CLINICAL TRIALS TRANSFORMATION INITIATIVE

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Use of RWD in Pre-Study Planning and Study Set up: A Manufacturer Perspective

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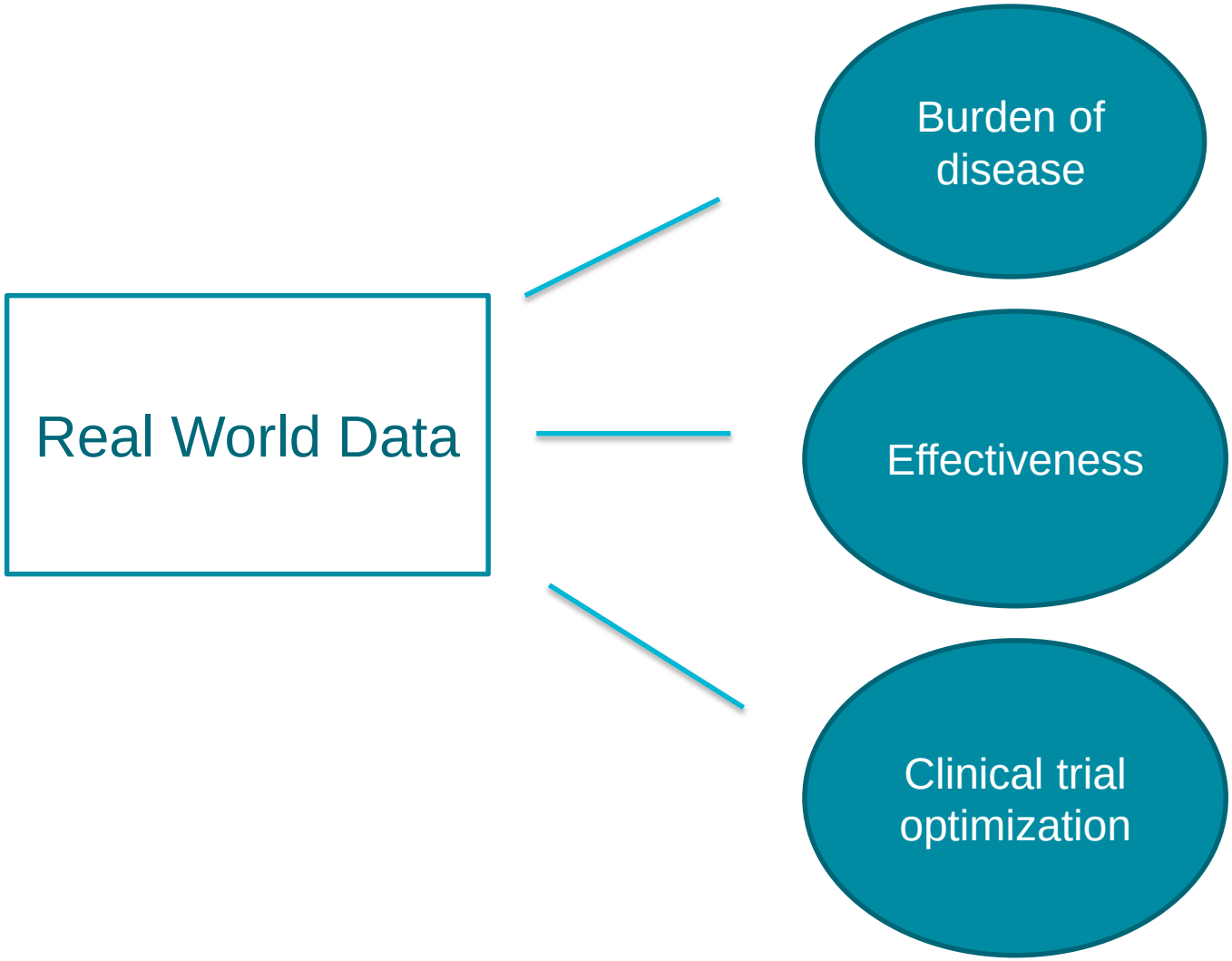
Real World Data Applications



