

THERAPIES IN THE PIPELINE

ANTIBODY TREATMENTS

Disease	Drug	Phase	Organization	Description
ALS	AL001	2	Alector/GlaxoSmithKline	Anti-sortilin (SORT1)
ALS	AP-101	2	AL-S Pharma AG/Neurimmune	Anti-SOD1
ALS	ANX005 (IV)	2	Annexon Biosciences	Anti-C1q
ALS	Actemra (tocilizumab)	2	Genentech	Anti-IL6 receptor
DMD	Pamrevlumab	3	Fibrogen	Anti-CTGF
DMD	Canakinumab (ILARIS)	1/2	Children's National Research Institute	Anti-IL1 β
FSHD	AOC 1020	1/2	Avidity Biosciences	Anti-DUX4 (siRNA-Ab conjugate)
FSHD	RO7204239	2	Hoffmann-La Roche	Anti-myostatin
MG	ALXN1830	1	AstraZeneca	FcRn blocker

Disease	Drug	Phase	Organization	Description
MG	gefurulumab (ALXN1720)	3	AstraZeneca	C5 complement inhibitor
MG	Rituximab	2	Genentech/Roche	Anti-CD20
MG	TAK-079	2	Takeda	Anti-CD38
MG	HBM9161 (Batoclimab)	3	Harbour BioMed	Anti-FcRn
MG	inebilizumab	3	Horizon Therapeutics/Viela	Anti-aquaporin-4 (AQP4)
MG	Nipocalimab (M281)	3	Janssen/Johnson & Johnson	Anti-FcRn
MG	satralizumab	3	Roche	Anti-AQP4
SMA	RO7204239/GYM329	3	Roche-Genentech	Anti-myostatin
SMA	SRK-015 (Apitegromab)	3	Scholar Rock	Anti-promyostatin

CELL THERAPY

Disease	Drug	Phase	Organization	Description
ALS	CK0803	1	Cellenkos	Tregulatory cells
ALS	HYNRCS-Allo	1	Corestem	Stem cells
ALS	NeuroNata-R	3	Corestem	Stem cells
ALS	MSCs	2	Mayo Clinic	Stem cells
ALS	NSI-566	2	Palisades Bio	SC-derived neural stem cells
ALS	RAPA-501	1	RapaTherapeutics	Tregulatory cells

Disease	Drug	Phase	Organization	Description
CMT	CLZ-2002	1	Cellatoz Therapeutics	MSC-derived schwann cells
CMT	EN001	1	ENCell	WJ-MSCs
DMD	DT-DEC01	1	Dystrogen	Chimeric cells (dystrophin)
DMD	CAP-1002	3	Capricor	Cardiosphere-derived cells
MG	Descartes-8	1/2	Cartesian Therapeutics	rCAR-T
MG	MuSK-CAART	1	Cabaletta Bio	CART-19

GENE THERAPY

Disease	Drug	Phase	Organization	Description
ALS	APB-102	1/2	Apic Bio/UniQure	<i>SOD1</i> gene
ALS	Engensis	2	Helixmith	<i>HGF</i> gene
CMT	VM202	1/2	Helixmith	<i>HGF</i> gene
DMD	RGX202	1/2	Regenxbio	Micro-dystrophin
DMD	PF-06939926	3	Pfizer	Mini-dystrophin
DMD	rAAVrh74.MCK.GALGT2	1/2	Nationwide Children's Hospital	<i>GALGT2</i> gene
FA	LX2006	1/2	LEXEO Therapeutics/Adverum Biotechnologies	<i>FXN</i> gene
LGMD	ATA-200	1/2	Atamyo Therapeutics	<i>FKRP</i> gene
LGMD	SRP-9003	1/2	Sarepta	β -sarcoglycan gene

Disease	Drug	Phase	Organization	Description
LGMD	SRP-6004	1	Sarepta	Dysferlin gene
LGMD	LION-101	1/2	AskBio (Bayer)	<i>FKRP</i> gene
Myopathies	AT132	1/2	Astellas Gene Therapies	<i>MTM1</i> gene (for XLMTM)
Pompe	ACTUS-101(AAV2/8LSPhGAA)	1/2	Actus Therapeutics/AskBio	<i>GAA</i> gene
Pompe	AT845	1/2	Astellas Gene Therapies/Audentes	<i>GAA</i> gene
Pompe	SPK-3006	1/2	Spark Therapeutics/Roche	<i>GAA</i> gene
Pompe	rAAV9-DES-hGAA	1	Lacerta Therapeutics	<i>GAA</i> gene
SMA	EXG001-307	1/2	Exegensis/Hangzhou Jiayin Biotech Ltd	<i>SMN1</i> gene
SMA	GC101	1/2	GeneCradle Therapeutics	<i>SMN1</i> gene (for SMA2)

