Advancements in PEDIATRIC ITP: What You Need to Know

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Initial Evaluation and Treatment Considerations for Pediatric ITP[1,2]

Management of newly diagnosed ITP in children depends on:

- Severity of bleeding
- Risk factors
- Degree of thrombocytopenia (low platelet count)
- Quality of life
- Preferences of the patient, caregiver, and family

Spontaneous disease resolution can occur in pediatric patients. It is not possible to predict which patients will have this outcome. **Treatment is indicated as follows:**

PLATELET COUNT	MANAGEMENT
Platelet count of < 20 x10^9/L and no or mild bleeding (skin manifestations) only	Outpatient
Platelet count of ≥ 20 x10^9/L and no or mild bleeding (skin manifestations) only	Outpatient

Initial Treatment Options[1,2]

Observation (watchful waiting): often appropriate for children with no or minor bleeding

Pharmacologic therapies, including:

- Intravenous immune globulin (IVIG)
- Anti-D immunoglobulin
- Glucocorticoids

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Evidence-Based Guidance From ASH 2019 Guidelines[1]

Newly Diagnosed ITP in Children

- Minor or no bleeding
- Observation is preferred over pharmacologic treatment
- **Strong recommendation:** Observation over IVIG or Anti-D immunoglobulin (moderate certainty)
- **Conditional recommendation:** Observation over corticosteroids (very low certainty)

Stratifying Bleeding[3]

GRADE		
0	None	No new hemorrhage of any kind
1	Minor	Few petechiae (< 100 total) and/or < 5 small bruises (< 3 cm), no mucosal bleeding
2	Mild	Many petechiae (> 100) and/or > 5 large bruises
3	Moderate	**Low risk:** Blood crusting in nares, painless oral purpura, oral/palatal petechiae, buccal purpura along molars only, mild epistaxis ≤ 5 minutes **High risk:** Epistaxis > 5 minutes, hematuria, hematochezia, painful oral purpura, significant menorrhagia
4	Severe	Mucosal bleeding or suspected internal hemorrhage (brain, lung, muscle, joint, etc.) that requires immediate medical attention or intervention
5	Life threatening/ Fatal	Documented intracranial hemorrhage or life-threatening or fatal hemorrhage at any site

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Evidence-Based Guidance From ASH 2019 Guidelines[1] (continued)

Persistent or Refractory ITP

- For patients unresponsive to first-line treatment
- TPO-RAs (thrombopoietin receptor agonists) are preferred:
  - Over rituximab
  - Over splenectomy
- Rituximab is preferred over splenectomy

Note: All recommendations for second-line therapy are conditional and based on very low certainty in the evidence of effects

Patient Stratification for Therapy Selection[1,2]

When stratifying patients with ITP, consider the following:

CATEGORY	CONSIDERATIONS
Newly Diagnosed, Mild Symptoms	Observation typically preferred
Newly Diagnosed, Moderate-Severe Bleeding	Initiate pharmacologic therapy (e.g., IVIG or corticosteroids)
Persistent or Chronic ITP	Assess response to first-line therapy; consider TPO-RAs or rituximab
Refractory to Second-Line Therapy	Multidisciplinary input for further treatment decisions (e.g., clinical trials)

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Monitoring and Long-Term Management[1-5]

Key Components of Follow-up

- Monitor **platelet counts** regularly
  - In an asymptomatic or mildly symptomatic patient, every week or 2 for several months to assess stability, then every few months or yearly
  - For patients receiving therapy for ITP, platelet counts should generally be monitored 1 week after a dose or drug change and then monthly once the patient stabilizes on the same therapy
    - » However, certain therapies, such as avatrombopag, may require modifications to this schedule, including more frequent early monitoring
  - Frequency of testing can be increased or decreased in response to treatment and/or depending on degree of thrombocytopenia
- Assess **bleeding symptoms**
- Track **side effects of ongoing therapies**
- Evaluate **health-related quality of life**
- Support patients and family through education and shared decision-making

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Monitoring and Long-Term Management (continued)

Investigational Therapies in Pediatric ITP[6]

Emerging therapies are under investigation and may offer future treatment alternatives, especially for chronic or refractory cases

DRUG	CLASS/MECHANISM	PHASE	ROUTE
Avatrombopag	TPO-RA	Phase 3	Oral tablet
Hetrombopag*	TPO-RA	Phase 3	Oral
Rilzabrutinib	BTK inhibitor	Phase 3	Oral
Daratumumab	Anti-CD38 antibody	Phase 2	Injection
Obinutuzumab	Anti-CD20 antibody	Phase 2	Injection

*Approved in China for second-line treatment of ITP

References

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