Valoctocogene Roxaparvovec –
Gene Therapy for Hemophilia A Undergoing Clinical Trials

Gene therapy for hemophilia is a treatment approach undergoing clinical studies and is not approved for commercial use by any regulatory authority.

**Valoctocogene roxaparvovec** is an AAV-based** gene therapy treatment for hemophilia A undergoing clinical trials. The safety and efficacy of the treatment has not been established.

The Most Advanced Hemophilia A Gene Therapy Clinical Trial Research Program:

- First and only to report two-year safety and efficacy data
- First to initiate a Phase 3 trial (December 2017), which will enroll 130 participants.

**Most clinical trials (six) underway, eligibility requires participants to have less than 1 percent Factor VIII activity level:**
- a. Two global Phase 3 trials: GENEr8-1 (4e13 vg/kg*dose) and GENEr8-2(4e13 vg/kg* dose)
- b. Phase 1/2 dose escalation study
- c. Phase 1/2 trial in participants with pre-existing adeno-associated vector (AAV) antibodies
- d. Two non-interventional studies: AAVs seroprevalence and baseline characteristics in participants with hemophilia A

**Gene Therapy for Hemophilia A Undergoing Clinical Trials**

- 6. https://clinicaltrials.gov/show/NCT03392974

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