



January 10, 2006

Mark B. McClellan, M.D., Ph.D.
Administrator
Centers for Medicare and Medicaid Services
Room 445-G, Hubert Humphrey Building
200 Independence Avenue, SW
Washington, DC 20201

Re: Orphan Drug coverage in Medicare Part D Formularies

Dear Dr. McClellan:

The National Organization for Rare Disorders (NORD) is committed to ensuring that Medicare beneficiaries with rare diseases have access to orphan drugs and biologics that they need to sustain and improve the quality of their lives. We are particularly concerned about the access for rare disease patients to orphan drugs under the new Medicare Part D outpatient program.

Part D Orphan Drug Coverage is Good, with a Few Notable Exceptions

Overall, we commend CMS for encouraging insurance plans to provide adequate coverage of orphan drugs in their Part D formularies. The majority of orphans researched had moderate to adequate coverage among the national plans. However, there are **seven orphan drugs** that we deem as “high priority access problems” because they are inadequately covered by the 10 national formularies (Attachment).

Since the public posting of the drug formularies on November 15, 2005, NORD has researched the inclusion of orphan drugs among the ten national PDP formularies. We initially focused on 98 outpatient drugs that have orphan status. Eventually, we were able to narrow this to 75 outpatient orphan drugs whose use is relevant to the Medicare Part D population (Attachment).

“Inadequate coverage” is defined as appearing on four or fewer national formularies. The seven priority drugs with inadequate coverage are: **H.P. Acthar Gel®**, **Galzin®**, **Syprine®**, **Cystadane®**, **Gastrocrom®**, **Xyrem®** and **Thiola®**. Below is a description of each drug, its intended therapeutic use and its importance to the Medicare beneficiary population.

H.P. Acthar Gel®: Acthar Gel® (corticotrophin) was approved in 1978 for acute exacerbations in multiple sclerosis (MS) patients. According to the National Multiple Sclerosis Society, an exacerbation (also known as an attack, a relapse, or a flare) is a sudden worsening of an MS symptom or symptoms, or the appearance of new symptoms that last at least 24 hours and are separated from a previous exacerbation by at least one month. The most common disease course in MS, called relapsing-remitting MS, is characterized by clearly defined acute exacerbations, followed by complete or partial recovery with no progression of the disease between attacks. This drug has been used to treat MS, infantile spasms, Crohns disease and adrenal insufficiency. In May 2003, Questor Pharmaceuticals applied for orphan designation for this product for the treatment of infantile spasms. H.P. Acthar Gel® is still widely used in the MS patient population.

H.P. Acthar Gel® is covered by only one national plan: Pacificare.

Galzin® & Syprine®: Wilson's disease is a rare genetic disorder characterized by excess copper stored in various body tissues, particularly the liver, brain, and eyes. Syprine®, (trientine HCL) removes excess copper from the body, particularly for patients who are intolerant of Cuprimine® (penicillamine), another older treatment for Wilson's Disease. Cuprimine® can have harsh side effects for patients; therefore, Syprine® provides a better-tolerated treatment for many Wilson's disease patients. As a result, Syprine® is an essential therapy for WD patients.

Once Wilson's disease patients have undergone the initial treatment to remove the excess copper, they are placed on a maintenance drug, Galzin® (zinc acetate). Galzin® provides pure elemental zinc to patients to prevent copper from building back up in body tissues. There are no therapeutic equivalents or other maintenance options. The approved orphan indication is for maintenance treatment of patients with Wilson's disease who have been initially treated with a chelating agent, such as Syprine® or Cuprimine®.

Galzin® is covered by only one national plan: CIGNA.

Syprine® is covered by only two national plans: CIGNA and Coventry-Advantra Rx.

Cystadane®: Cystadane® (betaine) is designated for treatment of homocystinuria. Elevated homocysteine blood levels are associated with clinical problems such as cardiovascular thrombosis, osteoporosis, skeletal abnormalities and optic lens dislocation. Cystadane® is indicated as an adjunct in treatment for homocystinuria. As a result of taking Cystadane®, toxic blood levels of homocysteine are reduced in these patients, usually to 20-30 percent or less of pre-treatment levels. The drug is critical for patients that do not respond to a vitamin B-6 and low methionine diet.

Cystadane® is covered by only three national plans: Aetna, Silverscript, and Member Health.

Gastrocrom®: Gastrocrom® (cromolyn sodium) received an orphan designation for the treatment of mastocytosis. Mastocytosis is caused by the presence of too many mast cells that can occur in two forms – cutaneous and systemic. The most common cutaneous (skin) form is also called urticaria pigmentosa, which occurs when mast cells infiltrate the skin. Systemic mastocytosis is caused by mast cells accumulating in the tissues and can affect organs such as the liver, spleen, bone marrow and small intestine.

Gastrocrom® is covered by only four national plans: MEDCO, CIGNA, United, and Coventry-Advantra Rx.

Xyrem®: Xyrem® (oxybate) is the first and only medication that has been approved by the US Food and Drug Administration for the treatment of cataplexy in patients with narcolepsy. It reduces the number of cataplexy attacks during which patients lose consciousness and their muscles are paralyzed. Since there is no other FDA approved treatment for cataplexy associated with narcolepsy, this drug is essential for all Part D formularies.

Xyrem® is covered by only four national plans: MEDCO, CIGNA, Silverscript and Coventry-Advantra Rx.

Thiola®: Thiola (tiopronin) is used in preventing a type of kidney stones that may develop due to too much cystine in the urine. Specifically, Thiola® is the only treatment for the prevention of cystine nephrolithiasis in patients with homozygous cystinuria. Patients are born with this disease and if they do not receive treatment, they begin producing kidney stones. Thiola® removes extra cystine from the body, preventing stones from developing.

Thiola® is covered by only four of the national plans: Aetna, CIGNA, United and Coventry-Advantra Rx.

PDP Implementation of Medicare Part D Needs Monitoring

We are pleased that our list of orphan drugs with Part D access concerns has steadily decreased as we have reviewed the formulary plans being offered. Nonetheless, we are concerned that these seven outpatient orphan drugs will not be readily available under Part D to rare diseases patients in need of these life-sustaining therapies. We urge CMS to notify the national plans that coverage of these drugs is essential.

Further, we will continue to monitor the implementation of Part D and keep you updated on our findings. Coverage of orphan drugs on published Medicare Part D formularies is a vital step in achieving access, but no guarantee that rare disease patients will get the treatments they need.

NORD is a federation of approximately 130 voluntary health organizations and approximately 60,000 individual patients, healthcare providers and clinical researchers. Collectively, we are committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research and service.

We thank you for the opportunity to provide insight into how the Medicare Part D program affects the rare disease community, particularly access to much needed therapies. We look forward to working with you in the coming months to assure access to orphan drugs and biologics under all parts of the Medicare program.

Sincerely,

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