

**MIME**

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# **New Complexities in Pricing Orphan and Ultra-Orphan Drugs**

September 28, 2010

# Agenda

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- ▶ Orphan drugs and their prices are less rare
- ▶ Changes in ultra-orphan markets
  - ▶ Increased competition
  - ▶ Big pharma enters
- ▶ When can lower prices increase volume?
- ▶ The myth: Downward price migration
- ▶ Where are your patients?

Why are we here? (....at this conference at least)

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Orphan Drug Legislation:

“Patients with rare diseases have the same right to effective therapies as those with more common diseases.”

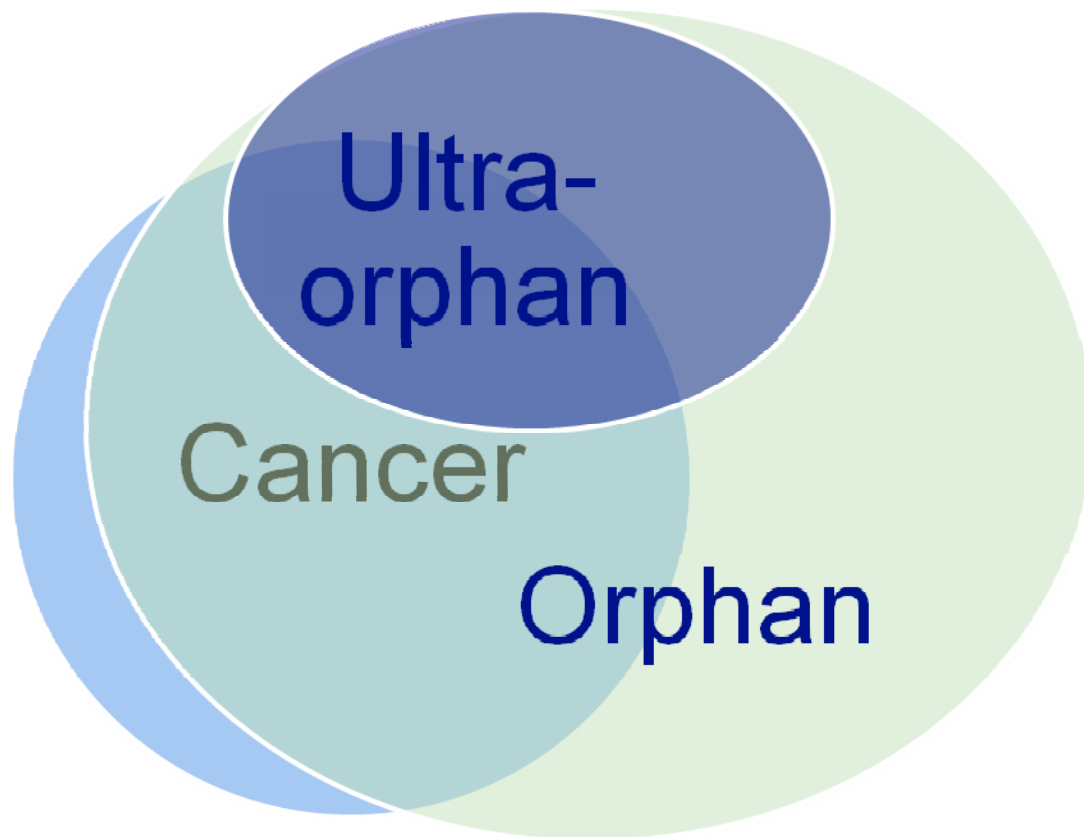
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The core of Orphan Drug Regulation consists of **non-economic societal values** representing the desire for provide equitable access to therapies independent of the rarity of the disease

**Key Takeaway: Orphan drug coverage is dictated by societal policy, not health economics**

# MME's View of the "Ultra-orphan World"

Orphan status is now common due to bio/pharma's research focus in oncology, but ultra-orphan remains "different" in payers' minds



- ▶ Payers "feel" a difference between orphans and ultra orphans
- ▶ Only the four "big" oncology indications (lung, female breast, prostate, and colorectal) are not orphan

# Orphan vs. Ultra-Orphan Drug (“UOD”) Definitions

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## OFFICIAL

Orphan drugs developed for rare diseases that affect a small proportion of the population (as proved to the FDA by developers)

<200,000 individuals in the USA and  
<1 person per 2,000 in Europe

## UNOFFICIAL

Ultra-orphan drugs treat < 20K patients world wide

Recognized by NICE for evaluation purposes as a prevalence of < 1:50,000 (or <1,000 in the UK)

► Ever wonder where the “6,000” number originated?

