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VIA FEDERAL EXPRESS

October 2, 2003

Mr. Thomas A. Scully, Administrator
Centers for Medicare and Medicaid Services
Room C5-14-03
7500 Security Boulevard
Baltimore, Maryland 21244-1850

RE: Medicare Program; Changes to the Hospital Outpatient Prospective Payment System and Calendar Year 2004 Payment Rates [CMS-1471-P]

Dear Mr. Scully:

These comments are submitted on behalf of the Alpha-1 Association and the Alpha-1 Foundation. The Alpha-1 Association is the patient advocacy and support organization representing the community of individuals affected by Alpha-1 Antitrypsin Deficiency. The Alpha-1 Foundation is dedicated to providing leadership and resources that will result in increased research, improved health, worldwide detection and a cure for Alpha-1. Alpha-1 is a devastating disorder, a pediatric liver disease that requires transplantation and an adult onset degenerative lung disease that leads to repeated infections and progressive loss of lung function. The median age of survival is 54. The most common signs and symptoms of Alpha-1 are recurring respiratory infections, shortness of breath or awareness of one's breathing, non-responsive asthma or year-round allergies, rapid deterioration of lung function without a history of significant smoking, decreased exercise tolerance, chronic liver problems, and elevated liver enzymes.

Because we fear the reduction of benefits will severely limit patient access to care, the Alpha-1 community requests that CMS carefully review the recommendations made in this document prior to issuing the final rule for 2004 OPPS before it's too late for individuals affected by Alpha-1 who require access to life saving therapy.

Aside from end-stage transplantation, currently only one treatment exists for the lung disease associated with Alpha-1. Alpha-1 proteinase inhibitor (Human) is indicated for chronic replacement therapy in individuals having congenial deficiency of Alpha₁-PI (Alpha-1 Antitrypsin Deficiency) with clinically demonstrable panacinar emphysema. Alpha-1 proteinase inhibitor arrests but does not reverse lung tissue damage however, for those patients eligible for therapy it is life-extending.

Many patients receive therapy in the hospital outpatient setting due to medical necessity and do not have other sites of service such as a physician's office available to them. CMS does not provide benefits for Alpha-1 patients in the home setting unless they are completely homebound. Maintaining access in the hospital outpatient setting remains vital for Alpha-1 patients.

1. Background on the Medicare Outpatient Prospective Payment System Payments for Alpha-1 Proteinase Inhibitor and the Problem Under the 2004 Proposed Rule

Under the Outpatient Prospective Payment System (OPPS), payments for Alpha-1 proteinase inhibitor¹ (Alpha-1) are determined prospectively based upon hospital reported charges for the biological. When OPPS was introduced in August 2000, payment for Alpha-1 was made on a pass-through basis at 95% of the national average wholesale price for the biological. This was sufficient to cover hospital costs for acquiring the biological and for storage, handling and pharmacy overhead. This payment rate of \$2.09-per 10 mg continued until March 31, 2002.

In April 2002, the payment rate for Alpha-1 dropped to approximately \$1.72-per 10 mg due to a pro rata reduction implemented by the Centers for Medicare and Medicaid Services (CMS) for all pass-through drugs and biologicals based upon CMS estimation that 2002 payments would exceed the permissible size of the pass-through payment pool set by law (then 2.5% of total OPPS payments). At this rate, hospitals were barely capturing their costs for the biological.

In 2003, CMS excluded payments for Alpha-1 from the OPPS and agreed to pay for this biological (plus 3 other orphan biologicals) under the so-called "reasonable cost" payment system². CMS explained: "We recognize that orphan drugs . . . are generally expensive and, by definition, are rarely used. We believe that if the cost of these drugs were packaged into the payment for an associated procedure or visit, the payment for the procedure might be insufficient to compensate a hospital for the typically high cost of this special type of drug." (57 Fed Reg

¹ Code J0256 " injection, alpha 1 - proteinase inhibitor - human, 10 mg."

² Payments for items and services under "reasonable cost" do not track the specific cost of a particular item or service covered under Medicare. Under the "reasonable cost" methodology, hospital costs are determined for any particular item or service by multiplying the billed charge by a hospital-and-department-specific ratio of costs-to-charges. Although the ratio of costs-to-charges is specific for a particular hospital and department, it reflects an **average** of the relationship between costs and charges for all items and services billed in that department. For example, if the charge for an orphan drug is \$500 and the ratio of costs-to-charges under pharmacy is 30%, then the "reasonable cost" for the orphan drug will be \$150--**regardless of the actual cost of the product**. If the actual cost of the product is \$400, the hospital will lose \$250.

