

National Organization for Rare Disorders  
(NORD)  
Rare Diseases & Orphan Products  
Breakthrough Summit  
Arlington, VA

*Special Training for Rare-Disease Patient Advocates*

*October 23, 2015*

*The Different Levels of Patient  
Engagement—Case Examples of  
Making the Most of Limited  
Resources*

# Presenters



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# Acknowledgements

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Chinenye Anyanwu, PharmD, MPH, *University of Maryland School of Pharmacy*

Jenna Koschnitsky, PhD, *Hydrocephalus Association*

# Learning Objectives

*At the end of this session, participants will be able to:*

- Identify examples of patient-engagement methods along the continuum - from low effort to highly involved
- Construct realistic short-term organizational PCOR goals and longer-term aspirations

# Session Overview

1. Patient engagement definitions and characteristics
  - Sophia Johnson, University of Maryland School of Pharmacy
2. Case example of patient engagement in research
  - Megeen White, Hydrocephalus Association

# Steps in CER Process

Topic Solicitation

Prioritization

Framing The Question

Select Comparators & Outcomes

Create Conceptual Framework

Analysis Plan

Data Collection

Reviewing and Interpreting Results

Translation

Dissemination

# CER to CER/PCOR

Goal: Help patients and caregivers make better informed decisions



# Methods Standards Associated with Patient Centeredness

PC-1:

**Engage** *people representing the population of interest* and other relevant stakeholders in ways that are appropriate and necessary in a given research context.

*...includes individuals who have the condition*

# Patient Engagement

Definition: **Bi-directional relationship** between patient and researcher/research team

Principles of engagement (the rubric):

- Reciprocal relationships
- Co-learning
- Partnership
- Trust
- Transparency
- Honesty

# Patient Engagement Along the Research Continuum

Research Step/ Engagement Opportunity	Purpose of Engagement
Topic Solicitation	Identify <b>topics important to patients</b> , caregivers and the community
Prioritization	<b>Relevance</b> and priority of topics, assuring urgency of topics
Framing the Question	Ascertain <b>relevant, useful questions</b> with real-world applicability
Selection of Comparators and Outcomes	Identify comparator treatments & outcomes of interest, making research <b>actionable in a real-world setting</b>

# Patient Engagement Along the Research Continuum

Research Step/ Engagement Opportunity	Purpose of Engagement
Creation of Conceptual Framework	Provide a <b>reality check</b> , supplement with factors not documented in literature yet
Analysis Plan	<b>Verify importance</b> of variables, identify proxy for concepts, inquire about potential confounders
Data Collection	Determine <b>best approaches</b> for data collection, assist with data source selection

# Patient Engagement Along the Research Continuum

Research Step/ Engagement Opportunity	Purpose of Engagement
Reviewing and Interpreting Results	Assess <b>believability</b> of results, suggest <b>alternate explanations</b> , provide suggestions to develop <b>sensitivity analyses</b>
Translation	<b>Interpret results in a meaningful way</b> , identify results that are easy versus difficult to understand and counterintuitive
Dissemination	<b>Facilitate engagement of other patients</b> , help other patients understand findings

# PCORI Engagement Rubric

1. **PLANNING THE STUDY:** Describe how patient and stakeholder partners will participate in study planning and design.
2. **CONDUCTING THE STUDY:** Describe how patient and stakeholder partners will participate in the study conduct.
3. **DISSEMINATING THE STUDY RESULTS:** Describe how patient and stakeholder partners will be involved in plans to disseminate study findings and to ensure that findings are communicated in understandable, usable ways.

# Levels of Engagement



- **Stakeholder-Directed**
  - Patient/Patient group led
- **Partnership**
  - Investigator/Co-investigator
- **Collaboration**
  - Advisory committee member
- **Consultation**
  - Consultant
  - Interviews
  - Focus groups
  - Surveys
- **Informal**
  - Unstructured discussions
- **Study participant**

# Current State of Engagement in PCORI Projects

- 90% of PCORI projects engaged patients
- 23% of patient engagement relationships >3 years
- Contributions include:  $\Delta$ s to methods, outcomes, goals, measurement tools, data interpretation



