Designing Clinical Trials from the Patient Point of View

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Disclosures

Nothing to disclose
From Science to Patient Experience

- Science: Insights poorly managed
- Evidence: Evidence poorly used
- Care: Experience poorly captured

Missed Opportunities, Waste, and Harm

https://theincidentaleconomist.com
Which Outcomes Do Patients Care About?

Examples:
- Health
- Relief from symptoms
- Health-related quality of life
- Function (ability to do what they want to do)
- Safety from medical harm
- Survival
What do Patients Value?

Patient-Centered Outcomes

- Quality of life
- Complexity of regimen
- Efficacy & effectiveness
- Safety: side effects/complications

Patient & Family Costs

- Medical out-of-pocket (OOP) costs
- Non-medical costs to the patient & family
- Future costs of care

Quality & Applicability of Evidence

- Quality of evidence
- Consistency of evidence
- Differences in treatment effects

FasterCures
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**Health-related quality of life**
- Functional/cognitive status
- Palliation of symptoms
- Symptom-free intervals
- Dosing/treatment schedule
- Treatment length
- Site of care
- Route of administration & invasiveness of procedure/device
- Improvements in end points
- Frequency, severity, duration
- Discontinuation rates

**OOP costs to patient**
- Supportive care agents
- Device maintenance
- Cost of travel
- Cost of child/elder care
- Cost of supportive care
- Required lifestyle/behavioral change
- Patient & family work productivity/lost wages
- Patient & family education/skill building
- Required hours of caregiving
- Complexity of patient support
- Administrative burden
- Subsequent healthcare utilization
- Changes in costs of therapies

**Adherence to generally accepted methods**
- Variability of study results
- Variance in demographics, comorbidities, disease stage
Change by Co-Production & Co-Design

A Continuum of Co Production

Partnership

Patient Voice/influence

Passive Patient

Source: new economics foundation
New Ways to Engage Patients & Caregivers

1. DISCOVERY
   - IDEA
   - BASIC RESEARCH
     The majority of the research at this stage is publicly funded at universities, colleges and independent research institutions in every state.

2. DEVELOPMENT
   - CLINICAL TRIALS
     Once a disease target is identified, drugs are designed and tested. Both public and privately funded research are involved.
   - PHASE I
   - PHASE II
   - PHASE III

3. DELIVERY
   - REGULATORY APPROVAL
     Human trials are completed. FDA approval. Industry is responsible for bringing a drug to market. Safety and evaluation continue after approvals.
   - PATIENT CARE

Research! America
<table>
<thead>
<tr>
<th>Defining product features</th>
<th>Optimize lead candidates</th>
<th>Preparation for clinical entry</th>
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<tbody>
<tr>
<td>- What symptoms of the disease are most disturbing to daily life?</td>
<td>- What levels of risk are you comfortable taking in trying novel treatments? What type of risks would you tolerate for what levels of efficacy?</td>
<td>- Are you willing to share your thoughts on the target profile and the preclinical results you have seen with regulators (e.g., FDA, EMA)?</td>
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<tr>
<td>- How do you prioritize the importance of the different disease symptoms?</td>
<td>- What is the maximum dosing frequency you could reliably manage without affecting your activities of daily life? How do these maxima change as efficacy increases?</td>
<td>- Are you willing to attend regulatory meetings alongside the sponsor?</td>
</tr>
<tr>
<td>- How might you measure those most troublesome symptoms?</td>
<td>- Which routes of administration could you consistently and reliably manage?</td>
<td>- Are you willing to participate in a patient registry and longitudinal studies that assess the progression of your disease?</td>
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<td>- At what age does the disease cause the greatest changes? What kind of patients are most severely affected and/or might best be able to show a positive drug effect, if there is one?</td>
<td>- How do you rate different routes of administration against each other?</td>
<td>- Are you willing to participate in clinical trials testing new drugs?</td>
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Efficient Foundation / Industry Partnership

**Foundation Provides**
- Communication with families and researchers
- Available pre-clinical resources
- Operational support
- Regulatory Advocacy
- Post-approval support

**Company Provides**
- Transparent and rapid communication
- Commitment to community
- Available safety-related data
- Regulatory expertise
- Compound library as pre-clinical tool
Patient Group Engagement Across the Clinical Trial Continuum

Patient groups have potential to enhance the quality and efficiency of clinical trials by providing:

**Discovery & Pre-Clinical**
- Financial support for research
- Natural history data
- Input on relevance of research to patients
- Access to translational tools
- Help defining eligibility criteria
- Input on meaningful endpoints & PROs
- Advocacy for policy & funding issues
- Education to patient community

**Phase 1 - 3**
- Benefit-risk & patient-preference studies
- Protocol design & study feasibility input
- Study recruitment & retention strategy input
- Increased awareness about trials
- Participant feedback on trial experience
- Input on informed consent content & processes
- Peer advocates for participants
- Clinical trial networks
- Data Safety Monitoring Board members

**Regulatory Review**
- Support to sponsors around key regulatory meetings
- Support preparing submissions for newborn screening for rare diseases
- Informing regulators on benefit-risk
- Public testimony at regulatory meetings

**Post-Approval**
- Phase 1-3 activities and...
- Support interpreting & disseminating study results
- Collaboration on post-marketing studies & surveillance initiatives
- Support developing access strategy & preparing for value or health technology review

*Updated 2018; adapted from Parkinson’s Foundation materials | †Patient group activities typically undertaken independently or with partners other than sponsors | ‡Includes early planning for trials
CTTI RECOMMENDATIONS: EFFECTIVE ENGAGEMENT WITH PATIENT GROUPS AROUND CLINICAL TRIALS

Part I. Background

With the increasing commitment to Patient-Focused Drug Development (PFDD) by FDA and patient engagement in translational research, there is a significant opportunity to improve the clinical trials enterprise and enhance participation by patient groups in the work of trial sponsors. The term PFDD, as used here in the broader sense, refers to the meaningful engagement of patients in the development of therapeutic products, and the various important roles patients can play in improving the entire enterprise, from study endpoint selection that reflects outcomes meaningful to patients, to recruitment and retention in clinical trials, and more effective postmarketing safety. Yet clarity is needed about how, when, and by whom patients or patient groups should be engaged during the therapy development process, and which patients or patient groups should be engaged. Also lacking are metrics by which the value of such engagement, in terms of regulatory and market success, might be measured. After decades of emphasis on mechanisms to speed “bench to bedside” development, PFDD and patient engagement in research should be considered an effort to extend the benefits of incorporating patient insight and experiences, as well as desires and preferences, from bench to bedside and back.
Meanwhile, at today’s Feline Engagement Committee, decisions were made on how to increase feline recruitment.