The reasonable cost methodology works only when either (1) all items and services are paid under this methodology or (2) hospital charges for all items in a particular department reflect the same relationship to cost. Neither circumstance holds under the Medicare OPPS. Very few services are paid under reasonable cost anymore, so relying on the averaging relationship in the ratio of costs-to-charges will not work--ie, products with higher mark-ups will not provide a cushion for products with lower mark-ups. Hospitals have told us that they cannot set charges for all drugs and biologicals to reflect the same relationship to cost because it would require fairly large dollar mark-ups for higher costs products, like most orphan drugs. Therefore, hospital charges for most orphan drugs are set so that the charge multiplied by the ratio of costs-to-charges is actually less than costs. The "reasonable cost" payment for most orphan drugs will generally be well below the actual cost of the product.

66718, 66772 [November 1, 2002].) Payments for Alpha-1 under the reasonable cost system would vary by hospital and would not be known for certain until the annual cost reports for Fiscal Year 2003 are finalized (which will not likely occur for at least one year). We can estimate the average reasonable cost payments from review of the average costs for Alpha-1 reported in the OPSS Public Use File for the period April through December 2002 at \$1.78-per 10 mg³.

In the OPSS Proposed Rule for 2004, CMS has indicated that it will no longer pay for Alpha-1 outside the OPSS system, but instead, will assign a prospective rate of \$1.16-per 10 mg for the biological. CMS has based its proposal on an estimated median cost for the biological of \$1.25-per 10 mg. CMS explained its decision for no longer excluding Alpha-1 (and 10 other orphan drugs and biologicals) as follows: "The aggregate number of Medicare beneficiaries who will receive the 11 drugs that meet our criteria for orphans is significantly higher than the number who receive the 4 we identified last year. Furthermore, as we identify more drugs that meet our criteria, we expect the number of beneficiaries who receive these drugs to grow. As the number of beneficiaries who receive these drugs increases, so do total payments for the drugs. Therefore, we no longer believe that paying for these drugs at reasonable cost, outside of OPSS, is appropriate." (68 Fed Reg 47966, 48004 [August 12, 2003].) In other words, CMS proposed no longer to pay on a reasonable cost basis simply because it determined that this would involve too many beneficiaries in the aggregate.⁴ In same Proposed Rule, however, **CMS acknowledged its rationale for excluding Alpha-1 and other orphan biologicals from OPSS was to ensure access--not to control payments**: "Our intent in implementing these policies was to avoid adversely affecting beneficiary access to needed treatment." (Ibid, at 47996.)

In the Proposed Rule, CMS also justifies its decision for paying for Alpha-1 (and the other 11 identified orphan drugs and biologicals) on a prospective payment basis using 2002 claims data as follows: "In the case of orphan drugs, we believe that our claims data for April 1, 2002 through December 31, 2002 do reflect the cost of orphan drugs," (Ibid at 48004.) CMS makes this statement without any supporting justification and without acknowledging that the Agency has received substantial amounts of data showing conclusively that the claims-based estimated costs for many orphan drugs and biologicals are substantially below the invoice costs for these products.

Under the Proposed Rule rates, a hospital would lose approximately \$25,000-per year⁵, on average, for each beneficiary with Alpha-1 Antitrypsin Deficiency it treats. Hospitals cannot be expected to continue providing treatment facing losses of this magnitude. When the 2003 Proposed Rule was issued in August 2002 with similarly low rates (\$1.04-per 10 mg), we already

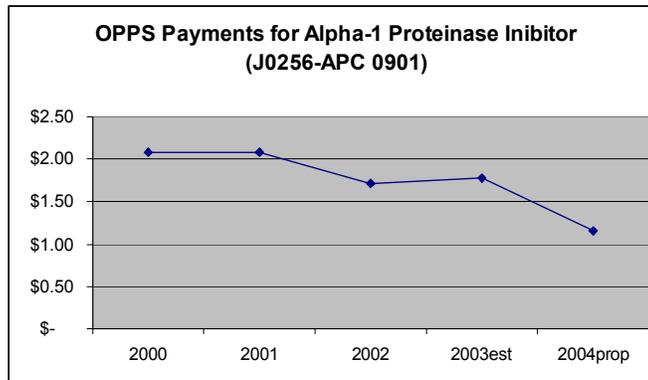
³ Although the payment, on average, would appear to be adequate to cover hospital costs, the median payment would be estimated at \$1.25-per 10 mg. This means that more than half of the hospitals providing alpha-1 would lose substantial amounts of money even under the "reasonable cost" payment system.