# Welcome To Ultra-Orphans...It Is Different

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- ▶ Please leave a lot, not all, of your bio/pharma thinking at the door
- ▶ Welcome to the community, you are no longer in a therapeutic market
- ▶ In this community...
  - Patients often know more about the KOLs than the manufacturers do
  - Patients have been waiting a long time for a therapy and the health care system has essentially abused them
- ▶ Can manufacturers really relate? Likely not, so they better truly listen to the patients
- ▶ “The Unwritten Social Contract” -- the company must conduct activities that are not required for other disorders
  - Comprehensive patient support programs that assure the patient will receive the drug, whether they are insured or not
    - “No patient left behind” mentality of ultra-orphan manufacturers
  - Disease awareness programs in the medical and patient community to identify patients and train clinicians

Borrowed from NORD's Diane Edquist Dorman

## Finding Equipoise...

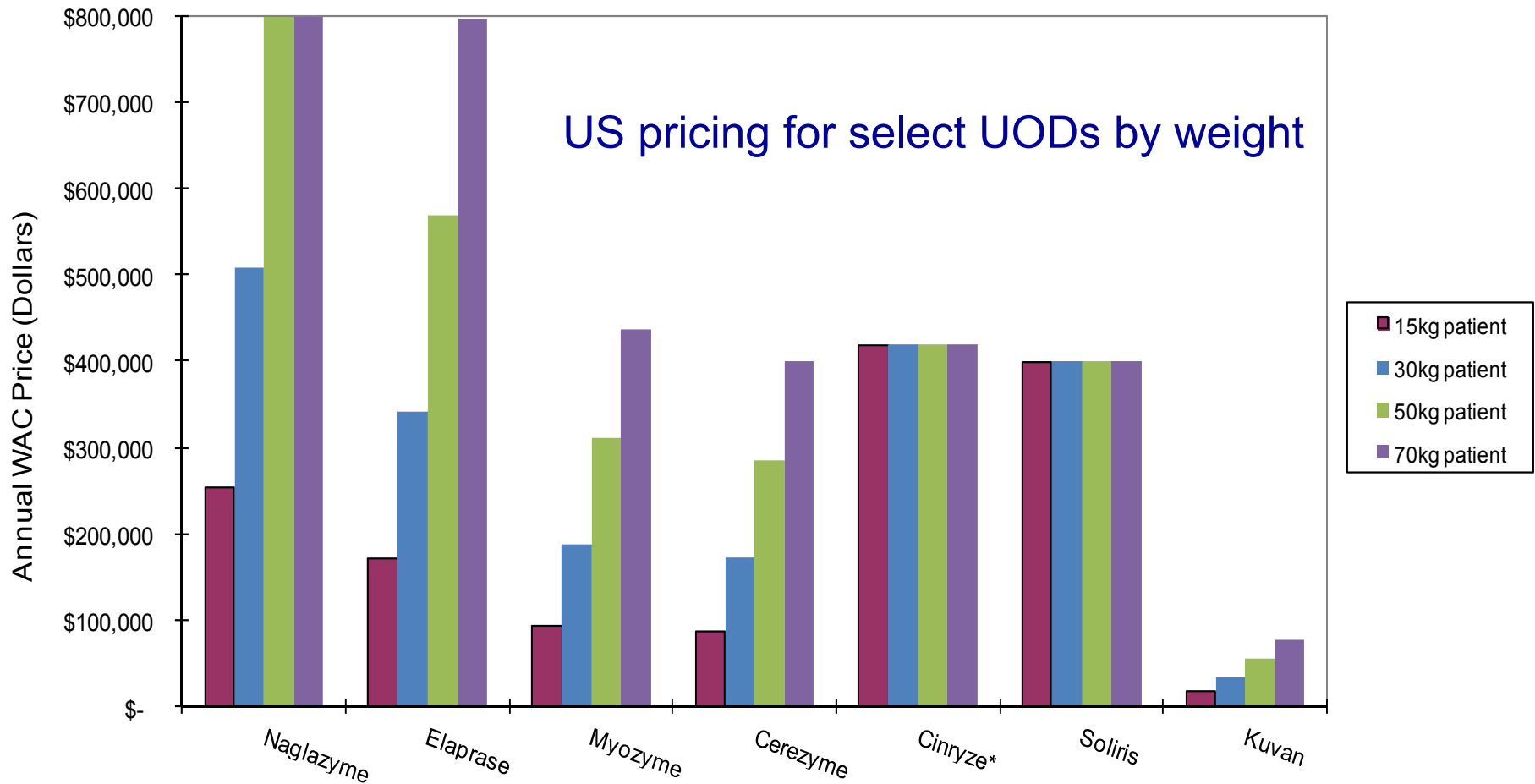


Another perspective, to paraphrase NORD's Diane Dorman

Good news...the FDA approved the therapy for which you have been waiting for years  
Bad news...the price is the same as a new house every year for as long as you live

# Who Is the Most Expensive?

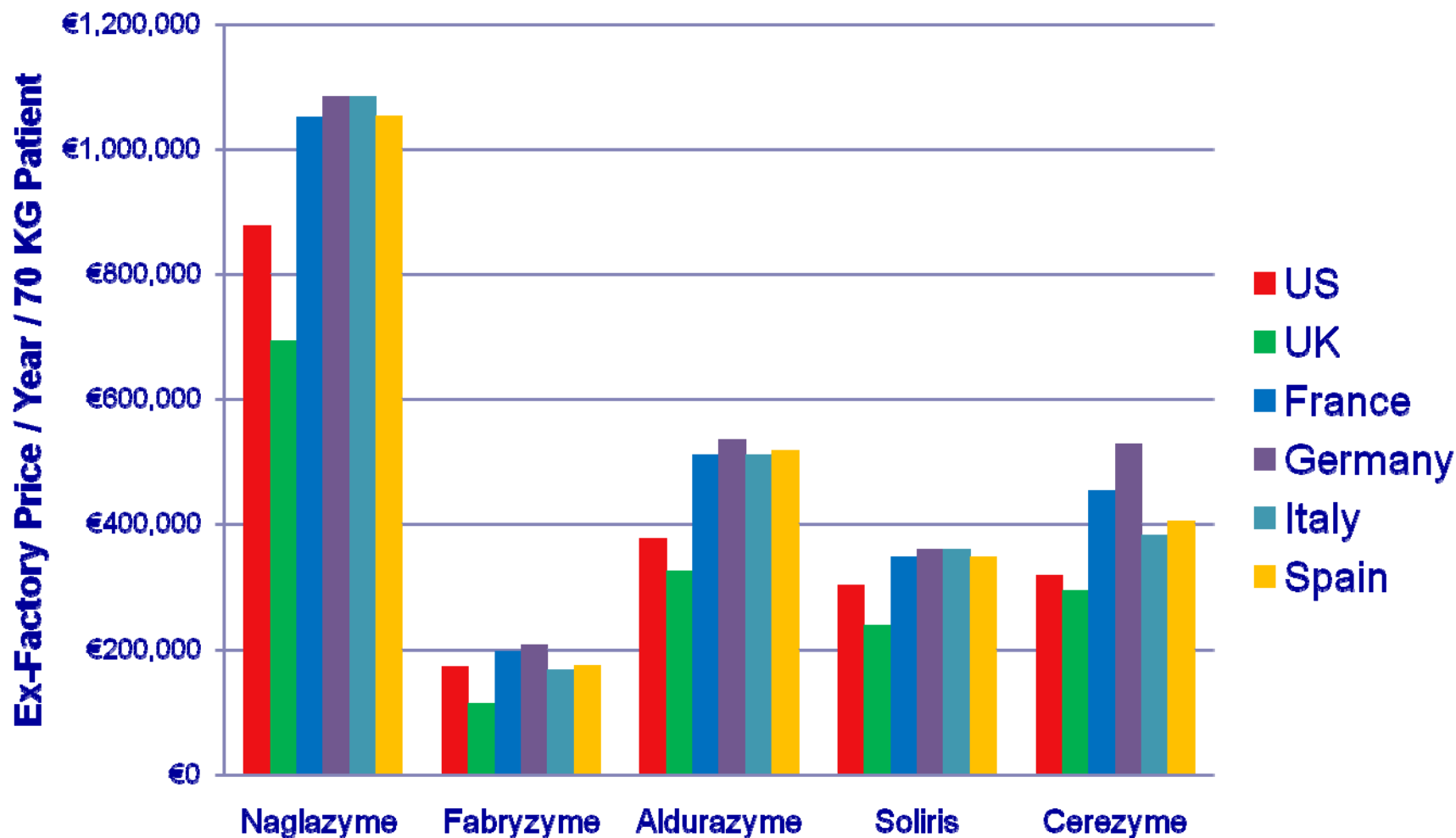
As patients weight increases so does cost of therapy



