A Brief Introduction to Pharmacoepidemiology

Tobias Gerhard, PhD
Assistant Professor, Ernest Mario School of Pharmacy
Institute for Health, Health Care Policy, and Aging Research
Rutgers University

Pharmacoepidemiology

The study of the use of and the effects of drugs in large numbers of people. It applies the methods of epidemiology to the content area of clinical pharmacology.

In: Strom BL, Kimmel SE, Hennessy S, editors. Pharmacoepidemiology

Pharmacopidemiology
The study of the frequency, distribution, and determinants of disease in humans, with “determinants” being drugs, vaccines, or devices.

In: Hartzema AG, Tilson HH, Chan KA, editors. Pharmacoepidemiology and Therapeutic Risk Management

Epidemiology in 2x2 Tables

<table>
<thead>
<tr>
<th>Exposure</th>
<th>Disease</th>
</tr>
</thead>
<tbody>
<tr>
<td>+</td>
<td>-</td>
</tr>
<tr>
<td>A</td>
<td>B</td>
</tr>
<tr>
<td>C</td>
<td>D</td>
</tr>
</tbody>
</table>

In the beginning, the epidemiologist created the cohort study and the 2x2 table, and exposure was dichotomized and disease was dichotomized, and thus the 2x2 table had four cells, the cell of A, the cell of B, the cell of C, and the cell of D. And the epidemiologist saw that it was straightforward and deemed that it was good.

- K. Rothman, 2001
US Regulatory Development

“History Matters”

• 1906: The Jungle –
  * Pure Food and Drug Act
  * Illegal to manufacture adulterated or misbranded foods or drugs

• 1938: Elixir of Sulfanilamide
  * Food, Drug, & Cosmetics Act
  * Safety, before marketing a drug

• 1962: Thalidomide
  * Kefauver-Harris Amendments
  * Efficacy, before marketing a drug (on-label/off-label)

US Regulatory Development (cont)

“History Matters”

• 1992: Lengthy approvals
  * PDUFA
  * Timeliness, funding, approval standards

• 2007: Vioxx and Other Drug Crises
  * FDAAA
  * Post-marketing drug safety, Sentinel, REMS

Clinical Development

• Observation
  * Anecdote → Collection of anecdotes → Data?
  * Spontaneous case reports → Pharmacovigilance
  * Hospital-based surveillance

• Inference
  * Resolving the counterfactual
    * Patient took drug → patient had adverse outcome
    * What would have happened if, counter to fact, patient had not taken drug?
    * How?

• Mechanistic understanding
  * Physiology/pharmacology
  * Genetics
Methods Development

- Case reports/series
- Cross sectional studies
- Ecologic studies
- Cohort studies (+/- randomized allocation)
- Case control studies
- Case-based studies
- Grand unified theories

Analytic Development

- World is flat (2 dimensions)
  - Case report(s)
  - The wheel (the 2x2 table)
- World is round (3+ dimensions)
  - Multivariable analyses
  - Survival analyses
  - Propensity scores
  - Etc...

Pharmacoepidemiologic Evolution/Drift

- Regulatory
- Industry
- Society

Methods/Analysis

Clinical

Concept/Definition
Presentations This Afternoon

- **Introduction**, Tobias Gerhard 8:30 - 8:40
- **Cohort Studies**, Almut Winterstein 8:40 - 9:30
- **Case-Control Studies**, Tobias Gerhard 9:30 - 10:20
- Break 10:20 - 10:50
- **Confounding and Bias**, Soko Setoguchi, FISPE 10:50 - 11:40
- **Introduction to Pharmacovigilance**, Syed Rizwanuddin Ahmad, FISPE 11:40 - 12:30

Thank you!

Contact:
tgerhard@rci.rutgers.edu