



Stakeholder Workgroup

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Proposed Mission

- “Investigate transparent and comprehensive models by which ME/CFS stakeholders can be effectively engaged in discussions regarding HHS priorities and utilize these investigations to enable the CFSAC to craft and submit informed recommendations to the Secretary on how to achieve effective, substantive, long-term engagement of stakeholders in **ME/CFS research efforts at NIH.**”
- Future efforts could include medical education & clinical care



Outline

- Definition
- Why stakeholder engagement is important
- Guiding principles for effective stakeholder involvement
- Various stakeholder engagement models researched
 - Select examples (PCORI, NCI)
- Initial recommendation for discussion



Stakeholder definitions

- **Anyone affected by an issue, who may or may not be formally involved in decision making about the issue.**
 - Our group focused primarily on the patient, but recognized the importance of all voices
- Anyone who might influence an organization's ability to achieve its mission or who can provide input on whether the mission is achieved.
- The Patient Centered Outcomes Research Initiative (PCORI) delineates between “patient partners” and “stakeholder partners”
 - Other groups also use various terms such as ‘research advocates’ and ‘patient research partners’



Faster Cures and PCORI definition clarifications

- PCORI differentiates between patient and stakeholder partners:
 - *“Patient partners” is intended to include patients (those with lived experience), family members, caregivers, and the organizations that are representative of the population of interest in a particular study.*
 - *“Stakeholder partners” may include members of constituencies based on professional, rather than personal, experience. For example, these constituencies can include: clinicians, purchasers, payers, industry, hospitals and health systems, policy makers, and training institutions.*
- Faster Cures has noted that:
 - *“The term ‘patient engagement’ is unevenly used and poorly defined.”*
 - 134 terms and 191 unique definitions in their review of patient engagement public documents

Stakeholder definitions cont.

- Stakeholders:
 - **Patients**
 - **Caregivers**
 - Agencies
 - Clinical care providers
 - Researchers
 - Charitable Organizations and NGOs
 - Medical Associations
 - Biotech and pharmaceutical companies



Engagement definition

- “The process used by an organization or group to engage relevant stakeholders for the purpose of achieving or improving acceptable outcomes”



Figure 13: Five Stage Stakeholder Engagement Framework
Redrawn from: [Krick et al. \(2005, 11\)](#)




What is effective stakeholder engagement?

- Stakeholders have meaningful involvement throughout the process
 - Formulating questions that are most relevant to patients lives and their concerns
 - Assisting with patient selection
 - Identifying outcomes that have the most meaning to the most patients
- Having a clear understanding of their roles in the process
 - Fostering open communication
 - Building trust through two-way communication
 - Building a partnership through reciprocal relationships
 - With consideration of the patients time and condition
 - Ensuring an environment that allows co-learning

Passive engagement methods are not enough


- We want to avoid passive engagement mechanisms such as:
 - Patient comments on completed works (i.e. P2P)
 - Listening sessions after studies have been designed
- If Stakeholder engagement is valued, it should be an active process such as:
 - Involvement in all phases of research
 - Two-way communication to decide on priorities from the outset





Benefits of stakeholder engagement in research


- **Authentic engagement of stakeholders:**
 - “elevates the moral plane of research by showing respect to patients and vulnerable populations”
 - “helps direct research toward questions that matter most to stakeholders”
 - “enhances study design by selecting outcomes that matter to end users and by choosing methodologies that optimize data collection and validity”
 - “elicits buy-in, getting participants excited about implementation and dissemination”



Benefits of stakeholder engagement in research

- **“If patient engagement were a drug, it would be the blockbuster drug of the century and malpractice not to use it.”**

