



## STATEMENT OF THE NATIONAL ORGANIZATION FOR RARE DISORDERS

Before the Medicare Coverage Advisory Committee

March 30, 2006

CMS Headquarters, Baltimore, MD

The National Organization for Rare Disorders (NORD) is pleased to have the opportunity to provide comments to the Medicare Coverage Advisory Committee (MCAC) regarding authoritative drug compendia for anti-cancer chemotherapeutic agents.

We are asking MCAC to provide the following direction to CMS:

Because off-label therapies for rare cancers may receive less attention from compendia and may experience additional structural delays in being evaluated, MCAC recommends that CMS work with patient groups, professional associations and compendia publishers to assure Medicare beneficiaries with rare cancers have coverage and access to innovative, state-of-the-art care on a comparable basis to beneficiaries with more common cancers. CMS should examine the need for similar action with regard to coverage and access to therapies for other rare diseases and conditions.

All of NORD's efforts to promote research and development of orphan drugs are diminished if patients cannot get access to new therapies or if the level of medical evidence supporting a particular use is not well-known. Thus, being listed in a compendium matters. It is a springboard to reimbursement, as well as a tool by which new and innovative therapies gain acceptance.

The focus of our statement is existing FDA-approved anti-cancer drugs—whether orphan or not—that may have additional uses in treating one or more rare cancers. However, we will be describing problems that are equally applicable to FDA-approved therapeutic agents being developed for a broad array of additional indications for rare diseases and conditions.

Because of the robustness of current cancer research, there is a long-lag time in oncology from research results to publication to compendia. This should be of concern to all cancer patients and, presumably, is one reason why the National Comprehensive Cancer Network is seeking official status for its compendium.

The lag time problem is magnified for rare cancers and orphan cancer drugs. Not surprisingly, we have observed that orphan indications are studied less often, are less likely to be presented at major medical conferences, and rarely have priority for publication after being presented. Also, orphan drug research is less likely to be featured in the front-line journals that are the primary material being reviewed by compendia committees.

While new orphan cancer indications wait to be presented, published and listed, rare disease patients often must wait, too. As a result, patients with rare cancers may wind up with reduced access to front-line oncology care because:

- Dissemination of innovative therapies is greatly slowed
- Effective treatments take much longer to gain reimbursement

This situation discourages researchers from working on orphan drug indications, resulting in further burden on the rare disease community.

Lag time is not the only problem with relying on the existing compendia systems to recognize orphan cancer drug indications. Large, randomized controlled trials are the acknowledged gold standard, but are often impossible when the target population is small or the resources limited for the investigation of an additional orphan indication.

The case for medical acceptance of orphan therapies—especially for second or third indications of an FDA-approved drug—is often based on multiple sources and types of evidence. Compendia listings should be explicit about the degree or level of evidence available, but not ignore orphan indications because they are based on studies that are not large, randomized and controlled.

All of our concerns are heightened by the lack of transparency involved in the existing compendia process. Patient groups, researchers, and industry cannot predict when or whether reviews will occur, what standards will be used, or whether all evidence was considered. In these circumstances, the quest for fair treatment for patients with rare cancers is hard to assess and impossible to remedy.

Ultimately, the solution may be a compendium specifically for rare diseases that will, when appropriate, recognize authoritative treatments on the basis of presentations at major medical conferences, publications of abstracts or other similar evidence. But this is truly a long-term response...it will take a number of years to reach this stage, if ever.

In the interim, and as noted at the beginning of my statement, we ask MCAC to provide the following direction to CMS:

Because off-label therapies for rare cancers may receive less attention from compendia and may experience additional structural delays in being evaluated, MCAC recommends that CMS work with patient groups, professional associations and compendia publishers to assure Medicare beneficiaries with rare cancers have coverage and access to innovative, state-of the-art care on a comparable basis to beneficiaries with more common cancers. CMS should examine the need for similar action with regard to coverage and access to therapies for other rare diseases and conditions.

NORD would welcome the opportunity to assist CMS in implementing this recommendation. Thank you for considering our comments and the needs of rare disease patients.

For more information, contact:

Diane Dorman  
Vice President, Public Policy  
National Organization for Rare Disorders  
1050 17th Street, NW, Suite 600  
Washington, DC 20036  
202-496-1296  
[ddorman@rarediseases.org](mailto:ddorman@rarediseases.org)

Steven Grossman  
President, HPS Group, LLC  
PO Box 10729  
Silver Spring, MD 20914-0729  
301-879-5600  
[sgrossman@hpsgroup.com](mailto:sgrossman@hpsgroup.com)