



Gene Therapy assets have a total of 44 catalyst events upcoming in the near term, specifically for: TSHA-102, PBGENE-PMM, Ixoberogene soroparovec, TN-201, TN-401, SGT-003, Quaratusugene ozeplasmid, ECUR-506, PBFT-02, 4D-175, Renizgamglogene autogedtemcel, BEAM-302, ABBV-RGX-314, BEAM-101, KB-301, KB-408, NTLA-2001, NTLA-2002, NTLA-3001, UX-701, Pariglasgene breccaparovec, PBGENE-HBV, BEAM-301, Rebisufligene etisparovec, 4D-710, 4D-150, IMNN-001, OCU-400, VERVE-102, VERVE-201

Asset	MoA	Company	1Q24	2Q24	3Q24	4Q24	1Q25	2Q25	3Q25	4Q25
4D-150	P2: Gene Therapy in Wet Macular Degeneration	4DMT			Data Report					
4D-175	P1: Gene Therapy in Geographic Atrophy Macular Edema Edema	4DMT		IND Submission						
4D-710	P1: Gene Therapy in Cystic Fibrosis	4DMT			Data Report					
4D-710	P2: Gene Therapy in Cystic Fibrosis	4DMT				Study Start				
ABBV-RGX-314	P2: Gene Therapy in Wet Macular Degeneration	REGENXBIO		Data Report						
BEAM-101	P1: Gene Therapy in Anemia, Sickle Cell	Beam Therapeutics			Data Report					
BEAM-101	P1: Gene Therapy in Anemia, Sickle Cell	Beam Therapeutics	Study Start							
BEAM-301	P1: Gene Therapy in Glycogen Storage Disease Type Ia	Beam Therapeutics	IND Submission							
BEAM-302	P1: Gene Therapy in alpha 1-Antitrypsin Deficiency	Beam Therapeutics	Study Start							
ECUR-506	P1: Gene Therapy in Hepatitis B, Chronic	PRECISION BIOSCIENCES				IND Submission				
IMNN-001	P2: IL12 in Ovarian Cancer	IMUNON				Data Report				
Ixoberogene soroparovec	P2: Gene Therapy in Wet Macular Degeneration	ADVERUM				Data Report				
Ixoberogene soroparovec	P3: Gene Therapy in Wet Macular Degeneration	ADVERUM					Study Start			
KB-301	P1: Gene Therapy in Depressive Disorder	Krystal	Data Report							
KB-408	P2: Gene Therapy in alpha 1-Antitrypsin Deficiency	Krystal			Data Report					
NTLA-2001	P3: Gene Therapy in Transthyretin Amyloidosis	Inteia Therapeutics	Study Start							
NTLA-2002	P3: Gene Therapy in Angioedemas, Hereditary	Inteia Therapeutics			Study Start					
NTLA-3001	P1: Gene Therapy in alpha 1-Antitrypsin Deficiency	Inteia Therapeutics				Study Start				
OCU-400	P3: Gene Therapy in Retinitis Pigmentosa	ocugen		Study Start						
PBFT-02	P1: Gene Therapy in Dementia	PassageBio			Data Report					
PBFT-02	P1: Gene Therapy in Dementia	PassageBio	Study Start							
PBGENE-HBV	P1: Gene Therapy in Hepatitis B	PRECISION BIOSCIENCES				IND Submission				
PBGENE-PMM	P1: Gene Therapy in Mitochondrial Myopathies	PRECISION BIOSCIENCES								IND Submission
Pariglasgene breccaparovec	P3: Gene Therapy in Glycogen Storage Disease Type Ia	ultragenyx	Data Report							
Quaratusugene ozeplasmid	P1: Gene Therapy in Non-Small-Cell Lung Cancer	Genprex			Study Start					
Rebisufligene etisparovec	P1: Gene Therapy in Sanfilippo Syndrome	ultragenyx	Data Report							

Renizgamlogene autogedtemcel	P2: Gene Therapy in Hemoglobinopathies Anemia, Sickle Cell					Data Report				
SGT-003	P1: Gene Therapy in Duscular Dystrophy, Duchenne					Data Report				
SGT-003	P1: Gene Therapy in Muscular Dystrophy, Duchenne			Study Start						
SGT-003	P1: Gene Therapy in Muscular Dystrophies Muscular Dystrophy, Duchenne			Study Start						
TN-201	P1: Gene Therapy in Cardiomyopathy, Hypertrophic					Data Report				
TN-401	P1: Gene Therapy in Arrhythmogenic Cardiomyopathies					Study Start				
TSHA-102	P1: Gene Therapy in Rett Syndrome			Study Start						
TSHA-102	P1: Gene Therapy in Rett Syndrome					Data Report				
TSHA-102	P1: Gene Therapy in Rett Syndrome		Study Start							
UX-701	P3: Gene Therapy in Wilson Disease					Data Report				
UX-701	P3: Gene Therapy in Wilson Disease		Data Report							
VERVE-102	P1: Gene Therapy in Hypercholesterolemia			Study Start						
VERVE-102	P2: Gene Therapy in Hypercholesterolemia									Study Start
VERVE-201	P1: Gene Therapy in Hypercholesterolemia					Study Start				

