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## ORPHAN DRUGS' MARKET ACCESS IN THE US

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- FDA orphan disease designation:
  - A drug to treat a rare disease/condition affecting < 200,000 people in the US
  - A vaccine, a preventive drug, or a diagnostic drug to be administered to < 200,000 persons per year
- The US Orphan Drug Act provides incentives to manufacturers of orphan drugs
- 6,000 orphan conditions affecting > 25 million Americans total
  - Orphan diseases in this analysis affect 2-50 patients per 100,000
- Sales of orphan drugs are expected to reach \$80B worldwide by 2011
- Six orphan drugs launched in the past three years cost >\$100,000 per year
  - 3/10 drugs in this analysis cost >\$100,000/year

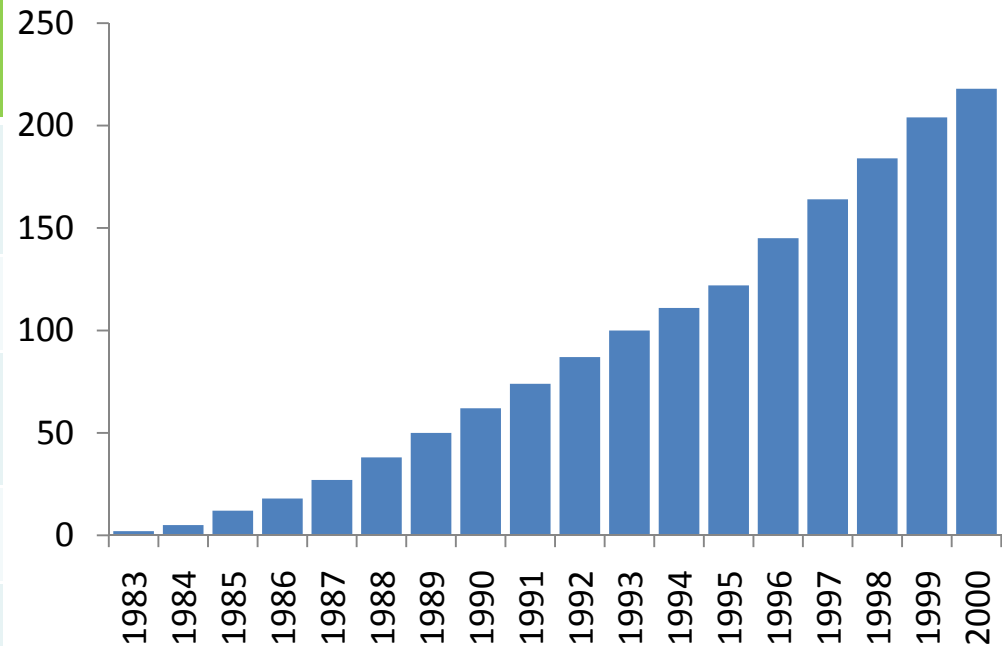
# Orphan Drug Market Growth

*The passage of the Orphan Drug Act in 1983 has unquestionably stimulated the development of orphan drugs and it is likely that the development of these expensive drugs will increase substantially in the near future*

## Select Orphan Drug Prices\*, 2004

Orphan Drug	Company	Disease	Average Price per Patient
Rituxan	Genentech, Biogen Idec	Non-Hodgkin's lymphoma	\$12,500 per year
Cerezyme	Genzyme	Gaucher 's disease	\$200,000 per year
Gleevec	Novartis	Chronic myeloid leukemia	\$37,000 per year
Avonex	Biogen Idec	Multiple sclerosis	\$14,000 per year
Velcade	Millennium	Multiple myeloma	\$22,000 for 4 months

## Total Orphan FDA Approvals, 1983-2000



\*ASP reported to CMS

Source: The Orphan Drug Act: Implementation and impact. HHS Office of Inspector General. [www.oig.hhs.gov/oei/reports/oei-09-00-00380.pdf](http://www.oig.hhs.gov/oei/reports/oei-09-00-00380.pdf) ; Anand. How Drugs for Rare Diseases Became Lifeline for Companies . The Wall Street Journal. [http://online.wsj.com/public/article/SB113202332063297223-ztfi1Ms5q2Cw\\_YKhwxKTHN3Tk9g\\_20051128.html?mod=blogs](http://online.wsj.com/public/article/SB113202332063297223-ztfi1Ms5q2Cw_YKhwxKTHN3Tk9g_20051128.html?mod=blogs)



