

## Indian Academy of Pediatrics (IAP)



# nRICH

**N**ewer **R**esearch and recommendations **I**n **C**hild **H**ealth

Lead Author

*Ritika Khurana*

Co-Author

*Sangeeta Mudaliar*



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**Dear fellow IAPans,**

**nRICH**

**Newer Research and recommendations In Child Health**-aims to bring you the abstracts of some of the breakthrough developments in pediatrics, carefully selected from reputed journals published worldwide.

Expert commentaries will evaluate the importance and relevance of the article and discuss its application in Indian settings. nRICH will cover all the different subspecialties of pediatrics from neonatology, gastroenterology, hematology, adolescent medicine, allergy and immunology, to urology, neurology, vaccinology etc. Each issue will begin with a concise abstract and will represent the main points and ideas found in the originals. It will then be followed by the thoughtful and erudite commentary of Indian experts from various subspecialties who will give an insight on way to read and analyze these articles.

I'm sure students, practitioners and all those interested in knowing about the latest research and recommendations in child health will be immensely benefitted by this endeavor which will be published online on every Monday.

Happy reading!

*Upendra Kinjawadekar*  
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# Use of Eltrombopag in Children With Chronic Immune Thrombocytopenia (ITP): A Real Life Retrospective Multicenter Experience of the Italian Association of Pediatric Hematology and Oncology (AIEOP)

Ritika Khurana<sup>1</sup>, Sangeeta Mudaliar<sup>2</sup>

Consultant, Pediatric hematology-oncology, B.J. Wadia hospital for Children, Mumbai, India <sup>1</sup>

Senior Consultant & Head of department, Pediatric hematology-oncology, B.J. Wadia hospital for Children, Mumbai, India <sup>2</sup>

## BASED ON ARTICLE

Giordano P, Lassandro G, Barone A, Cesaro S, Fotzi I, Giona F, Ladogana S, Miano M, Marzollo A, Nardi M, Notarangelo LD, Pession A, Ruggiero A, Russo G, Saracco P, Spinelli M, Tolva A, Tornesello A, Palladino V and Del Vecchio GC (2020) Use of Eltrombopag in Children With Chronic Immune Thrombocytopenia (ITP): A Real Life Retrospective Multicenter Experience of the Italian Association of Pediatric Hematology and Oncology (AIEOP). *Front. Med.* 7:66.

**Background:** Eltrombopag is a safe and effective thrombopoietin receptor agonist which is now widely used in pediatric patients with Chronic ITP. This study aims to consolidate data on patterns of use of eltrombopag in this population.

**Material and Methods:** This retrospective study was conducted in 17 centres affiliated to the Italian Association of Pediatric Hematology and Oncology (AIEOP) with a primary objective to study prevalence of eltrombopag use in Italian children (3-17 years) with chronic ITP. The secondary objectives were to assess efficacy of Eltrombopag in the first 6 months of use and adverse effect profile during entire duration of its use.

**Results:** Total of 386 patients with Chronic ITP were recruited, out of which 71(19%) had received eltrombopag (Eltrombopag monotherapy in 32 patients, i.e. 55%). Thirty-one patients (44%) were male and 40 patients (56%) were female. The median age of patients was 12 years (3–17 years) and median duration of use was 11 months (1–32 months). Starting dose of eltrombopag ranged from 12.5mg to 75mg (median dose 50mg/day). Significant improvement was seen in platelet counts and bleeding episodes six months post treatment with eltrombopag, including in the group which received only eltrombopag. Adverse effects like headache (7%) and thrombocytosis (6%) were observed in a minority of patients.

**Conclusion:** Eltrombopag is an effective and safe second line therapy for children with Chronic ITP.

## COMMENTARY

Management of children with chronic ITP is ever challenging. Though the ultimate aim of treating these patients is to avoid any significant bleeds and allow the child to have a decent quality of life. Having said that, the extremely low platelet counts in some patients keeps the parents and clinicians under stress.

Therapies for chronic ITP were traditionally the same over many years, which included steroids, IvIg

followed by other immunosuppressants like cyclosporin, Mycophenolate Mofetil, Rituximab etc and splenectomy. None of these are sinless and come with a baggage of many adverse effects like immunosuppression etc.

Eltrombopag, a Thrombopoietin receptor agonist, has evolved as a revolutionary drug in the treatment of chronic ITP since it's FDA approval for use in paediatric chronic ITP in August 2015. PETIT 1 & 2 trials clearly reflected it's benefit in terms of improved platelet counts and decreased bleeding episodes. The questions that remained unanswered on it's practical utility are beautifully answered by this Italian study. In this retrospective multicentre study conducted in 17 centres affiliated to the Italian Association of Pediatric Hematology and Oncology(AIEOP), Eltrombopag was used in 19% (71/386) patients between 3-17 years of age(median 12 years) with chronic ITP in a dose of 50-100mg/day for a median period of 11 months (1-32 months). Thirty two patients (45%) received Eltrombopag with concomitant medications and 39 patients (54.9%) received only Eltrombopag. Significant reduction in bleeding episodes and improvement in platelet counts (1,00,000 & 87,000 six months post treatment, respectively) were observed in both category of patients. This suggests that Eltrombopag monotherapy also has promising role in improving quality of life of patients with Chronic ITP.

No major side effects were observed during study period, except for headache in 7%, thrombocytosis in 6%, transient transaminitis in one patient, all of which resolved on dose reduction. Only one serious side effect of Cerebral sinus venous thrombosis in a 15 year old patient who also happened to have Factor V Leiden mutation. Platelet counts did not fall & bleeding episodes did not increase on reducing the dose for above indications suggesting that lesser dose may also suffice for certain pediatric population.

This study puts us in a more comfortable zone in administering TPO agonists to as many possible children with chronic ITP with an intention to bring down their bleeding episodes and reduce hospital visits, besides improving the platelet numbers. Here, the drug was available for free to patients which may be a constraint in most other countries where cost is a limitation for it's widespread use. Questions that are yet to be answered are how sustained will be the response post treatment discontinuation and any long term adverse effects with this drug.