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

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Disclaimer

The presenter is an employee of GlaxoSmithKline.

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Real World Data Applications

- Burden of disease
- Effectiveness
- Clinical trial optimization
Increased investment in Real World Data

- Administrative Claims Data
- EHR Linked to Claims Data
- EHR Data
Pre-study planning examples
Evaluating inclusion/exclusion criteria using administrative claims data

*Illustrative Example*

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

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

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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