# *Case Examples*

# The Cerebra Foundation

- National charity for “brain injured” children and young people
- Mission: Provide high-quality health and social-care information for the parents and carers of children (0 to 16 years) with neurological conditions
- Strive to improve the lives of children with neurological conditions, through research, information and direct on-going support
- Support programs include:
  - Online and face-to-face support
  - Grants to cover the cost of equipment and services
  - Regional family support forums

# The Cerebra Foundation

- Identifying unanswered **questions from CF inquiries**:
  - Members of CF sought information about osteopathy treatment for their children with cerebral palsy
  - Limited evidence on whether osteopathy treatments are effective
- Preparing for the RCT using a qualitative study:
  - Objective: *Design* based on patients experiences *a feasible RCT* that is responsive to their expectations and needs
  - Researchers sought **input from members of CF** to determine their views on **study design and outcomes**

Forsythe et al. A Systematic Review of Approaches for Engaging Patients for Research on Rare Diseases. J Gen Intern Med. 2014; 29(Suppl3): S788-800  
Edwards V, Wyatt K, Logan S, Britten N. Consulting parents about the design of a randomized controlled trial of osteopathy for children with cerebral palsy. Health Expectations. 2011;14(4):429–438.

# The Cerebra Foundation

## Qualitative study methods:

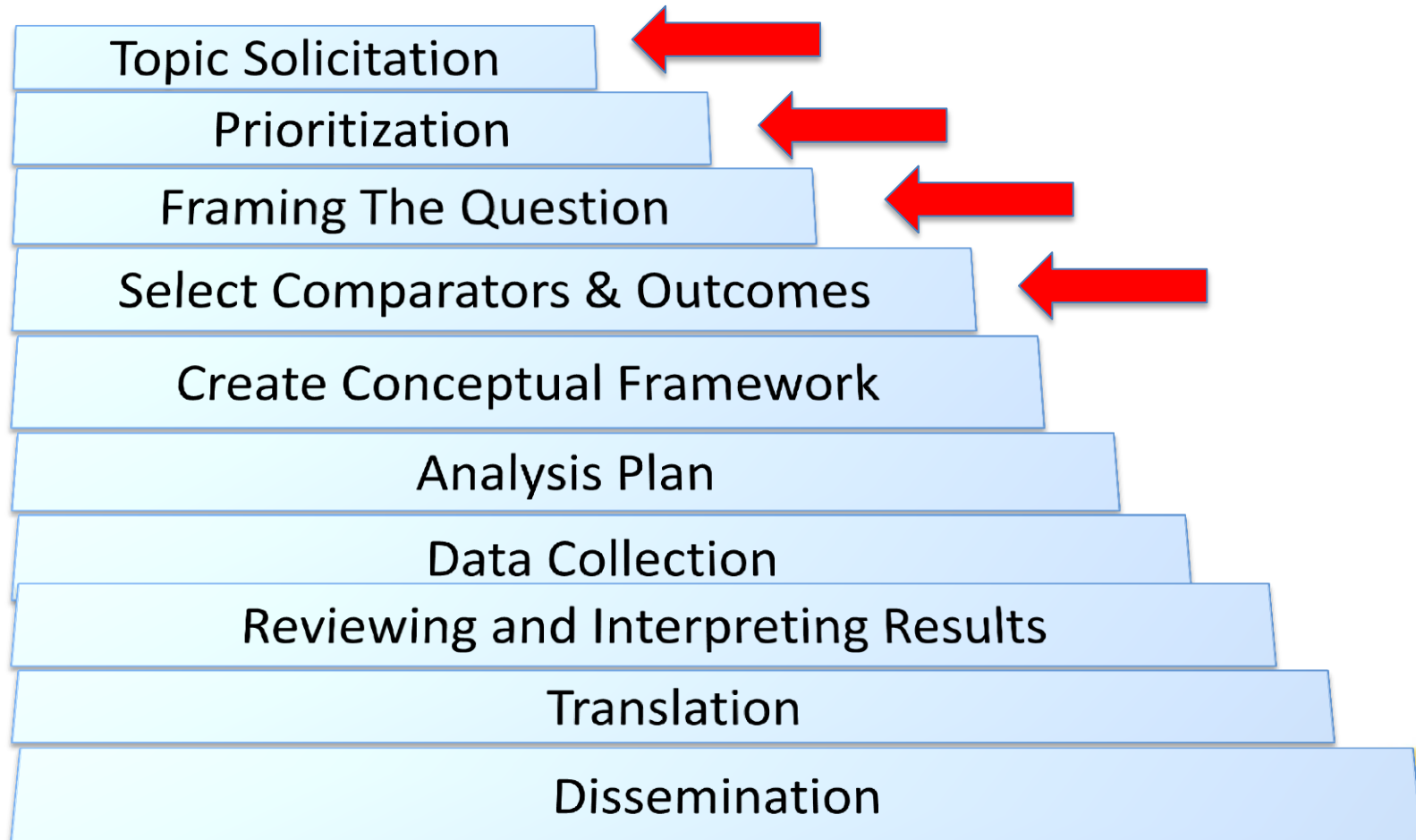
- CF recruited parents to participate
  - Wrote letters to members to see if they would be interested in participating
  - Recruited “newer” members only, as to **not overburden older members** who participated in other research projects
- Researchers conducted semi-structured interviews with parents **in their homes**

