Designing Clinical Trials from the Patient Point of View

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LGS FOUNDATION
LENNOX-GASTAUT SYNDROME

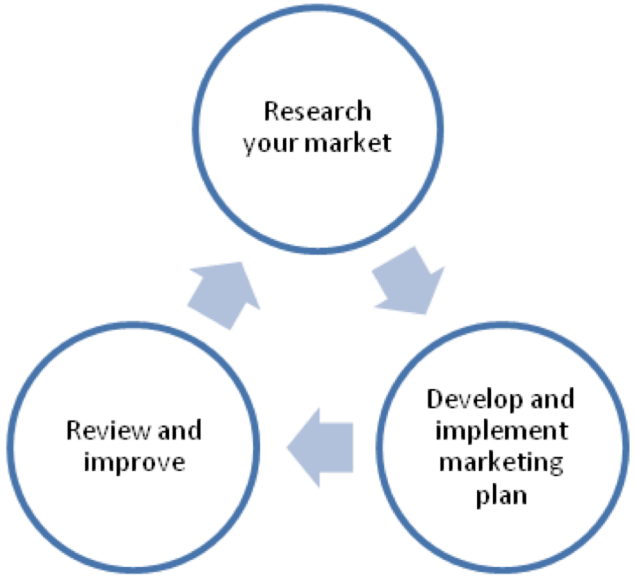
Disclosures

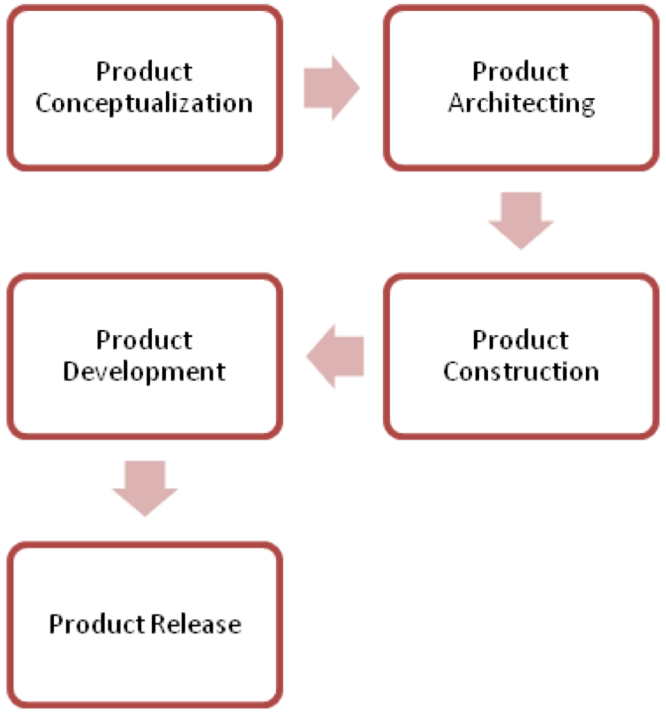
Nothing to disclose

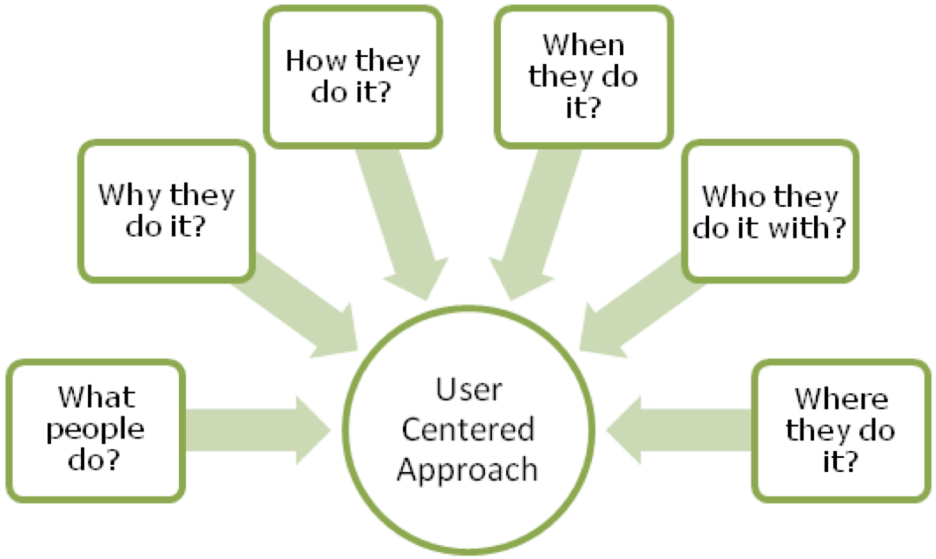
From Science to Patient Experience

https://theincidentaleconomist.com

Change by Reframing Value







Consumer-led research

Specific product development

User Centered Design

Marketing

Product Development

Ethnographic Style Research

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

pcori
Patient-Centered Outcomes Research Institute

What do Patients Value?

Patient-Centered Outcomes

Patient & Family Costs

Quality & Applicability of Evidence

PATIENT-CENTERED OUTCOMES

What are the **clinical, functional, and quality of life benefits/drawbacks** of different healthcare options to the patient?

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

PATIENT & FAMILY COSTS

What are the **overall costs of different healthcare options** to the patient and family?

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

QUALITY & APPLICABILITY OF EVIDENCE

What level of confidence does the patient have that a healthcare option **will have specific effects** for them?

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

What do Patients Value?

PATIENT-CENTERED OUTCOMES

What are the **clinical, functional, and quality of life benefits/drawbacks** of different healthcare options to the patient?

- Quality of life
  - Health-related quality of life
  - Functional/cognitive status
  - Palliation of symptoms
  - Symptom-free intervals
- Complexity of regimen
  - Dosing/treatment schedule
  - Treatment length
  - Site of care
  - Route of administration & invasiveness of procedure/device
- Efficacy & effectiveness
  - Improvements in end points
- Safety: side effects/complications
  - Frequency, severity, duration
  - Discontinuation rates

PATIENT & FAMILY COSTS

What are the **overall costs of different healthcare options** to the patient and family?