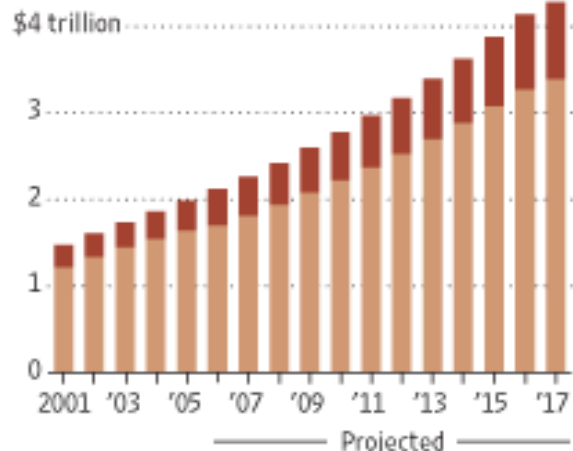
# US Healthcare Costs Driving Change

*As healthcare expenditures increase and the government looks for ways to decrease the bill, the burden of drug costs is being increasingly pushed to patients*

## Footing the Bill

The cost of insuring aging baby boomers will help push up national health expenditures in the next decade

Total health-care spending [ ■ - Medicare]



Source: Centers for Medicare & Medicaid Services

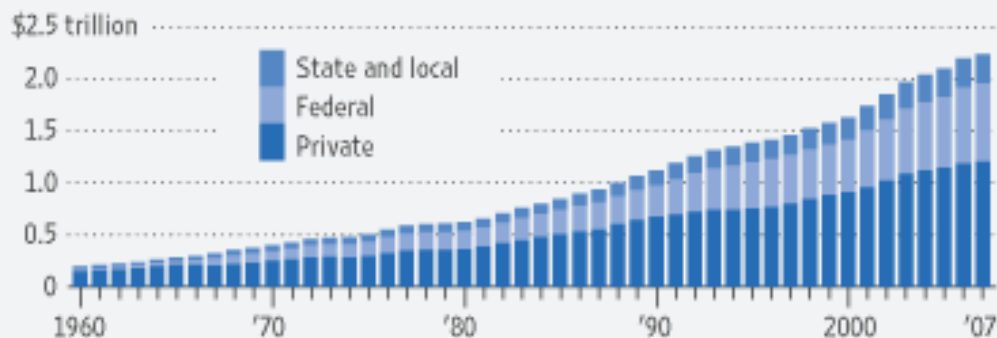
## Managing Care

President Obama is emphasizing cost control in his health-care proposal

What people view as the most important health-care issue



## Inflation-adjusted U.S. health expenditures\*



\*Based on annual change in monthly average of fourth-quarter consumer-price index