\* Assuming Cinryze is dosed at 500U every 3.5 days

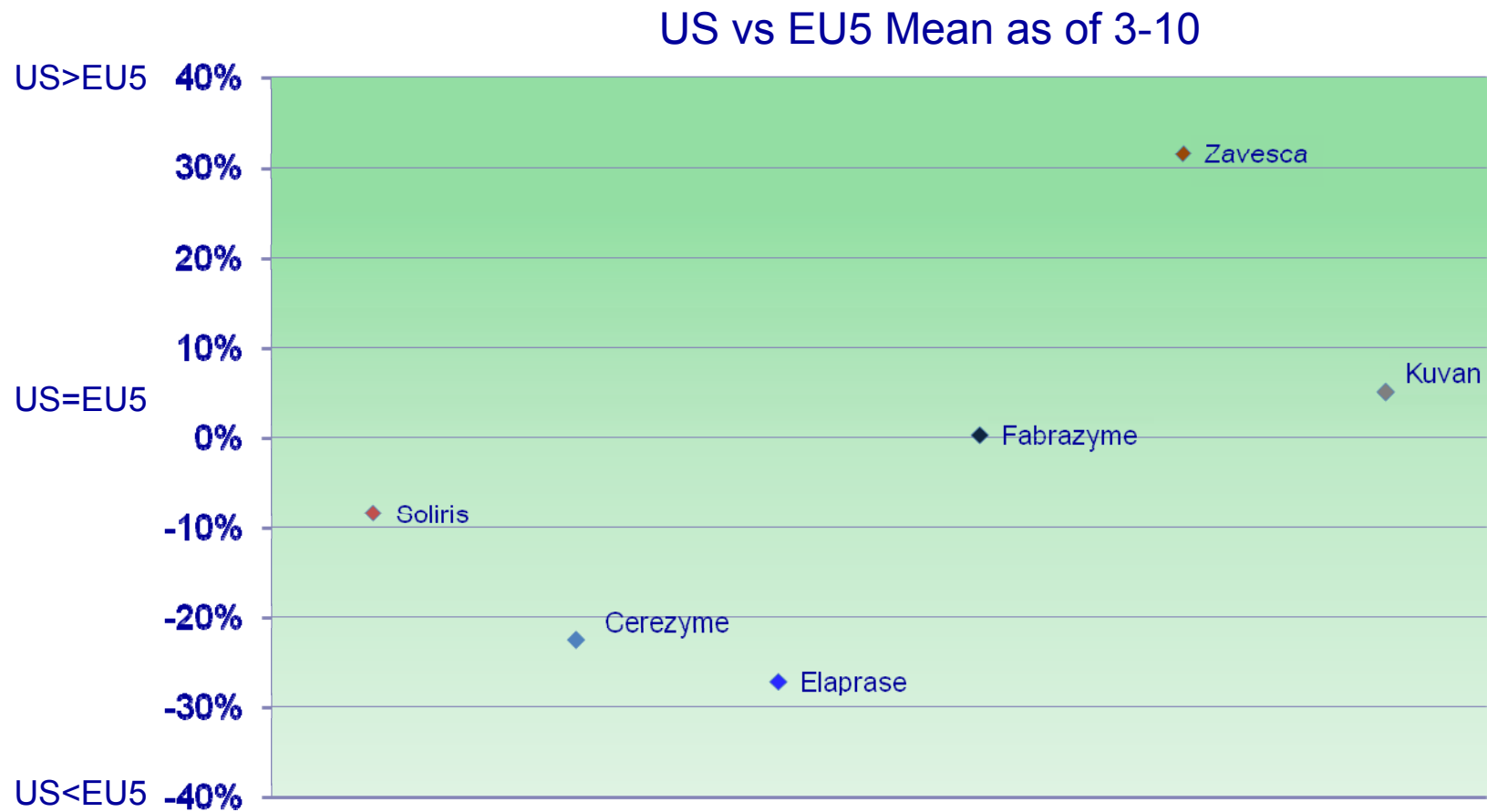
Data sources: Medispan MDDb March 2010

# US Is Always the Most Expensive, Right?



Sources: Various Country Websites: March 2010

# UOD Price Corridors Usually Very Narrow



# UOD Reimbursement Landscape

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- ▶ Many countries have accepted price ranges for novel therapies of ~\$200K to ~\$400K annually per patient for a novel ultra-orphan therapy
  - Vs. ~\$10K to ~\$90K annually a novel orphan therapies, including oncolytics
- ▶ Historically, payers do not restrict UODs beyond appropriate use due to the complicated nature of the diseases, low utilization, and low “abuse” potential
  - Patient assistance programs and reimbursement support further decrease the likelihood of payers significantly controlling product selection in these markets
  - Generally just want the drug to be used correctly

Could Alanis Morissette sing about this?

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Sept 22, 2010

**Two headlines back to back in the email update...**

**“Teva Files Second Lawsuit Against Mylan Over Generic Version Of Copaxone”**

**“Mylan Gets Court Order Blocking Competing Version Of Paxil”**

# New UOD Developments: Direct Competition?

ARCALYST®  
(rilonacept)

ILARIS®  
(canakinumab)

Cerezyme  
imiglucerase for injection

VPRIV™  
velaglucerase alfa  
for injection  
For Type 1 Gaucher Disease

CINRYZE®  
C1 esterase inhibitor (human)

BERINERT®  
C1 Esterase Inhibitor, Human

Chronic?

PROTALIX  
Biotherapeutics

zavesca®  
miglustat

Myozyme®  
(alglucosidase alfa)

Lumizyme™  
(alglucosidase alfa)

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When can lower prices  
increase volume?



**Orals = Medicare Part D**



# US Healthcare Reform Implications for Orphans

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## ► Changes that impact orphans:

- Prohibition on lifetime limits / annual caps / preexisting conditions
- Medicaid fee-for-service (FFS): Minimum 15.1% rebate increased to 23.1% retroactive to January 1, 2010
  - 340b prices changed commensurately as of July 1, 2010
- Managed Medicaid units now subject to Medicaid rebates
- 50% Medicare Part D rebate in “donut hole” applies effective January 1, 2011