Increased investment in Real World Data

Pre-study planning examples

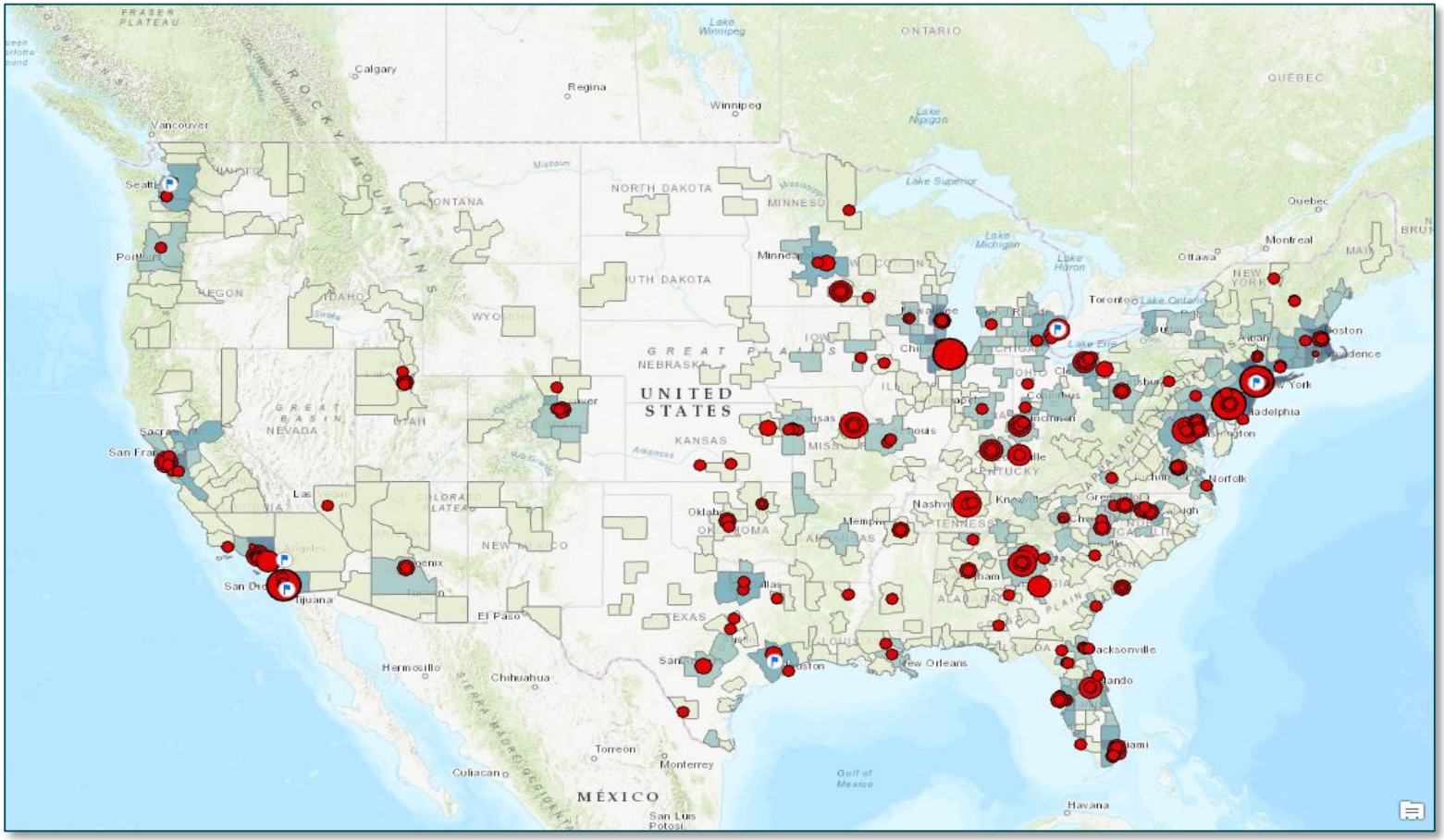
Evaluating inclusion/exclusion criteria using administrative claims data

Illustrative Example

Exclusion Criteria		
Exposure	Number	Percent
All eligible patients in 2016	31,721	100.0
Meeting age criteria	29,724	93.7
History of malignancy within the past 5 years	26,535	83.7
History of Condition X within the past 5 years	26,535	83.7
Condition Y	25,510	80.4
Condition Z	25,079	79.1
History of moderate-severe mental health illness	21,053	66.4
'Active' pregnancy	20,133	63.5
Co-infection criteria #1	19,051	60.1
Co-infection criteria #2	18,305	57.7
Co-infection criteria #3	16,870	53.2
Treatment A	16,359	51.6
Treatment B	2,815	8.9