# The Cerebra Foundation

## Qualitative study methods

- 20 semi-structured interviews
  - Study design
    - Parents provided feedback on the **feasibility** of four different study designs and whether families would participate
  - Selecting outcome measures
    - Parents were asked to consider what **changes** they would notice in their child to **show that a treatment helped them**

# Patient Engagement: The Cerebra Foundation



# National Association for the Relief of Paget's Disease (NARPD)

- Also known as the Paget's Association
- Only UK support group for people living with Paget's disease
- Aims to:
  - Offer support and information to Paget's disease sufferers
  - Sponsor research into the causes and treatment of the disease
  - Raise awareness of the disease among medical profession and public
- Founded in 1973, membership approximately 2000 (patients, caregivers, healthcare professionals, researchers)

Forsythe et al. A Systematic Review of Approaches for Engaging Patients for Research on Rare Diseases. *J Gen Intern Med.* 2014; 29(Suppl3): S788-800  
Langston AL, McCallum M, Campbell MK, Robertson C, Ralston SH. An integrated approach to consumer representation and involvement in a multicentre randomized controlled trial. *Clinical Trials.* 2005;2(1):80-87.

# National Association for the Relief of Paget's Disease (NAPRD)

## PRISM Trial:

- UK multicenter pragmatic RCT
- Compares two treatment strategies for Paget's Disease of the bone
- PRISM Trialist-NAPRD **Partnership**
  - Established at beginning of PRISM trial
  - Aid in the design, conduct, and delivery of the PRISM trial

Langston AL, McCallum M, Campbell MK, Robertson C, Ralston SH. An integrated approach to consumer representation and involvement in a multicentre randomized controlled trial. *Clinical Trials*. 2005;2(1):80–87.



# National Association for the Relief of Paget's Disease (NAPRD)

## Roles of NAPRD:

- Trial Steering Committee membership
  - Developed research question to make sure it was relevant and important to the organization
  - Reviewed study protocol
  - Influenced ongoing conduct of trial

