



## Yes, No, Maybe, Please Ask Again: The Frustrating Tale of Medicare Part D Formularies and the Coverage of Expensive Orphan Drugs


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*“The law, in its majestic equality, forbids the rich as well as the poor to  
sleep under bridges, to beg in the streets, and to steal bread.”*  
--Anatole France

As will be evident, my topic today is to explore how Medicare Part D is being implemented. Specifically, I will be examining the development of formulary policy, appeal rights, and coverage of expensive orphan drugs.

Slide 1



Yes,  
No,  
Maybe,  
Please Ask Again:

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The **Frustrating** Tale of Medicare  
Part D Formularies and the Coverage of  
Expensive Orphan Drugs

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As you will hear, our community won some key battles with a sympathetic staff at the Centers for Medicare and Medicaid Services (CMS). But now, we are at an impasse over what will happen next week, next month and next year. CMS believes that rare disease

patients will get what they need, without too many questions or problems. The National Organization of Rare Diseases (NORD) is skeptical and thinks more will need to be done to assure access to medically necessary orphan drugs.

## Slide 2



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“The law, in its majestic equality,  
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--Anatole France

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The message from France points out: if two people are treated equally, the impact on each may not be equal. Let's try to be imaginative here. Medicare has about 40 million beneficiaries; the disabled as well as those over 65.

Imagine that 10 million guests are coming for breakfast (sort of like New Orleans) and you are busy arranging for 16 million eggs and 20 million slices of bread, along with countless tables, chairs, cooking stoves, and cooks. Then to your amazement and horror, you discover that most of the 10 million people expect a meal that is individualized to their appetite and dietary restrictions. This is a lot like the predicament that CMS was put in when Congress passed the Medicare Modernization Act (MMA).

Suddenly, there was a new Part D benefit—for the first time extending outpatient drug coverage to Medicare beneficiaries. Getting it set up so that it is right for most people, necessarily means that CMS has to be tough with allowing exceptions, lest the exceptions multiply and take the Part D program down. Yet, there are obvious inequities when you treat everyone equally, as Anatole France has stated so succinctly.

***Let's face it: “Being CMS” is a difficult job.***

Under Dr. McClellan's leadership, we have had multiple opportunities to talk with him and his staff about how rare disease patients will be treated under Part D. They have always been gracious and, at a number of points, quite responsive to the needs of the rare disease community. We believe that they do care.

My frustrating and curious tale is about the big overhanging issue: when the Part D program is implemented, will rare disease patients have access to the expensive orphan drugs that they need?

It is hard to know where this story should start.

When progress has been achieved—as it has in this case—one does not need to study the history to know it’s path has been neither swift nor easy. In this regard, Medicare Part D, as implemented, is an iceberg with much of its mass submerged and invisible.

I will skip through history up to 2004, pausing only once. Even the discussion of 2004 and 2005 is but a fraction of the issues, meetings, and positions that have occupied CMS and, thus, occupied our time as well. From that history, I will note that both Houses of Congress passed a Medicare outpatient drug benefit in 1988. President Reagan signed the legislation. It was hailed as a milestone by policymakers, media and community groups.

### Slide 3



Remember the famous photo of angry seniors attacking House Ways and Means Committee chairman Dan Rostenkowski in his car?

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Some portion of the Medicare population did not agree. They were vocal, they were aggressive and they truly believed that they had been given a raw deal. There is an iconic shot of this imposing figure, Dan Rostenkowski, Chairman of the House Ways and Means Committee, in a limo in his Chicago district, being pelted with tomatoes by seniors.

Sadly, I could not find the exact shot, but you can imagine that a man like this—so imposing and so powerful--was humiliated by such extreme and unexpected acts of ingratitude. The next year, Congress repealed the program. . . long before it was to be implemented.

## Slide 4



### USP--First Battle of the Formularies

- Category view of drug coverage
- USP assigned responsibility
- At least 2 drugs needed to be available per category
- Original proposal outraged entire disease community
- Final product inadequate to needs
- CMS distanced itself, added other criteria to evaluate formularies.

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We pick up our tale in the middle of 2004. Congress wanted CMS to have the benefit of an outside group working at the typology of medical treatment. What are the important categories, such as analgesics and, are there important distinctions within that category that make it therapeutically divisible? Ultimately, the product was supposed to be a guide for the minimal formulary that would conform to the non-discrimination provisions of MMA.

Through Congressional report language, the task was assigned to the US Pharmacopeia (USP). USP made a valiant effort, but it is not a medical organization (although many of its key people are physicians). It is not a policy organization and it has no reason to have acquired any political savvy to provide a context for what they were doing.

The categories proposed by USP met with enormous resistance, particularly from disease groups of every stripe. Most were concerned about too little choice, old medications, and a barrier to multi-drug treatment regimens. As noted, the USP lacked the political savvy to have anticipated and dealt with this problem.

NORD was upset for the same reasons, but there were also unique orphan needs, totally unacknowledged. We had hoped there would be a separate category created, into which all designated and approved orphan drugs would be placed. Instead, there was no acknowledgement of orphans. In some categories, there was no room left for an orphan. In others, there was only one slot. For example, under the Enzyme replacements/Modifiers category, there was one slot, but several orphan drugs for different conditions and not interchangeable.

Ultimately, CMS accepted the USP product, but distanced themselves. When the dust settled, USP realized how orphans had been left out and they told us they would communicate their concerns to CMS. To our knowledge, they did not. CMS made clear it was going to use additional criteria and that final approved formularies would be much more robust than what USP had provided.

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### Appropriations Report Language

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The Committee is concerned that Medicare patients with rare diseases may have difficulties accessing care that involves orphan drugs. The Committee encourages CMS to carefully consider the impact on this population in proposing regulations.


