Webinar: Advancing Meaningful Patient Engagement in Drug Research, Development, and Approval

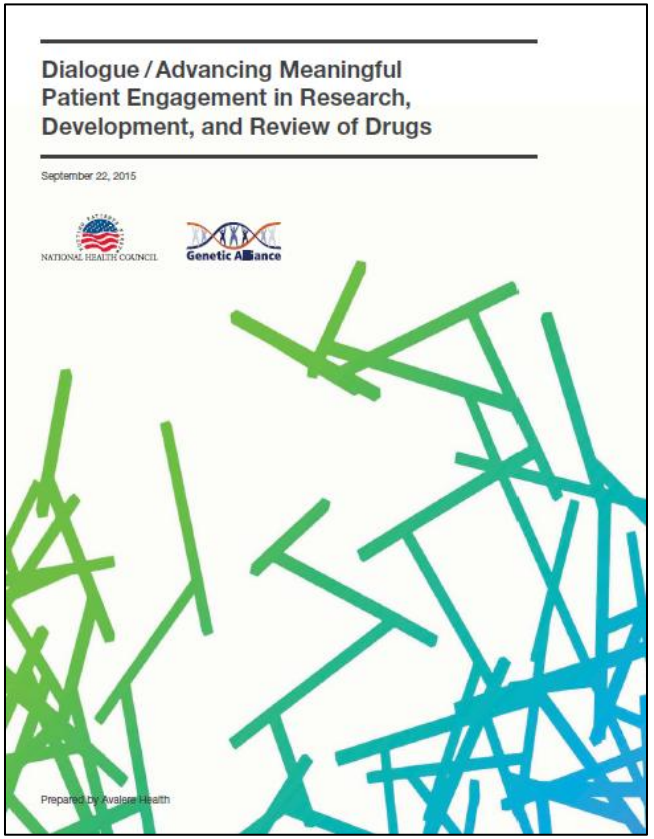
September 22, 2015

Agenda

- Welcome & Introductions
- Background
- Dialogue on Patient Engagement
- Understanding the Challenges
- Advancing Meaningful Patient Engagement
- Q&A

Today's Objective

- Share findings from the Dialogue on Advancing Meaningful Patient Engagement in Drug Research, Development, and Approval
- Propose solutions



Webinar Speakers

Eleanor Perfetto, PhD, MS (Moderator)
Senior Vice President of Strategic Initiatives, National Health Council

Marc Boutin, JD
Chief Executive Officer, National Health Council

Sharon Terry, MA
President and Chief Executive Officer, Genetic Alliance

Brenda Huneycutt, PhD, JD, MPH
Vice President, Avalere Health

Background

Speaker:

Marc Boutin, JD

Chief Executive Officer, National Health Council

Patients' Role in Health Care Is Evolving

Internal and external pressures

- Companies are facing increasing pressure to demonstrate how products address patient needs and enhance outcomes
- Patients and stakeholders want to get treatments to patients faster

Patients have not traditionally played a major role

- Patients were rarely engaged when critical decisions (e.g., study design) were made
- Patients' ability to provide information was limited by study design
- Predetermined study endpoints did not capture outcomes important to patients

Efforts Fragmented, Uncoordinated

Today, many stakeholders are engaging patients early with the intent to:

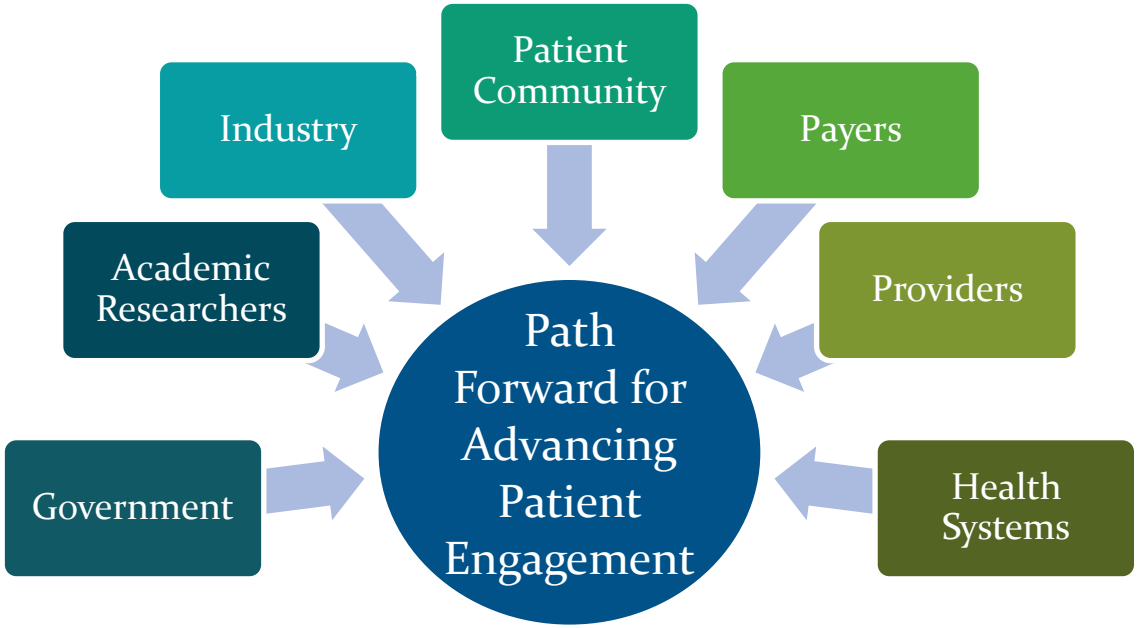
- Ensure products are designed to meet patient needs
- Ensure data informing regulatory approval and clinical use capture information important to patients

