Immune Thrombocytopenia (ITP) is an acquired autoimmune disorder characterized by a low platelet count resulting from platelet destruction and impaired platelet production. The incidence of ITP is estimated to be 2 to 5 per 100,000 persons in the general population and can be an isolated primary condition or it may be secondary to other conditions. The information in this pocket guide is intended to support patients, clinicians, and other health professionals in making evidence-based decisions about first- and second-line management of adults and children with ITP.

**Context**

**Adult**

**INPATIENT VS. OUTPATIENT MANAGEMENT**

<table>
<thead>
<tr>
<th>Platelet Count</th>
<th>Status</th>
<th>Management</th>
</tr>
</thead>
<tbody>
<tr>
<td>Platelet count &lt;20 x10^9/l and asymptomatic or minor mucocutaneous bleeding</td>
<td>Newly diagnosed</td>
<td>Inpatient</td>
</tr>
<tr>
<td>Platelet count ≥20 x10^9/l and asymptomatic or minor mucocutaneous bleeding</td>
<td>Established diagnosis</td>
<td>Outpatient</td>
</tr>
</tbody>
</table>

**Observation vs. Treatment**

Determining whether a patient with newly diagnosed ITP should be observed or requires pharmacological treatment depends on the degree of thrombocytopenia, patient comorbidities, medications, and age – all of which impact the risk of bleeding. Management approaches vary based on disease duration, access to care, quality-of-life implications, and patient and provider preferences, among other factors.

**TREATMENT**

**First-Line Therapies for Adults**

In adults with newly diagnosed ITP, the ASH guideline panel recommends against a prolonged course (>6 weeks) of prednisone in favor of a short course (<6 weeks) and suggests either prednisone (0.5 – 2.0 mg/kg/day) or dexamethasone (40 mg/day for 4 days) as the type of corticosteroid for initial therapy. The ASH guideline panel suggests corticosteroids alone rather than rituximab and corticosteroids for initial therapy.

**Second-Line Therapies for Adults**

In adults with ITP lasting ≥3 months who are corticosteroid-dependent or have no response to corticosteroids, the ASH guideline panel suggests the following as potential second-line therapies (see Figure 1):

- Thrombopoietin receptor agonist (eltrombopag or romiplostim)
- Rituximab
- Splenectomy

Follow the algorithm in Figure 1 to determine the most suitable second-line therapies based on presentation and patient preferences.

**Good Practice Statement**

For patients receiving corticosteroids, the treating physician should ensure the patient is adequately monitored for potential side effects regardless of the duration or type of corticosteroid selected. This includes close monitoring for hypertension, hyperglycemia, sleep and mood disturbances, gastric irritation or ulcer formation, glaucoma, cataract, myopathy and osteoporosis. Given the potential impact of corticosteroids on mental health, the treating physician should conduct an assessment of health-related quality of life (HRQoL) (depression, fatigue, mental status, etc.) while patients are receiving corticosteroids.

**Observation vs. Treatment (Newly Diagnosed ITP)**

<table>
<thead>
<tr>
<th>Platelet count ≤30 x 10^9/l and asymptomatic or minor mucocutaneous bleeding</th>
<th>Management with observation</th>
</tr>
</thead>
<tbody>
<tr>
<td>Platelet count &lt;30 x 10^9/l and asymptomatic or minor mucocutaneous bleeding</td>
<td>Treatment with corticosteroids</td>
</tr>
</tbody>
</table>

**Good Practice Statement**

The treating physician should ensure that patients have appropriate immunizations prior to splenectomy and that they receive counseling regarding antibiotic prophylaxis following splenectomy. The treating physician should also educate the patient on prompt recognition and management of fever and refer to current recommendations on pre- and post-splenectomy care.