# National Association for the Relief of Paget's Disease (NAPRD)

## Roles of NAPRD:

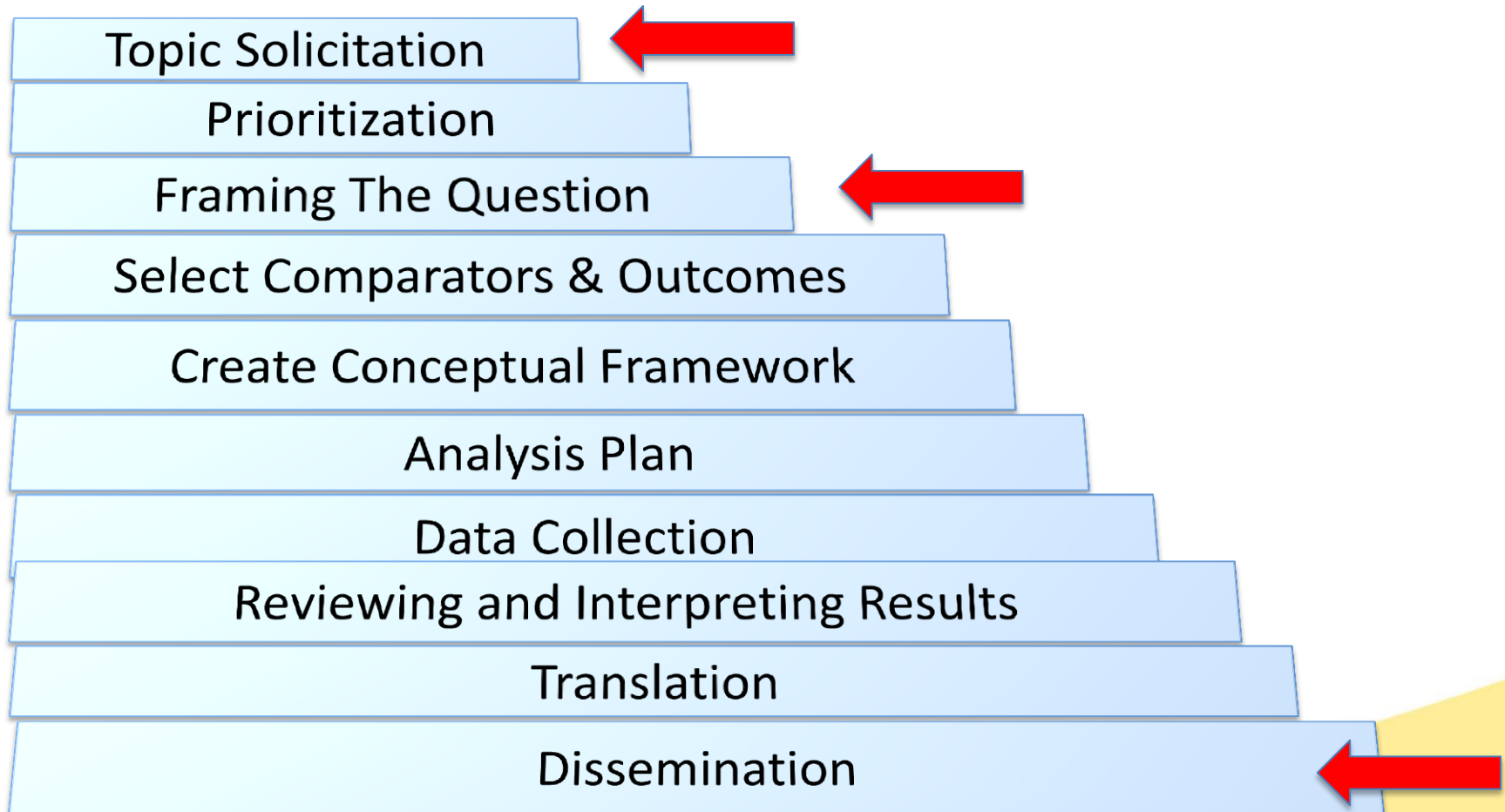
- Peer Reviewed materials for study participants
  - Ensure materials were appropriate for patients
- Advised trial team
  - Tips for presenting to patients and lay audiences
  - Provide recommendations for how to respond to study participant questions

# National Association for the Relief of Paget's Disease (NAPRD)

## Roles of NAPRD:

- **Promotion** amongst Paget's disease patients and partners
  - Shared database of Paget's disease specialists with trial team
  - Information included in quarterly newsletters
  - Educational conferences and meetings
  - Local support group meeting
  - Recruitment
  - “Patients Day” event

# Patient Engagement: Paget's Disease



# National Association for the Relief of Paget's Disease (NAPRD)

## Benefits of partnership:

- NAPRD
  - Increased awareness of Paget's disease
  - Increased access to relevant health research
  - Increased awareness of NARPD services
- PRISM Trial Team
  - Recruitment of patients
  - Well informed participants
  - Unsolicited patient advocacy of the trial
  - Interested and proactive collaborators