## ► Orphans exempt from:

- 340b expansion: Access to pricing for additional hospitals
- Biopharma industry fee (aka “excise tax”): \$2.5B aggregate annual industry fee apportioned based on each entity's relative share of branded prescription drug sales to any specific government program (includes Medicare Part D, Medicare Part B, Medicaid, VA, DOD or Tricare) effective January 1, 2011

## Downward Price Migration for Additional Indications

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- ▶ More myth than reality?
  - FDA approvals
  - Dosing
  - “Pressure”

## Where are your patients?

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- ▶ What if the insurance risk isn't spread over a large population (e.g. some EU countries or hereditary orphan diseases)?
- ▶ “Price taker” vs. “Price maker”



# Possible Future UOD World

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- ▶ Why be concerned?
  - Genzyme was THE ultra-orphan company and now there are many being sold and more in the pipeline
  - Average annual cost of many weight based products is growing as well with their patients living longer and increasing in weight
- ▶ Will an individual product be scrutinized more than others?
  - Ultra-orphans are generally viewed as a group, not as individual drugs
  - Society has essentially agreed at a conceptual level to pay for these therapies
- ▶ How will change occur?
  - We believe the biggest risk for the current ultra-orphan drug pricing/reimbursement models is in the cumulative budget impact at the policy level...
  - Thus risk is associated with the entire basket of ultra-orphan products vs. for any individual product
    - Although a “false step” by one could impact the entire group
  - Given the growth in biologics (especially in oncology), MME hypothesizes that actions here will subsequently be applied to UODs
    - As society questions the need to pay high prices for oncology products that add nominal extensions to life and are often associated with poor quality of life, society’s next arena to question is high priced product groups, such as ultra-orphans
    - CERs / QALYs may be applied to UODs, even though they clearly do NOT fit

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# Thank You

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