

May 17, 2005

Mark McClellan, MD, PHD
Administrator
Center for Medicare and Medicaid Services (CMS)
Hubert H. Humphrey Building
200 Independence Avenue, SW
Washington, DC 20201

Re: Proposed Specialty Tier: Medicare Prescription Drug Benefit. Final Rule, 70 Federal Register 4193, 4565, 42 C.F.R. 423.578(a)(7), January 28, 2005

Dear Dr. McClellan:

The final rule involving specialty tiers creates processes and standards that were not in the draft rule and which may have serious consequences for patients with rare diseases. Established in 1983, the National Organization for Rare Disorders (NORD) is a non-profit, voluntary health organization dedicated to the identification, treatment and cure of rare "orphan" diseases through programs of education, research, advocacy, and service to patients and families.

We are specifically concerned that the "specialty tier" emerged *de novo* in the final rule. Had there been a comment period, we would have raised the following issues:

- 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.

The final rule is also unfair because CMS did not provide *Notice of Intent* to create a specialty tier that would function outside the exceptions process.

CMS's explanation has been to assert that the exclusion is required to maintain the 75%/25% actuarial equivalence. Since, specialty tier drugs are almost exclusively ones that are rarely used, the total dollars involved should have no impact on actuarial equivalence. If CMS believes that the specialty tier is needed to maintain actuarial equivalence, NORD would like the opportunity to review the data and enhance our understanding of how this will impact rare disease patients.

There is a related, practical question that is of far more consequence to rare disease patients and which could have been resolved as part of creating the specialty tier. Beneficiaries pay several thousand dollars out of pocket as part of their co-pay until they reach the catastrophic threshold. The vast majority of beneficiaries will never reach this threshold during any one year... so their co-pay is likely to be paid in increments over most of the year.

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Those who are treated with high-cost drugs will be in the opposite situation, where all or almost all of their pre-catastrophic co-pay will be spent within 3 to 4 months. All at once, these beneficiaries will be required to lay out \$3600 plus any monies due under the 5% catastrophic co-pay. For many beneficiaries, it will be difficult – for some it will be impossible – to pay this large amount at one time. We anticipate that some patients, many of whom are on life-sustaining therapy, will have unpaid debt starting in January; others may not even start a therapy, knowing that they do not have the liquid assets to pay the required co-payment. Further, our analysis shows that varying the co-payment level or rate does not make any material difference in the hardship problem.

Our view is that the specialty tier provides no benefit to patients, plans or to CMS. It highlights, but does not assist, the many rare disease patients who are likely to pay out the entire co-pay early in the year. There is no reason for the specialty tier to exist; it just further complicates a complex implementation schedule. However, if it is to exist, it should be used as a way to delineate beneficiaries who will need to spread their co-pay over many months, even if it is due and collectable during the first quarter of the year.

We thank you for reviewing our comments. We would welcome the opportunity to work with your staff to develop a process whereby those with rare diseases and catastrophic costs are not forced to pay their entire co-payment at one time.

Sincerely,

Abbey S. Meyers
President

Cc: Diane E. Dorman, Vice President, Public Policy