RNA-TARGETED THERAPIES

Disease	Drug	Phase	Organization	Description
ALS	AROSOD-1	1	Arrowhead Pharmaceuticals	RNAi-SOD1
ALS	BIIB105/ION541	1/2	Biogen/Ionis	ASO-ATXN2
ALS	ION363	3	Ionis	ASO-FUS
ALS	QRL-201	1	QurAlis	ASO-STMN2
ALS	RAG-17	1	Ractigen Therapeutics	siRNA-SOD1
DMD	AOC-1044	1/2	Avidity Biosciences	Exon 44 skipping
DMD	DS-5141	1/2	Daiichi Sankyo	Exon 45 skipping
DMD	DYNE-251	1/2	Dyne Therapeutics	Exon 51 skipping

Disease	Drug	Phase	Organization	Description
DMD	Vesleteplirs(en exon 51)	2	Sarepta	Exon 51 skipping
DMD	SQY51	1/2	SQY Therapeutics	Exon 51 skipping
DMD	WVE-N531	1/2	Wave	Exon 53 skipping
DMD	ATL1102	2	Antisense Therapeutics	ASO-inhibitor of CD49d
DMD	PGN-EDO51	1	PepGen	Exon 51 skipping
DM	AOC 1001	1/2	Avidity Biosciences	siRNA-TfR1
DM	DYNE-101	1/2	Dyne	ASO-DMPK
SMA	BIIB115	1/2	Biogen/Ionis	ASO-STMN2

SMALL MOLECULE DRUGS

Disease	Drug	Phase	Organization	Description
ALS	Masitinib	3	AB Science	Tyrosine kinase inhibitor
ALS	CORT113176 (Dazucorilant)	2	Corcept Therapeutics	Glucocorticoid modulator
ALS	SPG-302	1	Spinogenix	Synapse regeneration
BMD	Vamorolone	2	ReveraGen BioPharma	MR antagonist
CMT	IFB-088	1/2	InFlectis Bioscience	UPR activator
CMT	FLX-787	2	Flex Pharma	TRP ion channel agonist
CMD	Omigapil	1	Santhera Pharmaceuticals	Anti-apoptotic agent
DM	Tideglusib	2/3	AMO Pharma Limited	GSK-3 inhibitor
DM	Mexiletine	3	Lupin	Sodium channel blocker
DMD	Tamoxifen	3	University Hospital, Basel	SERM modulator
DMD & BMD	EDG-5506	2	Edgewise Therapeutics	Muscle stabilizer
DMD & BMD	Givinostat	1/2	Italfarmaco	HDAC inhibitor

Disease	Drug	Phase	Organization	Description
FA	MIN-102 (leriglitazone)	2	Minoryx Therapeutics	PPAR gamma agonist
FA	Elamipretide	1/2	Children's Hospital of Philadelphia	Reduces oxidative stress
FSHD	Iosmapimod	3	Fulcrum Therapeutics	p38 MAPK inhibitor
LGMD	Ribitol(BBP-418)	3	ML Bio Solutions/BridgeBio	Precursor to alpha-dystroglycan
MG	Mysaterix	2	CuraVac	Antigen receptor
MG	amifampridine	3	Catalyst	K channel blocker
Myopathies	ARM210	1	ARMGO Pharma	RyR1
Myopathies	REN001 (Mavodelpar)	2/3	Reneo Pharmaceuticals	PMM
Pompe	MZE001	1	Maze Therapeutics/Sanofi	Substrate reduction/stabilizer
SBMA	NIDO-361	1	Nido Biosciences	Androgen receptor BF3
SBMA	AJ201	2	AnnJi Pharmaceutical/Avenue Therapeutics	Nrf1 and Nrf2 activation
SMA	reldesemtiv	2	Cytokinetics	Troponin activator