

Safety and Efficacy of RM-004 Cells for Hemoglobin H-Constant Spring Disease

NCT06107400

Status	RECRUITING
Phase	Early Phase 1
Sponsor	The 923rd Hospital of Joint Logistics Support Force of People's Liberation Army
Enrollment	5 participants

Key Eligibility Criteria

Inclusion (4)

- Subjects voluntarily sign informed consent by themselves or their legal guardians and complete the study procedures, follow-up examination and treatment.
- At the time of screening, subjects should be aged from 12 to 35 years old, regardless of gender.
- History of at least 100 mL/kilograms (kg)/year of packed red blood cells (pRBC) transfusions in the prior 2 years before screening.
- Subjects diagnosed with Hemoglobin H-Constant Spring disease (HbH-~~CS~~) with HBA2 c.427T>C mutation.

Exclusion (4)

- Subject who has an available HLA-matched/well-matched HSCT donor for allogeneic hematopoietic stem cell transplantation (HSCT).
- Prior HSCT or gene therapy.
- History of severe hemorrhagic disease.
- Clinically significant active bacterial, viral, fungal or parasitic infections per investigator's judgement at the time of screening.

Locations (1 total)

The 923rd Hospital of Joint Logistics Support Force of People's Liberation Army, Nanning, Guangxi, China