



ACCESS TO ORPHAN DRUGS THREATENED FOR MEDICARE PATIENTS TREATED IN HOSPITAL OUT-PATIENT CLINICS

The orphan drug “outlier” exception in the Medicare Hospital Outpatient Prospective Payment System (HOPPS) regulations expires this year. Congress must renew this exception. Without legislation:

- some treatments for rare disorders, will be bundled for payment with high-volume, non-orphan products that often cost considerably less; others will face drastic cuts to levels well below costs;
- hospital outpatient clinics will not stock expensive, low-volume drugs (such as orphan products, including blood clotting factors for individuals with hemophilia) if they know that they will not be adequately reimbursed; and,
- patients, including disabled children and adults under the age of 65 who are eligible for Medicare, may be denied treatments because healthcare providers may refuse to administer orphan drugs, blood clotting therapies and biologics to Medicare patients.

This is a critical access issue for those rare diseases and conditions where injectable and intravenous treatments are currently available and paid for by Medicare. Many existing orphan drugs, including blood-clotting factors for individuals with hemophilia, will become less available for Medicare recipients starting in January. Future treatments and possible cures may be inaccessible; i.e., promising protein and enzyme therapies for genetic diseases.

“Orphan products” don’t fit well under HOPPS, which is the payment system used by Medicare to reimburse hospitals for services and supplies provided in the outpatient setting. It is designed to reimburse hospitals prospectively, based on bundling of many products and services together in specific categories known as ambulatory payment classifications (APCs).

Orphan products are used by small populations, and their use is highly variable from one hospital outpatient center to another. Claims-based determination of the cost of orphan products is problematic because of the low volume of claims, unreported drug codes, and charges that do not reflect costs. Without legislation and under CMS's proposal, some orphan products will be bundled for payment with non-orphan products that often cost considerably less, resulting in a significant loss for hospitals that use orphan products; other orphan products will face substantial cuts to levels well below costs.

Congress should ensure that each orphan drug or biologic is considered individually because their costs vary widely and they cannot be compared to common drugs for more prevalent illnesses. Congress should amend the Social Security Act to exclude from HOPPS rare disease therapies: orphan drugs and blood clotting factors for individuals with hemophilia.

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Orphan products are treatments for rare disorders. As defined in the Orphan Drug Act of 1983, a rare disorder is a disease or condition affecting fewer than 200,000 Americans.

NORD MEDICARE HOPPS TASK FORCE

Actelion
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Alpha-1 Association
Alpha-1 Foundation
Alpha Therapeutic Corporation
American Porphyria Foundation
Amgen
Aventis Behring
Baxter Healthcare
Bayer Biologicals
Benign Essential Blepharospasm Research Foundation
Biogen
Biomarin
CMSC/Gimbel MS Center
Consortium of Multiple Sclerosis Centers
Cornelia de Lange Syndrome Foundation
Dystonia Medical Research Foundation
Elan
Engage Health, Inc.
Epilepsy Foundation
Exceptional Parent
Families with Spinal Muscular Atrophy
Genetic Alliance
Gentiva Home Health
Genzyme
Hemophilia Association of Capital Area
Hemophilia Federation of America
Hereditary Colon Cancer Association
Hereditary Hemorrhagic Telangiectasia (HHT) Foundation, International
Immune Deficiency Foundation
Mastocytosis Society
MedImmune
Medtronic
Narcolepsy Network, Inc.
National Hemophilia Foundation
National Gaucher Foundation
National MS Society
National Organization for Rare Disorders (NORD)
National Coalition for PKU and Allied Disorders
Novartis Corporation
Orphan Medical
Plasma Protein Therapeutics Association (PPTA)
Rx Crossroads
Society for Progressive Supranuclear Palsy
Tourette Syndrome Association
Transkaryotic Therapies
Wegener's Granulomatosis Association

Payment for Orphan Drugs and Biologicals Provided in Hospital Outpatient Departments

Rationale:

Orphan drugs and blood clotting factors for individuals with hemophilia do not fit well under the Hospital Outpatient Prospective Payment System (HOPPS). These drugs and biologicals are used rarely, which is inconsistent with the fundamental premise of "averaging" underlying HOPPS. This amendment seeks to protect access to drugs and biologicals provided to Medicare beneficiaries in the hospital outpatient setting by excluding, for a period of two years, the following products from HOPPS: (1) orphan drugs and biologicals, (2) drugs and biologicals that are the same products, but which have not or cannot obtain orphan designation, and (3) blood clotting factors for individuals with hemophilia. (See "Rationale for Products Included Under NORD Amendment.") The drugs and biologicals would be paid under the fee schedule that applies outside the hospital setting.

