Constraints and challenges in treating a young haemophiliac with exceptionally high inhibitor titers.

Authors:M. Economou[1], A. Papagianni[1], V. Gourtsa[1], A. Telli[1], P Dragoumi[1], E. Platokouki[2], N. Gombakis[1]

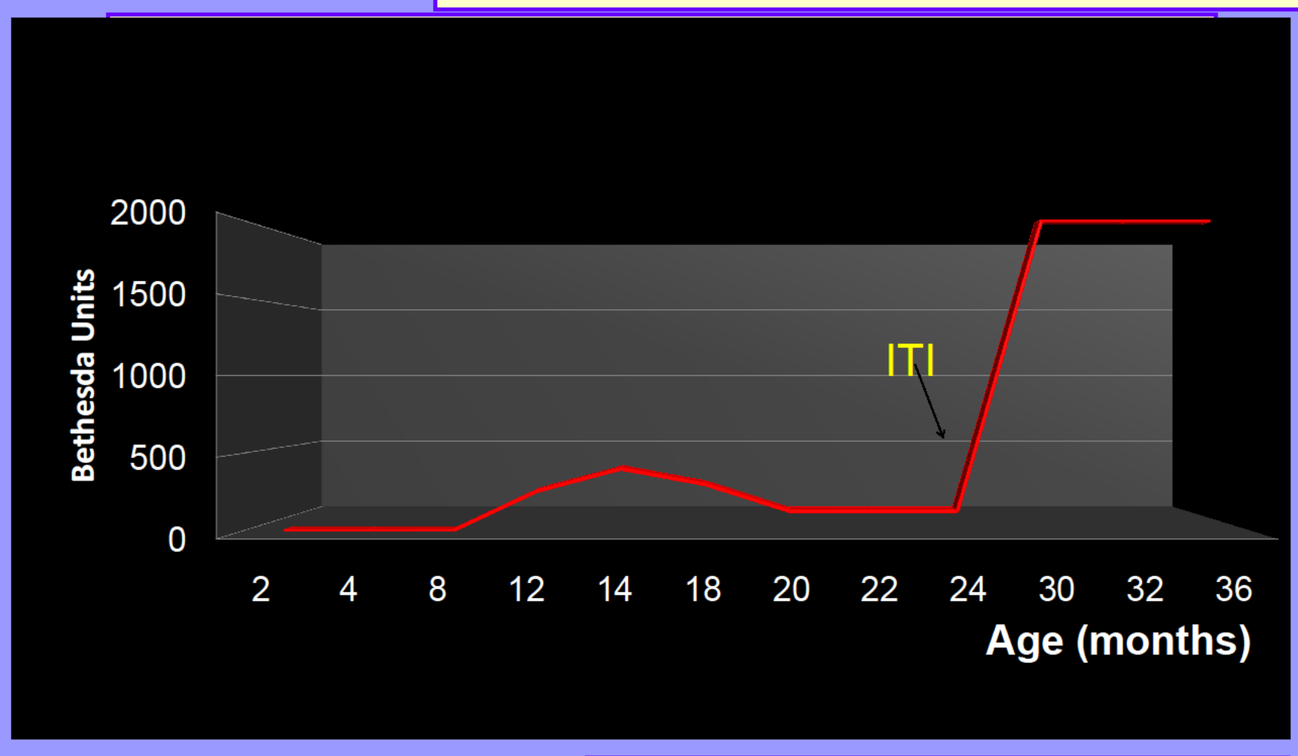
Hospital: 1. 1rst Pediatric Department,, Ippokration General Hospital, Aristotle University of Thessaloniki, Greece
2. Haemophilia Centre/Haemostasis Unit, Aghia Sophia Childrens' Hospital, Athens, Greece

Objectives:

To report the case of a 3.5 year old hemophiliac presenting with persistent and exceptionally high inhibitor titers unresponsive to conventional ITI, and to highlight difficulties in treating such patients.

Case Report:

A male patient, born to a healthy primiparous mother at 38 weeks gestation via normal delivery, was diagnosed with severe fVIII deficiency (1%) soon after birth. Diagnosis was made following soft tissue haematomas' formation at venepuncture sites and patient was promptly treated with recombinant factor VIII. During the first months of life he presented with repeated bleeding episodes requiring substitution therapy and by the age of 8 months inhibitor presence was established (1 B.U.). At 3 months follow up (11 months old) the titer was raised up to 256 B.U. DNA analysis revealed mutation INV22(+), known to be associated with a high risk for inhibitor development.



Management issues:

Bleeding episodes were initially managed with recombinant activated factor VII, which subsequently became unavailable due to hospital economic reasons. ITI was planned, but due to poor venous access and difficulty in maintaining a central venous line (the child pulled off his Hickman catheter twice) was not started until the age of 2. Because of ITI being performed by peripheral veins, a conventional non aggressive regiment was decided (80 IU/kg x 3/week). During 12 months of ITI, the inhibitor titer rose from 128 B.U./ml to 2000 B.U./ml and ITI was discontinued as unsuccessful. The child continues to bleed frequently, with minimal access to factor VII. Additionally, the very young age and the non presence of central venous line make aggressive ITI protocols that include immunosuppresants difficult to decide.

Conclusions:

The case highlights the difficulties in treating young patients with inhibitors, touching on both medical and economic issues and showing how these can affect patients' quality of life .

References:

1 **The Role of Immunomodulation in the Management of Factor VIII Inhibitors,** *David Lillicrap, Hematology 2006*

2.. **How we treat a hemophilia A patient with a factor VIII inhibitor,** *Christine L. Kempton and Gilbert C. White II, BLOOD, 1 JANUARY 2009*

DOI: 10.3252/pso.eu.WFH2012.2012