
Supplementary information

The clinical landscape for AAV gene therapies

In the format provided by the authors

Supplementary Table | Gene therapy trials in the analysis dataset

AAVDB	NCT Number	Title	Drug ID	Status	No of pts	Vector	Safety met	Efficacy met	Administration route	Therapeutic area	Phases	Primary Completion
1	NCT02341807	Safety and Dose Escalation Study of AAV2-hCHM in Subjects With CHM (Choroideremia) Gene Mutations	SPK-7001	Active, not recruiting	10	AAV2	Yes	No	Subretinal	Ophthalmology	Phase 1/2	01/10/2019
3	NCT02396342	Trial of AAV5-hFIX in Severe or Moderately Severe Hemophilia B	AMT-061	Active, not recruiting	10	AAV5	Yes	Yes	Intravenous	Hematology	Phase 1/2	01/05/2021
4	NCT01637805	Clinical Safety and Preliminary Efficacy of AAV-DC-CTL Treatment in Stage IV Gastric Cancer	NA	Unknown status	20	NA			NA	Oncology	Phase 1	01/10/2016
6	NCT03496012	Efficacy and Safety of AAV2-REP1 for the Treatment of Choroideremia	BIIB-111	Recruiting	14	AAV2	Yes	Yes	Subretinal	Ophthalmology	Phase 3	31/03/2020
7	NCT03252847	Gene Therapy for X-linked Retinitis Pigmentosa (XLRP) Retinitis Pigmentosa GTPase Regulator (RPGR)	A-004	Recruiting	36	AAV2	NA	NA	NA	Ophthalmology	Phase 1/2	01/11/2020
9	NCT03758404	Gene Therapy for Achromatopsia (CNGA3)	A-003	Recruiting	18	AAV2	NA	NA	NA	Ophthalmology	Phase 1/2	01/05/2021
10	NCT02418598	AADC Gene Therapy for Parkinson's Disease	AAV-hAADC-2	Terminated	2	AAV2	NA	NA	Intracranial	Neurology	Phase 1/2	31/03/2018
11	NCT03533673	AAV2/8-LSPhGAA in Late-Onset Pompe Disease	ACTUS-101	Recruiting	6	AAV2/8	NA	NA	Intravenous	Metabolic	Phase 1/2	01/12/2019
12	NCT03374202	VRC 603: A Phase I Dose-Escalation Study of the Safety of AAV8-VRC07 (VRC-HIVA070-00-GT) Recombinant AAV Vector Expressing VRC07 HIV-1 Neutralizing Antibody in Antiretroviral -Treated, HIV-1 Infected Adults With Controlled Viremia.	AA8-VRC07	Recruiting	25	AAV8	NA	NA	Intravenous	Virology	Phase 1	01/01/2020
13	NCT03001310	Gene Therapy for Achromatopsia (CNGB3)	A-002	Recruiting	27	AAV2/8	Yes	NA	Subretinal	Ophthalmology	Phase 1/2	01/02/2019
14	NCT03770572	Gene Transfer Study of AAV9-CLN3 for Treatment NCL Type 3	AAV9-CLN3	Recruiting	7	AAV9	NA	NA	Intrathecal	Neurology	Phase 1/2	01/12/2022
16	NCT01621581	AAV2-GDNF for Advanced Parkinson's Disease	AMT-090	Active, not recruiting	13	AAV2	Yes	Yes	Intracranial	Neurology	Phase 1	01/01/2022
17	NCT02240407	Re-administration of Intramuscular AAV9 in Patients With Late-Onset Pompe Disease	rAAV9-DES-hGAA	Recruiting	7	AAV9	NA	NA	Intramuscular	Metabolic	Phase 1	01/12/2019
18	NCT00229736	A Study of AAV-hAADC-2 in Subjects With Parkinson's Disease	VYAADC-01	Completed	10	AAV1	Yes	Yes but comparable to placebo in other studies	Intracranial	Neurology	Phase 1	01/03/2013
19	NCT04043104	A Phase 1/2 Open-Label, Dose Escalation Study to Determine the Optimal Dose, Safety, and Activity of AAV2hAQP1 in Subjects With Radiation-Induced Parotid Gland Hypofunction and Xerostomia	A-00X	Active, not recruiting	15	AAV2	NA	NA	Intrasalivary	Gastrointestinal	Phase 1/2	01/12/2021
21	NCT03489291	Dose Confirmation Trial of AAV5-hFIXco-Padua	AMT-061	Active, not recruiting	3	AAV5	Yes	Yes	Intravenous	Hematology	Phase 2	30/10/2018

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22	NCT01482195	Trial of Ocular Subretinal Injection of a Recombinant Adeno-Associated Virus (rAAV2-VMD2-hMERTK) Gene Vector to Patients With Retinal Disease Due to MERTK Mutations	rAAV2-VMD2-hMERTK	Recruiting	6	AAV2	Yes	Yes		Ophthalmology	Phase 1	01/08/2014
23	NCT03520712	Gene Therapy Study in Severe Haemophilia A Patients With Antibodies Against AAV5	Valrox	Enrolling by invitation	10	AAV5	NA	NA	Intravenous	Hematology	Phase 1/2	01/06/2024
