

J7513 Daclizumab parenteral, 25 mg; J9015 Aldesleukin, per vial; J9160 Denileukin diftitox, 300 mcg; J9216 Interferon, gamma 1-b, 3 million units; and Q2019 Injection, basiliximab, 20 mg.

We solicited comments on these proposals and requested that commenters submit information meeting the same criteria as comments for other drugs (as discussed above). We received numerous comments, all of which were in opposition to our proposals regarding payment for orphan drugs.

*Comment:* Every commenter who commented on the changes we proposed regarding payments for single indication orphan drugs opposed our proposal to discontinue payment for orphan drugs on a reasonable cost basis and to instead use the same methodology to set payment amounts for the single indication orphan drugs that we use to set rates for other drugs. Commenters stated that doing so would create serious access problems for patients who rely on an orphan drug for treatment of a rare disease because hospitals would no longer be able to afford to treat them. A number of commenters were particularly concerned by the decreased payment rate proposed for alpha-1-proteinase inhibitor. Some pointed out that the data we used to calculate payments for orphan drugs are especially flawed because of the low volume, high cost characteristics of orphan drugs, complicated by errors in the way hospitals bill for drugs generally. Recommendations from commenters included: applying the dampening rule to limit decreases to 10% of reasonable cost payments in 2003; establishing a payment floor; and, continuing to pay for orphan drugs on a reasonable cost basis.

*Response:* We carefully reviewed commenters' concerns about the impact our proposal would have on patient access to orphan drugs. We do not dispute that orphan drugs used solely to treat an orphan condition are generally expensive and, by definition, are rarely used. We also recognize that coding changes may have resulted in questionable billing data. However, we believe that it is important to balance these concerns with maintaining a consistent payment system for hospital outpatient department services overall, and to limit to the maximum possible extent payment for services or items outside the OPPS. We also discussed in the August 12 proposed rule our concerns about the increased number of drugs that meet our criteria for special payment status as single indication

orphan drugs and the resulting increase in the number of hospital outpatient services that would be paid outside the OPPS were we to continue to pay for these drugs on a reasonable cost basis. It was in light of these factors that we proposed to discontinue payment for single indication orphan drugs on a reasonable cost basis outside the OPPS and to use our claims data as the basis for setting payment rates for those drugs that we have identified as meeting our criteria for special payment status as single indication orphan drugs. We also proposed to pay separately for the single indication orphan drugs and to assign each of them to an APC.

Having weighed the concerns raised by commenters and our concerns about the increasing number of outpatient services that would be paid outside the OPPS were we to continue the current policy of paying for single indication orphan drugs on a reasonable cost basis, we have decided that beneficiaries, hospitals, and the Medicare program will be best served over the long term by our making payment for the single indication orphan drugs under the OPPS at 88 percent of the AWP. We arrived at 88 percent based on our analysis of claims data, and our intent that payment be sufficient to ensure that all beneficiaries have access to needed drugs. Among the 11 orphan drugs, the highest median cost in the claims data was approximately 78 percent of the AWP. After considering comments we received on the proposed rule, we were concerned that merely adopting the existing highest percentage of the AWP may not ensure that a sufficient payment amount is established in all cases prospectively. We therefore have provided for an additional margin of ten percentage points to account for possible future increases, and ensure sufficient payment. This results in the percentage of 88 percent that we have adopted in this final rule.

However, we received information consistent with our request for verifiable data (68 FR 47998) that indicates the payment amounts we proposed for alpha-1 proteinase inhibitor, for imiglucerase, and for alglucerase do not reflect the price at which these drugs are widely available to the hospital market. This information, combined with the concerns expressed by commenters generally that the payment amounts we proposed for the 11 drugs that meet our criteria for special payment as single indication orphan drugs are too low and may threaten beneficiary access to the drugs, have persuaded us to make final one modification to the method we proposed for setting payment rates for drugs that are paid as single indication

orphan drugs under the OPPS. That is, rather than using claims data to calculate payment rates for single indication orphan drugs that meet our criteria for special payment under the OPPS, we are setting payment for all but two of these drugs at 88 percent of their AWP as established in the April 1, 2003 single drug pricer (SDP). As discussed above, we received information about the widely available market price for imiglucerase and alglucerase, and, based on that information, we have priced these two drugs at 94 percent of their AWP.

We believe that this policy is a reasonable compromise. It enables us to set a prospective payment amount under the OPPS for qualified single indication orphan drugs. But, by increasing payment levels for these low volume drugs, we minimize the risk of compromising beneficiary access to treatment for life-threatening, rare diseases.

Therefore, we have set payment rates for single indication orphan drugs in accordance with the following policy, effective January 1, 2004:

- We are using the same criteria that we implemented in CY 2003 to identify single indication orphan drugs used solely for an orphan condition for special payment under the OPPS;
- We are discontinuing payment on a reasonable cost basis for single indication orphan drugs furnished in the outpatient department of hospital that is subject to the OPPS;
- We are making separate payment for single indication orphan drugs and assigning them to APCs;
- We are setting payment under the 2004 OPPS for single indication orphan drugs at 88 percent of the AWP listed for these drugs in the April 1, 2003 single drug pricer unless we are presented with verifiable information that shows that our payment rate does not reflect the price that is widely available to the hospital market.

*Comment:* Several commenters objected to our special treatment for only 11 orphan drugs, rather than including all of the drugs that the FDA designates as having orphan status. A few commenters recommended that we set the criteria for special treatment based on claims volume instead of our current criteria. That is, CMS would set a criterion for "high volume" drugs based on a threshold of 30,000 or more claims per year. Then, any FDA-designated orphan drug with less than the threshold volume of claims would be subject to special payment under the OPPS as an orphan drug.

*Response:* Using the statutory authority at section 1833(t)(1)(B)(i) of