ID: 122
Area Clinica
Poster
Parole chiave: NTDT, HU, Thalidomide, anemia

THE LONG-TERM AND EXTENSIVE EFFICACY OF LOW DOSE OF THALIDOMIDE IN A CASE OF UNTRANSFUSABLE PATIENT WITH NON TRANSFUSION-DEPENDENT THALASSAEMIA: THE FOLLOW UP DATA AND THE RECHALLENGE TO HYDROXYUREA

Paolo Ricchi, Silvia Costantini, Anna Spasiano, Massimiliano Ammirabile, Patrizia Cinque, Aldo Filosa
UOSD Malattie Rare Del Globulo Rosso, Italia; pabloricchi@libero.it

Introduction: We recently reported the case of an untransfusable patient with Non Transfusion Dependent Thalassaemia (NTDT) that, after the failure of hydroxyurea (HU) treatment, had the improvement in hemoglobin level and in a part of NTDT related complications, following the use of thalidomide as off label therapy. Here we report the follow up data related to the interruption of thalidomide treatment and to the re-challenging to HU.

CASE: LMC is a 50 years old female patient attending to our Thalassaemia Unit since she was 4 years old. At age 3-years-old she had been diagnosed with homozygous β-thalassaemia for the β0 39 nonsense C→T mutation and alpha-thalassemia coinheritance with a baseline haemoglobin almost completely (95%) represented by HbF. She was treated for 40 months with thalidomide 50 mg daily and the prolonged gain in Hb level (from 4.8 to 9.0 gr/dl) obtained was also associated with major improvement in exercise tolerance and in quality of life. At the end of January 2016 the patient had reached a further increase in hemoglobin level to 10.7 g/dL, but because of the worsening of neuropathy (grade 3) at sural nerve and the occurrence of atrial flutter, we decided to discontinue thalidomide treatment. In March 2016, the patient was restarted on HU 500 mg daily and thereafter 1000mg /daily. Her Hb level go down to 8.0 g/dL within the first month, reaching a hemoglobin nadir of 6.8 g/dL in May. Thereafter, in June her Hb reached 7.5 g/dL and currently she maintains a mean haemoglobin of 8.0 gr/dL without signs of mielosuppression. A normalization in the increase in eosinophil count previously described during thalidomide treatment (from 9 to 1%) and a significative increase in mean corpuscular volume (from 75 to 105 fL) weres observed. The previous thalidomide-induced correction of several long-term effects of chronic anemia persisted.

Conclusions: Despite the mechanism of action of thalidomide remain unclear, these preliminary follow up data may suggest that hematological response to HU was restored following thalidomide treatment and interruption, likely reflecting a persistent amelioration in ineffective erythropoiesis. Waiting for the results on activin antagonist based therapies, these data may suggest that thalidomide and HU could be valuable in the management of untransfusable patient with NTDT and an alternate use of both drugs, to reduce toxicity and further increase effectiveness, is hypothesizable .