CLINICAL TRIALS TRANSFORMATION INITIATIVE

June 12, 2018

Qualitative Interview Findings

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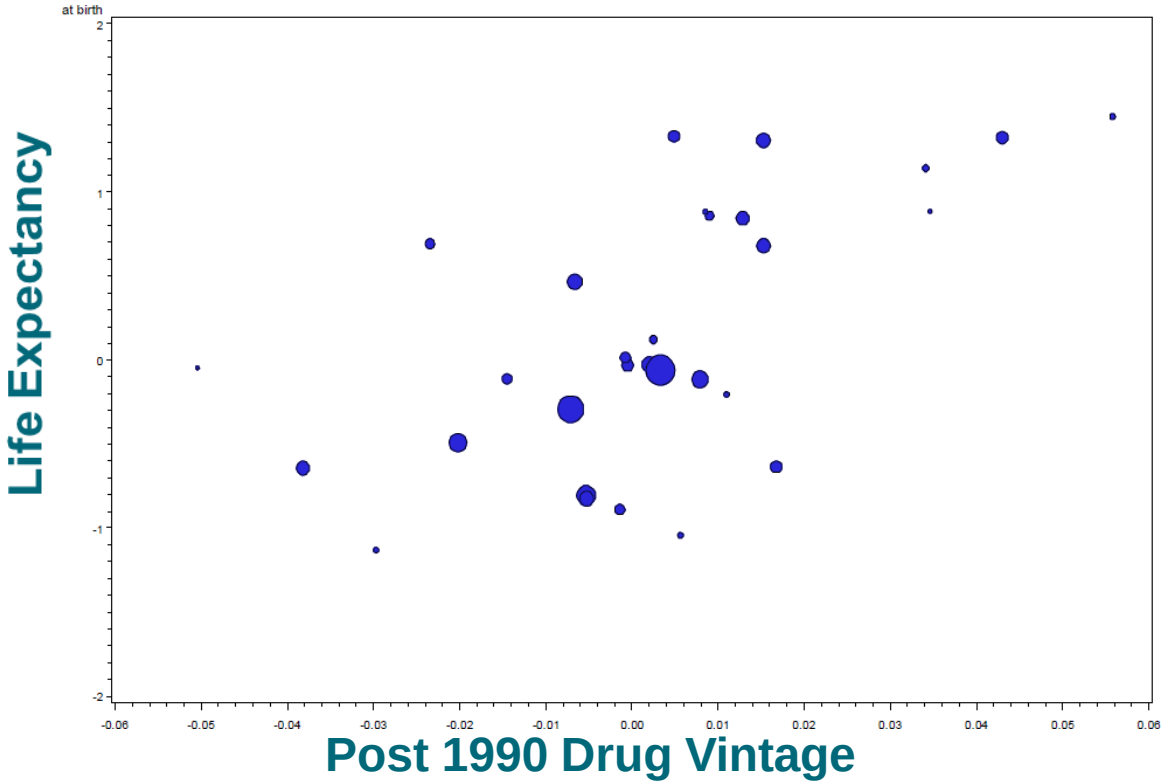
Disclaimer

The views and opinions expressed in this presentation are those of the individual presenter and do not necessarily reflect the views of the Clinical Trials Transformation Initiative or Janssen Pharmaceutical Companies of Johnson and Johnson.

My Inspiration

Gain in life expectancy from drugs launched after 1990: 1.27 years.



Lichtenberg et al. Pharmaceutical Innovation and longevity growth in 30 developing and high income countries, 2000-2009. National Bureau of Economic Research Working Paper Series.

CTTI

CTTI Social Scientist Team

CTTI's internal Social Scientist Team worked with the CTTI RWE project team to design and conduct qualitative in-depth interviews.

- Principal Investigator: Amy Corneli

  Interviewers: Brian Perry; Teri Swezey

- Data Analysts: Brian Perry; Carrie Dombeck; Brigid Grabert; Teri Swezey

Objectives and Methods

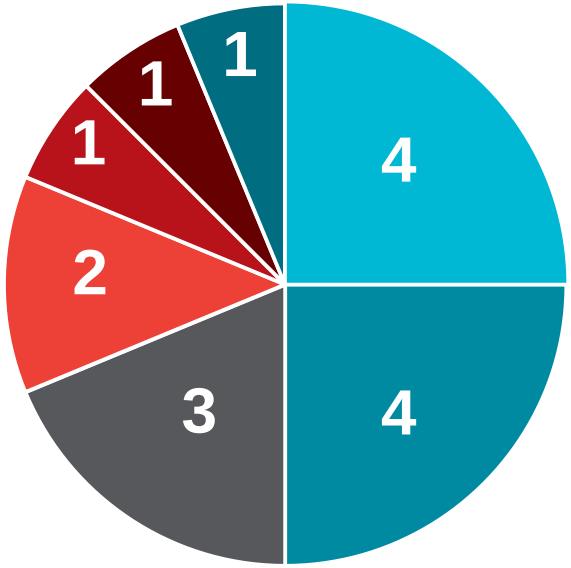
- Objectives:
  - Describe how RWD sources facilitate planning and execution of clinical research.
    - Identify challenges and solutions
  - Describe how RWD can generate evidence for regulatory decisions.
    - Identify guidance points for regulatory decisions
- Methods
  - 1:1/group expert interviews December 2017 – February 2018.

Interview Scope

- Describe:
  - Experience in using RWD in detail.
  - Current and future use of RWD in randomized trials.
    - Questions interviewees had for FDA or guidance needed from FDA.
  - Top recommendations for others considering using RWD in clinical research.

Participant Demographics

Number of Interviews



Professional Affiliation

- Academia
- Biopharmacuetical Company
- Payer-affiliated Research Group
- Health System Research Group
- CRO
- Government Organization
- Professional society

CTTI

RWD: Benefits & Future Opportunities

- Combine with traditional RCTs to increase efficiency
  - Inform patient population selection
  - Identify patients at risk
- Replace traditional RCT with pragmatic RCTs
  - Reduce cost
  - Decrease patient burden
  - Improve generalizability
  - Understand effectiveness
- Enhance rigor
  - Collect long-term data

RWD: Benefits & Future Opportunities

"The value of collecting real world evidence is that it's cheap, which means you can keep doing it. And you can do it longitudinally, and you can do it indefinitely, as long as you set up a system."

RWD: Weaknesses

"I think the biggest area where you have to be careful in is what questions you ask with respect to how do you determine the endpoint? And if you determine an endpoint that needs input from the patient, for example, that's problematic because you have to reach the patient."

data studies in the scope of regulatory trials is that if you have a product or compound for an indication for which it is not yet approved, you can't really do a real world data study because that information is not there. There's no real-world aspect to it."

RWD: Weaknesses

- Reliability/validation
- Quality
- Depth
- Subtle Outcomes

Challenges

- Pooling multiple sources/international sources
- Common data model
- Linking
- Regulatory pathway
- Data latency
- Patient privacy
- Data governance
- Defining: Evidence

THANK YOU.

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