Costs and Outcomes of Patients With Haemophilia a (HA) and Factor VIII Inhibitors Treatment: The Immune Tolerance and Economics Retrospective Registry (ITER) Results

A.Gringeri¹, L.Scalone², P.A.Cortesi², A.Rocino³, L.G.Mantovani⁴

1 University of Milan - IRCCS Cà Granda Maggiore Policlinic Hospital, Milano, Italy
2 University of Milano-Bicocca, Monza (MB), Italy
3 Ospedale S. Giovanni Bosco, Napoli, Italy
4 Federico II University of Naples, Naples, Italy

Objectives
Immune tolerance induction (ITI) is generally accepted as first choice treatment to eradicate inhibitors in hemophilia A patients. Little is known about the outcomes and cost consequences of this treatment option.

Methods
The Immune Tolerance and Economics Retrospective (ITER) study is an observational, retrospective, multicentre, multinational study aiming to estimate cost of treatment in hemophilia A patients, undergoing ITI. Data on hemostatic treatment given in the following time periods were collected: up to 12 months before the diagnosis of Inhibitors, between Inhibitors diagnosis and ITI start, during ITI, and 12 months after the end of ITI. Costs of treatment were calculated in the perspective of the third party payer and expressed as mean €/patient-month.

Results
Seventy-one valid patients, with median age at ITI start=3.8 (0.4-41) years, were enrolled. Before ITI the median Inhibitors peak titre was 18.5 (0.80-704) BU. ITI was applied for a mean of 1.85 (0.1-14.0) years and was successful in 84.5% pts. Before Inhibitors diagnosis, patients cost was 670.2 €/patient-month. Cost was 3,188€/patient-month between the Inhibitors diagnosis and ITI start (92.1% for bypassing agents), and 60,078€ during ITI (76.8% for ITI, 19.4% for extra FVIII treatment, 3.8% for extra treatment with bypassing agents). The mean cost after ITI was 13,211€/patient-month.

Conclusions
ITI applied on patients with the characteristics of those involved in the ITER study is successful in 84% of them at a mean cost of 60,000€/patient-month during ITI, plus 13,000€/patient-month through 1 year later. Further research is encouraged to value long term benefits and costs attributable to ITI versus other treatment options, in order to identify the most efficient treatment for the patients and for the health care system.