

Blood FOR Disorders Day 2018 Health Professionals Plunging Platelets: Thrombocytopenia in the Pediatric Patient Dr. Jayson Stoffman Pediatric Hematology/Oncology









#### Presenter Disclosure

Faculty / Speaker's name: Dr. J. Stoffman

- Relationships with commercial interests:
  - Grants/Research Support: Daiichi Sankyo, Hoffman-La Roche
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  - Other: None





# Learning Objectives

By the end of this presentation, the participant should be able to:

- 1. Explain common causes of thrombocytopenia in children
- 2. Describe the presentation, clinical course, and treatment options for ITP in children



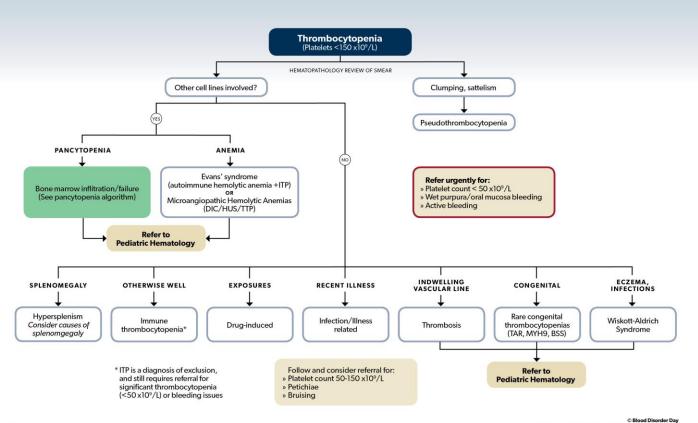


# Thrombocytopenia in pediatrics

- Pseudothrombocytopenia
- Impaired production:
  - Marrow infiltration or failure
  - Congenital disorders: TAR, BSS, MYH9-related, WAS
- Increased consumption:
  - Hypersplenism
  - Infection/illness
  - Drug-induced
  - Thrombosis
  - Autoimmune condition Evans syndrome, SLE, others...
  - Microangiopathic hemolytic anemias DIC, HUS, TTP
- Immune thrombocytopenic purpura



#### **Pediatric Thrombocytopenia**



Legend: DIC = Disseminated intravascular coagulation

**HUS** = Hemolytic uremic syndrome

TTP = Thrombotic thrombocytopenic purpura

TAR = Thrombocytopenia with absent radii

MYH9 = Myosin heavy chain 9-related thrombocytopenia

**BSS** = Bernard-Soulier syndrome

Pathways are subject to clinical judgement and actual practice patterns may not always follow the proposed steps in this pathway.

This algorithm is intended for general guidance only, and should be used with appropriate clinical judgement.





# Immune Thrombocytopenia Purpura

- Uncommon incidence of 3-8 to 7.2-9.5/100 000
- A diagnosis of EXCLUSION
- Sudden onset of purpura and petechiae in an otherwise normal child with an otherwise normal physical examination
- Isolated thrombocytopenia on the CBC
  - Smear usually shows few large platelets with no other abnormalities





## ITP – A tale of two conditions

#### SL – DOB April 2010

- 1 month history of increased bruising
- 1 nosebleed lasting 30 min
- Platelet count 2
- 3 weeks later Plt count 3

#### LM – DOB April 2012

- Large bruise after minor injury
- Mild viral URTI 3 weeks before
- Platelet count 3
- 3 weeks later Plt count 37
- 18 months later Plt count 118

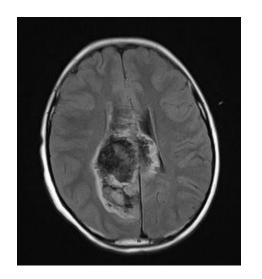




## The worst case scenario

#### AH – DOB May 2008

- 1 week history of purpura
- 1 day history of mucosal bleeding and altered LOC
- Platelet count 4

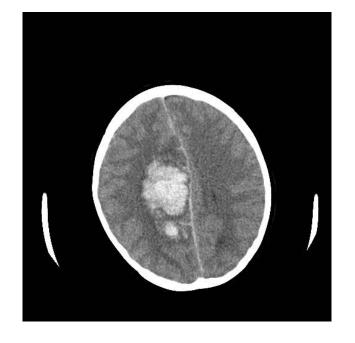






## Intracranial hemorrhage

- 0.4-0.6% of all ITP
- UK experience: 14 cases of ICH over 20 years
- World literature 1970-99:
  54 reported ICH







### What counts count?

- Normal platelet count: 150 450 X 10e9/L
- Thrombocytopenia: Plt < 100</li>
- < 50 Increased risk of bleeding with trauma/surgery</li>
- < 20 Petichiae and spontaneous bruising</li>
- < 10 Increased risk of severe hemorrhage</li>

Mechanism of thrombocytopenia in ITP is different





# Important definitions (IWG)

• Remission: Plt count > 100

- Acute: < 3 months</li>
- Persistent: 3-12 months
- Chronic: >12 months





### Treatment considerations

- 80% of children will have spontaneous remission
  - Predictors poorly understood: younger age, bleeding at Dx
- Burden of illness and treatment are important considerations
- Shared decision making between physician and parents
  - Value of HRQoL measures in choices under investigation





# Treatment options

#### First line

- Observation
- Corticosteroids
- lvlg
- Anti-D

#### **Second line**

- Splenectomy
- TPO-RA
- Rituximab





#### Treatment choices

- Patient/parent (and physician) preference and comfort
- Treatment profile
  - Likelihood of remission
  - Side effects
  - Route of administration





## Key messages

- Thrombocytopenia in children results from increased consumption or decreased production, due to a variety of causes.
- ITP in children is generally a benign and self-limited condition, and treatment should be directed at symptom management for improved quality of life.





# Key references

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