

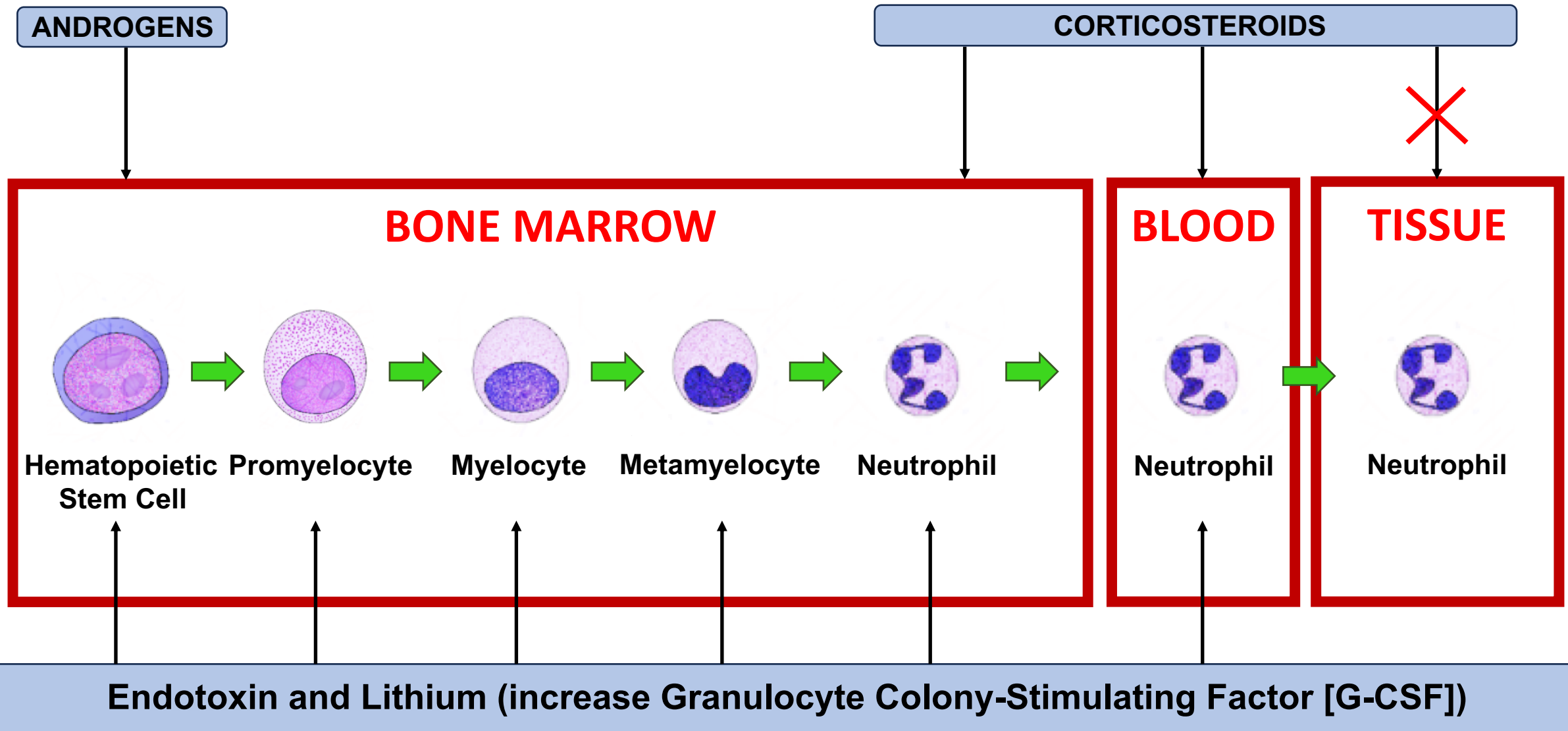
Novel Therapies for Chronic Neutropenia and Barriers for their Development

