

# Economics of Rare Disease Drug Development: Payers' Perspective

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# Making FDA Harder to Caricature

By [Michael McCaughan](#) / [Email the Author](#)

Depending on who is drawing the portrait, FDA managers are often portrayed as **classic bureaucrats who can be counted on to ask for irrelevant information** or otherwise **find any excuse to obstruct** an innovator and delay access to the next big breakthrough.

Or as **bullies** running roughshod over brave individuals raising critical public health and safety concerns, **driven by a reckless zeal to approve new drugs.**

The fact that the **same agency** is characterized in **completely opposite ways**, alas, does not seem to stop the public from being all too ready to believe whichever caricature is trotted out in the setting of a political proceeding or moment of media sensation.

Similarly,  
the **Payer Industry** can be characterized as  
colluding in **massive inflated costs**, or,  
in **strangling payment** for medical treatment.



Healthcare Spending per capita vs.  
Average Life Expectancy Among OECD Countries



# Reed Tuckson MD, April 17, 2013



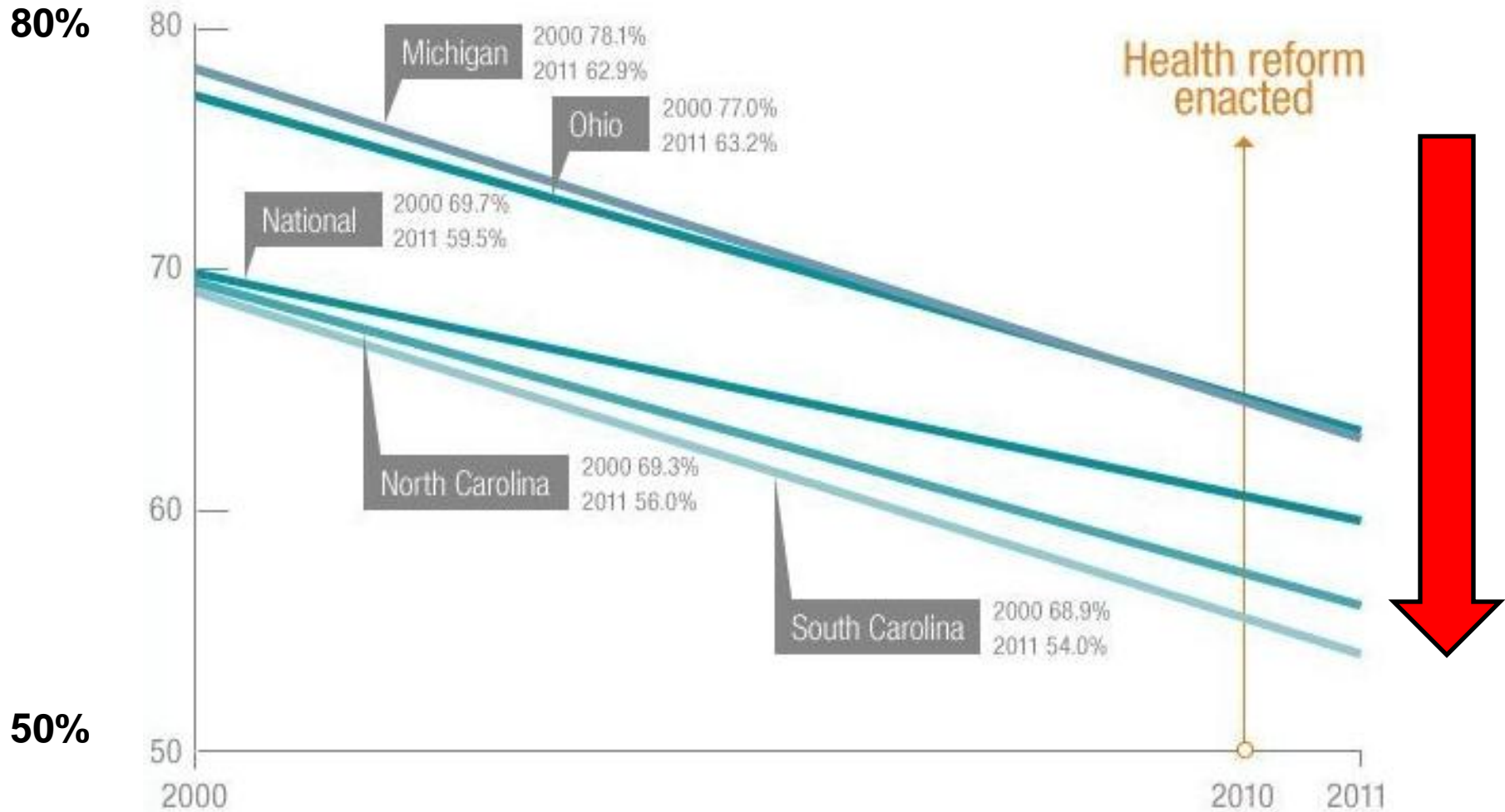
EVP, United Healthcare  
(Until Spring, 2013)