We recognize, however, that some orphan-designated drugs may also be used for conditions that are not rare diseases. Therefore, this amendment protects only rare disease uses for those orphan drugs and biologicals with annual Medicare hospital outpatient claims volume exceeding 30,000. This claims threshold was selected because it is at the midpoint of an apparent drop-off in claims volume among orphan drugs and biologicals from review of the 2001 HOPPS Public Use File claims data and because the next group of products below this threshold are used nearly exclusively for rare diseases. Orphan drugs and biologicals with annual Medicare hospital outpatient claims volume of 30,000 or fewer would be excluded from HOPPS without requiring administrative review of specific indications. The amendment covers only a small fraction of current spending on drugs and biologicals in the hospital outpatient setting.

Proposed Amendment:

PAYMENT FOR ORPHAN DRUGS AND BIOLOGICALS

(a) PAYMENT FOR ORPHAN DRUGS AND BIOLOGICALS.--

(1) Section 1833(t)(1)(B) of the Social Security Act (42 U.S.C. 1395l(t)(1)(B)) is amended by striking the period at the end of clause (iv) and inserting a semi-colon and by inserting at the end the following new clauses:

"(v) for periods prior to January 1, 2005 does not include a drug or biological that has been designated as an orphan drug under section 526 of the Federal Food, Drug and Cosmetic Act or a drug or biological which is described under the same product code, has the same non-proprietary name, or is the 'same drug' as that term is defined by the Food and Drug Administration under regulations promulgated under section 527 of the Federal, Food, Drug and Cosmetic Act, for which payment shall be made as described in section 1842(o); and
"(vi) for periods prior to January 1, 2005 does not include blood clotting factors for individuals with hemophilia for which a biologics license application under subsection (a) of section 351 of the Public Health Service Act has been submitted on or before December 31, 2002 and for which payment shall be made as described in section 1842(o)."

(2) In determining whether a drug or biological is excluded from the Hospital Outpatient Prospective Payment System under section (a)(1) above, the fact that a drug or biological may have uses that have not been designated as an orphan drug under section 526 of the Federal Food, Drug and Cosmetic Act, shall not be considered, *provided however* that for any drug or biological that would otherwise be

covered by section (a) (1), if the annual number of claims submitted by hospital outpatient departments for such drug or biological administered to Medicare beneficiaries who are registered on hospital records as outpatients exceeds 30,000, the exclusion shall apply only to the indications for which the drug has been designated under section 526 of the Food, Drug and Cosmetic Act or which are included on the Rare Diseases List maintained by the Office of Rare Diseases of the National Institutes of Health.

(b) REPORT

Not later than July 1, 2004, the Secretary shall submit to the Committees on Ways and Means and Energy and Commerce of the House of Representatives and the Committee on Finance of the Senate a report on payment for orphan drugs and biologicals and blood clotting factors for individuals with hemophilia in the hospital outpatient setting including recommendations for either continuing to exclude these drugs and biologicals from payment under the Hospital Outpatient Prospective Payment System or including these drugs and biologicals under such payment system. Such report shall include--

(1) Methods for appropriately reflecting the actual costs of orphan drugs and biologicals and blood clotting factors for individuals with hemophilia under the Hospital Outpatient Prospective Payment System. Such methods shall be designed to ensure that the payment rate established for each drug and biological adequately reimburses hospitals for the costs associated with acquiring and dispensing such product, including pharmacy service and overhead costs.

(2) The impact of making payment for orphan drugs and biologicals and blood clotting factors for individuals with hemophilia under the Hospital Outpatient Prospective Payment System on access to these therapies by patients with rare diseases.

