Oral Megadose Methylprednisolone for Diamond-Blackfan Anemia

To the Editor:

I have read the report of Olivieri et al1 in the May 1, 1994 issue of BLOOD. Although the investigators stated that "in standard practice, treatment options are limited to two: steroid therapy or RBC transfusions,"1 they ignore our very good results obtained with megadose methylprednisolone (MDMP; initial dose 30 to 100 mg/kg/d for 3 days followed progressive halving of the dose every 4 to 7 days, with continuation of 1 mg/kg/d until hemoglobin reaches 120 g/dL, each dose administered before 9 AM, in 10 minutes intravenously (previously) or orally recently),2,4 which was also not mentioned in the recent clinical annotation by Freedman.5 When refractoriness or resistance to one drug is observed, it would be logical to increase the dose as we have done.

I emphasize that all of our 12 children with congenital hypoplastic anemia since 1984 have, as reported recently by Hasan and Inoue,6 responded to MDMP with a mean of 18 (range 8 to 41) days and become transfusion independent. The treatment could be discontinued in seven of the children (2 to 9 years without further recurrences). Two of the 12 children died from causes unrelated to the treatment (gastroenteritis and pneumonia after discontinuation of MDMP) and tapering of the dose, which will take several months to be discontinued, has been started in three children. With the exception of cushingoid appearance, side effects of MDMP have not been observed, as previously stated.2,4,8

Marked elevation of granulocyte-macrophage colony-stimulating factor (GM-CSF) and erythropoietin (EPO) has been shown with MDMP administration in patients with acute leukemia and idiopathic thrombocytopenic purpura, which could be the explanation for its effect on DBA.8,9

Several unsuccessful therapeutic approaches in DBA, such as those of Olivieri et al, have been used, but MDMP has not been tried in a large series outside our experiences.

Because of its effectiveness in all patients, economy, and convenient administration (orally) MDMP should be used internationally to decide whether it is the treatment of choice. I am concerned about all transfusion-dependent patients with DB syndrome maintaining their lives.

I have observed that some references are less equal than others, as pointed out by Wiktor-Jedrzejczak.10 I wonder whether it could be related to references coming from underdeveloped countries? Although I work in Turkey, I am one of Dr Diamond’s former fellows (between 1961 and 1963) and I am very proud of my accomplishment in the treatment of a syndrome named for him.

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REFERENCES


9. Şayli TR, Özoğlu Ş: Serum erythropoietin and granulocyte-macrophage colony stimulating factor levels with megadose methylprednisolone treatment. (submitted)

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