⁴ However, CMS did not provide any estimate of the total number of beneficiaries receiving the 11 identified drugs, nor did the Agency offer any estimate of the total payments that would be subject to reasonable cost if all 11 drugs and biologicals were excluded from OPSS.

⁵ Considering product actual acquisition cost at \$2.20-per 10 mg and an average dose of 5,000 mg-per week.

learned of beneficiaries who were turned away from treatment with Alpha-1 due to the shortfall between costs and reimbursements.

The 2004 Proposed Rule payment is 45-percent lower than the payment in effect as of March 2002.



2. The Problem with the OPSS Data

The payment rate in the Proposed Rule for 2004 is inadequate because the hospital data comprising claims for Alpha-1 and the methodology used to identify the cost of Alpha-1 are seriously flawed. Review of the Public Use File data covering the period April through December 2002 (the relevant period for the 2004 ratesetting), we determined that 27-percent of claims were miscoded regarding dose units, product codes, procedure codes or diagnosis codes. Therefore, a substantial portion of the database cannot be relied upon to provide a valid estimate of the cost of Alpha-1.

Even if the erroneous claims are ignored, however, there remains a serious methodological problem. The claims-based cost estimate assumes that hospital charges bear the same relationship to cost for all pharmacy products. Review of hospital chargemasters and discussions with hospital chargemaster experts confirms that this is not the case. In general, the charges are lower relative to costs for relatively high cost drugs and biologicals, such as Alpha-1, compared to much less costly therapies.

This is shown clearly in the case of Alpha-1. The CMS charge data show a mean median cost of \$1.25-per 10 mg. However, the Actual Acquisition Cost is \$2.20-per 10 mg⁶. This means that most hospitals did not set their charges high enough to reflect the cost of Alpha-1.⁷ This has occurred because hospitals were not required to report use of Alpha-1 under code J0256 (injection, Alpha 1 - proteinase inhibitor - human, 10 mg) prior to implementation of the OPSS in August 2000 and there is still much confusion about billing and the relationship between

⁶ First Databank.

⁷ The average cost-to-charge ratio for alpha-1 (code J0256) was \$0.28 determined from the Public Use File for the period April through December 2003.

charges, near-term payments (what is paid on the claim submitted), and use of the charge data to determine payments in the long-term (how charge data from claims are used to set payments two years in the future).

3. Recommended Solutions to Assure Alpha-1 Patient Access

The 2004 Proposed Rule payment rate for Alpha-1 is substantially below the cost to provide Alpha-1 proteinase inhibitor (human) to patients with Alpha-1 Antitrypsin Deficiency. Unless the Final Rule rates are substantially higher, we fear patients will be turned away from treatment. This therapy is life-sustaining, untreated patients may have their lives foreshortened or die.

CMS can take one of several steps to assure access:

1. **CMS should exclude Alpha-1 from OPPS.** CMS recognized last year that it was necessary to exclude Alpha-1 from OPPS to assure patient access. Very little has changed to suggest that this is no longer necessary. If Alpha-1 continues to be paid outside OPPS, **CMS should modify the reasonable cost instructions to assure that payments cover the actual costs of Alpha-1.**⁸
2. If CMS decides to include Alpha-1 among the prospective payments of OPPS, **CMS should substitute the erroneous claims-based median cost estimate of \$1.25-per 10 mg with the Actual Acquisition Cost of \$2.20-per 10mg or other external data showing the cost of Alpha-1.**
3. At a minimum, **CMS should apply the dampening rule it has proposed for all other blood products to Alpha-1 so that the 2004 payments are no lower than 90-percent of the average reasonable cost payments made in 2003.** The proposed rate of \$1.16-per 10 mg is nearly 35-percent lower than the estimated reasonable cost payments from 2003. Alpha-1 proteinase inhibitor is prepared from pooled human plasma of normal donors by modification and refinement of the cold ethanol method of Cohn fractionation.

Access to the hospital outpatient setting is of critical importance to the Alpha-1 community; to date the Alpha-1 Association has received copies of over 100 letters from individual patients

⁸ This could be accomplished by instructing hospitals to set charges for alpha-1 at the true acquisition cost (invoice cost plus appropriate adjustment for handling, storage, overhead) and assigning claims reporting code J0256 to a specific cost center with a cost-to-charge ratio reflect a unitary relationship between costs and charges.

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who have responded to the CMS proposed rule. We hope that you will consider our recommendations and completely prevent the devastating impact the proposed rates will have on our community.

Sincerely,

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