24	NCT00534703	Investigation of the Safety and Feasibility of AAV1/SERCA2a Gene Transfer in Patients With Chronic Heart Failure	Mydicar	Terminated	5	AAV1	NA	No	Intracoronary	Cardiovascular	Phase 2	01/09/2015
26	NCT03507686	A Safety Study of Retinal Gene Therapy for Choroideremia	BIIB-111	Recruiting	15	AAV2				Ophthalmology	Phase 2	31/03/2020
27	NCT01208389	Phase 1 Follow-on Study of AAV2-hRPE65v2 Vector in Subjects With Leber Congenital Amaurosis (LCA) 2	Luxturna	Active, not recruiting	11	AAV2	Yes	Yes	Subretinal	Ophthalmology	Phase 1/2	01/11/2026
28	NCT01395641	A Phase I/II Clinical Trial for Treatment of Aromatic L-amino Acid Decarboxylase (AADC) Deficiency Using AAV2-hAADC	eladocogene exuparvec	Active, not recruiting	10	AAV2	Yes	Yes		Neurology	Phase 1/2	31/10/2020
31	NCT01620801	Hemophilia B Gene Therapy With AAV8 Vector	SPK-9001	Terminated	4	AAV8	Yes	Yes	Intravenous	Hematology	Phase 1	01/03/2016
32	NCT03882437	Gene Therapy for Male Patients With Danon Disease Using RP-A501; AAV9.LAMP2B	RPA-501	Recruiting	24	AAV9	NA	NA	Intravenous	Metabolic	Phase 1	01/04/2020
33	NCT03517085	Safety and Dose-Finding Study of DTX401 (AAV8G6PC) in Adults With Glycogen Storage Disease Type Ia (GSDIa)	DTX-401	Recruiting	12	AAV8	Yes	Yes		Metabolic	Phase 1/2	19/09/2020
34	NCT00999609	Safety and Efficacy Study in Subjects With Leber Congenital Amaurosis	Luxturna	Active, not recruiting	31	AAV2	Yes	Yes	Subretinal	Ophthalmology	Phase 3	01/07/2015
36	NCT00985517	Safety and Efficacy of CERE-120 in Subjects With Parkinson's Disease	CERE-120	Completed	57	AAV2				Neurology	Phase 1/2	01/01/2014
37	NCT02781480	Clinical Trial of Gene Therapy for the Treatment of Leber Congenital Amaurosis (LCA)	A-001	Completed	15	AAV2/5	Yes	Yes	Subretinal	Ophthalmology	Phase 1/2	01/12/2018
40	NCT03569891	HOPE-B: Trial of AMT-061 in Severe or Moderately Severe Hemophilia B Patients	AMT-061	Recruiting	56	AAV5	NA	NA	Intravenous	Hematology	Phase 3	01/08/2024
41	NCT00400634	Double-Blind, Multicenter, Sham Surgery Controlled Study of CERE-120 in Subjects With Idiopathic Parkinson's Disease	CERE-120	Completed	58	AAV2	Yes	No	Intracranial	Neurology	Phase 2	01/11/2008
42	NCT00252850	Safety of CERE-120 (AAV2-NTN) in Subjects With Idiopathic Parkinson's Disease	CERE-120	Completed	12	AAV2	Yes	NA	Intracranial	Neurology	Phase 1	01/03/2007
43	NCT01024998	Safety and Tolerability Study of AAV2-sFLT01 in Patients With Neovascular Age-Related Macular Degeneration (AMD)	AAV2-sFLT01	Completed	19	AAV2	Yes		Intravitreal	Ophthalmology	Phase 1	01/07/2014
44	NCT02926066	A Clinical Trial for Treatment of Aromatic L-amino Acid Decarboxylase (AADC) Deficiency Using AAV2-hAADC - An Expansion	GT-AADC	Unknown status	18	AAV2	Yes	Yes	Intracranial	Neurology	Phase 2	01/12/2018

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45	NCT03116113	A Clinical Trial of Retinal Gene Therapy for X-linked Retinitis Pigmentosa	BLIB-112	Recruiting	63	AAV8	Yes	Yes	Subretinal	Ophthalmology	Phase 2/3	01/08/2020
46	NCT01344798	Clinical Study of AAV1-gamma-sarcoglycan Gene Therapy for Limb Girdle Muscular Dystrophy Type 2C	AAV1-gamma-sarcoglycan	Completed	9	AAV1	Yes	Yes	Intramuscular	Musculoskeletal	Phase 1	01/06/2010
47	NCT03748784	ADVM-022 Gene Therapy for Wet AMD	ADVM-022	Recruiting	12	AAV7/8	Yes	NA	Intravitreal	Ophthalmology	Phase 1	01/06/2020
48	NCT02446249	Safety of a Single Administration of AAV2hAQP1, an Adeno-Associated Viral Vector Encoding Human Aquaporin-1 to One Parotid Salivary Gland in People With Irradiation-Induced Parotid Salivary Hypofunction	A-00X	Recruiting	50	AAV2	Yes	Yes	Intraperitoneal	Oncology	Phase 1/2	01/11/2022
