

NCL Subtype	Sponsor	Intervention	Status - July 2023
CLN3	Amicus Therapeutics	AT-GTX-502 Gene therapy (AAV9; CNS)	NCT03770572 Ph1/2 – active, not recruiting (USA) Assessments ongoing, not expanding NCL programs now "off strategy" -?
	Beyond Batten Disease Foundation/Theranexus	Batten-1 (miglustat)	NCT05174039 Ph 1/2 - active, not recruiting (USA) 6-month preliminary data announced June 14, 2023 Ph 3 pediatric study expected to commence end-2023 (international sites)
	Polaryx Therapeutics	PLX-200 (gemfibrozil)	NCT04637282 Ph 3 - not yet recruiting (no movement since Nov 2020) - No update at this time
CLN2	BioMarin	Cerliponase alfa - enzyme replacement; CNS	NCT04476862 Ph 4 -Observational/safety, ongoing (USA)
		Cerliponase alfa - Ocular	NCT05152914 Ph 1/2 - Enrolling by invitation (USA)
	LEXEO Therapeutics	LX1004 Gene therapy (AAVrh10; CNS)	Preclinical studies continue to explore alternate routes of administration
	Orphion Therapeutics	ORPH-301 Gene therapy (AAV2; ocular)	Preclinical - No update at this time
	REGENXBIO	RGX-181 Gene therapy (AAV9; CNS)	Investigator-led study - 1 patient treated (Brazil) Plans to present preliminary data Q3 2023
RGX-381 Gene therapy (AAV9; ocular)		NCT05791864 Ph 1/2; actively recruiting (UK)	
CLN1	JCR Pharmaceuticals	AGT-194 enzyme replacement IV	Investigator-led study - 1 patient treated (Germany) No further drug available; R&D actively continues
	Taysha Gene Therapies	TSH-118 Gene therapy (AAV9; CNS)	Ph 1/2 paused (since early 2022) Continuing to explore partnership opportunities
CLN5	Neurogene	NGN-101 Gene therapy (AAV9; CNS & ocular)	NCT05228145 Ph 1/2 actively enrolling (USA & UK)
CLN6	Amicus Therapeutics	AT-GTX-501 Gene therapy (AAV9; CNS)	Discontinued
CLN7	UT Southwestern	Gene therapy (AAV9; CNS)	NCT04737460 Ph1 active; not recruiting (USA) Assessments ongoing; plans to present data Q3-Q4 2023
	Neurogene	Preclinical + natural history study	Discontinued
Natural History Studies	University of Rochester, NY	All NCL types	NCT01873924 recruiting international patients – in-person and/or virtual visits
	National Institutes of Health (NIH)	CLN3; biomarker study	NCT03307304 recruiting international patients – in-person visits (+ virtual visits)
	UKE Hamburg- Eppendorf, Germany	All NCL types	NCT04613089 recruiting international patients – in-person and/or virtual visits

Abbreviations: AAV, adeno-associated virus serotype 9; CNS, central nervous system (ie. brain, spinal cord); [NCT](#) numbers denote Study ID at [clinicaltrials.gov](#)