

Medicines in Development – Cell Therapy and Gene Therapy

Alzheimer's Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AAV-hTERT (AAV-based gene therapy)	Libella Gene Therapeutics Manhattan, KS	Alzheimer's disease	Phase I www.libellagenetherapeutics.com
allogeneic mesenchymal stem cell therapy (itMSCs)	Stemmedica Cell Technologies San Diego, CA	Alzheimer's disease	Phase II www.stemedica.com
AstroStem autologous adipose tissue-derived stem cell therapy	Nature Cell Seoul, South Korea	Alzheimer's disease	Phase I/II completed www.stemcellbio.com
HB-adMSC (autologous adipose-derived mesenchymal stem cell therapy)	Hope Biosciences Sugar Land, TX	Alzheimer's disease	Phase I/II www.hope.bio
LMSC (mesenchymal stem cell therapy)	Longeveron Miami, FL	Alzheimer's disease	Phase I www.longeveron.com

Arthritis/Musculoskeletal Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AB-205 (genetically-engineered human cells derived from human umbilical vein tissue)	Angiocrine Bioscience San Diego, CA	arthroscopic rotator cuff repair	Phase I/II www.angiocrinebioscience.com
Cartistem® allogeneic mesenchymal stem cell therapy	Medipost America Seoul, South Korea	osteoarthritis of the knee	Phase I/II completed www.medi-post.com
CybroCell™ human dermal fibroblast-based cell therapy	FibroGenesis Houston, TX	intervertebral disc degeneration	Phase II www.fibro-genesis.com
HB-adMSC (autologous adipose-derived mesenchymal stem cell therapy)	Hope Biosciences Sugar Land, TX	rheumatoid arthritis	Phase I/II www.hope.bio
humantakinogene hadenovec (FX201) (gene therapy)	Flexion Therapeutics Burlington, MA	osteoarthritis	Phase I www.flexiontherapeutics.com
IDCT (allogeneic injectable discogenic cell therapy)	DiscGenics Salt Lake City, UT	lumbar degenerative disc disease (Fast Track)	Phase I/II www.discgenics.com

Arthritis/Musculoskeletal Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
JointStem mesenchymal stem cell therapy	Nature Cell Seoul, South Korea	osteoarthritis of the knee	Phase II completed www.stemcellbio.com
MACI (autologous cultured chondrocytes on a porcine collagen membrane)	Vericel Cambridge, MA	chondral or osteochondral defects of the knee (10-17 years)	Phase III www.vcel.com
Novocart® 3D autologous cartilage cells/ collagen matrix	Aesculap Biologics Breinigsville, PA	cartilage disorders	Phase III www.aesculapbiologics.com
PLX-PAD (emiplacel) (human placental stromal cell therapy)	Pluristem Therapeutics Haifa, Israel	muscle injury following arthroplasty for hip fracture	Phase III www.pluristem.com
remestemcel-L (mesenchymal stem cell therapy)	Mesoblast New York, NY	osteoarthritis/cartilage repair	Phase II www.mesoblast.com
rexlemestrocel-L (MPC-300-IV) (mesenchymal precursor cell therapy)	Mesoblast New York, NY	rheumatoid arthritis	Phase II www.mesoblast.com
tonogenchoncel-L (genetically-modified cell therapy)	Kolon TissueGene Rockville, MD	osteoarthritis of the knee	Phase III www.tissuegene.com

Arthritis/Musculoskeletal Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
XT-150 (DNA plasmid/IL-10 transgene gene therapy)	Xalud Therapeutics San Francisco, CA	osteoarthritis of the knee	Phase II www.xaludthera.com

Autoimmune Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ATA188 (allogeneic T cell immunotherapy)	Atara Biotherapeutics South San Francisco, CA	progressive multiple sclerosis	Phase I www.atarabio.com
ATA190 (EBV-specific T cell immunotherapy)	Atara Biotherapeutics South San Francisco, CA	progressive multiple sclerosis	Phase I www.atarabio.com
BCMA-CD19 cCAR-T cells (compound CAR-T cell therapy)	iCell Gene Therapeutics Stony Brook, NY	autoimmune disorders	Phase I www.icellgene.com
Descartes-08 (anti-BCMA CAR-T cell therapy)	Cartesian Therapeutics Rockville, MD	myasthenia gravis	Phase I/II www.cartesiantherapeutics.com
FCX-013 (fibroblast cell therapy) ORPHAN DRUG	Castle Creek Pharmaceuticals Basking Ridge, NJ Fibrocell Science Exton, PA	localized scleroderma (Fast Track)	Phase I/II www.castlecreekpharma.com www.fibrocell.com

Autoimmune Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
NurOwn® neurotrophic factor-producing mesenchymal stem cell therapy	BrainStorm Cell Therapeutics New York, NY	progressive multiple sclerosis	Phase II www.brainstorm-cell.com

