

Clinical Trial, Briefly Title and ClinicalTrials.gov link	Phase	Status	Sponsor reports	MPS Type	Interventions, Sponsor Study Name, & Treatment designation	Study Sites	Vector type	Route: ICM: Intracisternal magna IV: Intravenous IC: Intracranial
<a href="#">RGX-111 Gene Therapy in Patients With MPS I</a>	I/II	Recruiting	<a href="#">December 1, 2020</a>	Type I	<a href="#">REGENXBIO, RGX-111</a>	Children's Hospital of Orange County, Orange, California, United States Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, United States	AAV9	ICM
<a href="#">Ascending Dose Study of Genome Editing by the Zinc Finger Nuclease (ZFN) Therapeutic SB-318 in Subjects With MPS II</a>	I/II	Active, not recruiting	<a href="#">February 7, 2019</a>	Type I	<a href="#">Sangamo Therapeutics, Empowers Study, SB-318</a>	UCSF Benioff Children's Hospital Oakland, Oakland, California, United States	AAV6	IV
<a href="#">Gene Therapy With Modified Autologous Hematopoietic Stem Cells for the Treatment of Patients With Mucopolysaccharidosis Type I, Hurler Variant</a>		Active, not recruiting	<a href="#">September 1, 2020</a>	Type I	<a href="#">Orchard Therapeutics, OTL-203</a>	Ospedale San Raffaele, Milano, Italy	Second generation self-inactivating lentiviral vector transduced autologous HSC	IV-Transfusion
<a href="#">RGX-121 Gene Therapy in Patients With II (Hunter Syndrome)</a>	I/II	Recruiting	<a href="#">December 9, 2020</a>	Type II	<a href="#">REGENXBIO, RGX-121</a>	UCSF Benioff Children's Hospital, Oakland, California, United States Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, United States Children's Hospital of Pittsburgh - UPMC, Pittsburgh, Pennsylvania, United States Hospital de Clinicas de Porto Alegre, Porto Alegre, RS, Brazil	AAV9	ICM
<a href="#">RGX-121 Gene Therapy in Children 5 Years of Age and Over With II (Hunter Syndrome)</a>	I/II	Not yet recruiting		Type II	<a href="#">REGENXBIO, RGX-121</a>	Not yet listed	AAV9	ICM
<a href="#">Ascending Dose Study of Genome Editing by the Zinc Finger Nuclease (ZFN) Therapeutic SB-913 in Subjects With MPS II</a>	I/II	Active, not recruiting	<a href="#">February 7, 2019</a>	Type II	<a href="#">Sangamo Therapeutics, Champion Study, SB-913</a>	UCSF Benioff Children's Hospital Oakland, Oakland, California, United States Ann & Robert H. Lurie Children's Hospital of Chicago, Chicago, Illinois, United States NYU School of Medicine, Neurogenetics Division, New York, New York, United States University of North Carolina, Chapel Hill, North Carolina, United States Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio, United States	AAV6	IV
<a href="#">Phase I/II Gene Transfer Clinical Trial of scAAV9.U1a.hSGSH</a>	I/II	Recruiting	<a href="#">November 9, 2020</a>	Type IIIA	<a href="#">Abeona Therapeutics, The Transpher A Study, ABO-102</a>	Nationwide Children's Hospital, Columbus, Ohio, United States Women's and Children's Hospital, North Adelaide, South Australia, Australia Hospital Clinico Universitario de Santiago, Santiago De Compostela, Spain	AAV9	IV
<a href="#">Gene Transfer Study of ABO-102 in Patients With Middle and Advanced Phases of IIIA Disease</a>	I/II	Recruiting		Type IIIA	<a href="#">Abeona Therapeutics, Study ABT-003, ABO-102</a>	Children's Hospital of Pittsburgh, Pittsburgh, Pennsylvania, United States Adelaide Women's and Children's Hospital, North Adelaide, South Australia, Australia Hospital Clinico Universitario de Santiago, Santiago De Compostela, Spain	AAV9	IV
<a href="#">Gene Therapy With Modified Autologous Hematopoietic Stem Cells for Patients With Mucopolysaccharidosis Type IIIA</a>	I/II	Recruiting	<a href="#">December 8, 2020</a>	Type IIIA	<a href="#">Orchard Therapeutics, OTL-201</a>	Manchester University NHS Foundation Trust, Manchester, United Kingdom	Second generation self-inactivating lentiviral vector transduced autologous HSC	IV-Transfusion
<a href="#">Study of AAVrh10-h.SGSH Gene Therapy in Patients With Mucopolysaccharidosis Type IIIA (IIIA)</a>	II/III	Active, not recruiting	<a href="#">October 15, 2020</a>	Type IIIA	<a href="#">Lysogene, LYS-SAF302</a>	CHOC Children's, Orange, California, United States University of Minnesota, Minneapolis, Minnesota, United States Weill Cornell Medical College, New York, New York, United States Baylor college of medicine / Texas children's hospital, Houston, Texas, United States Armand Trousseau Public Hospital, Paris, France University Medical Center Hamburg-Eppendorf, Hamburg, Germany Amsterdam UMC, Amsterdam, Netherlands Great Ormond Street Hospital, London, United Kingdom	AAVrh10	IC
<a href="#">Gene Transfer Clinical Trial for Mucopolysaccharidosis (MPS) IIIB</a>	I/II	Recruiting	<a href="#">November 9, 2020</a>	Type IIIB	<a href="#">Abeona Therapeutics, The Transpher B Study, ABO-101</a>	Nationwide Children's Hospital, Columbus, Ohio, United States Armand-Trousseau Hospital, Paris, France Hospital Clinico Universitario de Santiago, Santiago De Compostela, Spain	AAV9	IV
<a href="#">Gene Therapy in Patients With Mucopolysaccharidosis Disease</a>	I/II	Recruiting	No Results Available	Type VI	TIGEM, MESIX, AAV2/8.TBG.hARSB	Federico II University, Napoli, Naples, Italy Erasmus Medical Center, Rotterdam, Netherlands Children's Hospital Hacettepe University, Ankara, Turkey	AAV8	IV