Each of the second-line treatments may be effective therapy and therefore the choice of treatment should be individualized based on duration of ITP, frequency of bleeding episodes requiring hospitalization or rescue medication, comorbidities, adherence, medical and social support networks, patient values and preferences, cost, and availability. For example, patients who place a high value on achieving a durable response may prefer splenectomy or thrombopoietin receptor agonists (TPO-RAs), patients who value avoidance of long-term medication may prefer splenectomy or rituximab, and patients who wish to avoid surgery may prefer a TPO-RA or rituximab. Patient education and shared decision-making are encouraged.
Patient places a high value on avoiding surgery

Patient places a high value on avoiding long-term medication

Primary treatment options: Rituximab, TPO-RA

Assess patient values and preferences

Primary treatment options: Rituximab, Splenectomy, TPO-RA

Assess patient values

Patient places a high value on achieving durable response

TPO-RA

Patient places a high value on avoiding long-term medication

Rituximab

Patient places a high value on avoiding surgery

Treatment options: Splenectomy, TPO-RA

Patient places a high value on achieving durable response

Treatment options: Rituximab, Splenectomy

Patient places a high value on avoiding long-term medication

Treatment options: Rituximab, TPO-RA

Patient places a high value on avoiding surgery

Figure 1. Selection of second-line therapy in adults with ITP should be individualized based on duration of disease and patient values and preferences. Other factors that may influence treatment decisions include frequency of bleeding sufficient to require hospitalization or rescue medication, comorbidities, compliance, medical and social support networks, cost, and availability of treatments. Patient education and shared decision-making is encouraged.

TPO-RA – thrombopoietin receptor agonist

All recommendations in Figure 1 are conditional recommendations.

NOT FOR DISTRIBUTION
### Pediatric

#### INPATIENT VS. OUTPATIENT MANAGEMENT

**Table 3 – Management of Newly Diagnosed ITP**

<table>
<thead>
<tr>
<th>Platelet Count</th>
<th>Management</th>
</tr>
</thead>
<tbody>
<tr>
<td>Platelet count of &lt;20 x10⁹/l and no or mild bleeding (skin manifestations) only</td>
<td>Outpatient¹</td>
</tr>
<tr>
<td>Platelet count of ≥20 x10⁹/l and no or mild bleeding (skin manifestations) only</td>
<td>Outpatient¹</td>
</tr>
</tbody>
</table>

¹ For patients with uncertainty about the diagnosis, those with social concerns, those who live far from the hospital, or those for whom follow-up cannot be guaranteed, admission to the hospital may be preferable.

#### Good Practice Statement

Patients not admitted to the hospital should receive education and expedited follow-up with a hematologist within 24-72 hours.

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#### OBSERVATION VS. TREATMENT

In children with newly diagnosed ITP who have no or minor bleeding, the ASH guideline panel recommends observation over both intravenous immunoglobulin and anti-D immunoglobulin, and suggests observation over corticosteroids.

#### TREATMENT

##### First-Line Therapies for Children

In children with newly diagnosed ITP who have non-life-threatening mucosal bleeding and/or diminished health-related quality of life, the ASH guideline panel suggests corticosteroids rather than intravenous immunoglobulin or anti-D immunoglobulin. For patients where corticosteroids are contra-indicated or otherwise not preferred, the ASH guideline panel suggests either intravenous immunoglobulin or anti-D immunoglobulin².

In children with newly diagnosed ITP who have non-life-threatening mucosal bleeding and/or diminished health related quality of life, the ASH guideline panel recommends against courses of corticosteroids longer than 7 days in favor of courses 7 days or shorter, and suggests prednisone (2 - 4 mg/kg/day; maximum, 120 mg daily, for 5-7 days) rather than dexamethasone (0.6 mg/kg/day; maximum, 40 mg/kg/day, for 4 days).

¹ This recommendation assumes corticosteroid dosing as outlined in the following paragraph. This recommendation is reserved only for children with non-major mucosal bleeding.

² This recommendation is reserved only for children with non-major mucosal bleeding.