49	NCT02852213	A Single-Stage, Adaptive, Open-label, Dose Escalation Safety and Efficacy Study of AADC Deficiency in Pediatric Patients	VYAADC-01	Recruiting	6	AAV2	Yes	Yes	Intracranial	Neurology	Phase 1	01/12/2020
50	NCT02407678	REP1 Gene Replacement Therapy for Choroideremia	BLIB-111	Active, not recruiting	30	AAV2	Yes	NA	Subretinal	Ophthalmology	Phase 2	01/08/2021
51	NCT00004533	Phase I Randomized Study of Adeno-Associated Virus-CFTR Vector in Patients With Cystic Fibrosis	AAV-CFTR	Completed	19	AAV2	Yes	No	Intranasal	Pulmonary	Phase 1	01/08/2002
52	NCT00516477	Safety Study in Subjects With Leber Congenital Amaurosis	Luxturna	Active, not recruiting	12	AAV2	Yes	Yes	Subretinal	Ophthalmology	Phase 1	01/07/2024
53	NCT00976352	Safety Study of Recombinant Adeno-Associated Virus Acid Alpha-Glucosidase to Treat Pompe Disease	rAAV1-CMV-hGAA	Completed	9	AAV1	Yes	No	Intravenous	Metabolic	Phase 1/2	01/12/2015
54	NCT00749957	Phase 1/2 Safety and Efficacy Study of AAV-RPE65 Vector to Treat Leber Congenital Amaurosis	rAAV2-CB-hRPE65	Completed	12	AAV2	Yes	Yes	Subretinal	Ophthalmology	Phase 1/2	23/09/2014
55	NCT03370172	Safety and Dose Escalation Study of an Adeno-Associated Viral Vector for Gene Transfer in Hemophilia A Subjects	TAK-754	Recruiting	10	AAV8	NA	NA	Intravenous	Hematology	Phase 1/2	30/06/2022
56	NCT02496273	Phase I Trial of CEA Specific AAV-DC-CTL Treatment in Stage IV Gastric Cancer		Active, not recruiting	60	NA	NA	NA	Ex vivo	Oncology	Phase 1	01/12/2026
57	NCT00087789	CERE-110 in Subjects With Mild to Moderate Alzheimer's Disease	CERE-110	Completed	10					Neurology	Phase 1	01/05/2010
58	NCT02553135	Choroideremia Gene Therapy Clinical Trial	BLIB-111	Completed	6	AAV2	Yes	Yes	Subretinal	Ophthalmology	Phase 2	01/02/2018
59	NCT01687608	Open-Label Single Ascending Dose of Adeno-associated Virus Serotype 8 Factor IX Gene Therapy in Adults With Hemophilia B	BAX-335	Active, not recruiting	30	AAV8	Yes	No		Hematology	Phase 1/2	28/10/2020
62	NCT03952637	Intravenous Gene Transfer With an AAV9 Vector Expressing Human α -Galactosidase in Type II GM1 Gangliosidosis	AXO-AAV-GM1	Recruiting	12	AAV9	NA	NA	Intravenous	Metabolic	Phase 1/2	30/04/2023

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63	NCT00151216	Safety Study of a Gene Transfer Vector for Children With Late Infantile Neuronal Ceroid Lipofuscinosis	AAV2CUhCLN2	Active, not recruiting	10	AAV2	Yes	No	Intracranial	Neurology	Phase 1	01/06/2020
64	NCT00643890	Study of AAV-GAD Gene Transfer Into the Subthalamic Nucleus for Parkinson's Disease	NLX-P101	Terminated	45	AAV2	Yes	Yes	Intracranial	Neurology	Phase 2	01/12/2010
65	NCT02317887	Study of RS1 Ocular Gene Transfer for X-linked Retinoschisis		Recruiting	24	AAV8	Yes	NA	Intravitreal	Ophthalmology	Phase 1/2	31/07/2023
66	NCT02651675	A Gene Therapy Study for Homozygous Familial Hypercholesterolemia (HoFH)	RGX-501	Recruiting	12	AAV8	Yes	NA	Intravenous	Metabolic	Phase 1/2	01/01/2019
67	NCT02935517	Safety and Efficacy Trial of AAV Gene Therapy in Patients With CNGA3 Achromatopsia	AGTC-402	Recruiting	24		NA	NA	Subretinal	Ophthalmology	Phase 1/2	01/08/2021
68	NCT02599922	Safety and Efficacy Trial of AAV Gene Therapy in Patients With CNGB3 Achromatopsia	rAA2tYF-PR1.7-hCNGB3	Recruiting	24	AAV2	NA	NA	Subretinal	Ophthalmology	Phase 1/2	01/06/2020
69	NCT03445715	ART-I02 in Patients With Rheumatoid Arthritis With Inflamed Wrists	ART-102	Active, not recruiting	15	AAV2/5	Yes	No	Intraarticular	Autoimmune	Phase 1	01/09/2019
70	NCT01054339	Safety & Efficacy Study of rAAV1-CB-hAAT for Alpha-1 Antitrypsin Deficiency	AGTC-0106	Completed	9	AAV1	Yes	Yes	Intramuscular	Musculoskeletal	Phase 2	01/10/2011