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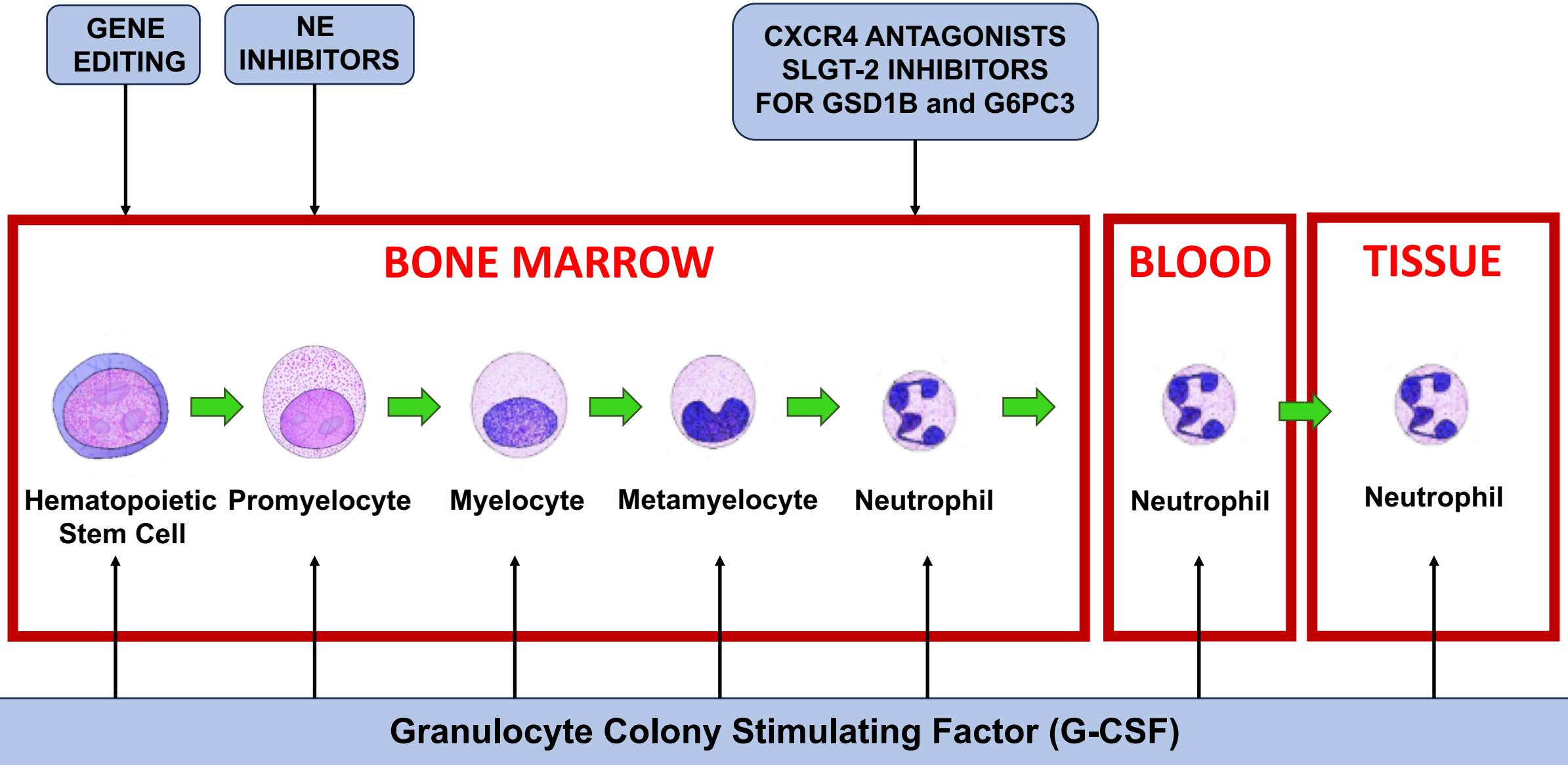
What are Novel Therapies?

- Something entirely new
- Drugs not previously used for this disease
- Diseases previously had no effective therapy
- Usually specific mechanism(s) of action known
- Other overlapping terms:
 - Targeted therapies
 - Precision medicine

Pre-G-CSF Therapies for Severe Chronic Neutropenia



Novel Therapies for Severe Chronic Neutropenia



Barriers to Novel Therapies for Rare Diseases

- Development costs and potential profitability
- Few patients have rare diseases; even fewer may qualify
- Unforeseen events, e.g., COVID 19, Laboratory support and supplies
- Regulations and bureaucracy
 - Hospital
 - University
 - Company/Sponsor
 - Clinical Research Organizations

Persistence and Patience