Using RWD and other data to inform study site selection

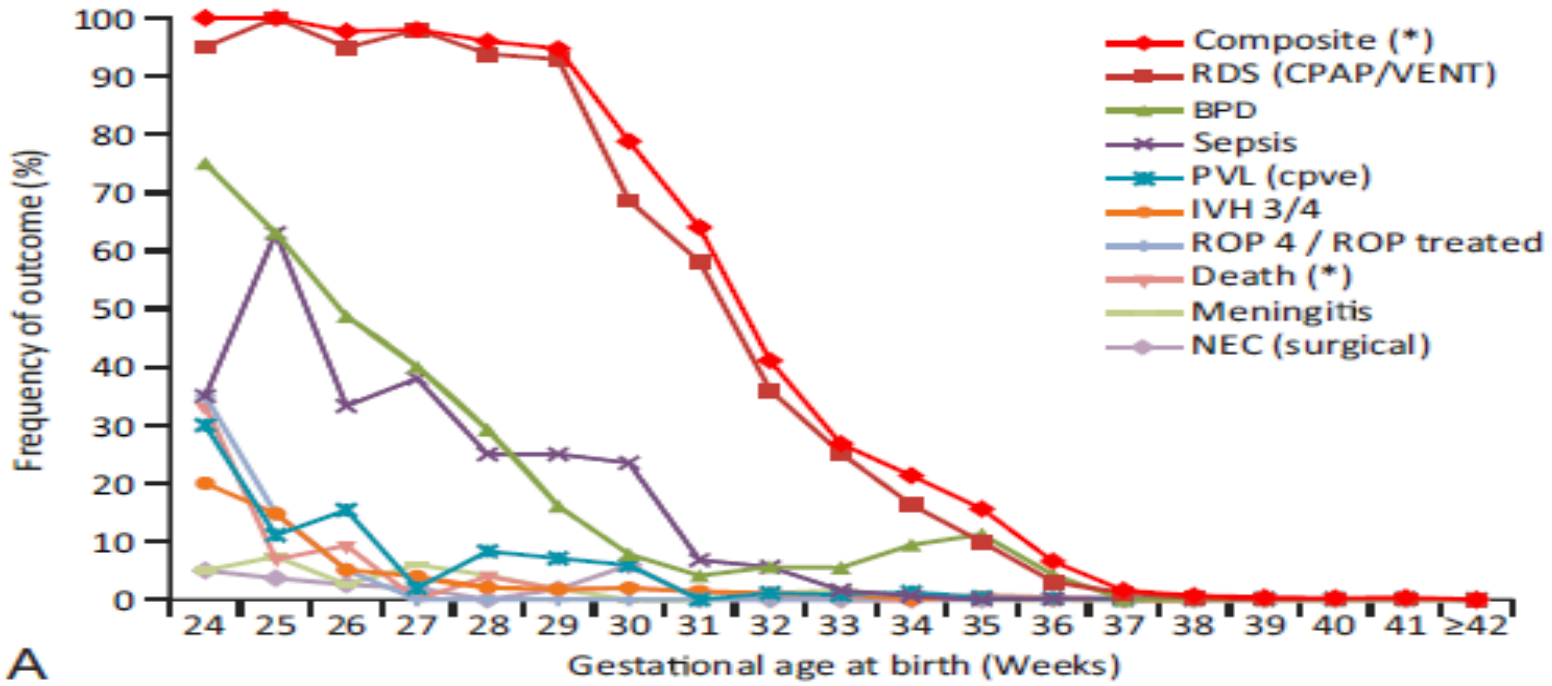
- Large administrative claims data can be used to generate patient densities
- Potential to add socioeconomic and demographic data
- Overlay study sites



Pre-Study Planning to Enhance Study Design

New study endpoint and sample size calculation

- FDA requires demonstration of neonatal benefit in Phase III studies for preterm labor, but limited data were available on event rates of neonatal morbidities and how these can be combined
- Data from Medical University of South Carolina Perinatal Information System were used to develop the composite endpoint