71	NCT02727764	A Single Dose Clinical Trial to Study the Safety of ART-I02 in Patients With Arthritis	ART-102	Active, not recruiting	12	AAV2/5	NA	NA	Intraarticular	Autoimmune	Phase 1	01/09/2022
72	NCT02416622	Safety and Efficacy of rAAV-hRS1 in Patients With X-linked Retinoschisis (XLR5)	BIIB-087	Active, not recruiting	27	AAV2	Yes	No	Intravitreal	Ophthalmology	Phase 1/2	01/04/2019
73	NCT02161380	Safety Study of an Adeno-associated Virus Vector for Gene Therapy of Leber's Hereditary Optic Neuropathy	scAAV2-P1ND4v2	Recruiting	30	AAV2	Yes	Yes	Subretinal	Ophthalmology	Phase 1	01/03/2023
74	NCT03001830	Gene Therapy for Haemophilia A.	AAV2/8-HLP-FVIII-V3	Recruiting	18	AAV2/8	Yes	Yes	Intravenous	Hematology	Phase 1	01/04/2019
75	NCT03328130	Safety and Efficacy Study in Patients With Retinitis Pigmentosa Due to Mutations in PDE6B Gene	HORAPDE-6B	Recruiting	15	AAV2/5	NA	NA	Subretinal	Ophthalmology	Phase 1/2	01/06/2020
76	NCT03199469	Gene Transfer Clinical Study in X-Linked Myotubular Myopathy	resamirigene bilparvovec	Recruiting	11	NA	Yes	Yes	Intravenous	Musculoskeletal	Phase 1/2	01/03/2024
77	NCT01937455	A Phase 1, Randomized, Blinded, Dose-escalation Study of rAAV1-PG9DP Recombinant AAV Vector Coding for PG9 Antibody in Healthy Male Adults.	rAAV1-PG9DP	Active, not recruiting	21	AAV1	Yes	No	Intramuscular	Virology	Phase 1	01/02/2018
78	NCT00454818	Efficacy and Safety Study of Genetically Targeted Enzyme Replacement Therapy for Advanced Heart Failure	Mydicar	Completed	9	AAV1	Yes	Yes	Intracoronary	Cardiovascular	Phase 1/2	01/08/2010
79	NCT01643330	A Study of Genetically Targeted Enzyme Replacement Therapy for Advanced Heart Failure	Mydicar	Completed	250	AAV1	Yes	No	Intracoronary	Cardiovascular	Phase 2	01/02/2015
80	NCT00430768	Experimental Gene Transfer Procedure to Treat Alpha 1-Antitrypsin (AAT) Deficiency	AGTC-0106	Completed	9	AAV1	Yes	Yes	Intramuscular	Musculoskeletal	Phase 1	01/01/2015

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82	NCT03061201	Dose-Ranging Study of Recombinant AAV2/6 Human Factor 8 Gene Therapy SB-525 in Subjects With Severe Hemophilia A	SB-525	Recruiting	20	AAV2/5	Yes	Yes	Intravenous	Hematology	Phase 1/2	15/11/2021
84	NCT03588299	Factor VIII Gene Therapy Study in Patients With Hemophilia A	DTX-201	Recruiting	30	NA	NA	NA	Intravenous	Hematology	Phase 1/2	16/03/2021
86	NCT02618915	Safety and Dose Finding Study of DTX101 (scAAV8OTC) in Adults With Moderate/Severe to Severe Hemophilia B	DTX-101	Terminated	6	AAVrh10	Yes	No	Intravenous	Hematology	Phase 1/2	18/10/2017
87	NCT02991144	Safety and Dose-Finding Study of DTX301 (scAAV8OTC) in Adults With Late-Onset OTC Deficiency	DTX-301	Recruiting	15	NA	Yes	NA	Intravenous	Metabolic	Phase 1/2	01/02/2021
89	NCT01966887	AAV1-CMV-Serca2a GENE Therapy Trial in Heart Failure	Mydicar	Terminated	10	AAV1	Yes	No	Intracoronary	Cardiovascular	Phase 2	01/09/2015
90	NCT00377416	Experimental Gene Transfer Procedure to Treat Alpha 1-Antitrypsin Deficiency	rAAV2-CB-hAAT	Active, not recruiting	12	AAV2	NA	NA	Intramuscular	Musculoskeletal	Phase 1	01/10/2006
91	NCT03634007	Gene Therapy for APOE4 Homozygote of Alzheimer's Disease	AAVrh. 10hAPOE2	Not yet recruiting	15	AAVrh10	NA	NA	Intracranial	Neurology	Phase 1	01/12/2020
92	NCT03223194	Gene Transfer Clinical Study in Crigler-Najjar Syndrome	AT342	Suspended	12	AAV8	Yes	NA	Intravenous	Metabolic	Phase 1/2	01/12/2019
93	NCT03066258	RGX-314 Gene Therapy for Neovascular AMD Trial	RGX-314	Recruiting	42	AAV8	Yes	Yes	Subretinal	Ophthalmology	Phase 1/2	01/11/2019
