

***“Balancing Access, Affordability and Innovation: Pharmaceutical Benefit Management Under Medicare Part D”***

**2006 Health Industry Forum**

**July 13, 2006**

# Demonstrating the Value of Pharmaceuticals

Tony Zook, President and CEO,  
AstraZeneca LP, U.S.

*Some seem to think that EBM is a panacea. It's not. Studies are hard to do, often very narrow, expensive, and research is a moving target. A frequent question from industry and government: do we need to change/restructure to meet the EBM challenge?*

Dan Mendelson, Avalere Health LLC

# Why is this hard?

**BusinessWeek** online

## Prescription For Upheaval

How the Medicare drug program is tightening the screws on Big Pharma

Nonetheless, it's a risk that Eli Lilly & Co. () has decided it needs to take. The company is plowing more than \$300 million into a trial designed to determine whether its new anti-clotting drug, called prasugrel, will work better than blockbuster Plavix, which generates \$4 billion in sales for Bristol-Myers Squibb Co. () If Lilly's drug doesn't win the trial, it'll likely be dead on arrival when it hits the market in 2008. "Lilly is either going to get zero or multibillions" of dollars in sales, says SG Cowen & Co. analyst Stephen M. Scala.



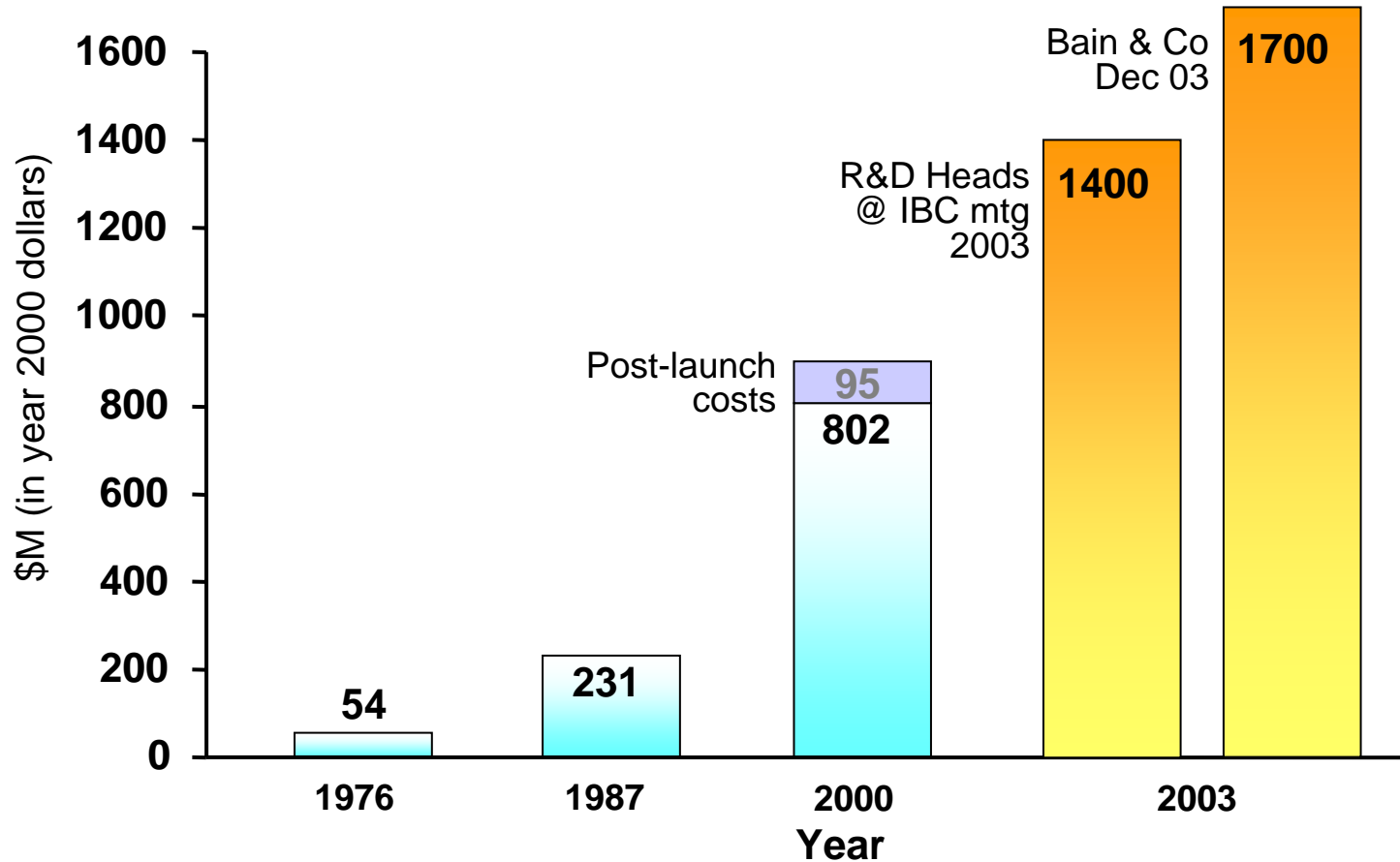
**THE WALL STREET JOURNAL**  
ONLINE

## Pfizer to Finance \$100 Million Safety Study of Celebrex

Cleveland Clinic announced that Pfizer was funding a massive, 20,000-patient study to finally answer questions about the possible cardiovascular risk posed by the blockbuster drug. The study, being run independently by the clinic, will compare three drugs: Celebrex (the Cox-2 drug from Pfizer), ibuprofen (sold under the brand name Motrin), and naproxen, sold under brand names such as Alleve. It will cost more than \$100 million.

# Unsustainable Economics ...

Average R&D costs per NCE drug launched



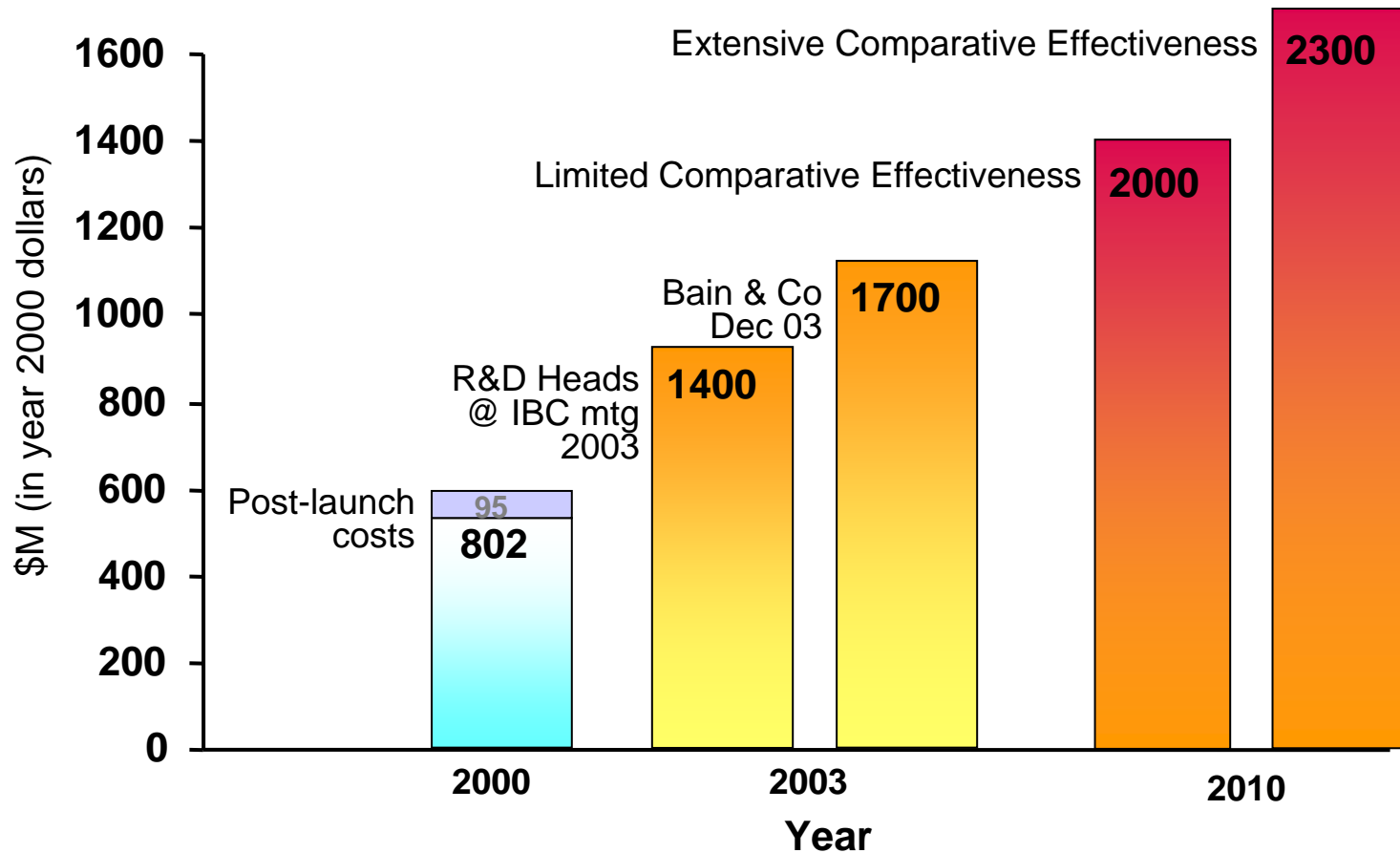
Historical data from DiMasi, Tufts Center for Drug Development