Leonard Kish

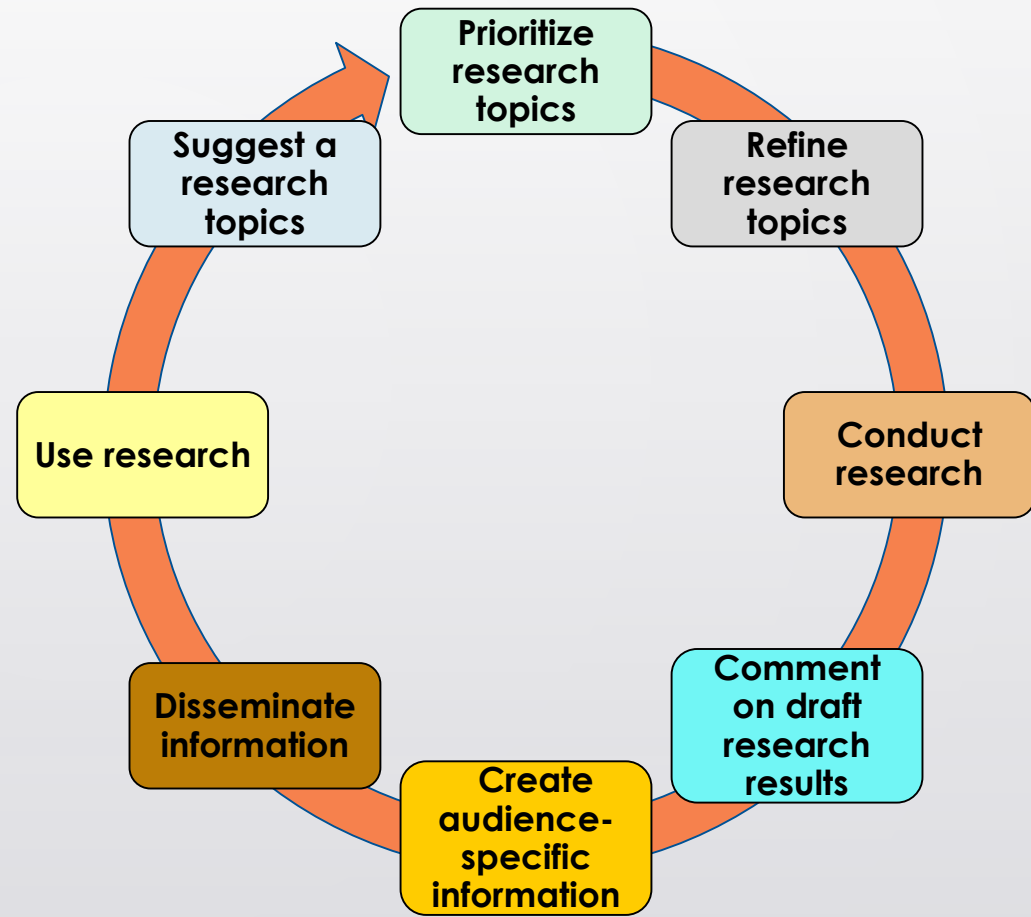


Why is stakeholder involvement in research, education and clinical care important?

- **Stakeholders bring unique, informed and experiential knowledge that will undoubtedly benefit research, education and clinical care**
- Stakeholders have a right to have a say in publicly funded research, education and health care activities
- Funding agency and organizations are becoming more interested in public input

Stakeholder involvement in research

- There are numerous steps in the research process where stakeholders can get involved



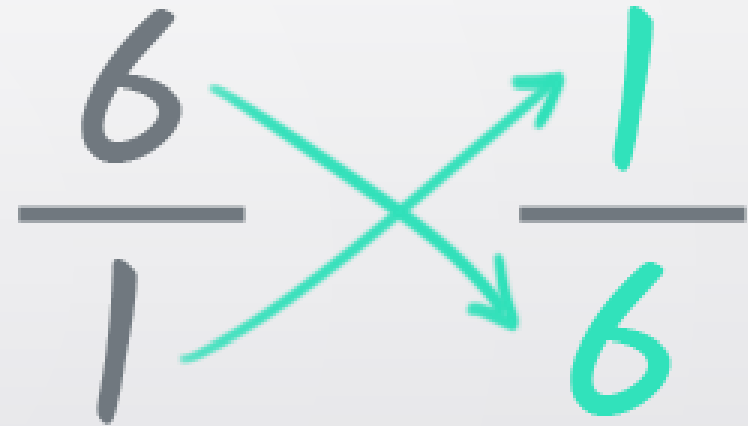
Guiding Principles

- There are several guiding principles for effective stakeholder engagement from different agencies such AHRQ, NIH, NHS, etc..
- Our group focused on the Patient-Centered Outcomes Research Initiative (PCORI) principles.



Reciprocal Relationships

- The roles and decision-making of all research partners, including the patient and other stakeholder partners, are defined collaboratively and clearly stated.



