HIGH-DOSE INTRAVENOUS METHYPREDNISOLONE FOR CONSTITUTIONAL PURE RED CELL APLASIA

To the Editor:

I read with interest the recent article by Lenarsky et al1 about bone marrow transplantation (BMT) for Diamond-Blackfan syndrome.

I reported a case of constitutional pure red cell aplasia (CPRCA) refractory to conventional prednisone (2 mg/kg) administration treated with high-dose intravenous (IV) methylprednisolone (HIVMP) (30 mg/kg for 3 days, 20 mg/kg for 4 days, then subsequently 10, 5, and 2 mg/kg for a week each, followed by 1 mg/kg until hemoglobin level reached 12 g/dL; each dose was given for 2 to 5 minutes).2 Eight other patients with Diamond-Blackfan syndrome resistant to conventional prednisone administration have also been treated with HIVMP successfully.3,4 Three of the infants died of causes not related to their disease (one died with a viral infection when he had been off treatment >2 months, and another died of pneumonia when his treatment was discontinued by the parents at home; the third infant died of pneumonia in the hospital); others are hematologically normal and do not require transfusion for >128 months (mean 21 months/patient). A young adult with CPRCA has been successfully treated with our regimen in the United States (J.R. Humbert, personal communication); in addition, two children have been treated in Switzerland (W.H. Hitzig, personal communication).

We have also used this regimen successfully in the treatment of several other hematologic disorders.5 With the exception of cushingoid appearance, the side effects of corticosteroids, such as hypertension, hyperglycemia, glycosuria, and corneal opacities, have not been observed in our patients; therefore, we believe that BMT in patients with CPRCA should be saved for cases unresponsive to the relatively cheap and easy treatment with HIVMP, which does not require a compatible donor and could be performed anywhere.

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REFERENCES

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High-dose intravenous methylprednisolone for constitutional pure red cell aplasia [letter]

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