Pediatric Perspectives

EMA Workshop – Challenges in drug development, regulation and clinical practice in Immune Thrombocytopenia

Disclosures

- Advisory board member for Octapharma, Dova, Principia, Argenx, Sanofi, Janssen and Shionogi
- Consultant for Novartis, Shionogi, Dova, Principia, Argenx, Rigel, Janssen, Sobi, and Bayer
- Research funding from Sysmex, Novartis, Rigel, Principia, Argenx, Dova, Octapharma, Janssen, Sobi and Astra Zeneca
- Coag Lab Research Funds from Rocket Pharmaceuticals and Werfen
- Medical Advisory Board Member for PDSA, CdLS foundation, 22q Society FWG^{+}BD
- Co-Chair of Hemostasis/Thrombosis GCEP and member Platelet Disorder VCEP for ClinGen
- Coagulation Laboratory Research Funding: Rocket Pharmaceuticals; Werfen

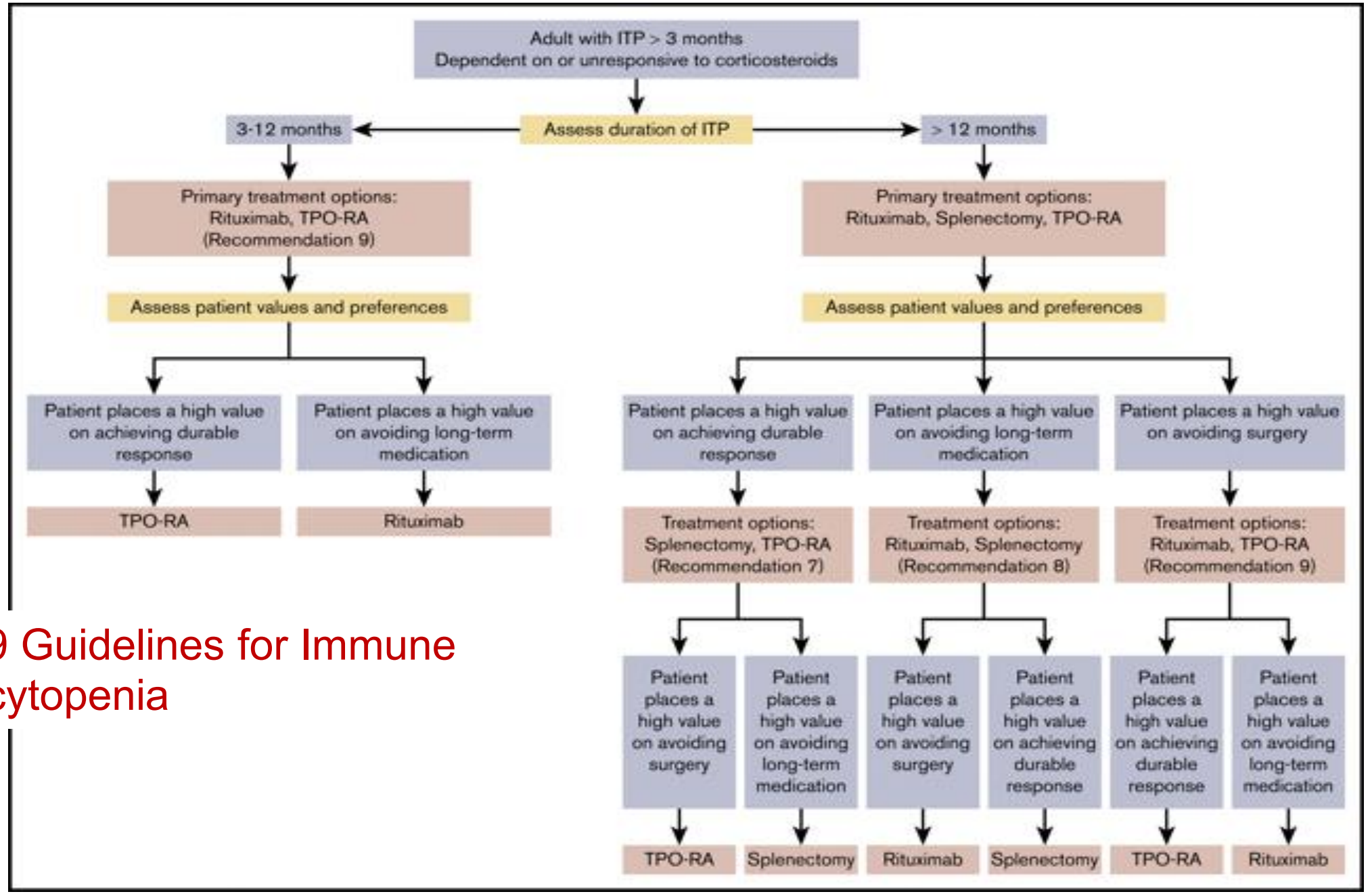
American Society of Hematology Guidelines

Generally, for newly diagnosed adults with very low platelet counts (<20 x10^9/L), guidelines ***suggest*** admission and ***treatment*** with corticosteroids even with minimal or no bleeding symptoms;

For patients with platelets 20-30 x10^9/L and newly diagnosed ITP without bleeding symptoms, ***suggest*** outpatient ***treatment*** with corticosteroids

For newly diagnosed patients with platelets >30 x 10^9/L without bleeding symptoms, ***recommends against*** corticosteroids in favor of observation (with some caveats)

ASH 2019 Guidelines for Immune Thrombocytopenia



Neunert et al. Blood,Adv 2019

Good Practice Statement

- The choice of second-line 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.**
- If possible, splenectomy should be delayed for as long as possible after diagnosis because of the potential for spontaneous remission.

Neunert et al. Blood Adv, 2019

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Guideline ***suggests*** observation over corticosteroids and ***recommends*** observation over IVIG or anti-D immunoglobulin in asymptomatic* pediatric patients with newly diagnosed ITP

Guideline ***suggestes against*** inpatient management in asymptomatic* pediatric patients with newly diagnosed ITP

Guideline ***recommends against*** corticosteroids >7 days duration, preferring prednisone 2-4 mg/kg (max 120 mg daily) over dexamethasone

Guideline prefers TPO-RA over rituximab and recommends against splenectomy in pediatric patients

*or mildly symptomatic

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Guidelines generally recommend treatment for bleeding symptoms OR for HRQoL

Because pediatric ITP is usually self-limited, goal is to ensure that therapeutic interventions do not cause more harm than the diagnosis

Second-line Therapy: Pediatrics

- In children with ITP who are unresponsive to first-line treatment, the panel **suggests** the use of thrombopoietin receptor agonists rather than rituximab

- In children with ITP who are unresponsive to first-line treatment, the panel **suggests** the use of thrombopoietin receptor agonists rather than splenectomy

- In children with ITP who are unresponsive to first-line treatment, the panel **suggests** the use of rituximab rather than splenectomy

All conditional recommendations based on very low confidence in the evidence

Neunert et al. Blood Adv, 2019

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Neunert et al. Blood Adv, 2019

Overall treatment goals are similar

Treatment Goals:

- Prevent serious bleeding and frequent minor bleeding
- Improve quality of life
- Minimize fatigue and depression
- Decrease absenteeism (work/school)
- Allow participation in desired activities: sports, travel
- Allow continuation of other medications

Song F, et al. *J Blood Med*. 2021;12:653-664.

Ideal therapy for pediatric patients

- Minimal short and no long-term toxicity
- High efficacy
- Minimal immune suppression
- Ease of delivery/tolerable

Thank you