Partnerships

- Time and contributions of patients and other stakeholder partners are valued and demonstrated in:
 - Fair financial compensation
 - Reasonable and thoughtful requests for time commitments
 - Research team are committed to diversity across all project activities and demonstrate cultural competency, including disability accommodations, when appropriate



Co-Learning

- The goal should be to:
 - Educate stakeholders about the research process
 - Educate researchers about patient-centeredness and patient/other stakeholder engagement
 - Incorporate patient and other stakeholder partners into the research process

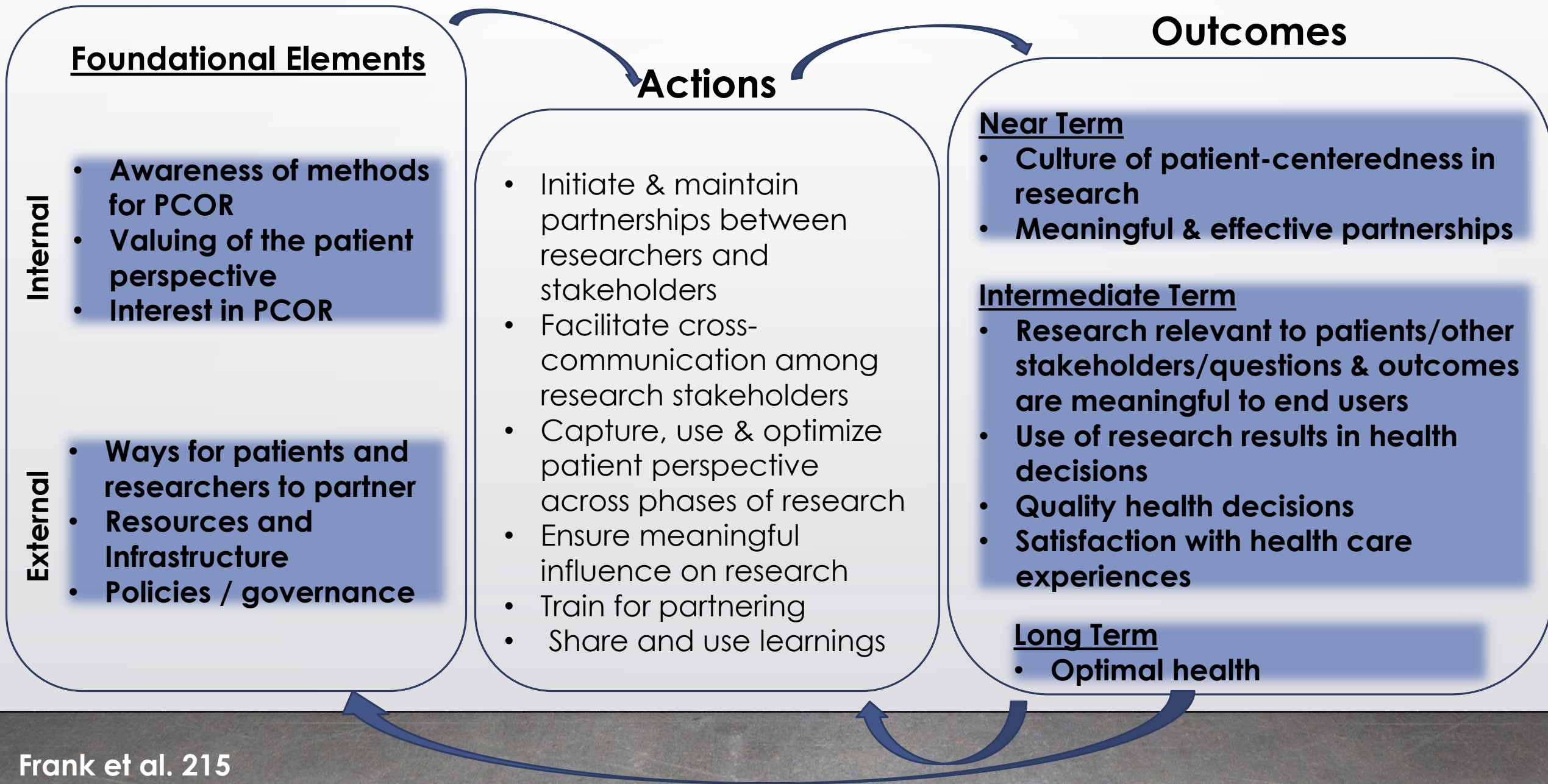


Transparency, Honesty, and Trust

- Major decisions are made inclusively and information is shared readily with all research partners.
- Patients, other stakeholders, and researchers are committed to open and honest communication with one another.
- Patient involvement creates “buy-in” for future success