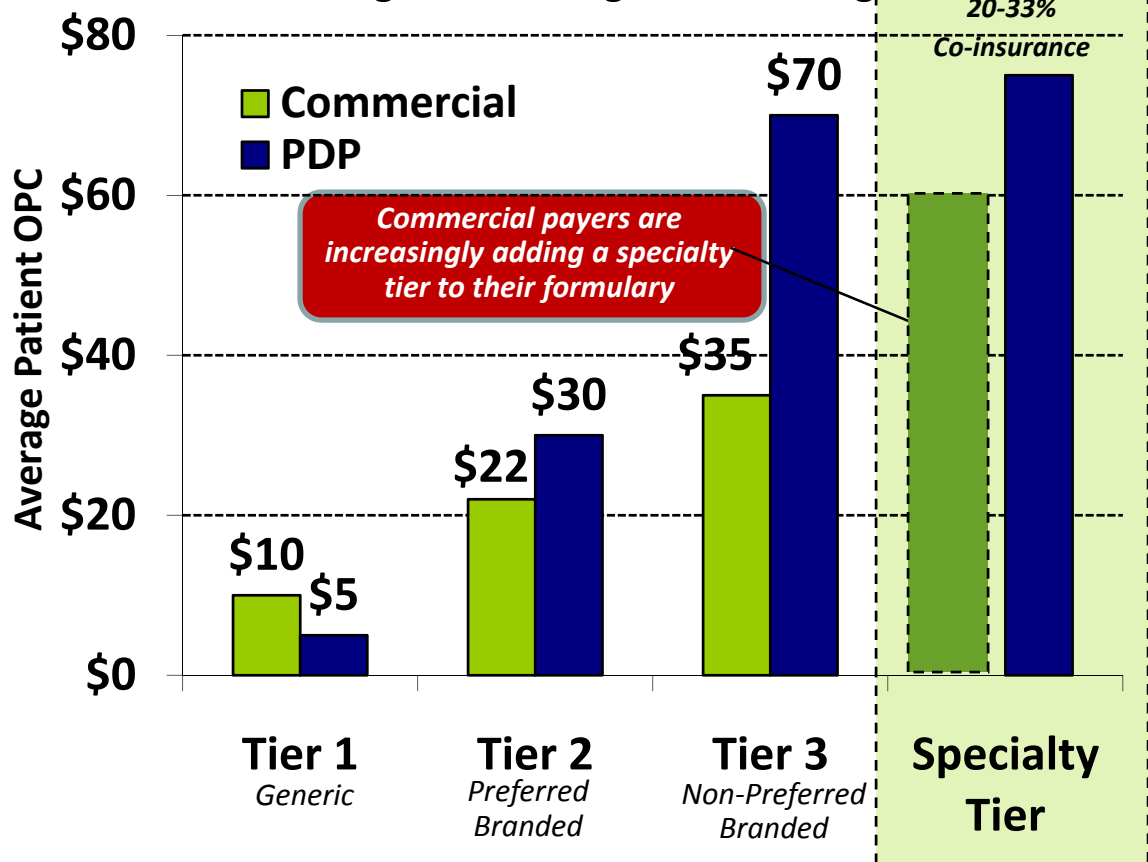
Sources: WSJ/NBC News telephone poll of 504 adults conducted Feb. 26-March 1; margin of error: +/-4.4 percentage points (issues); Centers for Medicare & Medicaid Services, Office of the Actuary (spending)

# Managed Markets Specialty Tiers

*Increasing use of a specialty tier for Medicare Part D formularies is a key issue for high-priced therapies*

*In 2008, 87% of National PDPs had a specialty tier*

Typical Commercial and Medicare Formulary Design and Average Cost Sharing



- ▶ In 2008, Medicare guidelines indicate that a drug may be placed in a specialty tier if costs exceed \$600 per month (up from \$500 per month in 2007)
- ▶ Co-pays and co-insurance limited by \$5,726 maximum after which the patient will pay the greater of 5% or \$2.25 for generic and \$5.60 for branded
- ▶ Catastrophic drug costs are covered by the patient (5%), payer (15%) and Medicare re-insurance (80%)

# Research approach



Are orphan drugs subject to barriers that impede patient access?

Are there correlations between a disease's prevalence, the drug's cost and the amount patients pay out of pocket?



Select drugs  
and plans to  
analyze

- 10 high-selling drugs and 10 large nationwide plans were analyzed
- Three utilization reviews (UR) were analyzed—step therapy, prior authorization, quantity limits



Analyze  
market  
access  
metrics

- UR implemented for each drug under each plan were determined by Medicare's Formulary Finder ([medicare.gov](http://medicare.gov))
- Average out of pocket costs (OPC) were calculated by averaging each drug's initial, gap and catastrophic level coverage under each plan ([medicare.gov](http://medicare.gov))
- Monthly retail price calculated for most commonly utilized dosages ([RxList.com](http://RxList.com)) using RedBook, 2009
- Orphan disease prevalence data from Orphanet (EU)

# Drugs and plans analyzed



## Select drugs

- Alglucerase: Gaucher's disease
- Amiodarone\*: ventricular tachycardia
- Bexarotene: cutaneous lymphomas
- Cinacalcet: hyperparathyroidism
- Imatinib: chronic myelogenous leukemia
- Imiglucerase: Gaucher's disease
- Miglustat: Gaucher's disease
- Modafinil: narcolepsy
- Sildenafil: pulmonary arterial hypertension
- Sotalol\*: ventricular tachycardia

*\*available as generic*



## Identify large nationwide plans

- Aetna Medicare
- Cigna Medicare
- Coventry AdvantraRx
- CVS Caremark SilverScript
- First Health Part D
- HealthNet Medicare
- Humana PDP
- Medco Medicare
- Sterling Medicare Rx
- UnitedHealthcare

# Utilization restrictions on orphan drugs



## Step Therapy (ST)

- ▶ Ensures appropriate utilization and controls costs
- ▶ Most limiting UR as it affects treatment algorithm