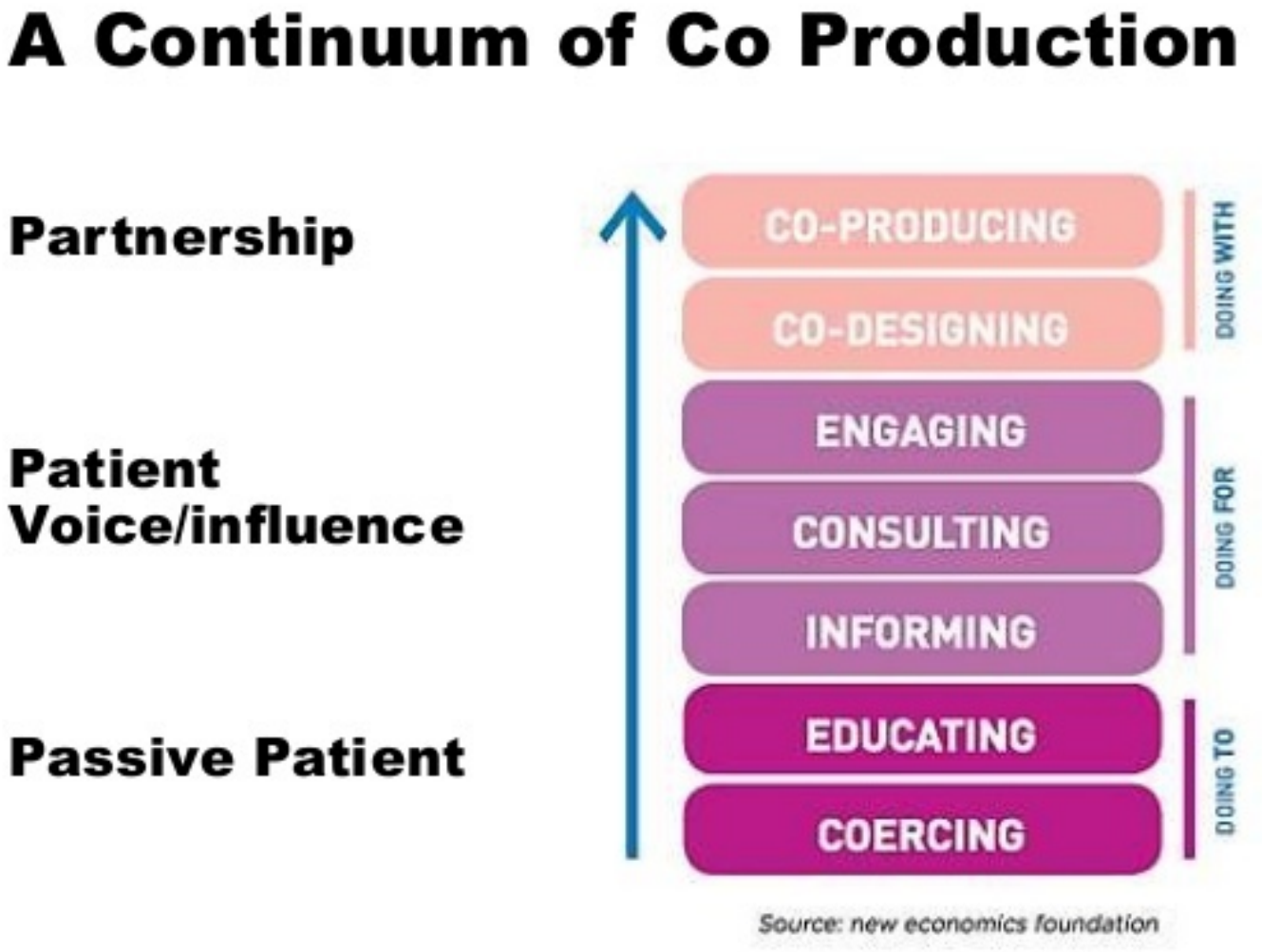
- Medical out-of-pocket (OOP) costs
  - OOP costs to patient
  - Supportive care agents
  - Device maintenance
- Non-medical costs to the patient & family
  - Cost of travel
  - Cost of child/elder care
  - Cost of supportive care
  - Required lifestyle/behavioral change
  - Patient & family work productivity/lost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

