

## **Intravenous Immunoglobulin versus Observation in Pediatric Immune Thrombocytopenia: First Results of a Multicenter Randomized Trial**

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**Background:** Management in children with newly diagnosed immune thrombocytopenia (ITP) consists of careful observation or treatment with corticosteroids or intravenous immunoglobulin (IVIg). In this multicenter randomized trial we studied efficacy and safety of careful observation versus IVIg as well as development of chronic disease.

**Patients and methods:** Children aged 3 months-16 years with newly diagnosed ITP, platelet counts  $\leq 20 \times 10^9/L$  and mild to moderate bleeding were eligible. Within 72 hours after diagnosis patients were randomized to receive either a single infusion of 0.8 g/kg IVIg or careful observation. Clinical data were collected and laboratory studies were performed at diagnosis and during one year of follow-up.

**Results:** Two hundred patients (109 males, 91 females) were randomized in 48 hospitals; 100 received IVIg and 100 received careful observation. Median age was 3.84 years (range 0-16 years). Median duration of symptoms prior to diagnosis was 3 days and median platelet count at diagnosis  $6 \times 10^9/L$ . One hundred fifteen children (57.5%) had skin bleeding only and 80 (40.0%) had mucosal bleeding as well. No statistical significant differences regarding baseline characteristics were found between the IVIg and observation group. Complete response to IVIg (platelet count  $\geq 100 \times 10^9/L$ ) was seen in 70.5% after one week and in 64.9% after one month. In the observation group, complete recovery was seen in 21.3% after one week and in 41.8% after one month. Twenty-nine severe adverse events were reported, 21 in the observation group and 8 in the IVIg group. All of these were transient. Preliminary data regarding long-term results have been presented at the meeting. Final results have to be awaited and are expected next year.

**Conclusion:** Although response rate is higher in patients that received IVIg, careful observation is a safe alternative in children with newly diagnosed ITP with spontaneous complete recovery within 1 month in 41.8%.