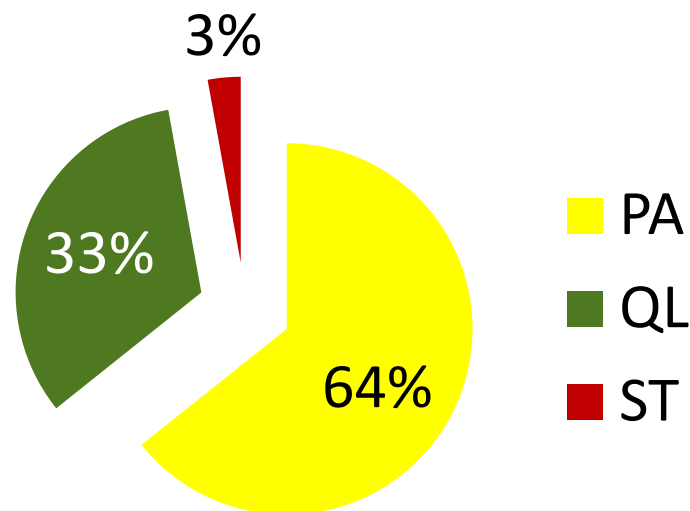
## Prior Auth. (PA)

- ▶ Prevents off-label use
- ▶ Can be costly as it requires a physician consultation

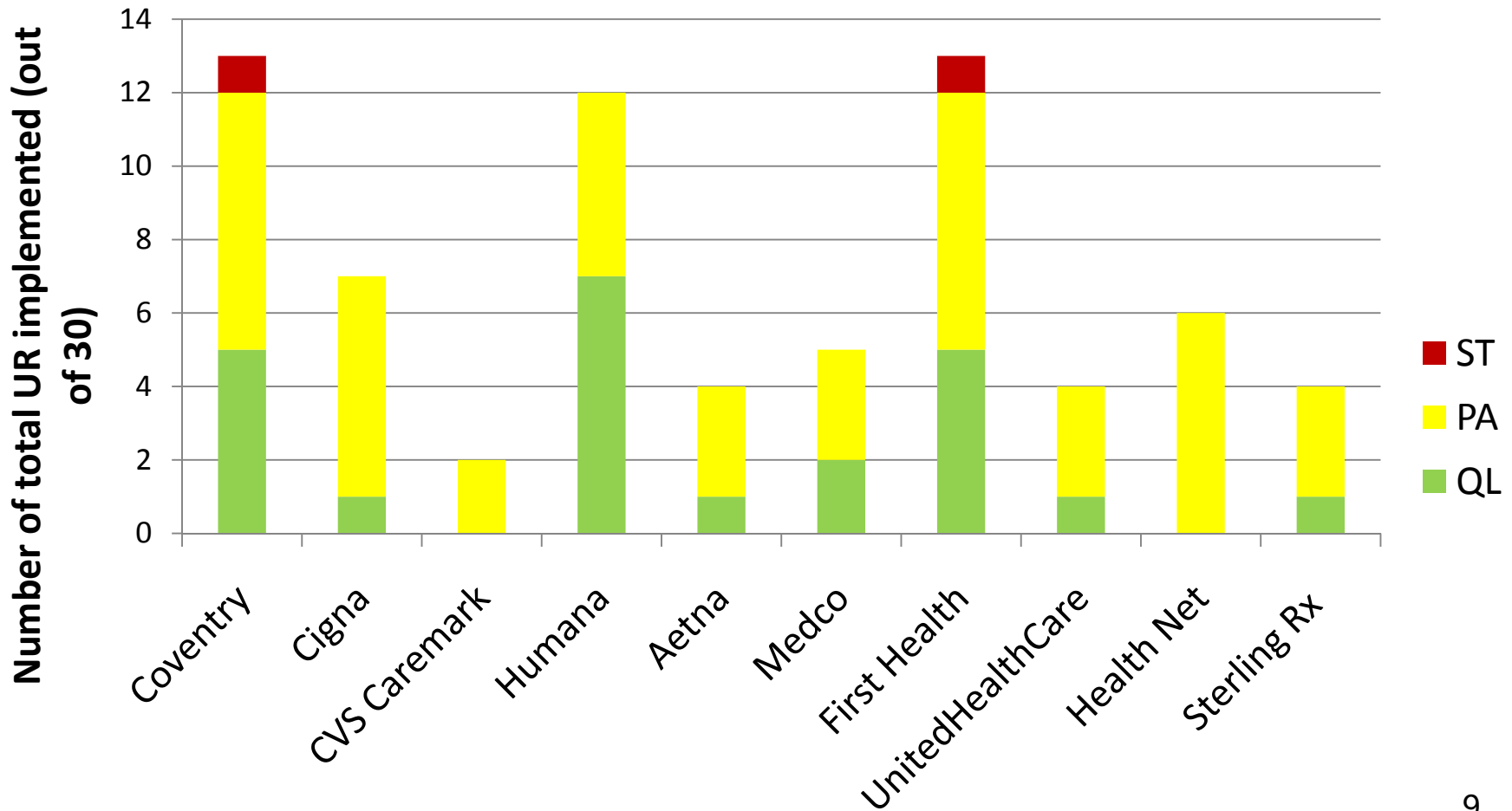
## Quantity Limits (QL)