94	NCT01414985	AAVrh.10 Administered to Children With Late Infantile Neuronal Ceroid Lipofuscinosis	AAVrh. 10CUCLN2	Active, not recruiting	8	AAVrh10	NA	NA	NA	Neurology	Phase 1/2	01/12/2020
95	NCT00643747	Safety Study of RPE65 Gene Therapy to Treat Leber Congenital Amaurosis	rAAV 2/2. hRPE65p. hRPE65	Completed	12	AAV2	Yes	Yes	Subretinal	Ophthalmology	Phase 1/2	01/12/2014
96	NCT01161576	Safety Study of a Gene Transfer Vector (Rh.10) for Children With Late Infantile Neuronal Ceroid Lipofuscinosis (LINCL)	AAVrh. 10CUCLN2	Active, not recruiting	25	AAVrh10	NA	NA	NA	Neurology	Phase 1	01/12/2020
98	NCT03461289	Single-Dose Gene Replacement Therapy Clinical Trial for Patients With Spinal Muscular Atrophy Type 1	Zolgensma	Active, not recruiting	33	AAV9	Yes	NA	Intrathecal	Neurology	Phase 3	01/11/2020
99	NCT00076557	Safety of a New Type of Treatment Called Gene Transfer for the Treatment of Severe Hemophilia B	Coagulin-B	Terminated	15	AAV2	NA	NA	Intramuscular	Hematology	Phase 1/2	04/04/2007
101	NCT02346422	A Phase 1/2 Study of High-dose Genetically Targeted Enzyme Replacement Therapy for Advanced Heart Failure	Mydicar	Terminated	9	AAV1	NA	NA	Intracoronary	Cardiovascular	Phase 1/2	26/06/2015
102	NCT03466463	Gene Therapy for Severe Crigler Najjar Syndrome	GNT0003	Recruiting	17	NA	NA	NA	Intravenous	Metabolic	Phase 1/2	31/12/2020
103	NCT03612869	Study of AAVrh10-h.SGSH Gene Therapy in Patients With Mucopolysaccharidosis Type IIIA (MPS IIIA)	LYSSAF-302	Recruiting	20	AAVrh10	NA	Yes	Intracranial	Metabolic	Phase 2/3	01/01/2022

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104	NCT01973543	Safety Study of AADC Gene Therapy (VY-AADC01) for Parkinson's Disease	VYAADC-01	Active, not recruiting	15	AAV2	Yes	Yes	NA	Neurology	Phase 1	01/12/2019
106	NCT03362502	A Study to Evaluate the Safety and Tolerability of PF-06939926 Gene Therapy in Duchenne Muscular Dystrophy	PF-06939926	Enrolling by invitation	12	NA	Yes	Yes	Intravenous	Musculoskeletal	Phase 1	13/06/2020
107	NCT01461213	Gene Therapy for Blindness Caused by Choroideremia	BIIB-111	Completed	14	AAV2	Yes	Yes	Subretinal	Ophthalmology	Phase 1/2	01/10/2017
108	NCT02725580	Batten CLN6 Gene Therapy	AT-GTX-501	Active, not recruiting	14	scAAV9	Yes	Yes	Intravenous	Neurology	Phase 1/2	01/03/2022
109	NCT03370913	Single-Arm Study To Evaluate The Efficacy and Safety of Valoctocogene Roxaparvovec in Hemophilia A Patients	Valrox	Recruiting	130	AAV5	Yes	Yes	Intravenous	Hematology	Phase 3	01/12/2022
110	NCT00821340	Clinical Trial of Gene Therapy for Leber Congenital Amaurosis Caused by RPE65 Mutations	rAAV2-hRPE65	Completed	3	AAV2	NA	Yes	Subretinal	Ophthalmology	Phase 1	29/06/2016
111	NCT01801709	Intracerebral Gene Therapy for Children With Early Onset Forms of Metachromatic Leukodystrophy	AAVrh. 10cuARSA	Completed	5	AAVrh10	Yes	No	NA	Metabolic	Phase 1/2	01/03/2018
112	NCT00481546	Phase I Trial of Gene Vector to Patients With Retinal Disease Due to RPE65 Mutations	rAAV2-CBSB-hRPE65	Active, not recruiting	15	AAV2	Yes	Yes	Subfoveal	Ophthalmology	Phase 1	01/06/2026
113	NCT03392974	Single-Arm Study To Evaluate The Efficacy and Safety of Valoctocogene Roxaparvovec in Hemophilia A Patients at a Dose of 4E13 vg/kg	Valrox	Recruiting	40	AAV5	NA	NA	Intravenous	Hematology	Phase 3	01/12/2022
114	NCT03316560	Safety and Efficacy of rAAV2tYF-GRK1-RPGR in Subjects With X-linked Retinitis Pigmentosa Caused by RPGR Mutations	AGTC-501	Recruiting	30	AAV2	NA	NA	Subretinal	Ophthalmology	Phase 1/2	01/03/2023
115	NCT02695160	Ascending Dose Study of Genome Editing by Zinc Finger Nuclease Therapeutic SB-FIX in Subjects With Severe Hemophilia B	SB-FIX	Active, not recruiting	12	AAV2/6	NA	NA	Intravenous	Hematology	Phase 1	01/01/2021