PCOR Principles – Trust, Honesty, Co-learning, Transparency, Partnership, Respect

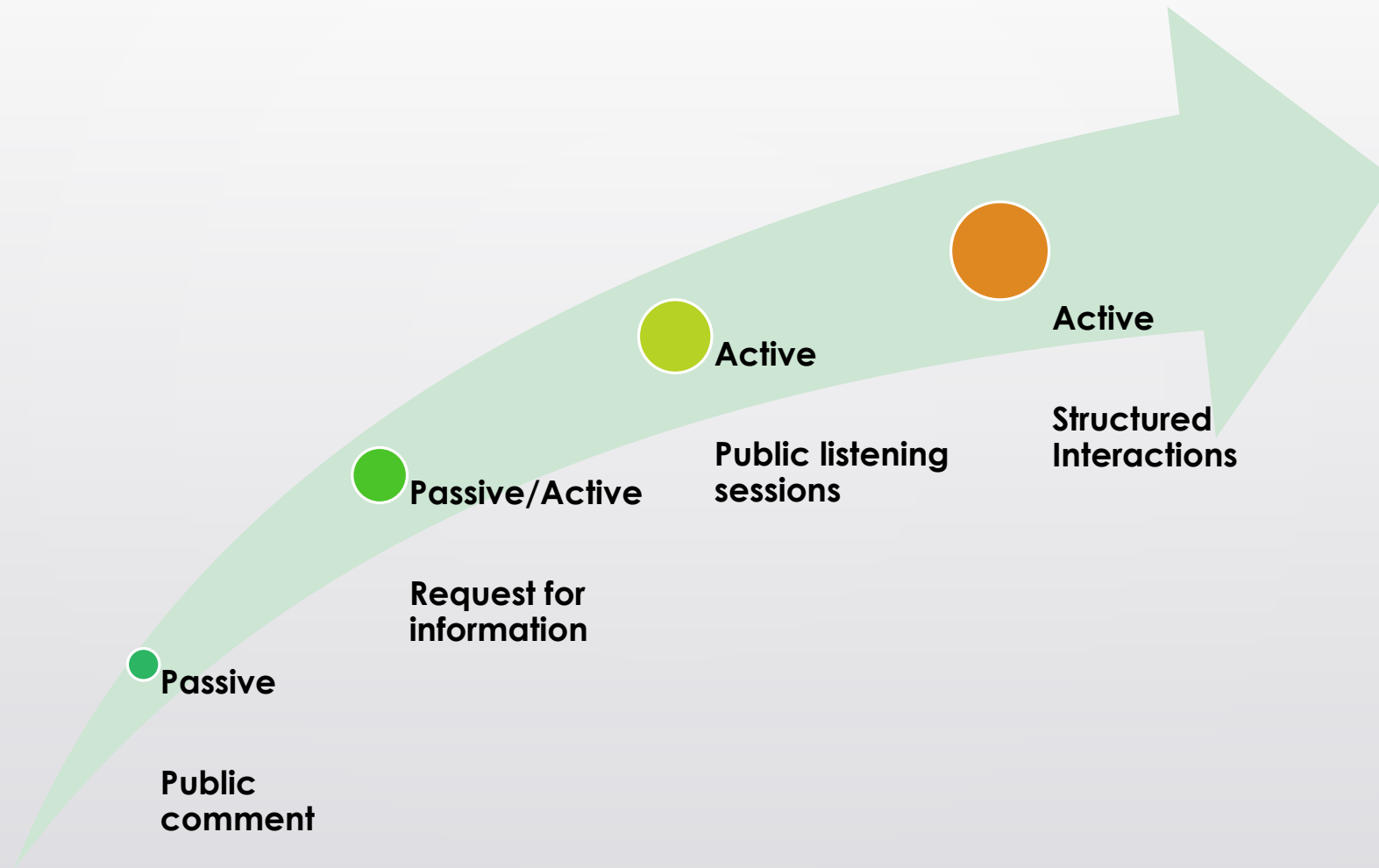




Examples of Patient Engagement at NIH and FDA

A continuum of engagement

Levels of engagement





U S Food and Drug Administration (FDA)

