**Therapeutic Approaches for Platelet Disorders**

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**Introduction**

- Blood consists of many types of cells, such as white blood cells, red blood cells, and platelets.
- When a blood vessel breaks, platelets will localise to the site of injury and trigger a coagulation cascade. During this, an enzyme, thrombin, cleaves fibrinogen into fibrin monomers, which then polymerise together to form the blood clot.
- Platelet disorders refer to either the surplus, deficiency, or dysfunction of platelets. Symptoms can include excessive bruising, prolonged bleeding, fatigue, chest pain, and vomiting.

**Types of Platelet Disorders**

1. **Bernard-Soulier disease**: characterised by dysfunction of the glycoprotein IIb-IIIa complex, leading to increased bleeding tendency.
2. **Hermansky Pudlak syndrome**: characterised by a deficiency or dysfunction of the glycoprotein IIb/IIIa receptor, resulting in impaired platelet aggregation and increased bleeding tendency.
3. **Jacobsen syndrome**: a rare chromosomal disorder characterised by multiple physical and developmental abnormalities, including heart defects, intellectual disability, and thrombocytopenia, caused by a deletion in the long arm of chromosome 11.
4. **TTP**: characterised by microvascular thrombosis, resulting in thrombocytopenia, haemolytic anaemia, and organ damage, often caused by a deficiency of the ADAMTS13 enzyme.

**Examples of Drugs: FDA Approval Timeline**

<table>
<thead>
<tr>
<th>Year</th>
<th>Drug</th>
<th>Type of Agent</th>
</tr>
</thead>
<tbody>
<tr>
<td>1986</td>
<td>Tranexamic acid</td>
<td>Antifibrinolytic agent</td>
</tr>
<tr>
<td>1991</td>
<td>Ticlopidine</td>
<td>Antiplatelet agent</td>
</tr>
<tr>
<td>1994</td>
<td>Abciximab</td>
<td>Antiplatelet agent</td>
</tr>
<tr>
<td>1997</td>
<td>Clopidogrel</td>
<td>Antiplatelet agent</td>
</tr>
<tr>
<td>1998</td>
<td>Epifibatide</td>
<td>Anti-coagulant agent</td>
</tr>
<tr>
<td>1998</td>
<td>Tirofiban</td>
<td>Antiplatelet agent</td>
</tr>
<tr>
<td>2008</td>
<td>Romiplostim</td>
<td>TPO-RA</td>
</tr>
<tr>
<td>2009</td>
<td>Prasugrel</td>
<td>Antiplatelet agent</td>
</tr>
</tbody>
</table>

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**Innovative Therapies**

- TPO-RA stimulate the production of platelets in the bone marrow and are used to treat thrombocytopenia, including TTP and myelodysplastic syndromes.
- Immunosuppressive therapy may be used in cases of immune-mediated platelet disorders like ITP; for which medications that suppress the immune system, such as corticosteroids, immunoglobulins, or immunosuppressants, may be prescribed to reduce platelet destruction.
- Anticoagulants or antiplatelet agents are used to prevent blood clots in conditions associated with platelet hyperactivity or increased thrombotic risk.
- Platelet transfusions may be necessary to increase the platelet count quickly in cases of severe thrombocytopenia or active bleeding. However, transfusions are typically reserved for emergencies or when other treatments are ineffective due to potential risks and complications associated with transfusions.

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**References**


**Abbreviations:**

- ITP: immune thrombocytopenic purpura
- TAR: thrombocytopenia with absent radius
- TPO-RA: thrombopoietin receptor agonists
- TTP: thrombotic thrombocytopenia purpura

**Logos:**

- EMJ Hematol.
- European Medical Journal