116	NCT02576795	Gene Therapy Study in Severe Haemophilia A Patients	Valrox	Active, not recruiting	15	AAV5	Yes	Yes	Intravenous	Hematology	Phase 1/2	01/02/2022
117	NCT00195143	Safety Study of Subthalamic Nucleus Gene Therapy for Parkinson's Disease	NLX-P101	Completed	12	AAV2	Yes	Yes	Intracranial	Neurology	Phase 1	19/09/2005
120	NCT03369444	A Factor IX Gene Therapy Study (FIX-GT)	FLT-180a	Recruiting	24	AAVS3	Yes	Preliminary	NA	Hematology	Phase 1	01/03/2019
121	NCT03300453	Intracerebral Gene Therapy in Children With Sanfilippo Type B Syndrome	AMT-110	Active, not recruiting	4	AAV2/5	Yes	Yes	Intracranial	Metabolic	Phase 1/2	01/11/2019
122	NCT00428935	Safety Study of Mini-dystrophin Gene to Treat Duchenne Muscular Dystrophy	Biostrophin	Completed	6	AAV2/5	NA	No	Intramuscular	Musculoskeletal	Phase 1	01/03/2009
123	NCT03173521	Gene Therapy in Patients With Mucopolysaccharidosis Disease	AAV2/8.TBG.hARSB	Recruiting	10	AAV2/8	Yes	Preliminary	Intravenous	Metabolic	Phase 1/2	30/04/2020
126	NCT00073463	Safety and Efficacy of Recombinant Adeno-Associated Virus Containing the CFTR Gene in the Treatment of Cystic Fibrosis	tgAAVCF	Terminated	100	NA	NA	No	NA	Pulmonary	Phase 2/3	28/01/2008

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127	NCT00876863	Randomized, Controlled Study Evaluating CERE-110 in Subjects With Mild to Moderate Alzheimer's Disease	CERE-110	Completed	49	AAV2	Yes	No	Intracranial	Neurology	Phase 2	01/12/2017
129	NCT02671539	THOR - Tübingen Choroideremia Gene Therapy Trial	rAAV2.REP1	Active, not recruiting	6	AAV2	Yes	No	Subretinal	Ophthalmology	Phase 2	01/03/2018
130	NCT02168686	Safety Dose Finding Study of ADVN-043 Gene Therapy to Treat Alpha-1 Antitrypsin (A1AT) Deficiency	ADVN-043	Active, not recruiting	20	AAVrh10	Yes	No	Intravenous	Metabolic	Phase 1/2	01/08/2019
131	NCT02077361	An Open Label Clinical Trial of Retinal Gene Therapy for Choroideremia	BIIB-111	Completed	6	AAV2	NA	NA	NA	Ophthalmology	Phase 1/2	30/08/2017
132	NCT01519349	Follistatin Gene Transfer to Patients With Becker Muscular Dystrophy and Sporadic Inclusion Body Myositis	rAAV1.CMV.huFollistatin344	Completed	15	AAV1	Yes	Yes	Intramuscular	Musculoskeletal	Phase 1	01/10/2017
133	NCT00494195	Gene Transfer Therapy for Treating Children and Adults With Limb Girdle Muscular Dystrophy Type 2D (LGMD2D)	MYO-102	Completed	6	AAV1	Yes	Yes	Intramuscular	Musculoskeletal	Phase 1	01/06/2011
135	NCT03837184	Single-Dose Gene Replacement Therapy Using for Patients With Spinal Muscular Atrophy Type 1 With One or Two SMN2 Copies	Zolgensma	Recruiting	6	AAV9	NA	NA	Intravenous	Neurology	Phase 3	01/04/2021
136	NCT03381729	Study of Intrathecal Administration of Onasemnogene Apeparovvec-xioi for Spinal Muscular Atrophy	Zolgensma	Recruiting	28	AAV9	Yes	Yes	Intrathecal	Neurology	Phase 1	01/06/2021
137	NCT02122952	Gene Transfer Clinical Trial for Spinal Muscular Atrophy Type 1	Zolgensma	Completed	15	AAV9	Yes	Yes	Intravenous	Neurology	Phase 1	15/12/2017
139	NCT01109498	Safety and Efficacy in LPL-Deficient Subjects of AMT-011, an Adeno-Associated Viral Vector Expressing Human Lipoprotein Lipase [S447X]	Glybera	Completed	14	AAV1	Yes	Yes	Intramuscular	Metabolic	Phase 2/3	01/06/2013
140	NCT03734588	Dose-finding Study of SPK-8016 Gene Therapy in Patients With Hemophilia A to Support Evaluation in Individuals With FVIII Inhibitors	SPK-8016	Recruiting	30	LK200	NA	NA	NA	Hematology	Phase 1/2	01/04/2020
142	NCT00979238	Dose-Escalation Study Of A Self Complementary Adeno-Associated Viral Vector For Gene Transfer in Hemophilia B	FLT-180a	Active, not recruiting	14	scAAV2/8	NA	Yes	Intravenous	Hematology	Phase 1	12/06/2016