- **Patient Representative Program**

- Advisory committees evaluating drug and device approvals
- Application process for program participation
- FDA provides in-person and web-based training
- Patient reps are “Special Government Employees”

- **Criteria (Citizen & 18 yrs)**

- Personal experience with disease
- Ability to be objective while representing concerns of other patients
- Willingness to communicate views
- Knowledge about treatment options
- No financial or ethical conflicts of interest



U S Food and Drug Administration (FDA)

- **Patient Focused Drug Development**
 - Through a series of ‘disease area’ meetings
 - Designed to better understand the patients’ experiences
 - Focused on risk and benefits
 - **ME/CFS was the inaugural PFDD meeting (April 2013).**
 - Summary report (“Voice of the Patient”)
- **Patient Engagement Advisory Committee**
 - FDA’s Center for Devices and Radiological Health
 - Committee will make recommendations on complex issues related to devices
 - How CDRH can better engage with and understand patients’ perspectives

NIH - National Cancer Institute

- **Office of Advocacy Relations**

- Connect the right advocate to the right activity
- Range of activities
 - Advisory boards
 - Peer review panels
 - Review educational materials
 - Serve on scientific steering committees
 - Meet with program staff

- **Selecting advocates**

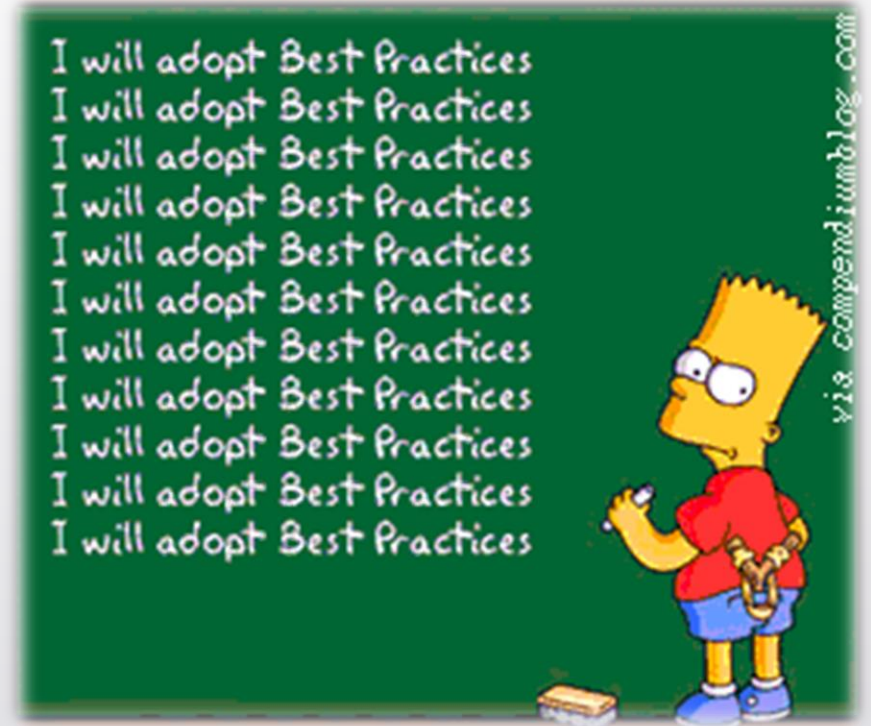
- More than two years of involvement in cancer research-related activities
- Personal experience with cancer
- Ability to demonstrate a collective patient perspective
- Residency in the United States
- Fluency in English

NIH - National Cancer Institute

- “Advocates are playing such an important role with their support of cancer research. They are providing critical analyses and insights that help ensure that research advances are more quickly and carefully translated to the patient and public health settings.”
 - *Dr. Christopher L. Hatch, Chief, Program Coordination and Referral Branch, NCI Division of Extramural Activities*

Agency differences across NIH

- It is important to recognize that:
 - No best practices for stakeholder engagement exist at NIH
 - No centralized process
 - Diversity in the approach among the independent institutes
 - Certain institutes embrace stakeholder engagement (e.g. NCI, Office of AIDS Research) – others not as much