In preparing this report, the Secretary shall consult with patients, physicians, providers of services and suppliers of orphan drugs and biologicals and blood clotting factors for individuals with hemophilia as well as other organizations involved in the distribution of such drugs and biologicals to such patients, physicians, providers of services and suppliers.

(c) MORATORIUM ON DECREASES IN PAYMENT RATES.--

Notwithstanding any other provision of law, effective for orphan drugs and biologicals and blood clotting factors for individuals with hemophilia furnished by hospital outpatient departments on or after January 1, 2005, the Secretary may not directly or indirectly decrease the rates of reimbursement in effect on December 31, 2004 for such orphan drugs and biologicals and blood clotting factors for individuals with hemophilia any earlier than six months after the date that the Secretary has submitted to Congress the report required under section (b).

(d) EFFECTIVE DATE.--

The amendments made by subsection (a) shall apply with respect to items furnished on or after January 1, 2003.

Technical Summary:

Orphan drugs and biologicals are agents, which are used for diseases that affect 200,000 or less in the U.S. The passage of the Orphan Drug Act of 1983 has led to a heightened interest in the development of orphan products, and there are currently over 200 orphan drugs and biologicals in the U.S. market, some treating conditions with as few as 100 patients in the U.S. Under the Medicare Hospital Outpatient Prospective Payment System, Medicare currently pays for orphan drugs and biologicals that are eligible for coverage under Medicare and provided in hospital outpatient departments separate from the prospective payment for the administration of these agents. The pass-through payments were introduced to assure that Medicare beneficiaries with rare disorders had access to potentially life saving orphan drugs and biologicals while the Centers for Medicare and Medicaid Services gathered data to determine how these agents should be paid under the HOPPS.

Pass-through payments are set to expire on January 1, 2003 for orphan drugs and biologicals that have been paid on a pass-through basis since HOPPS commenced in August 2000. Unless the law is changed, under the 2003 HOPPS Final Rule, payments for some orphan drugs and biologicals will be folded into the prospective payment for the administration of these agents; for others, drastic cuts in payments will be made to levels well below product costs. If this occurs, there may be serious limitations to patient access to orphan drugs and biologicals in the hospital outpatient setting.

Fundamental concepts behind a prospective payment system are (1) payments should cover, **on average**, the costs of reasonably prudent providers, (2) reasonably accurate data are available to set prospective payment amounts, and (3) the payment system will not preclude patients from receiving needed medicines. These concepts do not apply to orphan drugs and biologicals. The number of patients who would be eligible to receive any particular orphan drug or biological in any individual hospital outpatient department would be *extremely* low and *highly* variable from institution to institution. Medicare estimated costs rely on individual hospital practices with respect to setting charges. Although those practices may reflect costs for all pharmaceutical products provided by an institution, **on average**, these data are not reliable when used to estimate costs for rarely used products, like orphan drugs and biologicals.

Due to this extreme variability, these problems are best remedied by removing orphan drugs and biologicals provided in hospital outpatient departments from the HOPPS payment system until such time as an appropriate HOPPS payment methodology is established for these products. The amendment requires the Secretary to submit a report to Congress describing methods for appropriately reflecting the costs of orphan drugs and biologicals and blood clotting factors with individuals with hemophilia under HOPPS. Such report must include recommendations either to continue to exclude these drugs and biologicals from HOPPS or to include these drugs and biologicals under HOPPS payment. In preparing the report, the Secretary is required to consult with all interested stakeholders, including patients, physicians, providers, suppliers and distributors of orphan drugs and biologicals and blood clotting factors for individuals with hemophilia. Following the two-year period for exclusion from HOPPS, the amendment prohibits the Secretary from decreasing payments for orphan drugs and biologicals and blood clotting factors for individuals with hemophilia until Congress has had at least six months to consider the Secretary's report on payment for these products.

