Hydroxyurea Treatment in Beta-Thalassaemia Intermedia

KW Chik, V Lee, MMK Shing, CK Li

Abstract

Hydroxyurea is a cytotoxic, which is found to be useful in increasing the haemoglobin level of thalassaemia subjects, though the mechanism of actions is still unclear. Two patients with thalassaemia intermedia received hydroxyurea treatment were presented. Treatment with hydroxyurea increases the haemoglobin level by 1-2 g/dl in a sustained manner. There was improvement in patients' general condition without causing any significant side effect.

Key words

Hydroxyurea; Thalassaemia

Introduction

β-thalassaemia intermedia is due to decreased production of the β-subunit of adult haemoglobin, resulting in α/β globin imbalance. Induction of γ-globin production by hydroxyurea has been reported to be useful in the management of thalassaemia by reducing the degree of imbalance. The elevated fetal haemoglobin (HbF) will in turn ameliorating the degree of ineffective erythropoiesis and lessening the extent of erythroid hyperplasia. Hydroxyurea, an S-phase specific cytotoxic, has been used extensively to manage myeloproliferative conditions with satisfactory toxicity profile. Induction of HbF synthesis could be due to the rapid regeneration of erythroid precursors following a cyto-reduction phase.

Case Report

A four years old girl with β-thalassaemia intermedia initially presented with two years history of paleness, signs of thalassaemic facies and hepatosplenomegaly. The haemoglobin at diagnosis was 5.7 g/dl, with typical microcytosis and hypochromia. Haemoglobinopathy study revealed marked increase in HbF of 85%, and normal HbA2 level. Both of her parents were β-thalassaemia carriers. Blood transfusion was necessary. Splenectomy was subsequently performed at five years old. Her haemoglobin was maintained around 7-8 g/dl independently. Hydroxyurea (~10 mg/kg/day) was started because of the low haemoglobin level at six years old, now more than three years. Increase in haemoglobin by around 1 g/dl was noted without any need for further blood transfusion. The rise in haemoglobin was noted two months after hydroxyurea treatment.

The second subject was a boy diagnosed to have β-thalassaemia intermedia at age of one. His HbF was 79%, and HbA2 was normal. During the initial follow up, his haemoglobin was maintained satisfactorily around 9-10 g/dl. His haemoglobin dropped below 8 g/dl gradually as he grew up. Splenectomy was performed at age of eight. However, the rise in haemoglobin was minimal post-operatively. Hydroxyurea (~10 mg/kg/day) was started at age of 10, now more than a year. Thereafter his haemoglobin was increased by around 2 g/dl. The rise in haemoglobin...