Advancing Meaningful Patient Engagement in Drug Development
Evolution of PDUFA

1993

PDUFA I: Backlog reduction

1998

PDUFA II: FDAMA
Reduce review times

2003

PDUFA III: BTP

2008

PDUFA IV: FDAAA
• Unintended consequences
• Process for engagement

2013

PDUFA V: FDASIA
• Benefit/Risk framework
• Biomarkers/PROs
• PFDD
• Rare diseases

2018

PDUFA VI
Patient engagement throughout the development lifecycle

Initial patient group engagement

Shift to consumer engagement

Patient groups re-engage
Dialogue / Advancing Meaningful Patient Engagement in Drug Research, Development & Approval

Objective: Identify actionable solutions to advance meaningfully patient engagement across the research-to-care continuum

- Full-day event at FDA White Oak Campus
- Participants representing FDA, NCATS, Patient Organizations, Academia/Research, and Industry
Dialogue White Paper (to be released 9/22/2015)

• Close the information and communication gap
• Build consensus around a common vision
• Provide all stakeholders with a viable path forward
## Barriers to Meaningful Patient Engagement

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<th>Regulatory/Legal Uncertainty</th>
<th>Culture</th>
<th>Communication</th>
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| • What and How Information is Reviewed by FDA | • Using a Science-Based Approach  
• Financial Risks  
• Organizational Culture | • Proprietary Information  
• Translation and Patient Acknowledgement  
• Visibility |
Patient Engagement Strategy

1. Identify When to Engage
2. Determine Purpose for Engagement
3. Consider Who to Engage
4. Decide on Method(s) for Engagement
Patient Engagement: When to Engage

- Research Questions
- Discovery
- Pre-Clinical Development
- Clinical Development
- Evidence Communication
- Post Approval Surveillance
- FDA Approval Process
Patient Engagement: Purpose for Engagement

- Determine Patient Preference
- Understand Disease and Impact
- Capture Patient Reported Outcomes
- Inform Clinical Trial Design
Patient Engagement: Who To Engage?

- Patients
- Patient Advocacy Organizations
- Non-affiliated Patients
- Big Data Pools
Methods of Engagement

- Interviews
- Focus Groups
- Surveys
- Public Comment
- Advisory Panels
- Workshops
Continued Progress Will Require Collaboration

Advancing Patient Engagement

- Industry
- Patient Community
- Academic Researchers
- Payers
- Government
- Providers
- Health Systems
External Environment

Patient Engagement Movement

21st Century Cures Initiative

PDUFA Reauthorization
Patient-Focused Medicines Development

- CLINICAL TRIALS TRANSFORMATION INITIATIVE
- EPF European Patients Forum
- EUPATI European Patients’ Academy on Therapeutic Innovation

Other Stakeholders

- Innovative Medicines Initiative
- MDIC MEDICAL DEVICE INNOVATION CONSORTIUM
- CDRH Center for Devices and Radiological Health