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108TH CONGRESS
1ST SESSION

H. R. 2700

To amend title XVIII of the Social Security Act to revise the methodology by which payment for orphan drugs and biologicals is made under program prospective payment system for hospital outpatient department services under the Medicare Program.

IN THE HOUSE OF REPRESENTATIVES

JULY 10, 2003

Mr. COX (for himself, Mr. NORWOOD, Mr. ISSA, Mr. ENGEL, Mr. BOUCHER, Mr. BERMAN, and Mr. POMEROY) introduced the following bill; which was referred to the Committee on Energy and Commerce, and in addition to the Committee on Ways and Means, for a period to be subsequently determined by the Speaker, in each case for consideration of such provisions as fall within the jurisdiction of the committee concerned

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A BILL

To amend title XVIII of the Social Security Act to revise the methodology by which payment for orphan drugs and biologicals is made under program prospective payment system for hospital outpatient department services under the Medicare Program.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the ''Medicare Patient Access to Drugs for Rare Diseases Act of 2003''.

SEC. 2. FINDINGS AND PURPOSES.

(a) FINDINGS.—Congress makes the following findings:

(1) Rare diseases and disorders are those which affect small patient populations, defined as fewer than 200,000 individuals in the United States. Taken together, 25,000,000 Americans suffer from one of the 6,000 rare diseases and disorders.

(2) Because prescription drug manufacturers could not make a profit from marketing drugs for such small patient populations, very little ''rare disease'' research was conducted prior to 1983. Only 10 orphan drugs existed at that time.

(3) The Orphan Drug Act, signed into law in 1983, created financial incentives for the research, development, production and distribution of such orphan drugs.

(4) Since 1983, more than 240 new orphan drugs have been developed, approved, and marketed in the United States and more than 800 additional drugs are in the research pipeline.

(5) The tremendous success of the Orphan Drug Act cannot be taken for granted because—

(A) patient access to the more expensive orphan drugs is a continuing problem; and

(B) there is a need to stimulate more research for the millions of Americans and thousands of rare diseases for which there are not yet effective therapies.

(6) When Congress adopted the medicare hospital outpatient prospective payment system (HOPPS) in 1999, it defined orphan drugs based on the Federal Food, Drug and Cosmetic (FFD&C) Act and placed such orphan drugs in a category that provided sufficient reimbursement to assure continuing access for rare disease patients.

(7) Despite expressions of concern from Congress, the HOPPS regulation for 2003 does not continue this policy and, instead, uses a definition of orphan drugs that is not supported by the history of the Orphan Drug Act and forces most orphan drugs into categories in which they are reimbursed at levels significantly below hospital acquisition costs.

(8) Unless medicare provides adequate reimbursement for orphan drugs, hospitals are much less likely to have them available for beneficiaries with rare diseases, such as cervical dystonia, alpha–1 antitripsin deficiency, rare cancers, porphyria, sickle cell anemia, Tourette syndrome, cystic fibrosis, and amyotrophic lateral sclerosis (Lou Gehrig's disease).

(b) PURPOSE.—The purpose of this Act is to assure that medicare beneficiaries with rare diseases have continued access to orphan drugs in the hospital outpatient setting and that the FFD&C Act definition of rare diseases is used by the medicare program.

SEC. 3. PAYMENT FOR ORPHAN DRUGS AND BIOLOGICALS UNDER THE PROSPECTIVE PAYMENT SYSTEM FOR HOSPITAL OUTPATIENT DEPARTMENT SERVICES.

(a) PAYMENT FOR ORPHAN DRUGS AND BIOLOGICALS.—

(1) IN GENERAL.—Section 1833(t)(1)(B) of the Social Security Act (42 U.S.C. 1395l(t)(1)(B)) is amended—

(A) by striking the period at the end of clause (iv) and inserting a semi-colon; and

(B) by inserting at the end the following new clauses:

''(v) for periods before January 1, 2007, 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 Healthcare Procedure Coding System

product code (or product code under a successor coding system designated in regulations promulgated under section 1173(c)), 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; and

''(vi) for periods before January 1, 2007, 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.''.

(2) CONSIDERATIONS IN APPLYING EXEMPTION RULES.—

(A) IN GENERAL.—In determining whether a drug or biological is excluded from the prospective payment system under section 1833(t) of the Social Security Act (42 U.S.C. 1395l(t)) for hospital outpatient department services by reason of the amendment made by paragraph (1), the Secretary shall not take into account

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.

(B) EXCEPTION FOR HIGH VOLUME CLAIMS.—Notwithstanding subparagraph (A), for any drug or biological that would otherwise be covered by the amendment made by paragraph (1), if the number of claims submitted by hospitals for covered OPD services (as defined in section 1833(t)(1)(B) of such Act (42 U.S.C. 1395l(t)(1)(B)) without regard to clauses (v) and (vi) of such section) for such drug or biological administered exceeds 30,000 for the year from which claims are reviewed to determine payment rates for a given year, the exclusion under such amendments 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.

(C) TREATMENT FOR HIGH VOLUME CLAIMS.—In the case of a drug or biological

that, with respect to which the Secretary determines that more than 30,000 claims for the drug or biological has been submitted in a year for covered OPD services as described in subparagraph (B), that drug or biological shall be considered to exceed 30,000 claims for all succeeding years.

(3) PAYMENT METHODOLOGY.—In the case of a drug or biological covered by the amendment made by paragraph (1), payment for the drug or biological shall be made under section 1842(o)(1) of the Social Security Act (42 U.S.C. 1395u(o)(1)).

(4) EXEMPTION FROM INHERENT REASONABLENESS AUTHORITY.—Section 1842(b)(8)(A)(i)(I) of the Social Security Act (42 U.S.C. 1395u(b)(8)(A)(i)(I)) is amended by inserting after ''paid under section 1848'' the following: ''and other than drugs and biologicals and blood clotting factors for individuals with hemophilia excluded from the prospective payment system for covered OPD services under clauses (v) or (vi) of section 1833(t)(1)(B).''.

(b) REPORT.—Not later than July 1, 2006, the Secretary shall submit to the Committees on Ways and Means and Energy and Commerce of the House of Representa-

tives 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 or discontinuing the exclusion of such drugs and biologicals from payment under section 1833(t) of the Social Security Act (42 U.S.C. 1395l(t)). Such report shall include the following:

(1) Recommendations for methods to appropriately reflect the actual costs of orphan drugs and biologicals and blood clotting factors for individuals with hemophilia under such section. 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 such section 1833(t) on access to such drugs and biologicals 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, 2007, the Secretary may not directly or indirectly decrease the rates of reimbursement in effect on December 31, 2006 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, 2004.

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