



September 6, 2005

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

Re: Medicare Program; Competitive Acquisition of Outpatient Drugs and Biologicals Under Part B; Interim Rule CMS-1325-IFC “Provisions of the Interim Final Rule”

Dear Dr. McClellan:

On behalf of the National Organization for Rare Disorders (NORD), we request that CMS revise its position—stated in the CAP final rule—that some orphan drugs (ones defined by CMS as "single indication") should be excluded from the CAP program. We appreciate CMS' concern for rare disease patients and, in hindsight, recognize that there was some ambiguity in the position stated in our comments of May 23, 2005.

At this time, there is no evidence, nor plausible reason, to exclude orphan drugs—as a class—from the CAP program. Indeed, we can imagine situations where inclusion in the CAP program will result in increased access for rare disease patients (e.g. physicians may be more likely to order and administer an orphan drug that is available through the CAP program). We believe that the best policy would be to include all orphan drugs in the CAP program, while allowing consideration of specific drugs/treated populations where exclusion may be warranted.

We know there will be a problem where multiple drug products share a HCPCS code and are not interchangeable for all patients. There was sufficient concern about this for Congress to exclude intravenous immune globulin (IVIG) products from the CAP process. The same situation applies to antihemophilia clotting factors and alpha-1-proteinase inhibitor and they are appropriately excluded by the final CAP rule.

In sum, (1) all orphan drugs should be eligible for inclusion under CAP, (2) alpha-1-proteinase inhibitor, antihemophilia clotting factors and intravenous immune globulin should be excluded from CAP as requested by those knowledgeable about the use of those products and (3) a review mechanism should be established where relatively rapid decisions can be made by CMS to evaluate access problems and exclude a drug that would otherwise be included.

Dr. McClellan, this also seems an appropriate place to comment on CMS's decision to treat "single indication orphans" as a class under the CAP regulations. CMS originally developed this definition as part

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of the HOPPS regulatory process in 2002 in order to distinguish certain products that would be paid under methods expected to provide a higher percentage reimbursement under that program. At the time, NORD offered several alternative approaches to address the concerns about inadequate payment rates for all orphan drugs and biologicals under HOPPS.

We believe strongly that CMS should not have (indeed, has no need for) a definition of "orphan drugs" that is different than the statutory definition in the Food, Drug and Cosmetic Act. We expressed our concern in our comments on the proposed HOPPS rules in 2002, 2003, and 2004, as well as in several other meetings and communications with CMS. Shortly, we will be reiterating our opposition to treating "single indication orphan drugs" as a class in our comments on the proposed 2005 HOPPS rule. Simply put, "single indication orphan drugs" is not a meaningful way to distinguish among orphan drugs and rare disease patients. It is bad policy and inconsistent with actions taken by other government agencies.

While we disagree vehemently with the use of the CMS orphan drug definition in HOPPS and we have proposed alternatives, we at least understood the goals that CMS was trying to achieve when it developed the definition. No such rationale applies with regard to CAP. We fear that CMS has taken a definition created to meet a very specific (HOPPS) program goal and decided that those are the only orphan drugs/rare disease patients to whom CMS has legal and moral obligations. That may not be CMS' intent, but it is the consequence of your actions in the final CAP regulation.

We look forward to seeing revisions to the CAP final rule and would be happy to work with CMS officials to develop a process that will be timely, fair and not burdensome to CMS or to those asking for exclusions. Further, we would like an opportunity in the next few weeks to discuss the importance of CMS bringing its orphan drug definition into line with the statutory definition used by the rest of government.

We are grateful for your consideration of these requests. If you would like this assistance or need further information, please contact Diane Dorman at NORD's Washington, DC office: 202.496.1296 or ddorman@rarediseases.org.

Sincerely,



Abbey S. Meyers
President

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