

INFLUENCE
Policies & Programs

Our work can inform policy and legislation; programmatic planning, direction, and budgets; educational initiatives, such as curricula and training programs; and other activities.

FOSTER
Relationships & Collaboration

By bringing together a diverse group of participants around a particular topic, our activities foster new professional relationships, facilitate cross-sector collaborations, and enable professional development and networking, including the cultivation of new leaders.

INSPIRE
New Ideas & Shape the Field

Our work can advance and shape the field by framing issues and shining a light on important topics, and by generating novel approaches to overcome existing challenges, spurring progress and inspiring action.

IMPACT HIGHLIGHTS FROM OUR ROUNDTABLES AND FORUMS

INFLUENCE

Policies & Programs

A January 2015 report issued by Senator Lamar Alexander and Senator Richard Burr, *"Innovation for Healthier Americans: Identifying Opportunities for Meaningful Reform to Our Nation's Medical Product Discovery and Development,"* cited a workshop series of the Forum on Drug Discovery, Development, and Translation addressing clinical trials, which began in 2008, as a foundational resource in identifying and addressing the challenges facing the U.S. clinical trials enterprise. The report highlights concepts Congress might consider to better align public policy to support medical innovation and patient access to new medicines and technologies. One key concept explored in the report is the modernization of clinical trials.

FOSTER
Relationships & Collaboration

Discussions from the workshop "Sharing Clinical Research Data" generated the consensus study report Sharing Clinical Trial Data: Maximizing Benefits, Minimizing Risk (2015). The workshop was collectively hosted by the Forum on Drug Discovery, Development, and Translation; the Forum on Neuroscience and Nervous System Disorders; the National Cancer Policy Forum; and the Roundtable on Genomics and Precision Health. The consensus report provides guiding principles and a practical framework for the responsible sharing of clinical trial data, including recommendations regarding the optimal times to share the different types of data emerging over the course of a trial. To further the impact of this report and inspire further action in the area of data sharing, the collaborating forums and roundtable have launched an action collaborative to serve as a mechanism for interested stakeholders to convene and discuss the implementation of the report's recommendations, with workstreams focusing on building the information technology and technical infrastructure for data sharing and establishing data sharing goals for nonprofit funders of clinical trials.

INSPIRE
New Ideas & Shape the Field

A U.S. Centers for Disease Control and Prevention (CDC) webpage focusing on health literacy, titled *"Organizational Attributes: Be an Organization that Advances Health Literacy,"* builds on the discussion paper Ten Attributes of Health Literate Health Care Organizations (2012) that was authored by participants in the activities of the Roundtable on Health Literacy. CDC's Office of the Associate Director for Communication has interpreted the attributes listed in the discussion paper; the webpage offers a modified version to apply to organizations doing public health work