*Hydrocephalus Association*  
*Megeen White, RN, MS, OCN, CNL*

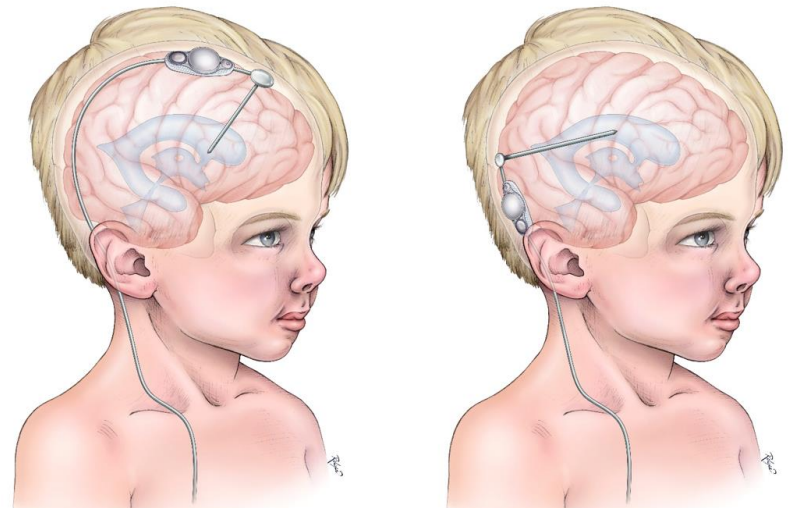
# Hydrocephalus Association

- National nonprofit organization focused on support and education, advocacy, and research for people with hydrocephalus
- **Mission:** Promote a cure for hydrocephalus and improve the lives of those affected by the condition
- Research programs include:
  - Discovery Science Awards
  - HA Network for Discovery Science
  - Hydrocephalus Clinical Research Network (pediatric focus)
  - Adult Hydrocephalus Clinical Research Network (adult focus)

# Hydrocephalus Association

## CSF Shunt Entry Site Trial:

- U.S. multicenter pragmatic RCT
- Compares two standard shunt entry sites
- Outcomes: shunt failure, quality of life
- Hydrocephalus Association-Hydrocephalus Clinical Research Network (HCRN) Partnership
  - Partnership established in 2012
  - Conduct high-quality, high-impact clinical research in hydrocephalus