147	NCT02484092	A Gene Therapy Study for Hemophilia B	SPK-9001	Active, not recruiting	15	SPK100	Yes	Yes	Intravenous	Hematology	Phase 2	04/08/2021
149	NCT02710500	rAAVrh74.MHCK7.DYSF.DV for Treatment of Dysferlinopathies	MYO-201	Active, not recruiting	6	AAVrh74	NA	NA	Intramuscular	Musculoskeletal	Phase 1	01/07/2019
154	NCT04046224	Dose-Ranging Study of ST-920, a rAAV2/6 Human Alpha Galactosidase A Gene Therapy in Subjects With Fabry Disease	ST-920	Recruiting	18	AAV2/6	NA	NA	Intramuscular	Metabolic	Phase 1/2	28/09/2021
155	NCT03562494	VY-AADC02 for Parkinson's Disease With Motor Fluctuations	VYAADC-02	Recruiting	42	NA	NA	NA	NA	Neurology	Phase 2	01/12/2020

AAVDB	NCT Number	Title	Drug ID	Status	No of pts	Vector	Safety met	Efficacy met	Administration route	Therapeutic area	Phases	Primary Completion
156	NCT02354781	Clinical Intramuscular Gene Transfer of rAAV1.CMV.huFollistatin344 Trial to Patients With Duchenne Muscular Dystrophy	rAAV1.CMV.huFollistatin344	Completed	3	AAV1	NA	NA	NA	Musculoskeletal	Phase 1/2	01/11/2017
157	NCT02702115	Ascending Dose Study of Genome Editing by the Zinc Finger Nuclease (ZFN) Therapeutic SB-318 in Subjects With MPS I	SB-318	Active, not recruiting	3	AAV2/6	Yes	NA	NA	Metabolic	Phase 1/2	01/01/2020
162	NCT03306277	Gene Replacement Therapy Clinical Trial for Patients With Spinal Muscular Atrophy Type 1	Zolgensma	Active, not recruiting	20	scAAV9	Yes	Yes	Intravenous	Neurology	Phase 3	12/11/2019
163	NCT03505099	Pre-Symptomatic Study of Intravenous Onasemnogene Apeparovect-xioi in Spinal Muscular Atrophy (SMA) for Patients With Multiple Copies of SMN2	Zolgensma	Recruiting	27	scAAV9	NA	NA	NA	Neurology	Phase 3	01/10/2020
164	NCT03580083	RGX-111 Gene Therapy in Patients With MPS I	RGX-111	Recruiting	5	AAV9	NA	NA	Intracranial	Metabolic	Phase 1	01/01/2020
165	NCT03368742	Microdystrophin Gene Transfer Study in Adolescents and Children With DMD	SGT-001	Recruiting	16	AAV9	Yes	NA	Intravenous	Musculoskeletal	Phase 1/2	01/03/2020
166	NCT03566043	RGX-121 Gene Therapy in Patients With MPS II (Hunter Syndrome)	RGX-121	Recruiting	6	AAV9	NA	NA	NA	Metabolic	Phase 1/2	01/08/2019
167	NCT02376816	Clinical Intramuscular Gene Transfer Trial of rAAVrh74.MCK.Micro-Dystrophin to Patients With Duchenne Muscular Dystrophy	SRP-9001	Completed	2	AAVrh74	NA	NA	NA	Musculoskeletal	Phase 1	01/09/2017
168	NCT03315182	Gene Transfer Clinical Trial for Mucopolysaccharidosis (MPS) IIIB	ABO-101	Recruiting	9	AAV9	Yes	NA	Intravenous	Metabolic	Phase 1/2	01/10/2020
169	NCT03333590	Gene Transfer Clinical Trial to Deliver rAAVrh74.MCK.GALGT2 for Duchenne Muscular Dystrophy	rAAVrh74.MCK.GALGT2	Active, not recruiting	6	AAVrh74	Yes	NA	NA	Musculoskeletal	Phase 1/2	01/11/2020
170	NCT04040049	A Fabry Disease Gene Therapy Study	FLT-190	Recruiting	NA	AAV8	NA	NA	Intravenous	Metabolic	Phase 1/2	01/03/2021
171	NCT03846193	First in Human Study to Evaluate the Safety and Efficacy of GT005 Administered in Subjects With Dry AMD	GT005	Recruiting	10	NA	NA	NA	NA	Ophthalmology	Phase 1/2	01/02/2021
172	NCT02716246	Phase I/II Gene Transfer Clinical Trial of scAAV9.U1a.hSGSH	ABO-102	Recruiting	22	scAAV9	Yes	NA	Intravenous	Metabolic	Phase 1/2	01/12/2019
175	NCT02790723	Safety of Intra-Articular Sc-rAAV2.5IL-1Ra in Subjects With Moderate Knee OA	AAV2IL-1Ra	Recruiting	9	AAV2/5	NA	NA	Intraarticular	Musculoskeletal	Phase 1	01/05/2021
176	NCT03153293	Safety and Efficacy Study of Gene Therapy for The Treatment of Leber's Hereditary Optic Neuropathy	AAV2-ND4	Active, not recruiting	142	NA	NA	NA	Intravitreal	Ophthalmology	Phase 2/3	30/06/2019