##### Good Practice Statement

For patients receiving corticosteroids, the treating physician should ensure the patient is adequately monitored for potential side effects regardless of the duration or type of corticosteroid selected. This includes close monitoring for hypertension, hyperglycemia, sleep and mood disturbances, gastric irritation or ulcer formation, glaucoma, myopathy and osteoporosis. Given the potential impact of corticosteroids on mental health, the treating physician should conduct an assessment of health-related quality of life (HRQoL) (depression, fatigue, mental status etc.) while patients are receiving corticosteroids.

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#### Second-Line Therapies for Children

In children with ITP lasting ≥3 months who have non-life-threatening mucosal bleeding and/or diminished health-related quality of life and do not respond to first-line treatment, the ASH guideline panel suggests the following options for second-line therapies presented in the order they should be pursued:

1. Thrombopoietin receptor agonist (eltrombopag or romiplostim)
2. Rituximab
3. Splenectomy

¹ Individual patient preference may place a higher value on the use of a daily oral medication (eltrombopag) or one that requires weekly subcutaneous injections (romiplostim). For pediatric patients, eltrombopag dosing should avoid consumption of calcium containing foods such as dairy products by four hours. This may limit the ability of some children to take this medication.

² If possible, splenectomy should be delayed as long as possible after diagnosis because of the potential for spontaneous remission in the first year.

##### Good Practice Statement

The treating physician should ensure that patients have appropriate immunizations prior to splenectomy and that they receive counseling regarding antibiotic prophylaxis following splenectomy. The treating physician should also educate the patient on prompt recognition and management of fever and refer to current recommendations on pre- and post-splenectomy care.

Each of these second-line treatments may be effective therapy and therefore the choice of treatment should be individualized based on duration of ITP, frequency of bleeding episodes requiring hospitalization or rescue medication, comorbidities, adherence, medical and social support networks, patient values and preferences, cost, and availability. Patient education and shared decision-making are encouraged.
Strength of Recommendations and Quality of Evidence

The methodology for determining the strength of each recommendation and the quality of the evidence supporting the recommendations was adapted from GRADE: an emerging consensus on rating quality of evidence and strength of recommendations. Guyatt GH, et al; GRADE Working Group. 2008;336(7650):924–926. More details on this specific adaptation of the GRADE process can be found in American Society of Hematology 2019 Guideline for Immune Thrombocytopenia.¹

<table>
<thead>
<tr>
<th>Strength of Recommendation</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>✔️ Strong recommendations</td>
<td>Most individuals should follow the recommended course of action. Formal decision aids are not likely to be needed to help individual patients make decisions consistent with their values and preferences.</td>
</tr>
<tr>
<td>☐ Conditional recommendations</td>
<td>Recognize that different choices will be appropriate for individual patients and that you must help each patient arrive at a management decision consistent with his or her values and preferences. Decision aids may be useful in helping individuals to make decisions consistent with their individual risks, values and preferences.</td>
</tr>
</tbody>
</table>

How to Use This Pocket Guide

ASH pocket guides are primarily intended to help clinicians make decisions about diagnostic and treatment alternatives. The information included in this guide is not intended to serve or be construed as a standard of care. Clinicians must make decisions on the basis of the unique clinical presentation of an individual patient, ideally though a shared process that considers the patient’s values and preferences with respect to all options and their possible outcomes. Decisions may be constrained by realities of a specific clinical setting, including but not limited to institutional policies, time limitations, or unavailability of treatments. ASH pocket guides may not include all appropriate methods of care for the clinical scenarios described. As science advances and new evidence becomes available, these pocket guides may become obsolete. Following these guidelines cannot guarantee successful outcomes ASH does not warrant or guarantee any products described in these guidelines.

The complete 2019 ASH Clinical Practice Guideline for Immune Thrombocytopenia¹ include additional remarks and contextual information that may affect clinical decisionmaking. To learn more about these guidelines, visit hematology.org/ITPguidelines.

Conflict of interest information for Drs. Neunert, Vesely, Mithoowani, and Kim may be found at hematology.org/pocketguidesCOI.


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For expert consultation on immune thrombocytopenia and other hematologic questions, submit a request to the ASH Consult a Colleague program at www.hematology.org/consult (ASH members only).