Additional examples of stakeholder approaches among the various institutes

- Office of AIDS Research:
 - Full interaction on strategic planning, Community Advisory Boards for AIDS Clinical Trial Group
- Office of Advocacy Relations at NCI:
 - Supports patient & stakeholder engagement
- NINDS Advisory Council:
 - Institute level advisory board, not disease specific
- NINDS Epilepsy Center Without Walls:
 - Requires patient involvement
- NIAMS:
 - Action Plan for Lupus - patient involvement
- NIAMS Coalition:
 - Outside consortium that is involved in NIAMS scientific meetings, retreats, roundtables, teleconferences





Stakeholder engagement is valued within numerous federal agencies

- *Quotes from P2P:*
 - *"Initiate a federal partners working group that meets on a regular basis to continue to foster communication and collaboration across the agencies, with periodic meetings open to ME/CFS stakeholders." (page 6)*
 - *"the federal partners proposed to adopt the patient-focused drug development (PFDD) framework utilized by the FDA." (page 6)*
 - *"Developing educational materials with broad stakeholder collaboration: Individuals with ME/CFS, advocates, medical professional and educational organizations, clinicians with expertise in ME/CFS, and government (HHS ex officio CFSAC members) could work together to develop educational materials." (page 8)*



CFSAC specific recommendation

- To improve and facilitate stakeholder engagement at meetings, CFSAC recommends that the following procedures be implemented related to Public Comment process:
 - The Supplementary Information in each Federal Register Notice of Meeting for CFSAC should identify key topics to be discussed, along with an invitation to the public to address their comment on those topics at their discretion.
 - The CFSAC Meeting Agenda should include ample time for questions and discussion by committee members following each individual public comment, whether that comment is provided in person or by phone.
 - Public Comment sessions should be scheduled based on content, as possible, in order that pertinent remarks be heard PRIOR to Committee discussion of that topic.
 - Written Public Comment by individuals who will not speak at the meeting should be provided to Committee members at least 48 hours in advance of the meeting.
- Additionally, CFSAC recommends that each Meeting Agenda include a minimum of one hour for open discussion with stakeholders in attendance.



Potential research recommendation

- The Working Group recommends formation of a Stakeholder Advisory Board for ME/CFS Research at NIH which would be actively involved in strategic planning, identification of research priorities, design and implementation of research studies, and progress toward medical treatments and fulfilling the strategic plan. The Advisory board would meet at least 3 times annually with the Trans-NIH Working Group and/or staff before decisions are finalized. The Advisory Board must include patients and/or caregivers and expert clinicians at the outset and seek to expand to research and drug development constituencies in the medium term. The Working Group is available to make further recommendations about a set of requirements for patients and clinician members and a process for selection of the Board.



Thank you

And thanks to the work group for all of their efforts



Resources

- Siegel, Joanna. "Innovative Methods in Stakeholder Engagement: An Environmental Scan." (2012).
- Arora, Roshni, et al. "Designing and implementing Medicaid disease and care management programs: A user's guide." *AHRQ Publication 07* (2008): 08.AHRQ Effective Health Care
- Program Stakeholders Guide: **How To Get Involved in the Effective Health Care (EHC) Program: A Concise Guide for Stakeholders**
- O'Haire, Christen, et al. "Engaging stakeholders to identify and prioritize future research needs." (2011).
- PCORI Stakeholder Engagement Presentation: Getting to Know PCORI: From Application to Closeout: A workshop for Sponsored Project Administrators – Patient and Stakeholder Engagement in Research, <http://www.pcori.org/events/2016/getting-know-pcori-application-closeout-january-2016>
- James Lind Alliance: <http://www.jla.nihr.ac.uk/>
- AHRQ Learning Modules (4 presentations): <http://effectivehealthcare.ahrq.gov/index.cfm/tools-and-resources/how-to-get-involved-in-the-effective-health-care-program/learning-modules-engaging-stakeholders-in-the-effective-health-care-program/>
- National Institute for Health Research (NHS): <http://www.nihr.ac.uk/nihr-in-your-area/kent-surrey-and-sussex/patient-and-public-involvement-and-engagement.htm>
 - INVOLVE program: <http://www.invo.org.uk/>
 - People in Research: <https://www.peopleinresearch.org/view-opportunities/>
- National Institutes of Health (NIH): <https://www.nih.gov/about-nih/what-we-do/get-involved-nih/public-involvement-nih>
 - NIMH Alliance for Research Progress: <https://www.nimh.nih.gov/outreach/alliance/index.shtml>