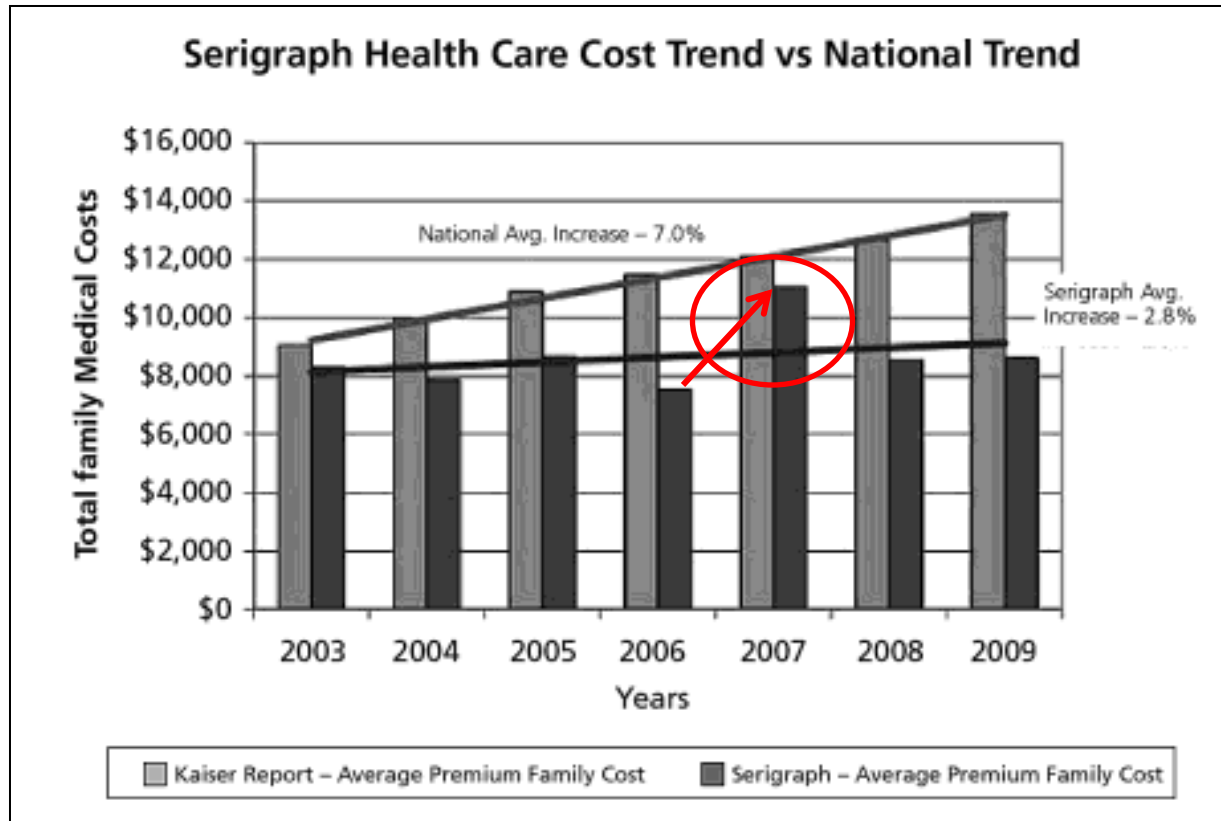
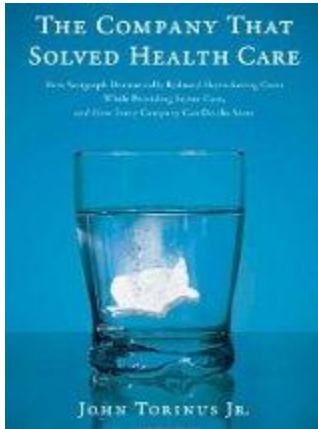
## EVIDENCE, COVERAGE, AND INCENTIVES

A PMC/BIO Solutions Summit

- Keynote speech
- (Not 5 min on 2<sup>nd</sup> day)
- Cost escalation has reached a ceiling.
- It is like sequestration – there is not one more dollar.
- Every state is in a budget crisis that will only get worse.
- Employers are at the end of their rope. Whatever you think the cost problem is, it is worse than you think.