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Anterior Entry Site

Posterior Entry Site

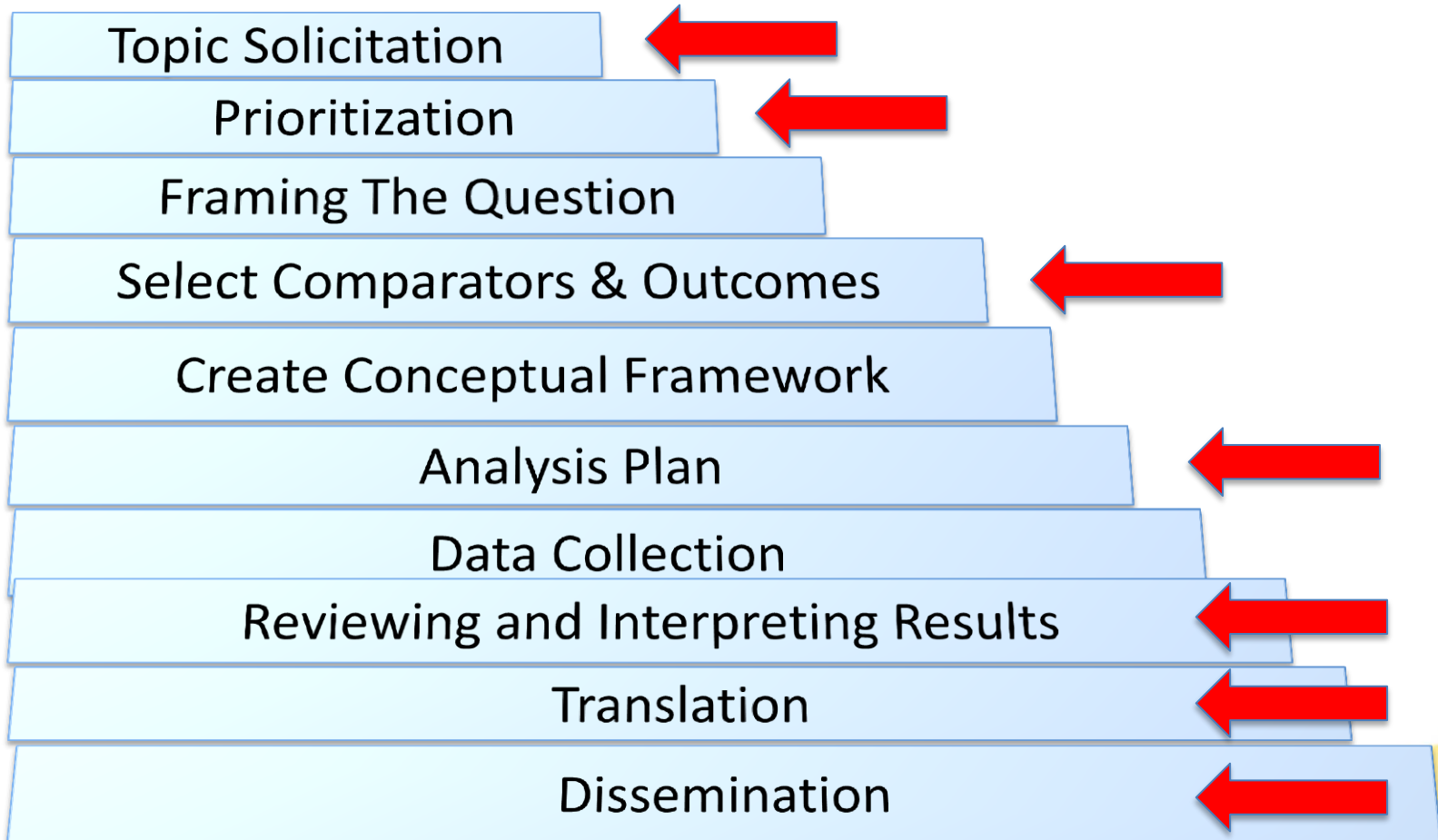


# Hydrocephalus Association

## Role of the Hydrocephalus Association:

- Patient Survey
  - Selection and prioritization of research topic
- Patient Partner Committee
  - Approval of design, outcome measures, consent form, web postings
  - Aid in translation of study results
  - Design and execute dissemination plan
- Steering Committee membership
  - Finalize case report forms (data forms)
  - Solve ongoing issues throughout the trial
- Investigator Committee membership
  - Review study progress
  - Involvement in analysis, interpretation, and translation of study results

# Patient Engagement: Hydrocephalus Association



# Summary of Introductory Principles

- **Patient engagement is critical !!!**
  - Integration of the patient voice will improve the value of the research and better inform patient and caregiver healthcare decision-making.
- Continuous patient engagement across stages of research process is optimal.
- Patients and patient groups need to be prepared to meaningfully engage!

# Questions

# Session Resources

- PCORI Engagement Rubric. PCORI (Patient-Centered Outcomes Research Institute) website. <http://www.pcori.org/sites/default/files/Engagement-Rubric.pdf>. Published February 4, 2014. Updated October 13, 2015.
- Workshop on Incorporating the Patient Perspective into PCOR. PCORI (Patient-Centered Outcomes Research Institute) website. <http://www.pcori.org/sites/default/files/Workshop-on-Incorporating-the-Patient-Perspective-into-PCOR1.pdf>
- Forsythe et al. A Systematic Review of Approaches for Engaging Patients for Research on Rare Diseases. J Gen Intern Med. 2014; 29(Suppl3): S788-800
- Mullins et al. Continuous Patient Engagement in Comparative Effectiveness Research. JAMA. 2012; 307 (15): 1587-1588.

# Session Resources

- Landy DC, Brinich MA, Colten ME, Horn EJ, Terry SF, Sharp RR. How disease advocacy organizations participate in clinical research: a survey of genetic organizations. *Genet Med*. 2012;14(2):223–228.
- Edwards V, Wyatt K, Logan S, Britten N. Consulting parents about the design of a randomized controlled trial of osteopathy for children with cerebral palsy. *Health Expectations*. 2011;14(4):429–438.
- Langston AL, McCallum M, Campbell MK, Robertson C, Ralston SH. An integrated approach to consumer representation and involvement in a multicentre randomized controlled trial. *Clinical Trials*. 2005;2(1):80–87.