

Clinical Trial of the Dual Vector Base Editor for the Treatment of the CHD3-R1025W Mutation

NCT06860672

Status	RECRUITING
Phase	Early Phase 1
Sponsor	Yongguo Yu
Enrollment	1 participants

Key Eligibility Criteria

Inclusion (4)

- Clinical diagnosis of Snijders Blok-Campeau syndrome
- Heterozygous mutation of c.3073C>T, p.(Arg1025Trp) in the CHD3 gene
- Normal liver, heart and immune function
- Normal coagulation and platelet counts

Exclusion (9)

- Brain tumor or intracranial space-occupying lesion
- Contraindications to administration of lumbar puncture or sheath injection administration
- Persistent status epilepticus or recurrent epileptic control instability
- Presence of unstable systemic disease including active bacterial, fungal or HIV, hepatitis A, hepatitis B infection
- Serum anti-AAV neutralizing antibody titer $\geq 1:50$ (ELISA immunoassay)
- ... and 4 more (see full listing online)

Locations (1 total)

Xinhua Hospital affiliated to Shanghai Jiao Tong University School of Medicine, Shanghai, Shanghai Municipality, China