



No. 5 – Non-interventionist” Approach to Childhood

An American Perspective reprinted from: Dec 99 (Reviewed and revised in 2012)

Title: **Increased Respectability of the “Non-interventionist” Approach to Childhood ITP**

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This column describes the continuing controversy about the management of children with a new diagnosis of ITP. We previously described the survey conducted in the United States in 1997 which indicated that the majority of pediatric hematology-oncology practitioners prescribe drug therapy to children newly-diagnosed with ITP whether they have significant hemorrhage or not. IVIG is the preferred treatment although corticosteroids and anti-D are also frequently employed. A published set of practice guidelines from the United States in 1996 also favored drug therapy for children with ITP with platelet counts less than 20,000 per mm³. Clearly the perceived idea of intracranial hemorrhage prompts many physicians to do everything possible to immediately raise the platelet count, despite the toxicities and costs of such treatment and even though there are no scientific data showing that initial drug therapy prevents life-threatening hemorrhage. Those practitioners favoring a “watchful waiting” or “non-interventionist” approach without use of drug therapy, seemed to represent a diminishing minority.

But...perhaps the pendulum has begun to swing in the other direction, thanks to two recent contributions by eminent British pediatric hematologists, whom readers of this column will immediately identify. First in the July 1999 issue of the *British Journal of Haematology*, Professor John Lilleyman wrote an eloquent commentary regarding diagnosis and management of ITP in which he critically reviewed existing publications and concluded that there is no firm evidence to support exposing children to potentially toxic and costly remedies which are aimed at transiently raising the platelet count. His commentary is, in our view, required reading for all practitioners who deal with ITP.

Then more support was provided by Dr. Paula Bolton-Maggs on September 12, 1999 at the annual meeting of the American Society of Pediatric Hematology/Oncology (ASPH/O) in Montreal, Quebec in a presentation entitled “The Management of Childhood ITP – Controversy Revisited”. In a convincing manner (it is clear that she and Professor Lilleyman have worked with one another!) she reviewed the management of childhood ITP, focusing on the results of her 1997 *Lancet* paper which described a UK audit of childhood ITP cases. She pointed out that British practice guidelines on ITP published in 1992 were infrequently followed by practising physicians. Thus, just because the experts recommend something doesn’t mean that these recommendations are followed in practice. Practitioners recognize that recommendations based only on opinion, without firm evidence, may not be correct. Then Dr. Bolton-Maggs described her own management of children with ITP, which rarely included drug therapy. She reminded the audience that there has been too much emphasis on the platelet count, rather than observations on actual bleeding, and too little emphasis on the expense, inconvenience, and toxicities of drug therapies. At the end of her presentation, she had much of the highly attentive audience convinced that a “watchful waiting” approach to ITP is acceptable.

Another important presentation to the ASPH/O meeting attendees was by Dr. R. Dickerhoff from St. Augustin, Germany. She recounted her experience over the past decade with 52 consecutive children with newly diagnosed ITP who were untreated (except for a 3-day course of prednisone in 5 cases). What were her results? In six weeks more than half (30 of the 52) of the children had recovered with a normal platelet count, and 47 of them had normal platelet counts within six months. Only two children were still thrombocytopenic at the end of one year. There were no intracranial or other life-threatening hemorrhages. One physician asked “But what would have happened if an untreated child with ITP were in an automobile accident?” Our response would be, “Thank goodness automobile accidents are rare,

and when they do occur at high speed, tragedy often results whether the child has a normal platelet count or not. Seat belts are probably more important than the platelet count.”

Drs. Dickerhoff, Bolton Maggs, and Lilleyman all emphasized that the term “non-treatment” is really a misnomer. All doctors carefully evaluate the child with ITP, counsel the parents regarding risk of hemorrhage, prescribe limitation of activities during the period of acute bleeding, and urge that aspirin and aspirin-containing drugs be avoided. In our view, non-interventionist physicians spend more time with the parents of children with ITP than do those whose solution to the disease is simply to order a dose of IVIG or anti-D.