QUALITY & APPLICABILITY OF EVIDENCE

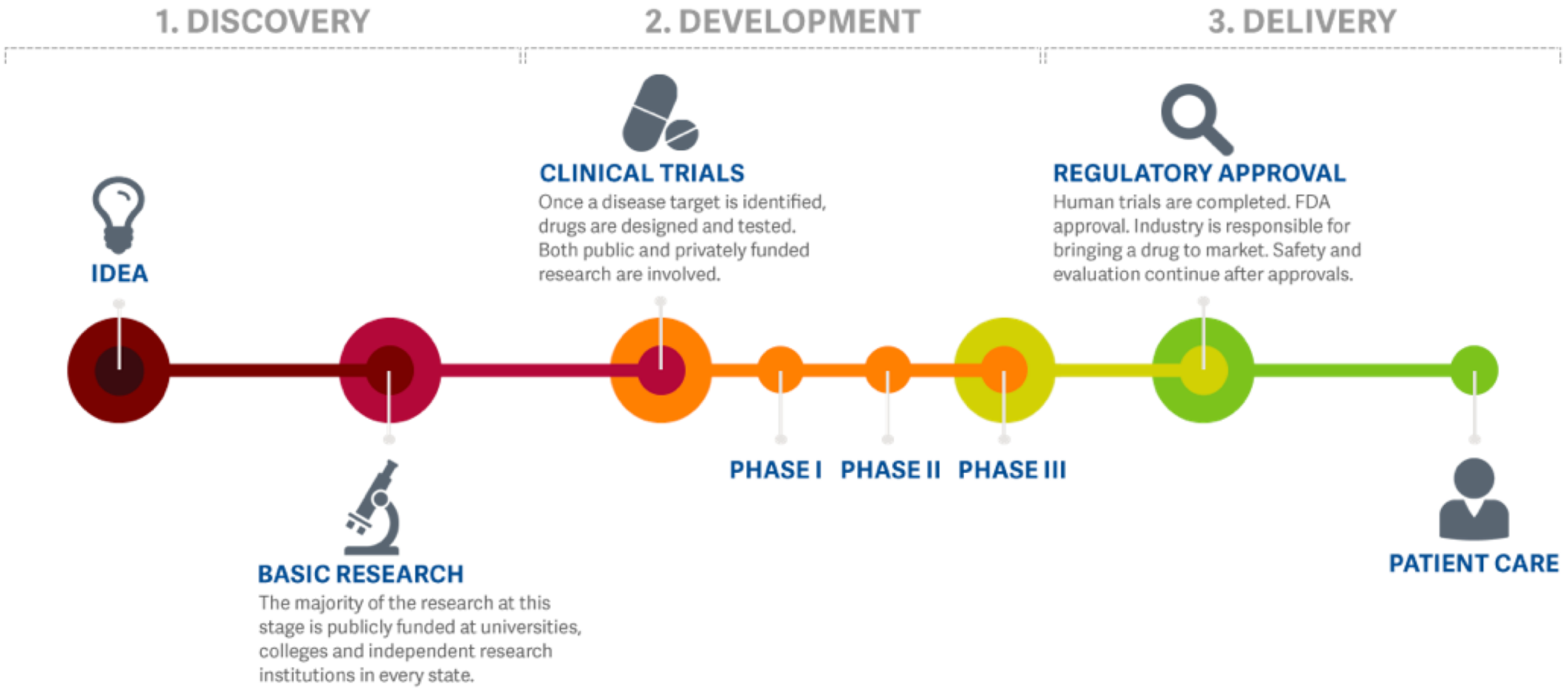
What level of confidence does the patient have that a healthcare option **will have specific effects** for them?

- Quality of evidence
  - Adherence to generally accepted methods
- Consistency of evidence
  - Variability of study results
- Differences in treatment effects
  - Variance in demographics, comorbidities, disease stage

Change by Co-Production & Co-Design



New Ways to Engage Patients & Caregivers



Value Questions for Patient and Caregiver KOLs

Defining product features	▪ What symptoms of the disease are most disturbing to daily life? ▪ How do you prioritize the importance of the different disease symptoms? ▪ How might you measure those most troublesome symptoms? ▪ 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? ▪ Would you be satisfied to hold the disease progression in check or is an actual improvement in symptoms the only path forward?
Optimize lead candidates	▪ What levels of risk are you comfortable taking in trying novel treatments? What type of risks would you tolerate for what levels of efficacy? ▪ 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? ▪ Which routes of administration could you consistently and reliably manage? ▪ How do you rate different routes of administration against each other?
Preparation for clinical entry	▪ Are you willing to share your thoughts on the target profile and the preclinical results you have seen with regulators (e.g., FDA, EMA)? ▪ Are you willing to attend regulatory meetings alongside the sponsor? ▪ Are you willing to participate in a patient registry and longitudinal studies that assess the progression of your disease? ▪ Are you willing to participate in clinical trials testing new drugs?

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:

- 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†

- 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†

Discovery & Pre-Clinical‡ → **Phase 1 - 3** → **Regulatory Review** → **Post-Approval**

- 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†

- *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

CLINICAL TRIALS TRANSFORMATION INITIATIVE

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.[1] 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.

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Thank you

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