PBRNs CAN HELP INDUSTRY AND PATIENTS

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Chief Science Officer
National Pharmaceutical Council
• Health policy research group
• Funded by the pharmaceutical industry
• Non-advocacy
• Focus on:
  – Balancing the needs of the population with those of the individual
  – Value of Rx in a changing environment
  – Role of real-world evidence
PBRNs Can Help Patients and Industry

1. Answer the unanswered questions
2. Clarify treatment heterogeneity
3. Not without regulatory guidance
1. Answer the Unanswered Questions

Can it work?

Many other unanswered questions
Patients

Intervention

Comparisons

Outcomes

Time

Setting

Framework can identify unanswered questions
Framing the Research Question

Patients
• Who has been studied in RCTs?
• Older?
• With comorbidities?
• Lesser disease severity?
Framing the Research Question

• Patients
• Interventions
  – How critical is compliance?
  – How critical is the provider?
Framing the Research Question

- Patients
- Interventions
- Comparators
  - Need for active therapies
  - Multiple comparisons
Framing the Research Question

• Patients
• Interventions
• Comparators
• Outcomes
  – Medication compliance
  – Total cost of care
  – QOL
  – Workplace productivity
Framing the Research Question

- Patients
- Interventions
- Comparators
- Outcomes
- Time
  - Long term as care continues
Framing the Research Question

- Patients
- Interventions
- Comparators
- Outcomes
- Time
- Setting
  - Small group vs. large multi-specialty group
  - Academic center vs. community based care
2. Clarify Treatment Heterogeneity

- Treatment Heterogeneity
  - Cross overs
  - N-of-1
3. Regulatory Clarity Needed

• Can industry communicate the results?
• Will PBRN studies add to or replace registrational trials?
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