

**Highlights from the Fall 2008  
Applied Pharmacoeconomic and Outcomes Research Forum**

The fifth Applied Pharmacoeconomic and Outcomes Research Forum was held October 6<sup>th</sup> at the University of California San Diego Faculty Club. The event was hosted by the Skaggs School of Pharmacy and Pharmaceutical Sciences and supported by grants from Allergan and Biogen Idec.

The topic for the Forum was “***Real World Data for Decision Making: Moving Beyond Clinical Trials***”.

***Speakers:***

**Real World Data: Insight into the ISPOR Task Force Report**

***Andreas Pleil, PhD***

Senior Director

Worldwide Medical & Outcomes Research

Pfizer Global Pharmaceuticals

Member ISPOR Task Force on Real World Data

**How real world data has affected therapeutic decisions - Examples**

***T. Jeffrey White, Pharm.D., M.S.***

Director, Clinical Analytic Strategies

WellPoint NextRx

***Mark Bounthavong, Pharm.D.***

Pharmacoeconomics Clinical Specialist

VA San Diego Healthcare System

Forum participants included representatives from managed care, government, pharmaceutical and biotech companies, medical centers and academia. Each was invited because of mutual interest in expanding the practical application of pharmacoeconomic and outcomes research to enhance decision-making.

Speaker slides are posted at:

[Applied Pharmacoeconomics and Outcomes Research Forum - Fall 2008](#)

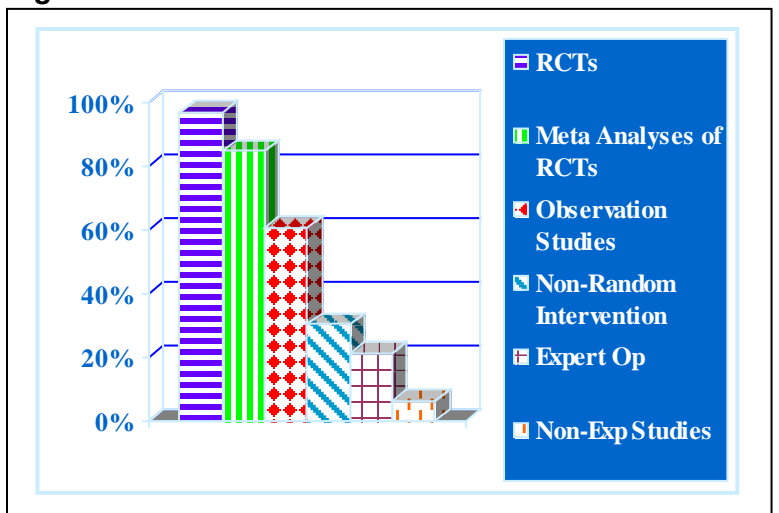
**Pre-Meeting Survey Results (Figures 1 and 2)**

Prior to the meeting, participants were asked to respond to two questions related to their preference for types of evidence [Randomized Controlled Trials (RCTs) vs. Real World Data] and data sources. When considering decisions among pharmaceuticals, Randomized Controlled Trials (RCTs) or Meta Analyses of RCTs were the most frequently chosen preferred evidence types as opposed to more real world types of evidence (e.g. observations studies). For formulary decisions most respondents indicated their preferred data source would be “large simple trials”.

**QUESTION #1**

Considering the decision making process related to which pharmaceuticals to utilize in a patient population, which three **Evidence Types** do you consider most useful?

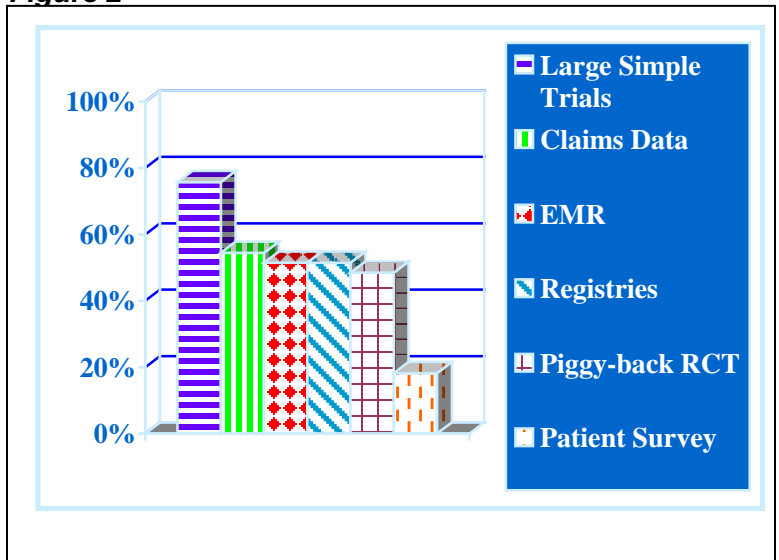
**Figure 1**



**QUESTION #2**

If you could have access to only three **DATA SOURCES** for formulary decision making, which would you choose?

