PRACTICE AID

Investigational Gene Therapy for GBA-Associated Disease[1-6]

Full abbreviations, accreditation, and disclosure information available at **PeerView.com/GWQ40**

PeerView Neurology

GCA GAUCHER COMMUNITY ALLIANCE

Gaucher Disease

Study Identifiers	Patient Population	Investigational Product	Study Phase and Design	Study Status and Estimated Completion	Key Endpoints
PROCEED NCT05487599	Adults with peripheral manifestations of GD	LY3884961	Phase 1/2 multicenter, 5-yr, open-label, dose-finding study	Recruiting; 10/2030	• **Primary:** safety/tolerability • **Secondary:** change in spleen volume, PLT count, GCase levels, GluSph levels
PROVIDE NCT04411654	Infants with GD2	LY3884961	Phase 1/2 multicenter, 5-yr, open-label, dose-finding study	Active, not recruiting; 5/2028	• **Primary:** safety/tolerability, immunogenicity against AAV9 and GCase in blood and CSF • **Key secondary:** time to death, clinical event, milestones

GBA-Associated Parkinson's Disease

Study Identifiers	Patient Population	Investigational Product	Study Phase and Design	Study Status and Estimated Completion	Key Endpoints
PROPEL NCT04127578	PD and ≥1 *GBA1* mutation	LY3884961	Phase 1/2a multicenter, 5-yr, open-label, dose-finding study	Recruiting; 12/2030	• **Primary:** safety/tolerability, immunogenicity against AAV9 and GCase in blood and CSF • **Key secondary:** change in glycolipid, GCase levels, GCase enzyme activity in blood and CNS
ACTIVATE NCT05819359		BIA-28-6156	Phase 2, two-part, dose-finding study	Active, not recruiting; 7/2026	• **Primary:** time to clinically meaningful motor aspects of experiences of daily living • **Key secondary:** MDS-UPDRS components, CGI, H&Y score, PDQ-39
NCT07011771		CAP-003	Phase 1/2, 2-year, open-label, dose-escalation study	Not yet recruiting; 11/2029	• **Primary:** safety/tolerability • **Key secondary:** GluSph levels, GCase level changes

1. https://clinicaltrials.gov/study/NCT05487599. 2. https://clinicaltrials.gov/study/NCT04411654. 3. https://www.clinicaltrials.gov/ct2/show/NCT04127578. 4. Hordeaux J et al. *Sci Transl Med.* 2020;12:eaba9188. 5. https://www.clinicaltrials.gov/study/NCT05819359. 6. https://clinicaltrials.gov/study/NCT07011771.