What Are the Problems?

- **Absence of a common definition** for meaningful patient engagement across stakeholders
- **No standardized methods** for engaging with patients and collecting patient information
- **Fragmented understanding of the fundamental barriers** to meaningful patient engagement
- **Lack of actionable solutions for stakeholders** to address fundamental barriers

Goal for the Dialogue

Create the opportunity for stakeholders to establish a common vision that drives meaningful integration of the patient voice in the product research, development, and approval processes.



Dialogue on Patient Engagement

Speaker:

Brenda Huneycutt, PhD, JD, MPH
Vice President, Avalere Health

Dialogue on Advancing Meaningful Patient Engagement in Drug Research, Development, and Approval

- Co-hosted by NHC and Genetic Alliance on March 2, 2015
- Held at Food and Drug Administration White Oak Campus
- Individuals representing the federal government, patient organizations, academia/research, and industry

Dialogue Objective: Identify actionable solutions for all stakeholders to provide a cohesive path forward in advancing meaningfully engagement across the research-to-care continuum

Dialogue Stakeholder Participants

Patient Groups
- Genetic Alliance
- FasterCures – A Center of the Milken Institute
- Leukemia & Lymphoma Society
- National Health Council
- National Multiple Sclerosis Society
- Parent Project Muscular Dystrophy
- Parkinson's Disease Foundation

Academia/Research
- Duke Translational Medicine Institute
- Emory University School of Medicine
- Johns Hopkins Bloomberg School of Public Health
- Northwestern Feinberg School of Medicine
- Patient-Centered Outcomes Research Institute
- University of Maryland School of Pharmacy

Government
- Food and Drug Administration (FDA), Center for Drug Evaluation and Research
- National Center for Advancing Translational Sciences (NCATS)

Industry
- Biotechnology Industry Organization
- Eli Lilly and Company
- Merck & Co.
- Novartis
- Orexigen Therapeutics
- OncoMed Pharmaceuticals
- Pfizer Inc
- Pharmaceutical Research and Manufacturers of America
- Sanofi

Other
- Avalere Health, LLC
- BioCentury
- Reagan-Udall Foundation for the FDA

Dialogue Key Discussion Areas

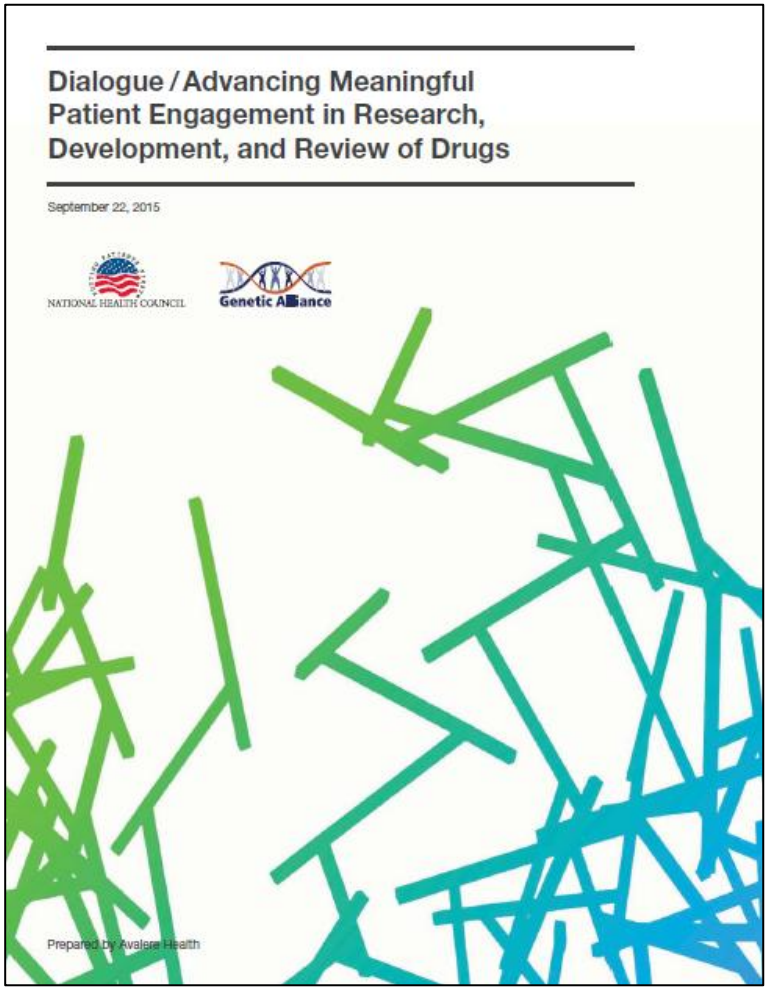
Topic 1: What is Meaningful Patient Engagement? Arriving at a Common Vision