Also covered under this amendment are blood-clotting factors for hemophilia patients. Blood clotting factors for individuals with hemophilia have been excluded from the inpatient prospective payment system for a number of years in recognition of the fact that patients with hemophilia incur substantially higher costs than patients without hemophilia for nearly all hospital encounters where the encounter requires administration of blood clotting factors. Although hemophilia is a rare disorder, some blood clotting factors have never been designated as orphan drugs and biologicals. This amendment puts these blood-clotting factors for patients with

hemophilia on parity with other orphan drugs and biologicals by also excluding these products from the HOPPS provided that a biologics license application for the product was submitted to FDA by December 31, 2002. Thereafter, manufacturers of blood clotting factors would be required to obtain orphan product designation for the exclusion to apply (unless the product is the same drug as one that has already been designated as an orphan drug).

Recognizing that some orphan-designated drugs may also be used for conditions that are not rare diseases, this amendment identifies two groups of orphan drugs and biologicals based upon the annual Medical hospital outpatient claims volume: (1) orphan drugs and biologicals with 30,000 or fewer claims ("low volume orphan drugs") and (2) orphan drugs and biologicals with more than 30,000 claims-per year ("high volume orphan drugs"). Low volume orphan drugs would be excluded from HOPPS completely. High volume orphan drugs would be excluded from HOPPS only for their rare disease uses. The 30,000 annual claims threshold was selected because it is at the midpoint of an apparent drop-off in claims volume among orphan drugs and biologicals from review of the 2001 HOPPS Public Use File claims data and because the next group of products below this threshold are used nearly exclusively for rare diseases. In the annual proposed rulemaking under HOPPS, CMS would identify high volume orphan drugs and the rare diseases for which these drugs would be excluded from HOPPS. Interested stakeholders would have the opportunity to review and comment upon the claims data supporting CMS's identification of high volume drugs and on the list of rare diseases for which these drugs would be excluded from HOPPS.

Rationale for Products Included Under NORD Amendment

The amendment includes three categories of drugs and biologicals for exclusion from HOPPS:

1. Orphan-designated drugs and biologicals
2. Non-orphan-designated drugs and biologicals that are the same drugs as orphan-designated drugs.
3. Blood clotting factors for individuals with hemophilia.

Although there may appear to be three discrete categories, in fact, there is substantial overlap among the three groups. There would be very little difference in budget impact if the amendment included only orphan-designated drugs and biologicals rather than all three categories.

1. Orphan-designated drugs and biologicals

These are the innovator products for patients with rare diseases. As innovator products, they are eligible for orphan designation and approval. The amendment tracks the Federal Food, Drug and Cosmetic Act and its delegation to the Food and Drug Administration for this determination. This is consistent with other Medicare references to orphan drugs and biologicals under HOPPS.

