



March 6, 2006

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Administrator  
Centers for Medicare and Medicaid Services  
Room 445-G, Hubert H. Humphrey Building  
200 Independence Avenue, SW  
Washington, DC 20201

Re: Draft Transition Process Requirements for Part D Sponsors, February 2006

Dear Dr. McClellan:

On behalf of the many Medicare beneficiaries with rare diseases, we appreciate the opportunity to comment on CMS' guidance dealing with formularies and therapeutic transition.

CMS staff has been extraordinarily responsive to concerns of rare disease patients. We are grateful that you understand how difficult it is to be a patient with different and unusual needs in a large new system.

Our continuing concern is that CMS' written materials assume the same insight and the same good will among the plans implementing the Part D benefit. We urge you to insist that plans meet the needs of rare disease patients. Do it prospectively and in writing, so that plans will not have the excuse that they are "not required" to be responsive or that it is not built into their actuarial analysis.

#### Greater Assurance of Access for Rare Disease Patients

We share your frustration that so many decisions need to be made for 2007, before we have more experience with the Part D program. Some of our concerns would be lessened if we had more confidence in the efficacy and promptness of the appeals process in dealing with the needs of patients with rare diseases. Because decisions need to be made now, protections must be greater.

Using the rubric of "all or substantially all," CMS has chosen to protect patients who need immunosuppressant, antidepressant, antipsychotic, anticonvulsant, antiretroviral and antineoplastic therapies. As you acknowledge in your guidance document, patients in need of these therapies represent a vulnerable population. CMS wants to "mitigate the risks and complications associated with an interruption of therapy."

As a result, these patients are largely spared the appeals process. They know they will get the right medicine, even in situations (such as with anti-depressants) where there are multiple products, but a chance that substitution will result in therapeutic failure. Drug plans cannot get it wrong, because they have been explicitly told how to do it right. Further, the actuarial playing field has been made level for them. All plans must provide drugs in these categories without formulary restriction and without requiring appeal.

Patients with rare diseases are no less vulnerable. For many, access to orphan drugs is, literally, their life-line. In most cases, no other therapy exists. Similar or alternative therapy is not a viable option. A change in size or frequency of dose can be devastating. It seems logical that all formularies should cover orphan drugs. Appeals should be rare and always result in the patient receiving their medically-necessary medications.

The reality is less sanguine. The appeals process is an unnecessary burden on the care team and brings uncertainty for rare disease patients. It is an invitation for the system to fail by denying treatment, reducing dose or altering the treatment schedule. Interruption of therapy is always a danger.

If “all or substantially all” did not already exist, we would be asking for something like it for orphan drugs. But it does exist and assuring the access needs of rare disease patients requires only that CMS add FDA-designated orphan drugs to the six drug categories that all plans must cover. We urge you to do so.

#### Eliminate Discrimination Against Patients with High-Cost Medications (many of whom are rare disease patients)

We continue to have serious concerns about the specialty tier. As originally stated in our May 17, 2005 comments to CMS:

- The specialty tier is inherently discriminatory against beneficiaries with rare diseases who require “unique therapies that are often very expensive;”
- The specialty tier is inconsistent with the MMA language and congressional intent by eliminating the exceptions process for beneficiaries needing drugs that have been put in the specialty tier;

Those concerns have yet to be addressed. Further, the specialty tier does not meet CMS’ current guidance on evaluating tier-based discrimination:

CMS will review tier placement to provide an assurance that the formulary does not substantially discourage enrollment of certain beneficiaries.....The CMS review will focus on identifying drug categories...[placed] in non-preferred tiers in the absence of commonly used therapeutically similar drugs in more preferred positions.”

This is a serious concern and we urge CMS to be aggressive in reviewing plans to eliminate tier-based discrimination.

Unfortunately, the specialty tier—in principle and as drafted—allows plans to practice tier-based discrimination with impunity.

Patients who need high-cost drugs (greater than \$500 per month negotiated price) will be among the most expensive for the system. These patients have the largest burden and should be given the most help by the Part D program. For example, case management is a proven, cost-effective approach to managing costs, while meeting the medical needs of these patients. It is an appropriate system response.

Instead, patients with high-cost drugs have been placed in the tier with the largest cost-sharing requirements. Further, unlike other Part D decisions adverse to the patient, placement in a specialty tier is not appealable. Those who will be required to spend the most are simultaneously cut off from any possible break on their cost-sharing requirements.

As a matter of principle, the specialty tier is wrong. The lack of an appeals process is a further encouragement for plans to practice tier-based discrimination. By its own standards, CMS should not be sanctioning this discriminatory behavior.

Protecting Patients with Large Out-of-Pocket Costs

For patients with high medication costs, regardless of tier, there is the risk that their responsibilities will outstrip their ability to pay. In many instances, patients will have incurred \$3600 in TrOOP expenses within a few weeks or months, but not have the ability to pay that much money quickly. This will be even worse for patients with the very highest drug costs, where the 5% catastrophic co-pay can be a large and continuing burden. We urge that plans be required to adopt practices that would permit patients with large drug costs to spread their TrOOP costs over a reasonable period of time.

NORD thanks CMS for the opportunity to comment. We are available to clarify any points and would be delighted to help in any way to make sure that the problems we have outlined are solved.

Sincerely,

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