178	NCT03520751	Phase I/IIa Trial of scAAV1.tMCK.NTF3 for Treatment of CMT1A	scAAV1.tMCK.NTF3	Not yet recruiting	9	scAAV1	NA	NA	Intramuscular	Neurology	Phase 1/2	01/04/2020
179	NCT03375164	Systemic Gene Delivery Clinical Trial for Duchenne Muscular Dystrophy	SRP-9001	Active, not recruiting	4	AAVrh74	NA	NA	Intramuscular	Musculoskeletal	Phase 1/2	01/04/2021
182	NCT03041324	Ascending Dose Study of Genome Editing by the Zinc Finger Nuclease (ZFN) Therapeutic SB-913 in Subjects With MPS II	SB-913	Active, not recruiting	9	AAV2/6	Yes	No	NA	Metabolic	Phase 1/2	01/02/2021

AAVDB	NCT Number	Title	Drug ID	Status	No of pts	Vector	Safety met	Efficacy met	Administration route	Therapeutic area	Phases	Primary Completion
183	NCT00126724	Study of Intra-articular Delivery of tgAAC94 in Inflammatory Arthritis Subjects	tgAAC-94	Completed	120	AAV2	NA	No	Intraarticular	Autoimmune	Phase 1/2	01/10/2008
187	NCT02082860	Phase I Gene Therapy Clinical Trial Using the Vector rAAV2/5-PBGD for the Treatment of Acute Intermittent Porphyria	AMT-021	Completed	8	AAV2/5	Yes	No	NA	Hematology	Phase 1	01/07/2014
188	NCT01899092	Safety and Efficacy Study of Single Doses of TT-034 in Patients With Chronic Hepatitis C	TT-034	Completed	9	NA	Yes	No	Intravenous	Virology	Phase 1/2	01/09/2016
189	NCT02652767	Efficacy Study of GS010 for the Treatment of Vision Loss up to 6 Months From Onset in LHON Due to the ND4 Mutation	GS-010	Active, not recruiting	36	AAV2	Yes	No	Intravitreal	Ophthalmology	Phase 3	01/01/2019
190	NCT02652780	Efficacy Study of GS010 for Treatment of Vision Loss From 7 Months to 1 Year From Onset in LHON Due to the ND4 Mutation	GS-010	Completed	37	AAV2	Yes	No	Intravitreal	Ophthalmology	Phase 3	01/01/2018
191	NCT03003533	A Gene Transfer Study for Hemophilia A	SPK-8011	Recruiting	30	LK200	Yes	Yes	NA	Hematology	Phase 1/2	01/08/2019
194	NCT03652259	Gene Transfer Clinical Trial for LGMD2E (Beta-sarcoglycan Deficiency) Using scAAVrh74.MHCK7.hSGCB	MYO-101	Recruiting	9	AAVrh74	Yes	yes	Intravenous	Musculoskeletal	Phase 1/2	31/12/2020
195	NCT01976091	Gene Transfer Clinical Trial for LGMD2D (Alpha-sarcoglycan Deficiency) Using scAAVrh74.tMCK.hSGCA	MYO-102	Completed	6	AAVrh74	Yes	NA	Intravenous	Musculoskeletal	Phase 1/2	14/03/2019
196	NCT03293524	Efficacy & Safety Study of Bilateral IVT Injection of GS010 in LHON Subjects Due to the ND4 Mutation for up to 1 Year	GS-010	Active, not recruiting	90	AAV2	NA	NA	Intravitreal	Ophthalmology	Phase 3	30/06/2020
197	NCT00891306	Efficacy and Safety of Human Lipoprotein Lipase (LPL)[S447X] Expressed by an Adeno-Associated Viral Vector in LPL-deficient Subjects	Glybera	Completed	6	AAV1	Yes	yes	Intramuscular	Metabolic	Phase 2/3	01/08/2010
199	NCT03065192	Safety and Efficacy Study of VY-AADC01 for Advanced Parkinson's Disease	VYAADC-01	Active, not recruiting	16	AAV2	Yes	Yes	Intracranial	Neurology	Phase 1	01/12/2021
201	NCT01494805	Safety and Efficacy Study of rAAV.sFlt-1 in Patients With Exudative Age-Related Macular Degeneration	AVA-101	Completed	40	AAV2	NA	NA	NA	Ophthalmology	Phase 1/2	01/05/2017
206	NCT03952156	Gene Therapy Clinical Study in Adult PKU	HMI-102	Recruiting	21	AAVHSC15	NA	NA	Intravenous	Metabolic	Phase 1/2	01/06/2021
207	NCT03326336	Dose-escalation Study to Evaluate the Safety and Tolerability of GS030 in Subjects With Retinitis Pigmentosa	GS-030	Recruiting	18	AAV2/7m8	NA	NA	NA	Ophthalmology	Phase 1/2	01/09/2020
211	NCT00617032	Phase 1 Dose Escalation Study of Intra-Articular Administration of tgAAC94		Completed	15	AAV2	Yes	No	NA	Autoimmune	Phase 1	01/11/2005
213	NCT01474343	Intracerebral Gene Therapy for Sanfilippo Type A Syndrome	LYSSAF-301	Completed	4	AAVrh10	Yes	NA	NA	Metabolic	Phase 1/2	01/05/2013