# Employer Offered Insurance





- 1400 employees
- Self insured
- Generally very tight cost control
- **2007: 4 bad events (2 car accidents, 2 cancer)**



## Cancer Physicians Attack High Drug Costs



Eric Gaillard/Reuters

Chemotherapy drugs being prepared at a cancer treatment center in Nice, France.

By ANDREW POLLACK

Published: April 25, 2013 |  114 Comments



Getty Images

Pharmaceuticals

## Orphan Drugs Could Lose Their Government Subsidies

By Simeon Bennett

April 11, 2013

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### Cost Per Year for U.S. Patients With Rare Maladies



As more medicines win approval to treat diseases that affect no more than 5 in 10,000 people, austerity-conscious governments in Europe are applying the same pressure to so-called orphan drugs that they do to widely prescribed medicines for heart disease and diabetes. Photographer: Antoine Antonio/Bloomberg

Bloomberg News

## Orphan Drug Prices Under Siege in Austerity-Minded Europe

By Simeon Bennett

April 07, 2013

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## ORPHAN DRUG PRICING AND PAYER MANAGEMENT IN THE UNITED STATES: ARE WE APPROACHING THE TIPPING POINT?

Rebecca Hyde; Diana Dobrovolny

Patient access to orphan drugs is rarely denied. The diseases that orphan drugs treat are rare and usually have no treatment alternatives. However, orphan drugs are not immune to management tactics and benefit design trends that payers apply to other expensive biologics, injectables, or specialty drugs.

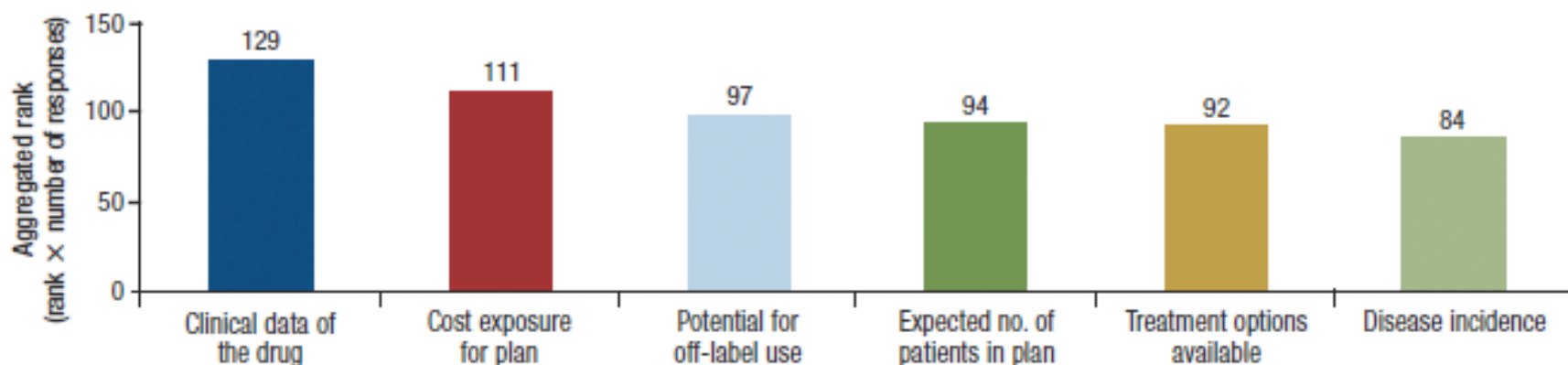
- Scrutiny of orphan drug utilization up to and exceeding the \$50,000 per-patient per-year threshold
- Increased focus on appropriate use of orphan drugs, often restricting use to approved indications
- Rising burden on patients through cost-sharing (ie, coinsurance, higher copayments), as well as existing annual or lifetime maximum payments.