**Figure 2**



## **Key Forum Summary Points**

### **Real World Data: Insight into the ISPOR Task Force Report**

Dr. Pleil, who served on the ISPOR Task Force on Real World Data, reviewed the highlights of the Task Force report and offered insight into the challenges and deliberations of the Task Force.

- Task Force Charge:
  - Develop a framework to assist health care decision-makers in dealing with “real world” data and information in “real world” health care decision-making, especially related to coverage and payment decisions.
- What is Real World data?
  - “Real World” data is anything OTHER than RCT generated data.....data derived from:
    - Prospective observational studies
      - Non-interventional observations
    - Database studies
      - Prospective registries create a database
      - Retrospective databases created for other reasons
    - Medical records
      - Data abstraction
- What is Real World evidence?
  - In general, “real world evidence” is what happens to data. Building the evidentiary portfolio requires the systematic unbiased collection of data. The validity of the evidence is dependent on the accuracy of the data and the appropriate organization to allow interpretation, analysis, and conclusions.
- Importance and Limitations of Real World Data
  - Although RCTs have many advantages and remain the gold standard, decision makers making coverage and payment decisions may rely on multiple sources of real world data as well.
  - Most significant concern is bias
    - Typically there is a selection bias in treatment decisions and this bias can lead to differences in outcomes (rather than due to treatment)
- Some Remaining Questions
  - Central policy question: what is the appropriate role of the public sector in producing and judging evidence?
  - Who should collect, pay for, and evaluate RW data?
  - Why should these data be collected?
    - Confirm RCT's?
    - Focus on safety, not efficacy?
    - Rationalize rationing?
    - Can these data help at the patient level to improve individual outcomes?

**Examples: How real world data has affected therapeutic decisions**

### **WellPoint NextRx**

Dr. White shared the role of Real World data within WellPoint and some factors and examples that may yield results contrary to those surmised from RCT data.

- WellPoint uses Outcomes-Based Formulary Management
  - Considers the complete burden of disease
    - Clinical Burden, Epidemiology, Natural History of Disease, Total Cost of Care, Productivity Impact, Quality of Life Impact
  - Leverages the formulary process to improve patient outcomes
    - Improve Quality of Care (clinical status, quality of life)
    - Reduce Total Cost(pharmacy, medical, ancillary, home health, nursing home, etc.)
    - Optimize Care (cost effectiveness)
    - Improve Productivity
- Compliance is a very important factor in driving differences between RCT results and Real World Study results
  - Compliance within RCTs usually much higher than in clinical practice
  - Real World compliance about 50%
  - Differs significantly by therapeutic category – thus affecting possible degree of difference between RCT and Real World study results
- Wellpoint Real World studies have revealed
  - Virtually no difference in the incidence of hip fractures between Fosamax and Actonel
    - RCT data (REAL Study) had previously shown Fosamax with a higher rate than Actonel
  - Consistently lower mean % LDL-C reduction for statins than reported in
    - STELLAR Clinical Trial
    - Package Insert

### **VA San Diego Healthcare System**

Dr. Bounthavong presented the group with an interesting example of how results of studies using Real World data can differ from results from RCTs and results of other Real World studies as well.

- Switching patients from Donepezil to Galantamine
  - Concern regarding destabilizing patients due to switch were raised
  - Published Clinical Trials and Real World data showed patients tolerated switching between these two products
    - Trial data
      - Well tolerated; little discontinuation of galantamine after switch
    - Real World data – another VA system
      - 2.0% experienced adverse drug reactions
      - 5.8% switched back to donepezil (VA average switch back after conversions = 10%)
- San Diego VA conversion process reviewed by experts and approved by Pharmacy & Therapeutics committee
  - Results

- 15.5% patients switched back to donepezil
  - This was ~3 times the switchback rate at the other VA and 50% more than the average
  - The conversion was halted
- In retrospect
- Past clinical trials and data from other VA did not evaluate or measure patient reported outcomes.
    - Burden of switch is not only to the patient, but to the care giver
    - Patient/caregiver perspectives should have been measured
  - Results of Real World studies in one health care system may not be the same when conducted within another patient population

***Purpose of Applied Pharmacoeconomic and Outcomes Research Forums***

The number of individuals in the Southern California region with interest in pharmacoeconomics (PE) is growing rapidly across the healthcare system - from those involved with creation of PE data within pharmaceutical and biotech companies to those incorporating results into decision making within a plethora of managed care organizations. The region provides an excellent opportunity to gather individuals to debate issues, and propose solutions that are vetted from multiple perspectives – not just individual silos defined by employer.

The Applied Pharmacoeconomic and Outcomes Research Forum was created to facilitate this cross perspective communication. The goals of the forum are to:

1. Discuss commonly encountered obstacles to conducting or utilizing results of applied pharmacoeconomic studies and explore solutions from various perspectives of the health care system.
2. Create an environment and foundation to foster the creation of a Southern California Pharmacoeconomic and Outcomes Research Interest Group

Current steering committee members are:

Charles Daniels, RPh, PhD  
UCSD Healthcare Department of Pharmacy

Darlene Fujimoto, PharmD  
Biogen Idec

Ted Ganiats, MD  
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