2. Non-orphan-designated drugs and biologicals that are the same drugs

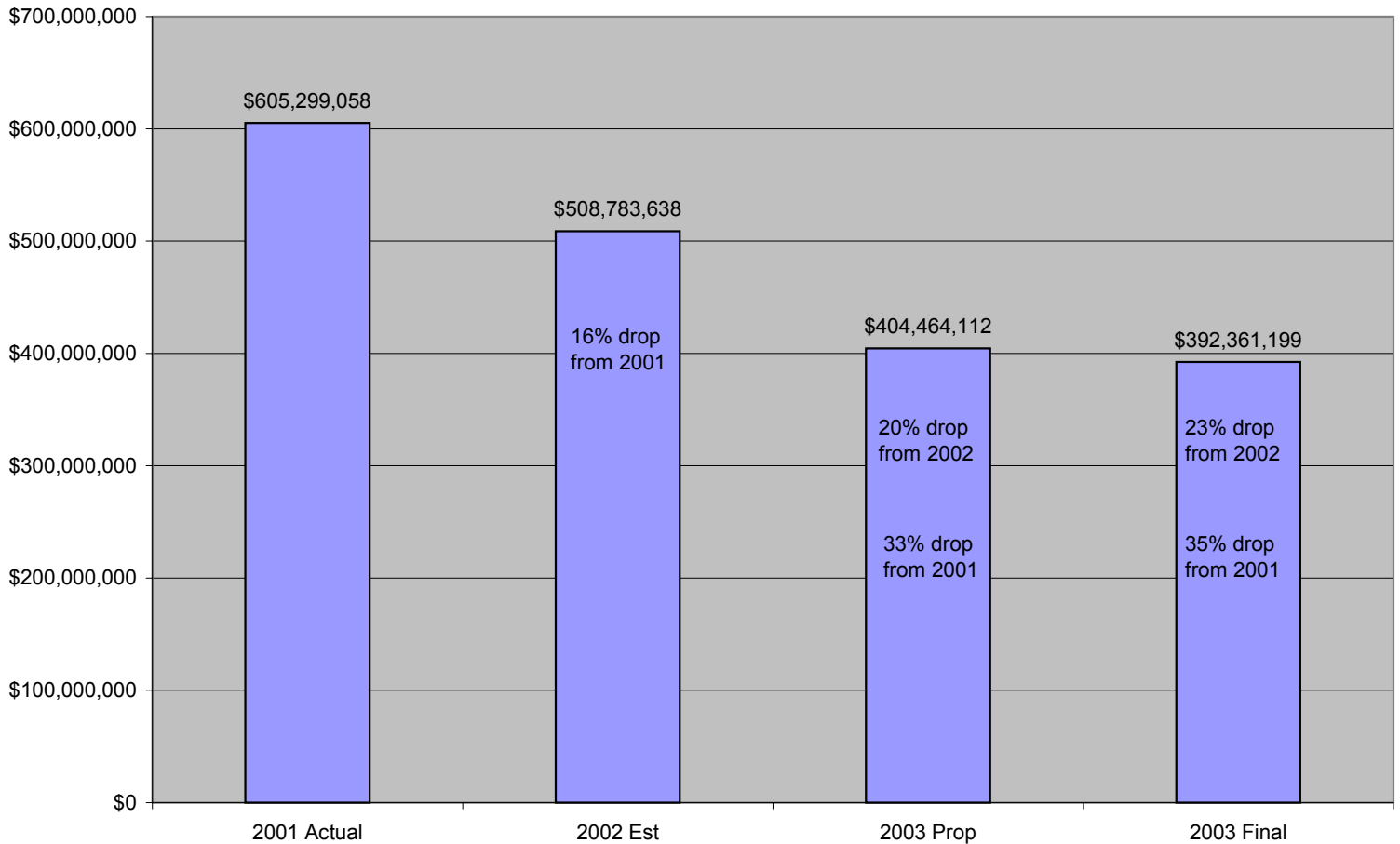
Once an innovator product has obtained approval as an orphan drug, no later follow-on product that is "the same drug" can obtain orphan designation and approval. Nevertheless, the follow-on drug may be the same active drug for the same population with a rare disease. Medicare generally uses the same product code to report the follow-on drug. The amendment appropriately includes these follow-on products to avoid what otherwise would be an unfair situation--hospitals would be eligible for payment only if they stock a particular brand of an orphan product. Removing this category from this amendment would have no budget impact because providers would simply shift to the specific brand eligible for payment.

3. Blood clotting factors for individuals with hemophilia

Nearly all of the blood clotting factors for individuals with hemophilia are orphan-designated drugs or are the "same drugs" as orphan designated drugs (i.e., follow-on brands of clotting factors that are orphan-designated drugs). We included this group separately, however, because we identified two clotting factor product types that have no orphan-designated products despite the fact that the agents are used in very small populations: Factor VIII anti-inhibitor (for patients with inhibitors to hemophilia clotting factors) and Factor IX complex (for patients with Hemophilia B). These agents are critically important for the few patients who require them, but they have very little impact on the budget for the amendment (2001 Medicare claims show these agents cost less than one-quarter of 1-percent [.0025] of total costs for the orphan drugs included under the amendment).

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Payment Impact for Orphan Drugs & Blood Clotting Factors



Prepared by McDermott Will & Emery. For this analysis, payments from 2001 were taken directly from the 2001 Public Use File (PUF) claims data files. Estimated payments for 2002 were computed using 2001 payments for the first quarter of 2002 and the pro rata reduced rates for the second through fourth quarters of 2002. 2003 Proposed and Final payments were taken from the Proposed and Final Rules, respectively. Only products with payments in 2001 are included. Costs from the PUF claims data were used for the 2003 Final payments for the 3 products excluded from HOPPS. Product utilization was held constant from the 2001 PUF claims data files.