Resources continued

- Boote, Jonathan, Wendy Baird, and Anthea Sutton. "Public involvement in the systematic review process in health and social care: a narrative review of case examples." *Health Policy* 102.2 (2011): 105-116.
- Boote, Jonathan, Wendy Baird, and Claire Beecroft. "Public involvement at the design stage of primary health research: a narrative review of case examples." *Health policy* 95.1 (2010): 10-23.
- Jones, Ian Rees, et al. "Is patient involvement possible when decisions involve scarce resources? A qualitative study of decision-making in primary care." *Social science & medicine* 59.1 (2004): 93-102.
- <http://www.fastercures.org/assets/Uploads/Final-FasterCures-Patient-Input-Language-WEB.pdf>
- <http://www.pcori.org/funding-opportunities/what-we-mean-engagement/pcoris-stakeholders>
- **L. Kish, The blockbuster drug of the century: An engaged patient. HL7 Health Standards 28 August 2012.**
www.hl7standards.com/blog/2012/08/28/drug-of-the-century.
- Woolf, Steven H., et al. "Authentic engagement of patients and communities can transform research, practice, and policy." *Health Affairs* 35.4 (2016): 590-594.
- <http://effectivehealthcare.ahrq.gov/index.cfm/tools-and-resources/how-to-get-involved-in-the-effective-health-care-program/learning-modules-engaging-stakeholders-in-the-effective-health-care-program/>



Resources continued

- <http://www.pcori.org/sites/default/files/Engagement-Rubric.pdf>
- Frank, Lori, et al. "Conceptual and practical foundations of patient engagement in research at the patient-centered outcomes research institute." *Quality of Life Research* 24.5 (2015): 1033-1041.



Questions for CFSAC

- Does CFSAC want us to develop solutions along the full spectrum from passive to active involvement?
- Does CFSAC want us to limit our focus to patients vs propose solutions for a broader group of stakeholders?
- What are CFSAC's ideas about engagement and how they would like us to move forward?



Extra information for those interested
and for discussion purposes



Common Data Elements project



Once a committee is established

The following guides are important for effective stakeholder engagement

Strategies for public involvement in research priorities

Health Policy 95 (2010) 10–23



Contents lists available at ScienceDirect

Health Policy

journal homepage: www.elsevier.com/locate/healthpol



Review

Public involvement at the design stage of primary health research:
A narrative review of case examples

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Facilitating strategies

- There were four main strategies identified to facilitate public involvement
 - Cultural (insert disease as well) sensitivity
 - Methods and language should consider the population of interest
 - Clear explanation of health research methods
 - This may require training up-front
 - Independent facilitation
 - Important when multiple stakeholder groups are involved
 - Funding for involvement in research design
 - A no brainer and something that needs to be integrated into the funding of research and education proposals
 - Accessibility
 - Being considerate to the unique needs of ME/CFS patients



Barriers and tensions

- Tensions between different stakeholder groups
 - Differing agendas
 - Conflicts between investigators and public concerning research design
- Public understanding of health research methods
 - Problems communicating the importance of certain research design elements
- Time and cost
 - Public needs to be compensated for their efforts
 - Funds need to be included for this purpose
- Representativeness
 - Who is speaking for whom
- Language and jargon
 - This gets at co-learning and being able to communicate an idea and a research design in a way that all can understand

INVOLVE example

- INVOLVE is an effort by NHS to increase public involvement in research
 - **Something we might consider?**
- I could not identify a similar mechanism within HHS





INVOLVE – “Active involvement examples”

- **Active Involvement**

- as joint grant holders or co-applicants on a research project
- identifying research priorities
- as members of a project advisory or steering group
- commenting and developing patient information leaflets or other research materials
- undertaking interviews with research participants
- user and/or career researchers carrying out the research.

- **Passive Engagement**

- science festivals open to the public with debates and discussions on research
- open day at a research centre where members of the public are invited to find out about research
- raising awareness of research through media such as television programmes, newspapers and social media
- dissemination to research participants, colleagues or members of the public on the findings of a study.



Building and maintaining trust

- A reoccurring theme throughout all of the materials reviewed was the importance of trust
 - Decisions need to be made inclusively
 - Communication must be open and honest
 - Dissemination of information back to community needs to be meaningful and useable
 - The entire process must be transparent