# A Scoping Review of Patterns and Characteristics of Adaptive Design Trials in Haematology

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## Introduction

- An adaptive design (AD) trial allows preplanned modifications based on the interim results, while maintaining the trial's integrity and validity (1).
- Haematological diseases are heterogeneous and rare.
- Some trials fail to recruit sufficient participants and obtain funding (2,3).
- AD trials shorten the trial duration and accelerate the approval of therapeutic agents by the regulatory bodies.
- This study aimed to map the existing literature on the patterns and characteristics of AD trials for haematological diseases from July 31, 2015, to July 31, 2025.

## Methods

- This scoping review was conducted following the Joanna Briggs Institute (JBI) methodological framework (4).
- 4 databases: PubMed, Embase, Cochrane Library, and ClinicalTrials.gov.
- The eligibility criteria were studies that explored AD trials in participants with haematological disease worldwide.
- Studies were screened and selected following the Preferred Reporting Items for Systematic Reviews and Meta-Analyses extension for scoping reviews (PRISMA-ScR) flowchart.
- The data were extracted according to the template provided by the JBI guideline and analysed using descriptive statistics and narrative summary.

## Results

- Among 867 records, 37 reports describing 33 studies were included.
- 29 clinical trials, 5 protocols, and 1 each for methodology, to the editor and correspondence
- Studies were conducted across 72 countries.
- The most common method used was the continued reassessment method (CRM), followed by multiple adaptive designs and platform designs.

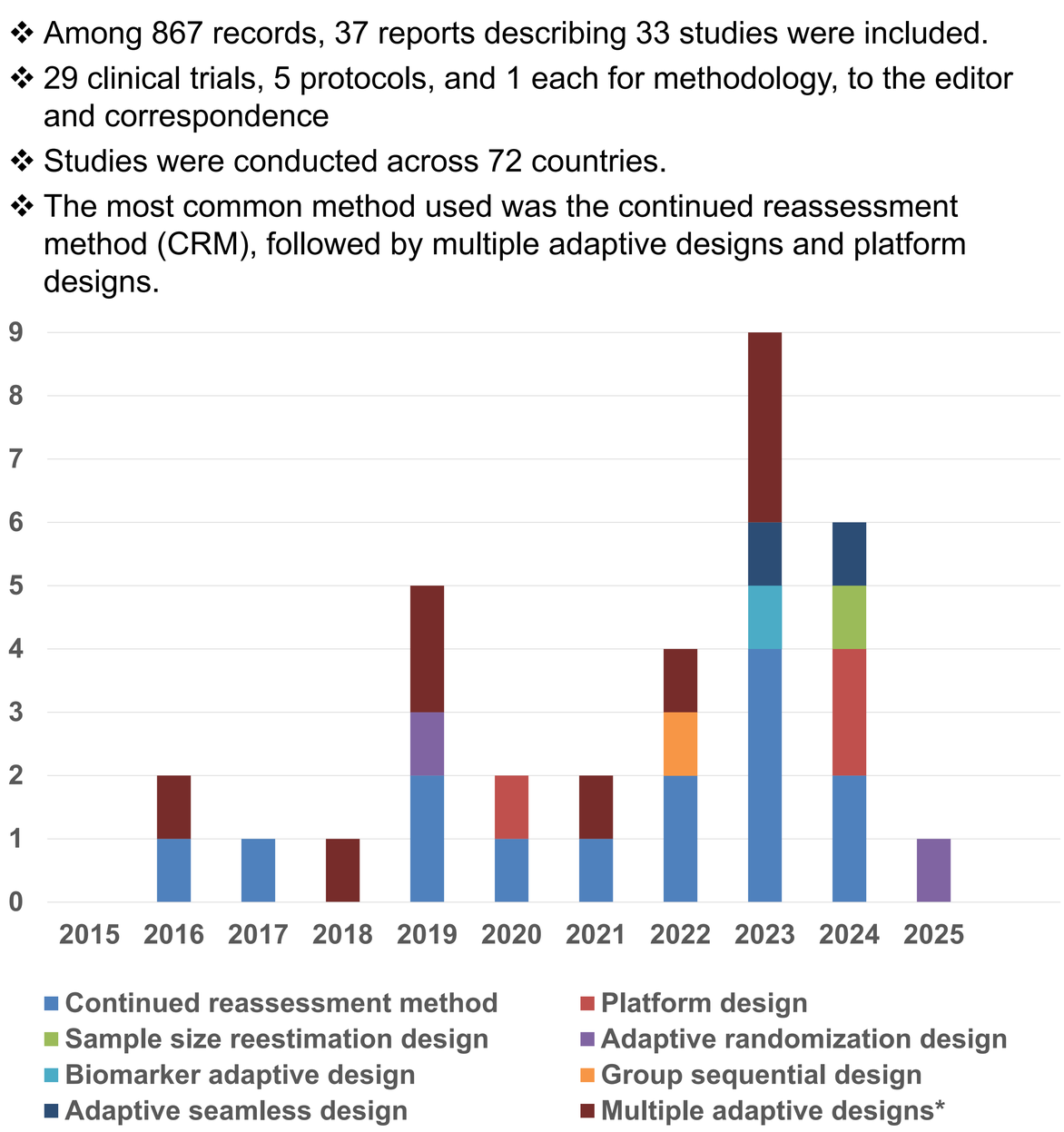


Figure 1: Patterns of adaptive trials in haematology over 10 years

*Multiple adaptive designs: CRM & adaptive seamless phase 1/2 (n=3); "pick the winner or drop the loser" design & adaptive seamless phase 1/2 (n=1); adaptive randomisation & group sequential design (n=1); platform design & adaptive seamless phase 2/3 (n=1), adaptive randomisation & adaptive seamless phase 1/2 (n=1), platform design & adaptive randomisation (n=2)