# ...made worse

<b>Trial Type</b>	<b>Cost</b>	<b># Patients</b>
Comparative Efficacy	\$300M	13,000
Comparative Risk	\$100M	20,000
Epidemiology	\$20M	507

# ...through additive costs of comparative outcomes research

Average R&D costs per NCE drug launched



Historical data from DiMasi, Tufts Center for Drug Development

# Why is this hard?

- Larger patient populations significantly increase resource need including logistical and manufacturing issues
- The sterile environment of the traditional clinical trial
  - Needed to reduce bias and other confounding elements

Moreover...

**The traditional clinical trial  
doesn't reflect actual  
clinical use.**



# A New World is Emerging



## From:

- Serendipitous drug discovery
- Cumbersome, time consuming, expensive drug development
- Access/reimbursement considerations an afterthought
- Market driven by marketing and sales throw-weight
- Product differentiation defined by label and delivered through marketing
- Value of innovation assumed
- Healthcare sectors act independently or in conflict



## To:

- Discovery driven by molecular understanding of disease
- Drug development never stops – seamless transition from research to market
- Access/reimbursement an integral part of drug development
- Market driven by evidence
- Product differentiation defined through real-world comparative effectiveness
- Value of Innovation demonstrated
- Healthcare sectors act in concert



# But at what cost?

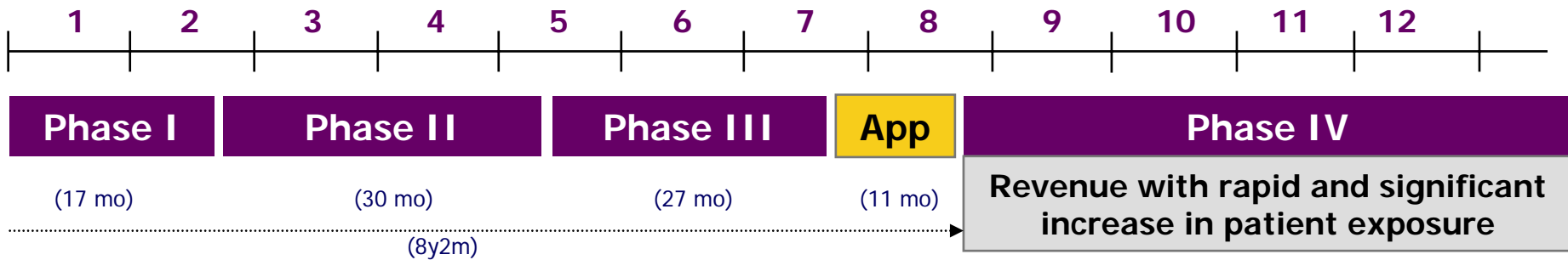
- The cost of generating effectiveness information is high,
- But, the cost of not getting a favorable formulary position and reimbursement can be even higher



- Bottom-line:
  - The current model creates an untenable situation for the industry to support effectiveness research
  - The need for effectiveness research constitutes a significant business opportunity to rethink the process by which drugs are approved and reimbursed
    - Are there alternative standards of evidence which are sufficient to support these decisions?
    - Can we trade the generation of effectiveness data for earlier conditional approvals?