Topic 2: How Are Patients Being Engaged? Current State and Best Practices for Engaging Patients in Drug Development and Approval

Topic 3: What are the Barriers to Patient Engagement? Exploring Cultural, Financial, Capacity, and Regulatory Barriers

Topic 4: Opportunities to Advance Meaningful Patient Engagement: Areas for Promising Investment and Expansion, and Appropriate Roles of Different Stakeholders

Dialogue White Paper



Understanding the Challenges

Speaker:

Sharon Terry, MA

President and Chief Executive Officer, Genetic Alliance

Barriers to Meaningful Patient Engagement

Regulatory/Legal Uncertainty

- What and How Information is Reviewed by FDA
- Patient Interactions

Culture

- Using a Science-Based Approach
- Financial Risks
- Organizational Culture

Communication

- Proprietary Information
- Translation and Patient Acknowledgement
- Visibility

Barriers: Regulatory/Legal Uncertainty

What and How Patient Information Is Reviewed by FDA

- How FDA will link information, data, or outcomes to inform regulatory decisions
- How FDA will evaluate and weigh sponsor-submitted patient information
- How FDA will use patient preference in the benefit-risk framework

Patient Interactions

- Perceived uncertainty among product sponsors on what constitutes appropriate interactions with patients
- Concerns that interactions with patients could be misinterpreted as promotional activities

Barriers: Culture

Using a Science-Based Approach

- View that methods/approaches for engaging patients and collecting information are not scientifically robust or methodologically rigorous
- Perception that patient perspectives are anecdotal, emotional, and subjective, which may detract from clinical data

Financial Risks

- Significant investments required to implement processes and build capacity across both patient groups and product sponsors
- Unclear and/or uncertain returns on early investments for all stakeholders

Barriers: Culture

Organizational Culture

- Skepticism regarding benefits of patient engagement
- Internal resistance to changes
- Uncertainties in the environment

Barriers: Communication

Proprietary Information

- Limited public information on patient engagement activities in the pre-market space
- View among product sponsors and other organizations that engagement activities with patients is proprietary

Translation and Patient Acknowledgement

- Perception by patients and other stakeholders that engagement is purely a formality
- Lack of a feedback loop to convey study information/results and keep patients continuously engaged beyond study participation
- Limited translation and communication efforts to facilitate patient understanding of study importance or impact of their contributions

Barriers: Communication

Visibility

- Lack of organized, centralized warehouse for information related to patient engagement, such as methods, best practices, and success stories
- Limited visibility or mechanisms for disseminating results

Advancing Meaningful Patient Engagement

Speaker:

Marc Boutin, JD
Chief Executive Officer, National Health Council

Key Themes and Potential Solutions

- Create Regulatory Guardrails
- Promote a Culture Shift
- Facilitate Open Communication

Actionable Steps

	Patients	Academia	Industry	Regulatory
Generate buy-in and sponsorship for patient engagement at the executive and senior leadership levels	x		x	
Create accountability at all levels within an organization for collecting, understanding, and integrating patient perspectives by establishing expectations and measuring the impact		x	x	x
Organize internal infrastructure and staffing to be coordinated around patient engagement activities and to prevent information silos	x		x	
Train and educate researchers on patient engagement	x	x		
Develop methods standards that can be applied across multiple disease areas	x	x	x	x
Catalog validated methods for gathering patient information	x	x	x	x
Establish processes or models to systematically engage patients at any point in the research-to-approval continuum	x	x	x	x
Develop and implement tools and resources that complement methods for patient engagement and facilitate implementation	x	x	x	
Develop and test metrics to evaluate patient engagement	x	x	x	
Develop a platform, repository, or system for sharing best practices, research, examples of impact (e.g., public-private partnership or "center of excellence")	x	x	x	
Establish a public-private partnership to build capacity and infrastructure, advance scientific methods for patient engagement, and serve as a central clearinghouse for pre-market patient-centered studies	x	x	x	x
Direct funds from public and private research funders through patient groups so patient groups have the opportunity to solicit, evaluate, prioritize, and even directly fund patient-centered studies or projects	x	x		

