14 Forums and Twelve Years …

1. AMCP Dossiers: Useful for Decision Making or Not? A Debate
2. Biologics: Drawing (or Crossing) the line of Cost vs. Benefit: A Case in Oncology
3. Quality Adjusted Life Years (QALY’s) for Decision Making: Views from Canada & US
4. Using Large Databases to Inform Decision Making: Experience in 3 Healthcare Systems
5. Real World Data for Decision Making: Moving Beyond Clinical Trials
6. Pharmacoeconomics of Pharmacogenomics
7. Pharmacoeconomics & Pricing: Now & After Health Care Reform
8. What is the Business Case for Quality Improvement Initiatives?
10. Biosimilars: Challenges and Opportunities for All.
11. Patient Reported Outcomes: Useful for Whom?
13. New World of Biosimilars in the U.S. - Current Challenges to Inform Future Directions
14. Drugs to treat rare and ultra-rare diseases: Beyond the price and headlines.
Forum Objectives

• Discuss commonly encountered obstacles to conducting or utilizing results of PE studies for decision making

• Explore solutions from various perspectives
  • Managed Care
  • Government
  • Pharmaceutical/Biotech Firms
  • Medical Centers
  • Academia

• Create an environment & foundation to foster larger PE/Outcomes Research Interest Group
Steering Committee

Welcome “Trainees”

**Michael S. Broder MD, MSHS**
Partnership for Health Analytic Research

**Charles Daniels, RPh, PhD**
UCSD Health Department of Pharmacy

**Jan D. Hirsch, RPh, PhD**
UCSD, Skaggs School of Pharmacy & Pharmaceutical Sciences

**Mirta Millares, PharmD, FCSHP, FASHP**
Kaiser Permanente – CA Region

Thanks Mirta

**Anthony P. Morreale, Pharm.D., MBA, BCPS**
PBM Services, Department of Veterans Affairs

**Michael Pollock**
Reynolds Pollock & Associates

**Robert Schoenhaus, PharmD**
Sharp HealthCare
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- abbvie
- PHAR
  Partnership for Health Analytic Research, LLC
Drugs for Rare & Ultra-Rare Diseases
Beyond the Price and Headlines.

- Price tag on gene therapy for rare form of blindness: $850K
- Nearly every $100,000 drug approved in the last three years was for a rare disease
- Biotech company's stock jumps 40% on promising drug to treat rare lung disease
- U.K. Clears Glaxo's $700,000 ‘Bubble Boy Disease’ Gene Therapy
Quiz Time!
Smart Phones Down
Laptops & Tablets Closed
Top 5 Sci-Fi Drugs
- Dune
- The Spice
- Melange

7 Most Famous TV Docs
- Flashback… & Memory Test

10 Most Important Drugs
- **SANDOZ**
  - Cyclosporine discovery: travel policy “digging on location”

Biosimilars of Animal Kingdom
- Alpaca
- Llama
The Power of Serendipity

Slice of potato left in a lab

Petri Dish

- Colored growths
- Different bacteria

Proposed first solid growth medium where types of bacteria could be isolated

Selective Culturing

1881

Robert Koch

Julius Petri

His colleague created
1895

Mysterious green glow on photosensitive plate across room when experimenting with encased cathode ray tube

Experimented on wife

Her hand

Wilhelm Roentgen

X-rays
1928

No growth in areas accidently contaminated with ????

Penicillin

EXTRA TRIVIA
Fleming born same year Petri dish invented by Julius Petri

Alexander Fleming

Julius Petri
The Power of Serendipity

More after the Break!
Overview current issues surrounding rare and ultra-rare diseases

Michael S. Broder, MD, MSHS
President, Partnership for Health Analytic Research

Stakeholder Perspectives
Pharmaceutical Manufacturers
Denise Globe
VP and Head US Health Economics and Outcomes Research, Novartis

Health Plan/Payers
Steven G. Avey, MS, RPh
Vice President Specialty Pharmacy Programs, MedImpact

Patient Advocacy Groups
Kristina Bowyer
Executive Director, Patient Advocacy
Ionis Pharmaceuticals, Inc.

Panel Q & A/Discussion  All
1940

Hungry cow + Spoiled sweet clover = Sick cow

Karl Link & Mark Stahmann + Warfarin 5 mg Tablets =
1949

RAF Pilot + Plastic Shrapnel in Eye + No Effect on Eye = Harold Ridley

Intraocular Lens
1959

40 Compounds Synthesized. All Pharmacologically Inert.

Lab Clean-Up. Found one on shelf.

It Worked!

Benzodiazepines

Leo Sternbach
1982

Patient Culture + Three Day Weekend = New “Bug”

Forgot 3-Day Weekend + Made a Broth & Drank It! = Gastritis

Helicobacter pylori
Peptic Ulcer Disease

Barry Marshall
Robin Warren
Overview current issues surrounding rare and ultra-rare diseases
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