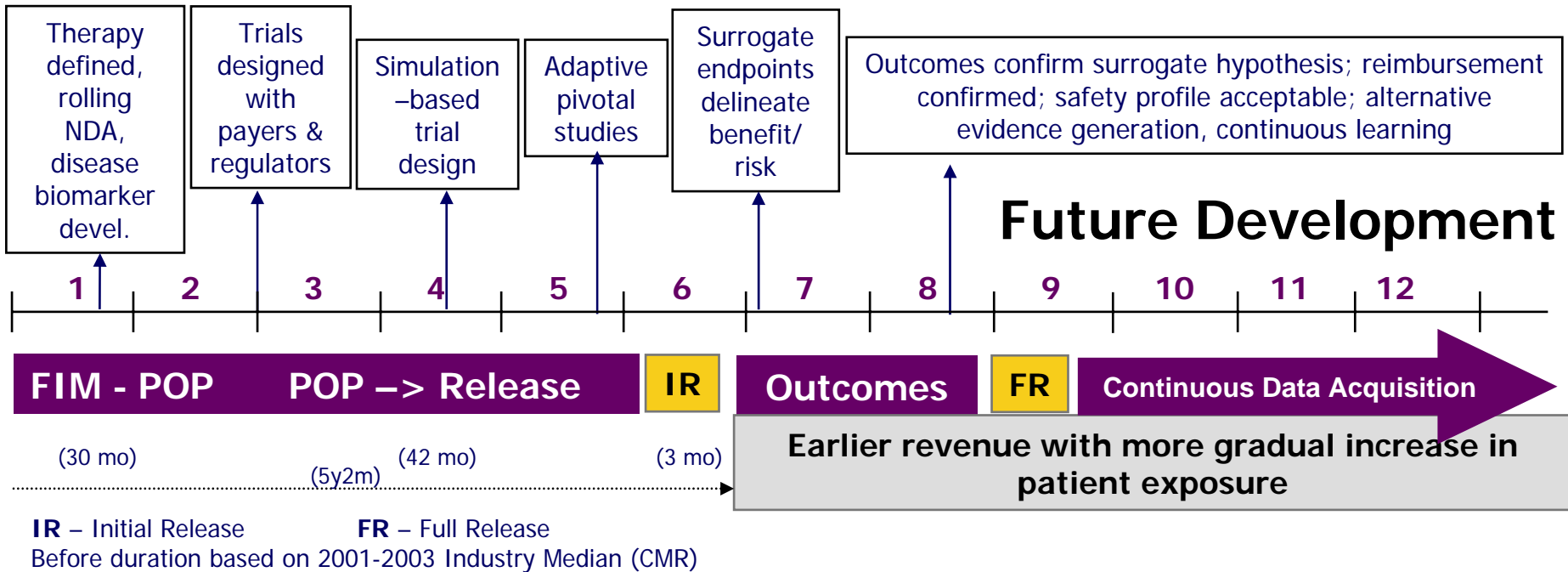
# Evidence-based Drug Development?

## Traditional Development



Duration based on 2001-2003 Industry Median (CMR)

# Evidence-based Drug Development?



# What about the value of innovation?



## Company Puts a Big Price Tag On Drug to Treat Blood Disease

Dec. 29, 2005 -- Celgene says it is pricing Revlimid at between \$4,500 and \$4,700 a month -- which adds up to between \$54,000 and \$56,400 annually. The drug was just approved in the US for some patients with myelodysplastic syndrome. Celgene CEO John Jackson defended the price in an interview on CNBC, saying patients with this disorder currently require frequent blood transfusions.



## Cancer's Cost Crisis

...The question is simple: **How many \$20,000 cancer drugs can society really afford to stack on top of one another?** "Absent a thoughtful national discussion, the answer is none," says Michael A. Friedman, chief executive of City of Hope, a cancer center in Los Angeles. **"We will quickly run out of resources, leading to de facto rationing." The rising costs, he says, are "utterly insupportable."** Whatever solutions emerge, it's likely that drug costs will have to come down and that some patients will be denied medicine because their chances are too slim. Says Saltz: "We're going to have to stop taking the stance that every patient has to get every last shot at survival. **The days of developing drugs and not worrying what they cost are over.**"



## The Most Expensive Drugs—Rx For An Industry, As Biotech Drug Prices Surge US Is Looking For A Solution - Proposals Include New Role For Generics; Let Uncle Sam Bargain...