Create Regulatory Guardrails

PRIMARY ACTIONS

- Formalize regulatory asks for negotiation in PDUFA
- Prioritize development of guidances
- Enhance FDA division alignment on the use of tools for evaluating patient information

SUPPORTIVE ACTIONS

- Align stakeholder advocacy strategies
- Increase transparency
- Create more opportunities to collect feedback

Promote a Culture Shift

PRIMARY ACTIONS

- Generate buy-in
- Create accountability for collecting/integrating patient perspectives
- Create public-private partnership

SUPPORTIVE ACTIONS

- Develop/implement tools and metrics
- Train/educate researchers
- Help patient groups solicit/fund patient-centered studies

Facilitate Open Communication

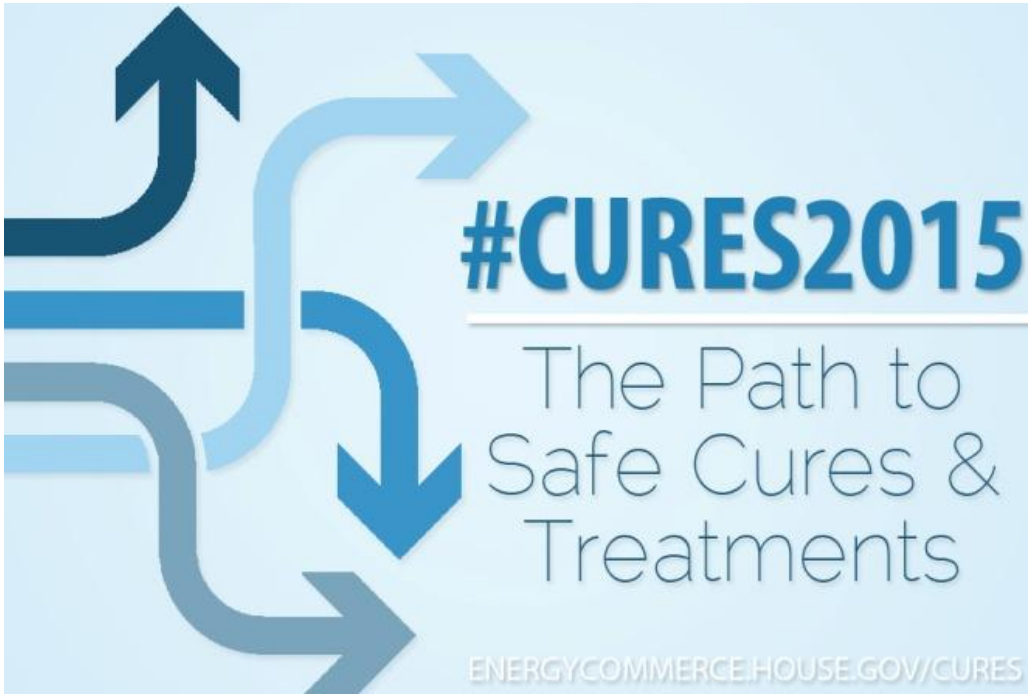
PRIMARY ACTIONS

- Make information comprehensible
- Document impact of patient perspective studies
- Make publicly available experiences, advice, best practices, lessons learned, and other resources

SUPPORTIVE ACTIONS

- Create a feedback system
- Utilize open-source production model whenever possible
- Create partnerships to encourage information sharing

21st Century Cures Act Provisions



- Implementing a structured benefit-risk framework for drug evaluation that integrates patient experience data
- Issuing draft guidance within three years of enactment

Findings Will Inform Next Steps

Disseminate Dialogue Findings

Prioritize Actions

Q & A

Eleanor Perfetto, PhD, MS (Moderator)
Senior Vice President of Strategic Initiatives, National Health Council

Marc Boutin, JD
Chief Executive Officer, National Health Council

Sharon Terry, MA
President and Chief Executive Officer, Genetic Alliance

Brenda Huneycutt, PhD, JD, MPH
Vice President, Avalere Health

For more information, please contact

Eleanor Perfetto
Senior Vice President, Strategic Initiatives
National Health Council
eperfetto@nhcouncil.org

To download a copy of the white paper, go to

www.nationalhealthcouncil.org

www.geneticalliance.org