- ▶ Ensures appropriate dosing and prevents abuse
- ▶ QL may be considered the least limiting tool

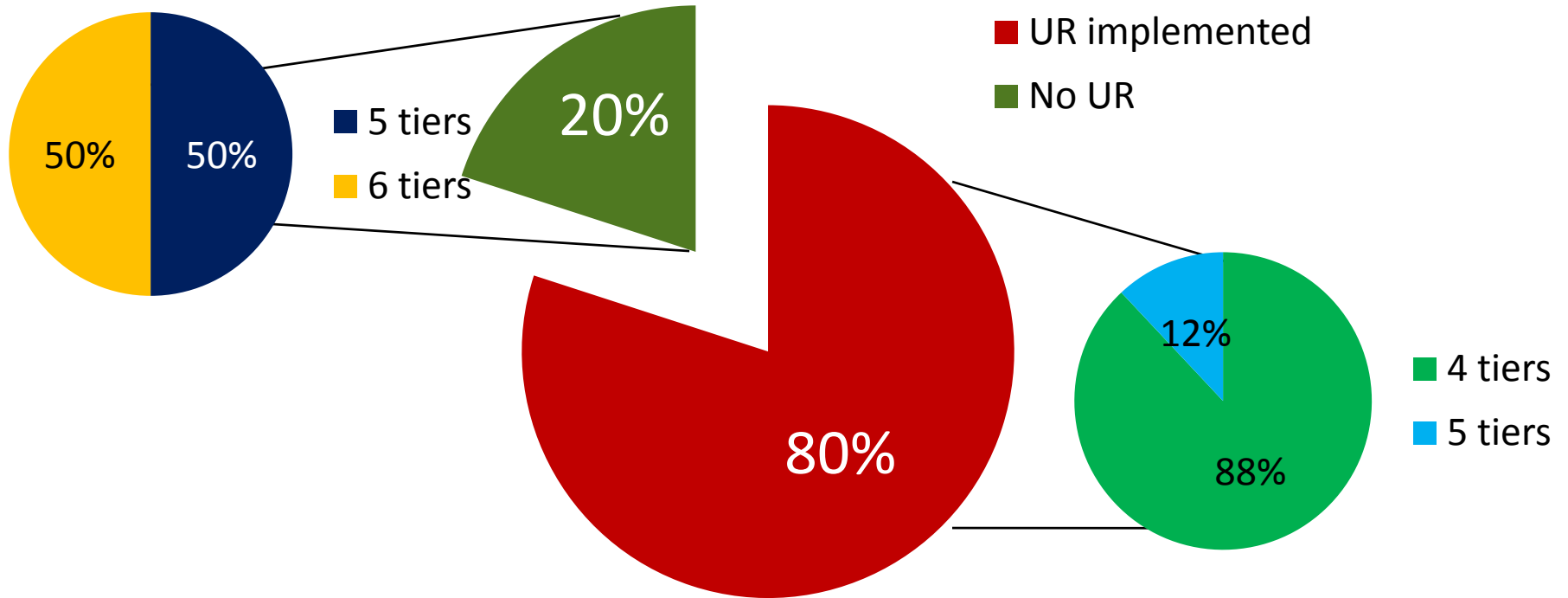
## Utilization Restrictions Implemented



# Utilization controls by individual Medicare plans



# Plans' formulary structures



# The effect of drug price on utilization restrictions

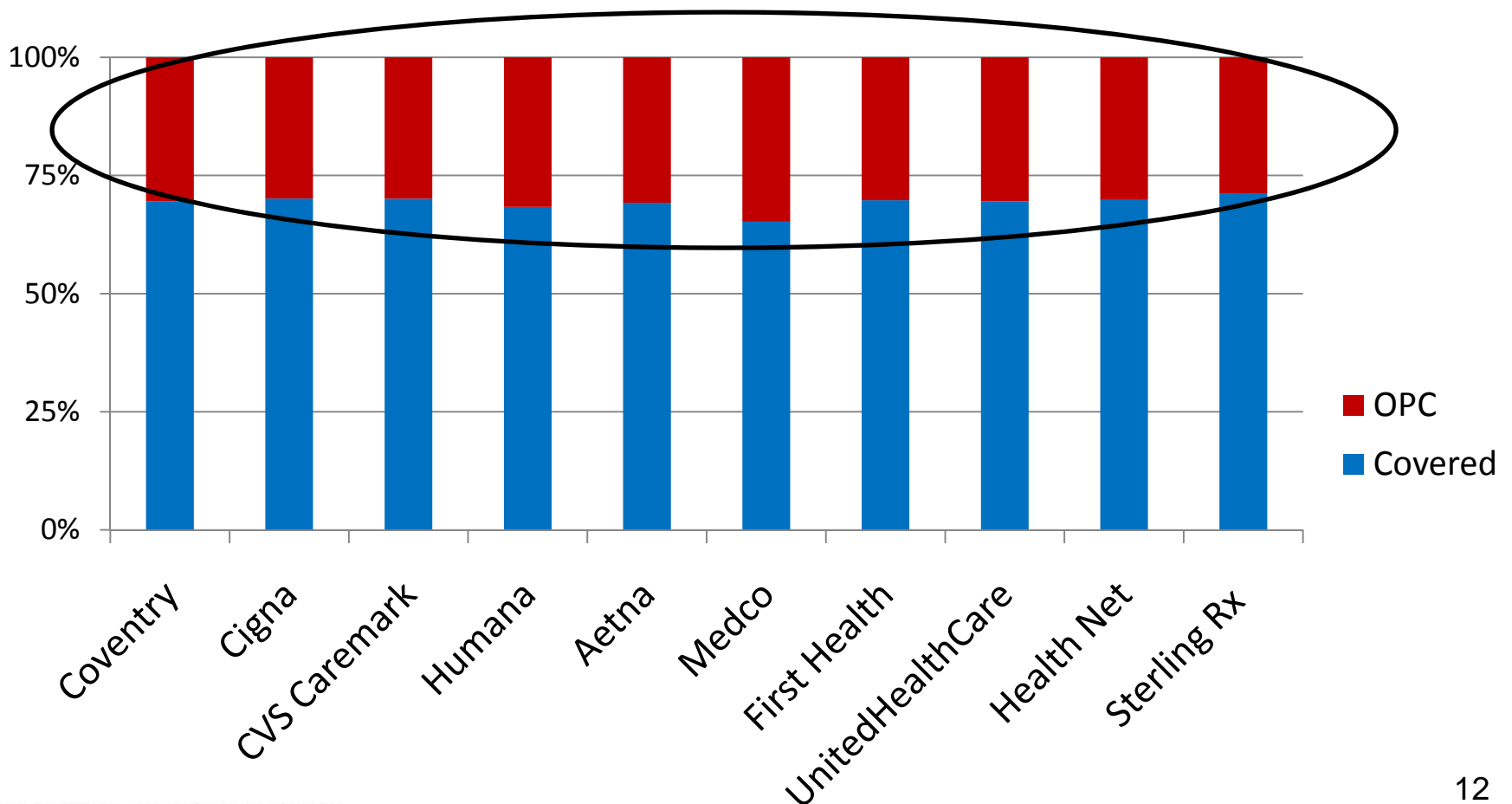
Drug	Monthly Retail Price	Number of Plans implementing UR		
		ST	PA	QL
amiodarone	\$269.50	0	0	0
sotalol	\$284.73	0	0	0
cinacalcet	\$392.40	2	2	3
modafinil	\$503.97	0	10	7
sildenafil	\$1,350.68	0	9	5
imatinib	\$3,934.34	0	7	4
bexarotene	\$5,568.48	0	5	0
miglustat	\$11,346.00	0	5	4
alglucerase	\$45,676.80	0	5	1
imiglucerase	\$45,676.80	0	7	1

