

# Global Status of LSD Treatment Development -2024- (16 Diseases) -1- [Provisional Translation]

Method	Company	Country	Mucopolysaccharidosis								Galactosialidosis	Mucopolipidosis	GM1-Gangliosidosis	GM2-Gangliosidosis	Fabry	Gaucher	Pompe	
			I	II	III			IV		VI								VII
					A	B	C	A	B									
Gene Therapy	REGENX Bio	US		⊙														
	4 DMT	US												⊙				
	IMMUSOFT	US	⊙	●											●			
	M6P THERAPEUTICS	US				●						●		●	●	●		
	Ultragenyx	US			⊙													
	Asklepios BioPharmaceutical (Bayer)	Germany														⊙		
	Phoenix Nest	US					●											
	Amicus	US												●		●		
	Orchard Therapeutics (Kyowa Kirin)	UK (Japan)	●		●													
	SPUR Therapeutics	UK													⊙			
	GENETHON	France														⊙		
	ESTEVE R&D	Spain		●	⊙	●												
	UniQure	Netherlands												⊙				
	Prevail Therapeutics (Eli Lilly)	US													⊙			
	Institute for Gene Therapy	Japan											●					
	AUDENTES (Astellas)	US (Japan)														⊙		
	Passage Bio	US											⊙					
	LogicBio (CANbridge)	US (China)													●	●		
	Sangamo Bio	US													⊙			

Global Status of LSD Treatment Development -2024- (16 Diseases) –2- 【Provisional Translation】

● Pre-clinical  
◎ Clinical

Method	Company	Country	Mucopolysaccharidosis									Galactosialidosis	Mucolipidosis	GM1-Gangliosidosis	GM2-Gangliosidosis	Fabry	Gaucher	Pompe
			I	II	III			IV		VI	VII							
					A	B	C	A	B									
Enzyme Replacement Therapy	M6P Therapeutics	US						●		●						●	●	●
	Denali Therapeutics	US	●	⊙	●													
	Crosswalk Therapeutics	US														●		●
	JCR Pharmaceuticals	Japan	⊙	●	⊙	●					●	●		●	●	●	●	●
	Eli Lilly	US															⊙	
	CANbridge (WuXi Biologics)	China														●	⊙	
	Hanmi	Korea														●		
	Allievex	US			●	⊙								●	●			
	GC BioPharma	Korea			●											●		
	Chiesi	Italy														●		
Chaperones	GC BioPharma	Korea												●				
Others	Gain Therapeutics	Spain												●			●	
	Sanofi	France														⊙	⊙	
	Amicus	US																⊙
	Zywie LLC	US															●	
	AceLink Therapeutics	US												●	●	⊙	⊙	
	idorsia	Switzerland														●		
	azafaros	Netherlands												●	⊙			
	Paradigm	Australia	⊙							⊙								
	Evotec	Germany															●	
	Maze Therapeutics (Shionogi introduced)	US																●
	Bio Arctic	Sweden															●	

• Y Support Fund

• Mucopolysaccharidosis Fund

Donation Recipient

June 2010: National Center for Child Health and Development – Substantial donation for “The diagnosis and research funding of rare and intractable pediatric diseases such as lysosomal diseases”

October 2014: Gifu University School of Medicine – Donation for “Research on improving bone and cartilage symptoms in all types of mucopolysaccharidosis-related diseases”

September 2017: Kawasaki Medical School: Substantial donation for “Research and development into elucidating the pathology and treatment of rare and intractable hereditary lysosomal disorders, particularly mucopolysaccharidoses”

June 2019: Hiroshima University Genome Editing Innovation Center – Donation for “Research on genome editing”

August 2022: Kyoto University Hospital, Department of Diabetes, Endocrinology, and Nutrition – Donation for “CNP treatment research regarding mucopolysaccharidosis”