



# Applied Pharmacoeconomic and Outcomes Research Forum

February 5<sup>th</sup> 2018



UNIVERSITY *of* CALIFORNIA  
SAN DIEGO

SKAGGS SCHOOL *of* PHARMACY  
*and* PHARMACEUTICAL SCIENCES

# 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?
9. Specialty Pharmacy Distribution Channels & Processes: Challenges & Opportunities Today & Under Health Care Reform
10. Biosimilars: Challenges and Opportunities for All.
11. Patient Reported Outcomes: Useful for Whom?
12. Health Services Research: Applications & Challenges Across Healthcare Systems
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*

# Sponsors - Thanks!

## *PE Forum Sponsorship Fund & Exhibitors*



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abbvie



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

# Flashback... & Memory Test

2013

## Top 5 Sci-Fi Drugs



Dune  
The Spice  
Melange

2015

## 7 Most Famous TV Docs



2017

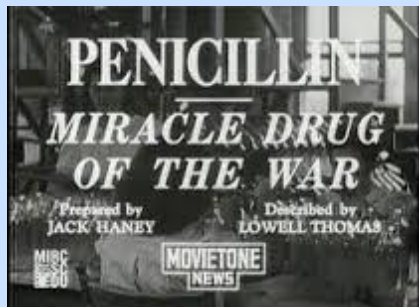
## Biosimilars of Animal Kingdom

Alpaca



2014

## 10 Most Important Drugs



2016

## Pharma Trivia



 **SANDOZ**

Cyclosporine  
discovery: travel  
policy “digging  
on location”

Llama







# The Power of Serendipity

[http://www.medscape.com/slideshow/accidental-discoveries-6008976?src=WNL\\_infoc\\_170816\\_MSCPEDIT\\_TEMP2&impID=1410982&faf=1](http://www.medscape.com/slideshow/accidental-discoveries-6008976?src=WNL_infoc_170816_MSCPEDIT_TEMP2&impID=1410982&faf=1)

#1

1881



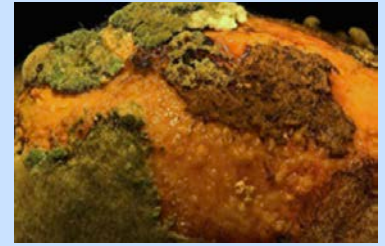
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Slice of potato left in a lab

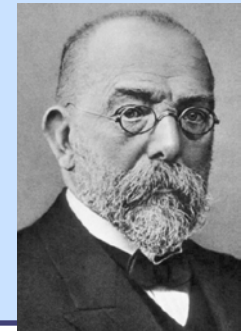
- Colored growths
- Different bacteria



Robert Koch



Petri Dish



Julius Petri

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

Selective Culturing

His colleague created

1895



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

Experimented on wife

Her hand



Wilhelm Roentgen

X-rays



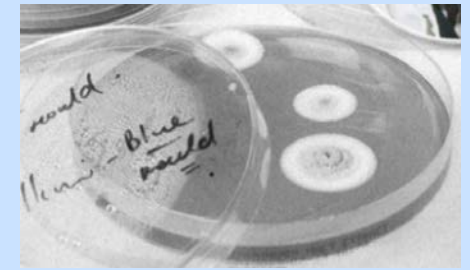
1928



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No growth in areas accidentally contaminated with ????

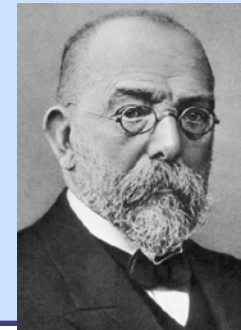
## Penicillin



Alexander Fleming

### EXTRA TRIVIA

Fleming born same year **Petri dish** invented by **Julius Petri**



Julius Petri



# The Power of Serendipity

More after the Break!





# Agenda

## **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

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Sick cow

+



Karl Link & Mark Stahmann

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1949



RAF Pilot

+



+



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No Effect on Eye



Harold Ridley

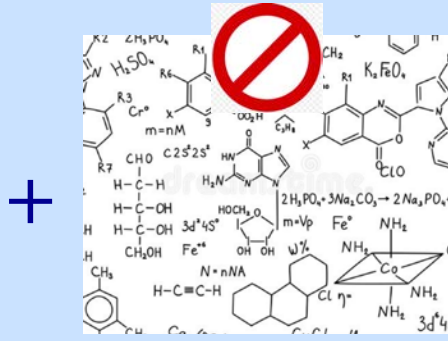
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Intraocular Lens



1959



40 Compounds Synthesized.  
All Pharmacologically Inert.

Lab Clean-Up. Found one on shelf.



Leo Sternbach



It Worked!

Benzodiazepines

1982



Patient Culture

+



Forgot 3-Day Weekend

=



New "Bug"



Made a Broth & Drank It!

+



=



Gastritis



Barry Marshall  
Robin Warren

**Helicobacter pylori**  
**Peptic Ulcer Disease**



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