Least restrictive

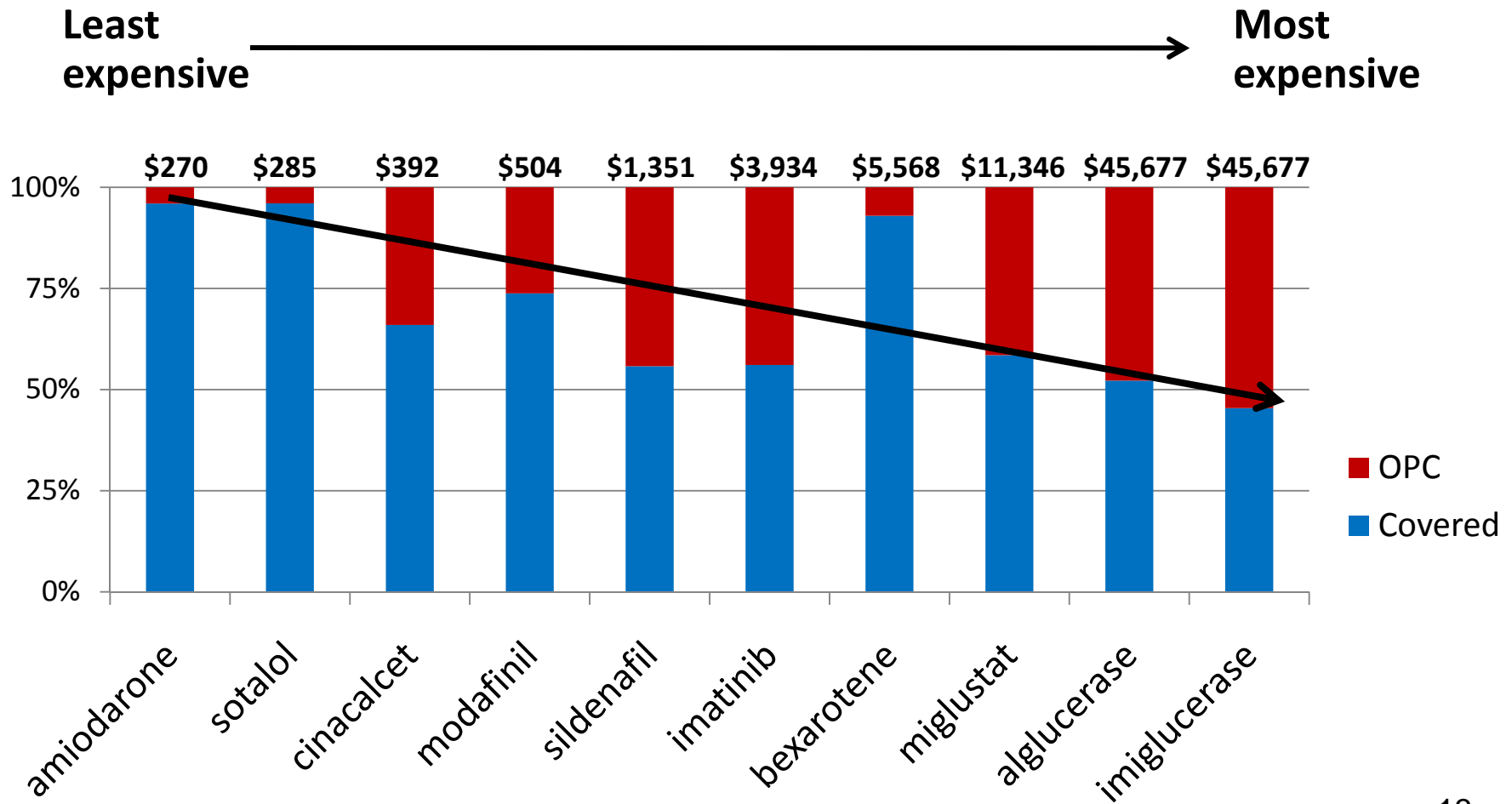


Most restrictive

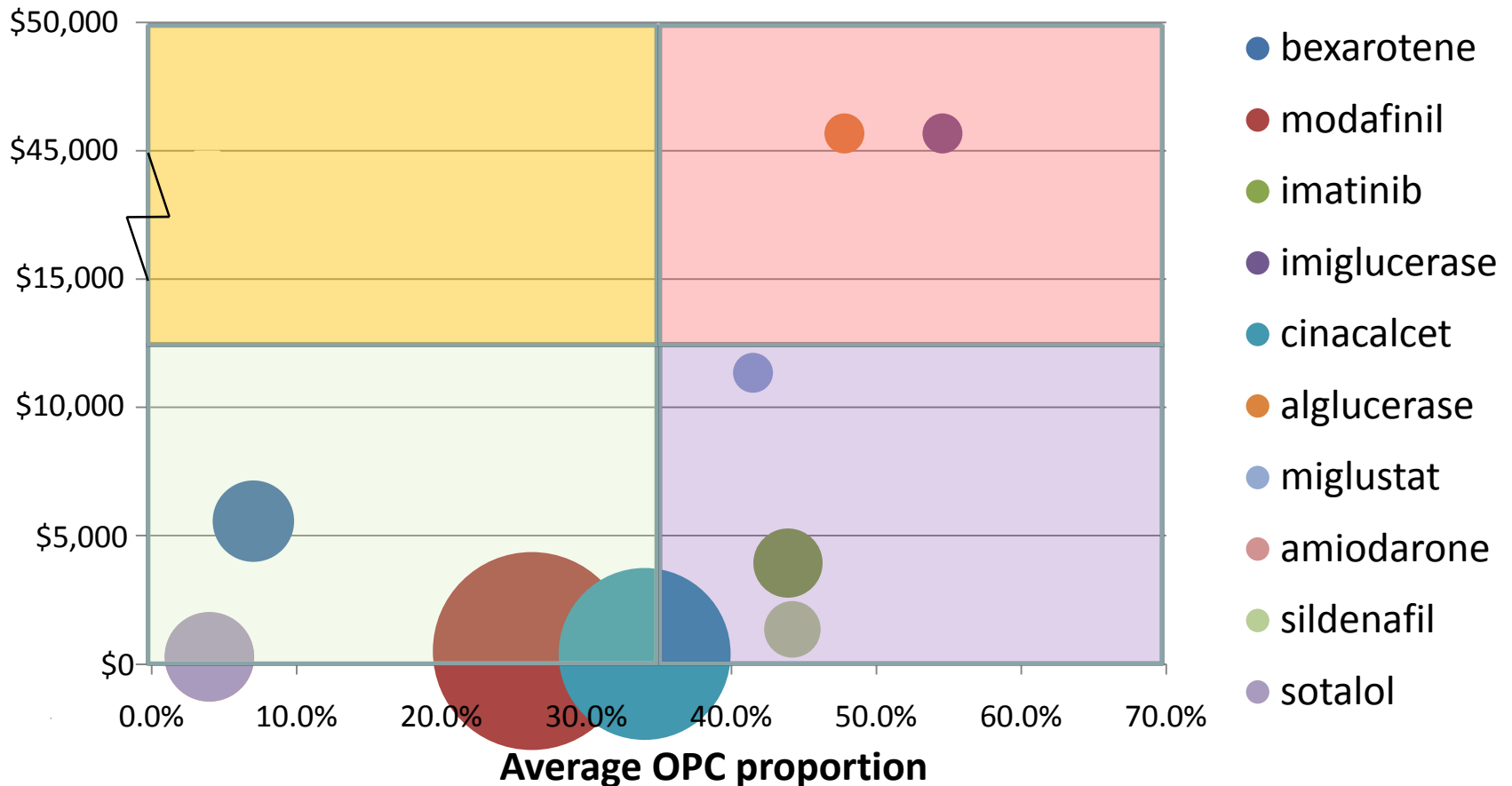
# Cost burdens among Medicare prescription drug plans



# Cost burdens for individual orphan drugs



# Are OPC greater for the most expensive drugs?



**NOTE: Bubble size = drug indication's prevalence per 100k**

# Conclusions

- With some exceptions, drug OPC are proportional to the total cost of the drug
- Utilization restrictions are more frequent and more limiting for more expensive drugs
  - EXCEPTIONS: potential for abuse, potential for off-label use
- With patients paying approximately 50% of the cost for the most expensive orphan drugs and pervasive utilization restrictions, there are still significant barriers to access
- Drugs treating the rarest disorders garner highest prices, which precipitate the highest OPC burden

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