The Committee encourages the Administrator to solicit the views of the FDA Office of Orphan Products Development and the NIH Office of Rare Diseases, as well as stakeholder groups, before determining whether an access problem exists or would be made worse by proposed regulations.

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With the USP process looking bad from the beginning and always fearful of CMS allowing formularies unfriendly to rare disease patients. NORD pursued another way to get CMS focused on this problem. The NORD Washington office was successful in getting this language (see slide 5) inserted into the report accompanying the FY 2005 appropriation for the Department of Health and Human Services.

This proved useful in demanding attention, but never resulted in the formal consultation process we sought.

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### CMS-Second Battle of the Formularies

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- o CMS provided direction to PDPs to formulate their bids
- o Based on "good" public and private sector formularies. Orphan drug coverage varied under these plans
- o CMS reviewed hundreds of PDP formularies.
- o CMS claimed their hard negotiating achieve broad drug availability

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Once USP delivered its report, it was time for CMS to provide directions to the prescription drug plans. As guidance, CMS named about six drug plan formularies—some private and some public that were considered to provide excellent coverage. We took each of these model formularies and evaluated orphan drug coverage. It varied from quite good to nearly abysmal. Therefore, we have no clue as to how we stand with the PDP formularies that will be in operation on January 1, 2006.

Since these were closed negotiations, we can judge them only by what CMS told us. They told NORD that criteria were tough, orphan drug coverage was good, and appeals

would clean up the rest. This was also what they had told every other disease-related cause.

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### Specialty Tier—A Bump or a Mountain?

- PDP's allowed tier for costly drugs
- Co-pays may exceed other tiers

For rare disease patients with high-cost treatments:

- tier will accelerate payment of deductibles/co-pays
- patients may have to pay \$4,000 or more during the first quarter of year
- No different impact than w/o special tier

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To give plans more flexibility, CMS permitted plans to create a tier different from the branded tier and the generic tier. The idea was to give drug plans the opportunity to separate out more expensive drugs (\$1,000 and above), charge a higher co-pay and keep a closer eye on utilization and costs. At first this was seen as a way to discriminate against patients needing more expensive medicines, especially since the decision to place a drug in the specialty tier is not appealable.

However, for most rare disease patients with costly medications, none of this matters much. The higher co-pay gets them to their out-of-pocket ceiling faster, at which point, beneficiaries will only owe 5% of the total cost, while the payer and the government will be picking up the vast majority of the cost. For patients with tens of thousands of dollars of drug bills, it is only the difference between an extra week or two longer that they owe the deductible or a week or two less that they don't have to worry about it.

NORD has chosen to focus on the timing problem. Many orphan patients will have to come up with full payment of their deductible in a matter of weeks or a few months. This may turn out not to be a burden. Or, it may be a problem that has no solution (e.g. spreading out payments makes sense, but it may be contrary to other laws dealing with preventing fraud and abuse).

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### “All or Substantially All” The Biggest Exception

- CMS carved out 6 disease areas, such as cancer and psychiatric drugs
- Focused on high volume categories
- PDPs directed to cover “all or substantially all” drugs in these categories
- As “low volume” products, CMS did not consider orphan drugs for inclusion
- NORD: CMS should treat orphans as an “all or substantially all” category

Relatively late in the process, CMS woke up to realize that the first round of appeals was likely to be voluminous, extremely time-consuming, and result in very few denials. A patient on Paxil, who is stable and doing well, should not be shifted to Zoloff or Prozac without support from his/her medical provider. To avoid this, CMS carved out six disease areas and told PDPs to cover “all or substantially all” of the products in that category.

While much is different between orphans and anti-depressants, some of the issues are similar. CMS has repeatedly told us not to worry because every orphan not on the plan’s formulary will be paid for anyway, based on doctor assertion of medical necessity and the likelihood orphan patients will win on appeal. It may work out this way, but almost all the risk would be eliminated by putting orphan drugs—“all or substantially all” of them--on automatic acceptance.

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### Yes, No, Maybe, Please Ask Again

- Protections are great on paper:
    - a statutory non-discrimination provision
    - medical necessity trumps formulary status
    - Multi-tiered appeals process
  - Will it work in “real life?”
    - PDPs profit when drugs are denied
    - Medical necessity should overrule all other considerations, but it frequently doesn’t
    - Rationing by inconvenience
- Do patients know that a “no” from a PDP means: “maybe” or “please ask again?”
- Will patients persist through multiple appeals?

The real and the theoretical....only time will tell.

## Slide 10



### The Ultimate Disagreement

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- CMS reassured NORD:
  - criteria were tough,
  - orphan drug coverage was good, and
  - appeals process will quickly cover the rest
- NORD continuing concerns:
  - PDP's won't have orphans on formularies
  - PDP's will try to save money through denials
  - PDP's will force appeals, so patients give up

In a competitive market, is "medical necessity" enough to force access?<sub>10</sub>

CMS has chosen to believe that the same rules applied to everyone will work for rare disease patients. NORD has advocated special rules to assure this will work for rare disease patients. Like Anatole France, NORD has not wanted to take the chance that the same rules will provide the same results.

## Slide 11



### "Here Come the Beneficiaries"

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- PDP's offered many low premium policies
- Plan marketing starting now
- Enrollment begins November 15
- Those enrolled by December 31, will start coverage January 1, 2006

**NORD—and, we hope, CMS--will be watching carefully to assure that rare disease patients are getting medically necessary treatments**

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As the initial marketing and enrolment phase begins next week. NORD, and we hope CMS, will be watching carefully to assure that rare disease patients are getting the vital medicines they need.

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