Pimenta et al. A Retrospective database analysis of neonatal morbidities to evaluate a composite endpoint for use in preterm labor clinical trials. Am J Perinatol Rep 2018; 8:e25-e32

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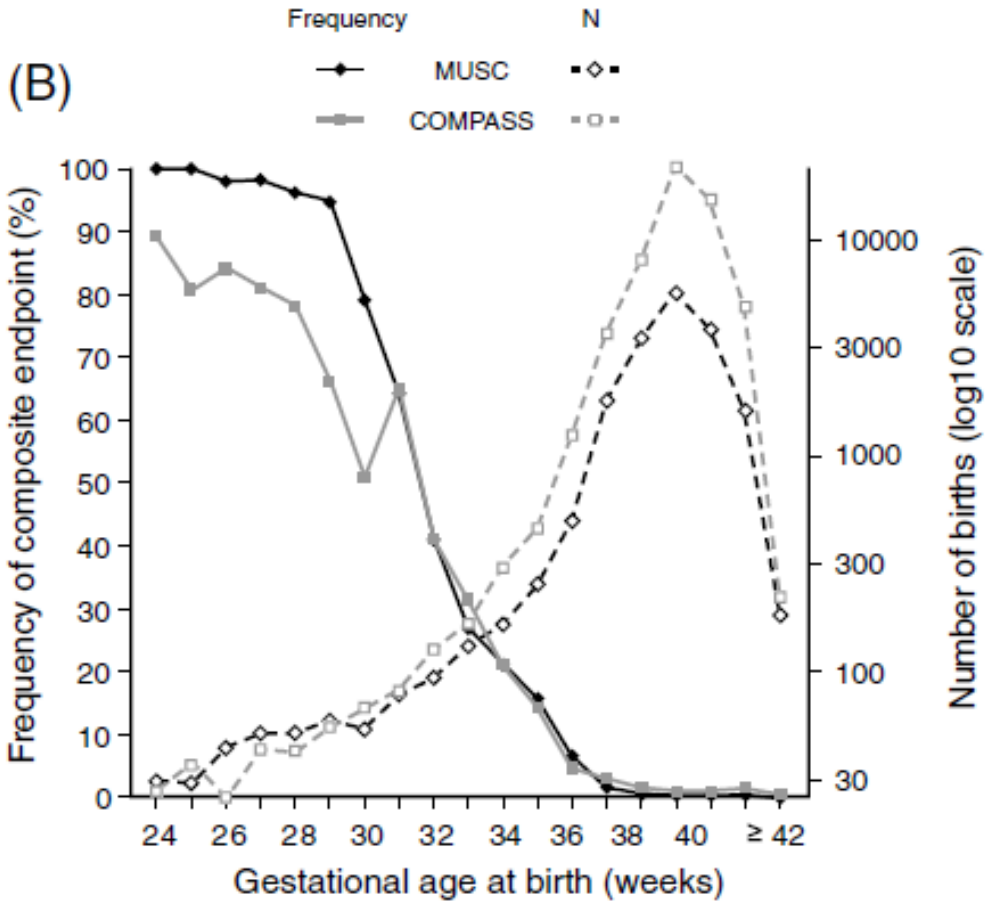
Pre-Study Planning to Enhance Study Design

New study endpoint and sample size calculation

- Generalizability of composite endpoint was evaluated using EHR data from COMPASS IDNs
- Opportunity to evaluate neonatal outcomes in a simulated clinical trial population using real world data
- Lower rates of neonatal outcomes based on composite endpoint found in COMPASS vs MUSC
- Adaptive trial design implemented for phase 3 with interim assessment that would allow sample size readjustment to accommodate heterogeneity of preterm labor population



Pimenta et al. Assessing a composite endpoint for new tocolytics in clinical trials: Data from 4 US Integrated delivery networks. Pharmacoepidemiology Drug Safety 2018; 27:213-220.

Opportunities and Challenges

- Opportunities
  - RWD can provide insights into study feasibility and design
  - Assist in identify where to find potential study subjects
  - Define and model relevant outcomes

- Challenges
  - Administrative claims data lacks clinical details
  - EHR data heterogeneity across systems
  - Additional data validation needed for broader use of EHR data

THANK YOU.

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