Patient Engagement Across the Drug Discovery and Development Continuum

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Outline

• Framing the Issue
• Benefits of Engagement & Costs of Non-Engagement
• Patient Inspired Innovation
• Beyond drug discovery and development
Patient Engagement in Research and Development

- Gaining momentum as way to improve healthcare outcomes

- Patient input recognized as just as critical to improving healthcare as engagement post-approval

- Patients have greater stake
  - Increased access to information
  - Greater transparency of clinical trial data and results
  - Economics of relationship has changed

- FDA and other stakeholders (industry, academic researchers, etc) beginning efforts to engage patients earlier to:
  - Meet needs of patients, and
  - Capture information relevant to patient values and preferences
Bi-Directional Exchange Influences Each Stage of the Product Lifecycle

**Discovery/Pre-Clinical**
- Understanding unmet need
- Informing treatment design

**Clinical Development**
- Designing protocols to optimize recruitment, retention, compliance, medication adherence
- Selecting endpoints meaningful to patients

**Approval**
- Contributing to decisions on licensure and labelled indications

**Post-Approval through Patent Expiration and Beyond**
- Developing packaging, health-literate labeling, medication guides, education tools
- Supporting reimbursement and access
- Facilitating treatment adherence
- Contributing to risk-benefit profile
- Informing public-health policy
Barriers and Challenges to Patient Engagement

- No common definition of meaningful patient engagement
- Cost
- Lack of defined metrics
- Current organizational culture
- Regulatory restrictions
- Need to engage 3rd parties to facilitate
- Potential Legal risks
Consequences of not Engaging Patients

Opportunity Costs:

- Failure to realize greater efficiencies in recruitment and retention
- Making wrong choices, wasted effort pursuing wrong target or path
- Gaps in understanding on part of patient and missed opportunity for education
- Gaps in understanding that can lead to gaps in trust
Engagement Rubric:

… tool providing guidance to researchers on ways to engage patients, family members, caregivers, and patient groups throughout the research process… planning, conducting, disseminating study results

Updated February 11, 2015
Patient Inspired Innovation at Merck

… listening, learning and collaborating with experts and expert groups in all sectors …
• Enhances corporate responsibility, reputation, and transparency by focusing on the patient
• Contributes to assessments of patient safety
• Champions and enables Merck’s strategy of patient-inspired innovation
• Facilitates access to medicines
• Addresses health-care disparities
• Promotes health literacy and treatment adherence
Patient-Inspired Innovation: Guiding Principles

• Patient and caregiver at the center of our decision-making
• Opportunity to innovate more profoundly by listening to patients and caregivers, in addition to health care professionals
• Patient perspective and “bi-directional exchange” with patients critical to sustained innovation from discovery through commercialization
• There are no average patients; we need to
  • Engage a wide range of patients
  • Use their perspectives and experience to refine our strategic decisions

“Nothing about me without me”
--- Patient
- Bi-directional interactions and exchange with patients throughout discovery and development make communications with patients more relevant and impactful.

- One of the greatest barriers to patient engagement across the continuum is communicating with patients -- much of which can be described under the umbrella of health literacy.

- Application of principles of health literacy important to patient engagement strategies and innovation.

- Improved health literacy as an enabler of more diverse participant recruitment, retention, comprehension, adherence and experience in clinical trials.
## We Must Collaborate Globally!

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<td>Health Literate Patient Rx labels; Universal Medication guide pilot</td>
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Patient Engagement Beyond Drug Development: Multiple Interfaces

Patient:
- Which treatment is best for me? How does my doctor and health system perform comparatively?

Insurer/payer:
- Understand cost-effectiveness of approaches

Regulatory and Reimbursement Authorities:
- Patient perspectives formally sought at time of registration or reimbursement

Pharmaceutical company:
- How our drugs fit into clinical practice; comparative safety and effectiveness; value and benefit: risk perspective from patients
“IT TURNS OUT THAT WHAT IS REALLY BOTHERING THE PATIENT AND WHAT IS REALLY BOTHERING THE DOCTOR CAN BE RADICALLY DIFFERENT THINGS.”

Janet Woodcock, M.D.
Director, Center for Drug Evaluation and Research
FDA
The Goal

... end to end, global, bi-directional engagement with patients/caregivers to optimize and realize greater innovation and efficiency

... putting the individual patient and caregiver at the center of our decision-making.