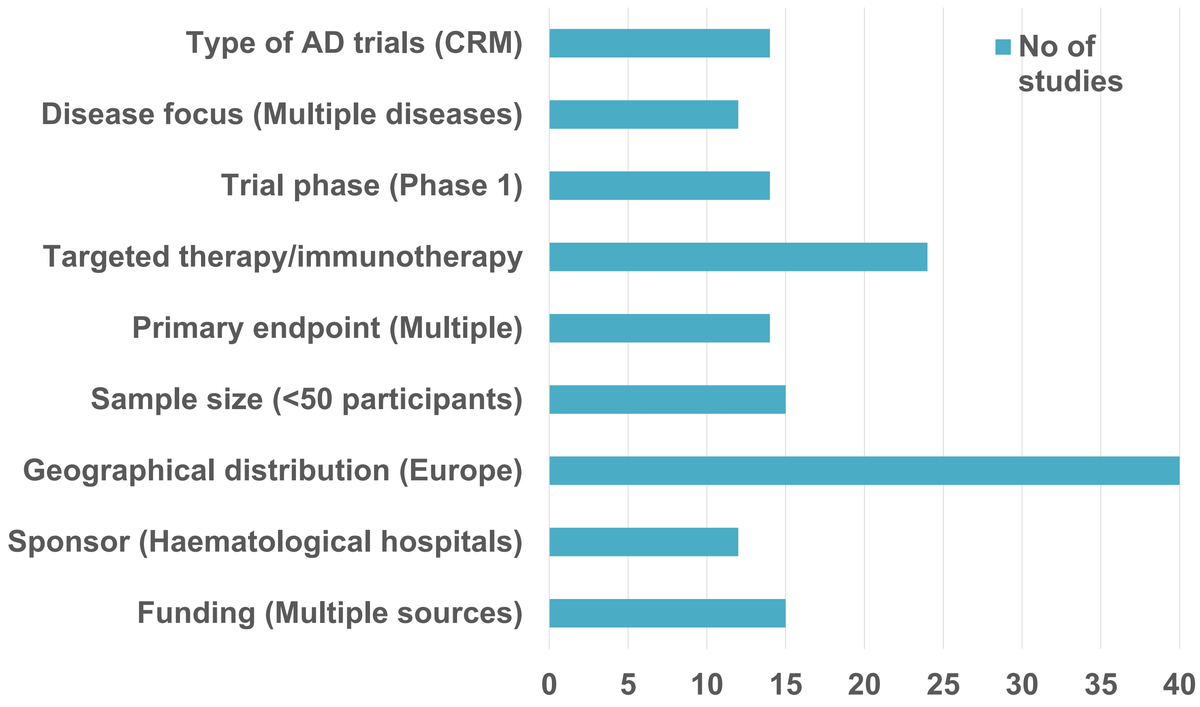


Figure 2: The characteristics of adaptive design trials

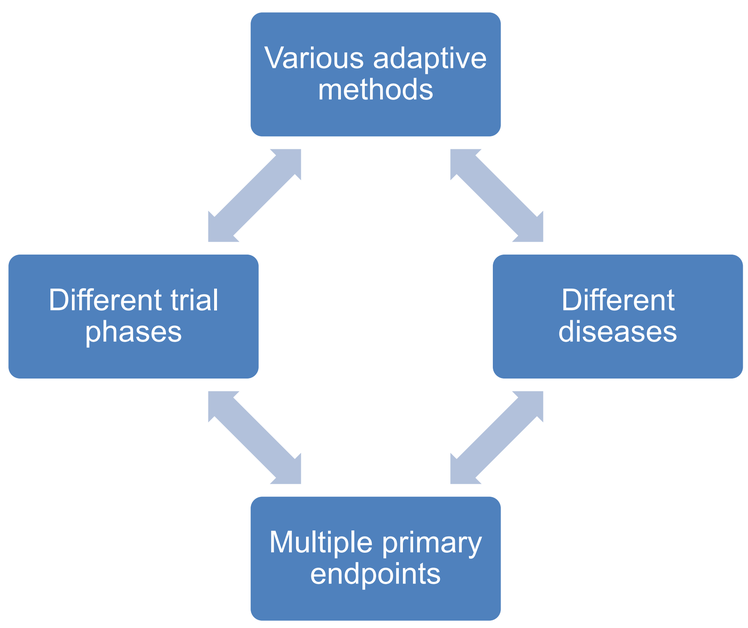


Figure 3: The unique features of adaptive design trials in haematology

## Conclusion

- There was an uneven distribution of AD trials globally, and non-malignant blood diseases remained underrepresented.
- These inform policymakers, researchers and industries to consider AD trials in these areas.

## References

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