Asset	Biological Target	Phase	Ticker	Indications	Catalyst Date	Catalyst Type
TSHA-102	Gene Therapy,MECP2	P1	TSHA	Rett Syndrome	2Q24	Study Start
TSHA-102	Gene Therapy,MECP2	P1	TSHA	Rett Syndrome	1Q24	Study Start
PBGENE-PMM	Gene Therapy,PMM	P1	DTIL	Mitochondrial Myopathies	YE25	IND Submission
Ixoberogene soroparvec	Gene Therapy	P2	ADVM	Wet Macular Degeneration	YE24	Data Report
TN-201	Gene Therapy,MYBPC3	P1	TNYA	Cardiomyopathy, Hypertrophic	2H24	Data Report
Ixoberogene soroparvec	Gene Therapy	P3	ADVM	Wet Macular Degeneration	1H25	Study Start
TN-401	Gene Therapy,PKP2	P1	TNYA	Arrhythmogenic Cardiomyopathies	2H24	Study Start
SGT-003	Gene Therapy,DMD	P1	SLDB	Duscular Dystrophy, Duchenne	4Q24	Data Report
SGT-003	Gene Therapy,DMD	P1	SLDB	Muscular Dystrophy, Duchenne	2Q24	Study Start
Quaratusugene ozeplasmid	Gene Therapy,TUSC2	P1	GNPX	Non-Small-Cell Lung Cancer	2H24	Study Start
ECUR-506	Gene Therapy,PCK9	P1	DTIL	Hepatitis B, Chronic	YE24	IND Submission
PBFT-02	Gene Therapy,Progranulin	P1	PASG	Dementia	2H24	Data Report
PBFT-02	Gene Therapy,Progranulin	P1	PASG	Dementia	1H24	Study Start
4D-175	Gene Therapy,CFH	P1	FDMT	Geographic Atrophy Macular Edema Edema	2Q24	IND Submission
TSHA-102	Gene Therapy,MECP2	P1	TSHA	Rett Syndrome	2H24	Data Report
Renizgamlogene autogedtemcel	Gene Therapy,CD34	P2	EDIT	Hemoglobinopathies Anemia, Sickle Cell	YE24	Data Report
BEAM-302	Gene Therapy,SERPINA1	P1	BEAM	alpha 1-Antitrypsin Deficiency	1H24	Study Start
ABBV-RGX-314	Gene Therapy,VEGF	P2	RGNX	Wet Macular Degeneration	2Q24	Data Report
BEAM-101	Gene Therapy,CD34	P1	BEAM	Anemia, Sickle Cell	1H24	Study Start
KB-301	Gene Therapy,COL3A1	P1	KRYS	Depressive Disorder	1H24	Data Report
KB-408	Gene Therapy	P2	KRYS	alpha 1-Antitrypsin Deficiency	2H24	Data Report
NTLA-2001	Gene Therapy,TTR	P3	NTLA	Transthyretin Amyloidosis	1Q24	Study Start
NTLA-2002	Gene Therapy,KLKB1	P3	NTLA	Angioedemas, Hereditary	2H24	Study Start
NTLA-3001	Gene Therapy,SERPINA1	P1	NTLA	alpha 1-Antitrypsin Deficiency	YE24	Study Start
UX-701	Gene Therapy,ATP7B	P3	RARE	Wilson Disease	2H24	Data Report
Pariglasgene breccaparvec	Gene Therapy,G6PC	P3	RARE	Glycogen Storage Disease Type Ia	1H24	Data Report
PBGENE-HBV	Gene Therapy,HBV	P1	DTIL	Hepatitis B	YE24	IND Submission
UX-701	Gene Therapy,ATP7B	P3	RARE	Wilson Disease	1H24	Data Report

Asset	Biological Target	Phase	Ticker	Indications	Catalyst Date	Catalyst Type
BEAM-101	Gene Therapy,CD34	P1	BEAM	Anemia, Sickle Cell	2H24	Data Report
BEAM-301	Gene Therapy,G6PC	P1	BEAM	Glycogen Storage Disease Type Ia	1H24	IND Submission
Rebisufligene etisparvovec	Gene Therapy,SGSH	P1	RARE	Sanfilippo Syndrome	1H24	Data Report
4D-710	Gene Therapy,CFTR	P1	FDMT	Cystic Fibrosis	3Q24	Data Report
4D-150	Gene Therapy,VEGF	P2	FDMT	Wet Macular Degeneration	2H24	Data Report
IMNN-001	IL12 Agonist	P2	IMNN	Ovarian Cancer	YE24	Data Report
4D-710	Gene Therapy,CFTR	P2	FDMT	Cystic Fibrosis	4Q24	Study Start
SGT-003	Gene Therapy,DMD	P1	SLDB	Muscular Dystrophies Muscular Dystrophy, Duchenne	2Q24	Study Start
OCU-400	Gene Therapy,NR2E3	P3	OCGN	Retinitis Pigmentosa	2Q24	Study Start
VERVE-102	Gene Therapy,PCSK9	P2	VERV	Hypercholesterolemia	YE25	Study Start
VERVE-102	Gene Therapy,PCSK9	P1	VERV	Hypercholesterolemia	2Q24	Study Start
VERVE-201	Gene Therapy,ANGPTL3	P1	VERV	Hypercholesterolemia	2H24	Study Start