Spending on specialty pharmaceuticals -- biotechnology drugs and other expensive medicines prescribed by medical specialists -- is growing twice as fast as traditional prescription drugs, according to Medco Health Solutions Inc., one of the largest pharmacy benefits managers. Insurers project it will grow by between 20% and 50% annually. It reached \$42 billion in 2004 and will rise to almost \$69 billion next year, when spending on specialty pharmaceuticals is projected to make up 25% of the nation's pharmacy bill, according to Health Strategies Group, a New Jersey consulting firm.

... Dec 28, 2005

# The Paradox of Innovation

- As newer agents are released, they improve the quality and quantity of life, but at significant additive costs
- The more new therapeutics move cancer and other “specialty” diseases from being acute to chronic conditions, the more attention will be paid to managing the costs of therapy for such conditions (“cancer as the new diabetes”)

The notion that innovation is valuable is no longer a given, but must be demonstrated in the clinic, with the patient, for the payer.

# Haven't we seen this before?

- 15 years ago the pharmaceutical industry invested substantially in Health Economics expecting a push for “evidence” that never materialized, or worse yet, didn’t get used.



# Haven't we seen this before? ...Not like this

- Healthcare expense growth is now pervasive
  - Affecting all stakeholders; patients, employers, private payers, states, and the federal government.
- The quality of care being delivered today is increasingly recognized as suboptimal
  - recommended care not implemented effectively and misaligned incentives not serving the patient.

The current situation is not sustainable, and no single part of healthcare, any single industry, any one company or agency can do it alone.

# Creating The Brave New World of Future Partners



Pharma and Biotech



Payers



Patients



Diagnostic/Device



Providers



Technology Providers



U.S. Food and Drug Administration



Government Agencies



# Asking the Right Questions...

- ...when assessing the comparative effectiveness of various therapeutic options.
  - All of the evidence needed to fully assess the options is rarely available to decision makers.
  - The appropriate method must match a clear policy goal
    - Comparative randomized trials frequently fail to find differences when there indeed are some.
    - Decision modeling approaches frequently identify differences where there are none.



# Matching Policy Goals and Certainty of Evidence



- Serious Life-threatening Health Condition
  - Greater willingness to accept less than perfect information as decisions must be made by doctors and patients
- Asymptomatic Population at Risk for Developing an Illness
  - Greater desire for certainty before widespread use



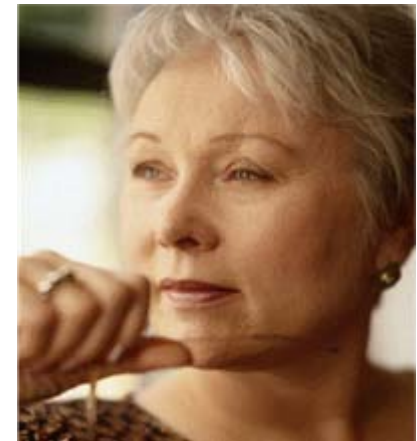
# One Size Doesn't Fit All

- Evidence by its nature is population based,
- But each individual patient responds differently to a given therapy

- We need to work to identify the needs of patient subpopulations and individuals and tailor the delivery of care appropriately.
  - Recognize clinical differences in response to therapy
  - Eliminate financial incentives that are counter-productive
  - Ensure appeals processes are efficient

# A Patient's Future?

- Patients and providers receive updates on changing treatment recommendations and how they could affect a patient's health based on the patient's health profile
- Evidence-based treatment guidance, and exceptions to guidance, are revisited quarterly based on population data feeds regarding sub-segments of the patient population
- Messaging and decision support regarding the unique requirements for a patient's care are consistent across care settings
- Providers have data access and tools to understand appropriate care for Mary and readily navigate payment administration even when treatment's are unique
- Coordinated performance metrics are established for all participants in the care continuum



# So what is NPC Doing?

- NPC supports the development of quality standards and performance metrics by groups including NCQA and JCAHO
- We are working with AHRQ to resolve methodology issues for evidence-based studies.
- We are conducting analyses of drug benefit design and management tools like formularies, prior auth, step therapy and the like. These techniques are here to stay and they can improve value - but analysis may reveal where refinements are necessary to meet the needs of individuals and subpopulations
- Navigating the health plans' rules present challenges for physicians, pharmacists and most of all for patients. NPC has for many years been an effective voice to assure that administrative procedures are fair, transparent and sound.
- Through its consumer education campaign, NPC and its partners give consumers the knowledge and practical tools to choose their drug plan wisely and use their drug benefits successfully