LONG-TERM SAFETY AND EFFECTIVENESS OF DEFERIPRONE IN A POPULATION AT LARGE

Felisi M., 3° anno di corso del Dottorato in Farmacologia Clinica e Terapia Medica, XV Ciclo. Durata del Dottorato in anni: 3.Sede di servizio: Università degli Studi di Bari.

In 1997 the Italian Ministry of Health included deferiprone (L1), the oral iron-chelator for thalassemia major, in the list of unauthorized medicinal products to be used under controlled conditions. As a consequence, the Italian Register for the Controlled Use of L1 was instituted to collect and evaluate deferiprone safety and effectiveness in the long-term use within a non-experimental population.
86 Centers for Thalassemia Treatment joined the Register enrolling 532 patients in the period July 1997-July 2000. 187 patients (32%) experienced a total of 269 events owing to temporary or definitive treatment interruption. Incidence of agranulocytosis (neutrophils<$0.5x10^9$/l) and neutropenia (neutrophils<$1.5x10^9$/l) were 0.4/100 and 2.1/100 patient-years respectively. Neutropenia occurred predominantly in non-splenectomized patients and in subjects with less of 18 years.
ALT increase, gastroenteric problems and arthralgia were the other major recorded events.
Ferritin levels showed a significant decrease in time after 3 years of therapy; the decrease was significant just after 1 year therapy for patients with baseline levels greater than 4000 µg/L.
Our results support deferiprone effectiveness and its safety when used under controlled conditions. Weekly haematological assessment and active event surveillance revealed necessary to detect early neutropenias and to prevent agranulocytosis, whose incidence rates resulted lower than previously reported.