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**Figure 2** Factors Driving Benefit Design or Restrictions for Orphan Agents, in Order of Importance

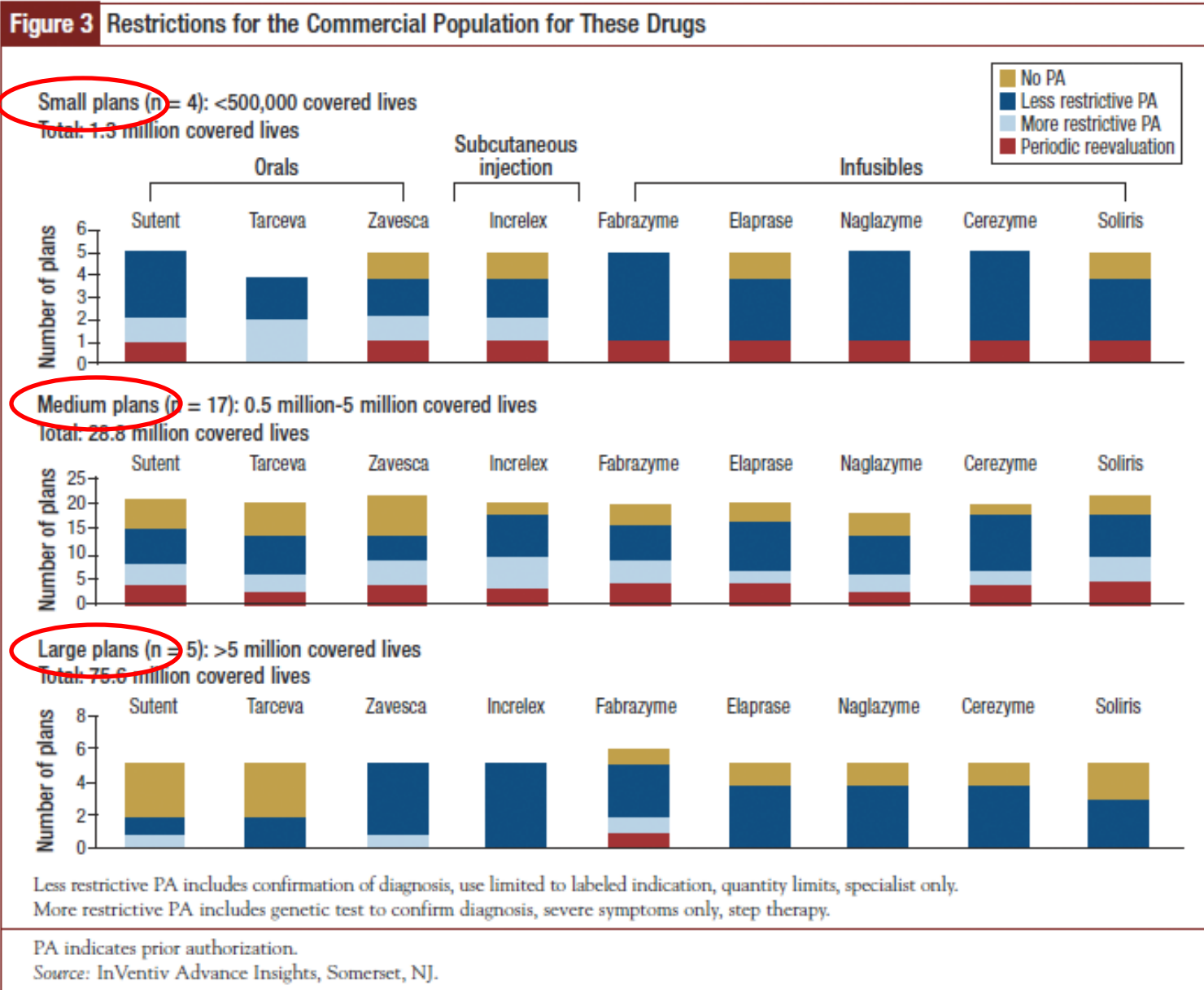


N = 26 health plans.

Source: InVentiv Advance Insights, Somerset, NJ.

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# EVIDENCE MATTERS



## Is There an Easier Path to Acceptance for Orphan Drugs?

*Ruth Brown, MS, MHSA, Senior Research Scientist*

- Identifying the target population
- Conducting a landscape overview of the current market
- Determining the standard of care and comparators
- Identifying competitor therapies, their value messages, technology reviews, and market access
- Determining the burden of illness
- Identifying the key stakeholders
- Assessing the evidence needs
- Understanding the reimbursement requirements and trends
- Identifying the value messages and connecting with supporting evidence
- Recommending studies to fill data gaps and support the value message
- Communicating and disseminating evidence and value

**Only the most innovative therapies are able to command attractive prices, and these are likely to face rigorous price negotiations, restrictions on use, or patient access schemes.**