Bladder Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AMDC (autologous muscle-derived stem cell therapy)	Cook MyoSite Pittsburgh, PA	stress urinary incontinence	Phase III www.cookmysite.com
		underactive bladder	Phase I/II www.cookmysite.com
URO-902 (hMaxi-K gene therapy)	Urovant Sciences Irvine, CA	overactive bladder	Phase II www.urovant.com

Blood Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AMT-060 (AAV-based gene therapy) ORPHAN DRUG	uniQure Lexington, MA	hemophilia B	Phase I/II www.uniqure.com

Blood Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AMT-061 (AAV-based gene therapy)	uniQure Lexington, MA	hemophilia B	Phase III www.uniqure.com
ARU-1801 (gamma-globin gene transfer) ORPHAN DRUG	Aruvant Sciences Basel, Switzerland	sickle cell anemia	Phase I/II www.aruvant.com
BAX-335 (AAV-based gene therapy) ORPHAN DRUG	Takeda Deerfield, IL	hemophilia B	Phase I/II www.takeda.com
BAY2599023 (DTX 201) (AAV-based gene therapy) ORPHAN DRUG	Bayer Pharmaceuticals Whippany, NJ	hemophilia A	Phase I/II www.pharma.bayer.com
BIVV003 (ex vivo ZFN gene-edited cell therapy)	Sangamo Therapeutics Richmond, CA Sanofi Bridgewater, NJ	sickle cell disease	Phase I www.sangamo.com www.sanofi.com
CSL200 (γ -globin gene therapy)	CSL Behring King of Prussia, PA	sickle cell anemia	Phase I www.cslobehring.com

Blood Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CTX001 (autologous CRISPR/Cas9 gene edited hematopoietic stem cell therapy)	CRISPR Therapeutics Cambridge, MA Vertex Pharmaceuticals Boston, MA	beta thalassemia, sickle cell disease (Fast Track)	Phase I/II www.crisprtx.com www.vrtx.com
fidanacogene elaparvovec (AAV-based gene therapy) ORPHAN DRUG	Pfizer New York, NY Spark Therapeutics Philadelphia, PA	hemophilia B (Breakthrough Therapy)	Phase III www.pfizer.com www.sparktx.com
LentiGlobin™ beta-globin gene therapy ORPHAN DRUG	bluebird bio Cambridge, MA	transfusion-dependent beta-thalassemia (Fast Track) (Breakthrough Therapy)	Phase III www.bluebirdbio.com
		sickle cell disease (Fast Track)	Phase I/II www.bluebirdbio.com
OTL-300 (ex vivo lentiviral gene therapy)	Orchard Therapeutics Boston, MA	transfusion-dependent beta thalassemia	Phase I/II www.orchard-tx.com
PF-07055480 (SB-525) (AAV-based gene therapy) ORPHAN DRUG	Pfizer New York, NY Sangamo Therapeutics Richmond, CA	hemophilia A (Fast Track)	Phase II www.pfizer.com

Blood Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
RIVO-CEL™ rivogenlecleucel (BPX-501) ORPHAN DRUG	Bellicum Pharmaceuticals Houston, TX	inherited blood disorders	Phase I www.bellicum.com
romyelocel-L (allogeneic myeloid progenitor cell therapy)	Cellerant Therapeutics San Carlos, CA	neutropenia in AML	Phase II completed www.cellerant.com
RP-L102 (lentivirus-based gene therapy) ORPHAN DRUG	Rocket Pharmaceuticals New York, NY	Fanconi's anemia (Fast Track)	Phase II www.rocketpharma.com
RP-L301 (lentiviral-based gene therapy) ORPHAN DRUG	Rocket Pharmaceuticals New York, NY	pyruvate kinase deficiency (Fast Track)	Phase I/II www.rocketpharma.com
SB-FIX (in vivo gene editing) ORPHAN DRUG	Sangamo Therapeutics Richmond, CA	hemophilia B (Fast Track)	Phase I/II www.sangamo.com
SPK-8011 (factor VIII gene therapy) ORPHAN DRUG	Spark Therapeutics Philadelphia, PA	hemophilia A	Phase III www.sparktx.com

Cancer**Drug Name****Sponsor****Indication****Development Phase**

AB-205 (genetically-engineered human cells derived from human umbilical vein tissue)	Angiocrine Bioscience San Diego, CA	relapsing and/or refractory lymphoma (+HDT-ASCT)	Phase I/I www.angiocrinebioscience.com
		life-threatening leukemia, myelodysplastic syndromes (MDS)	Phase I/II www.angiocrinebioscience.com
ACTR087/BCMA (antibody-coupled T-cell receptor immunotherapy and B-cell maturation antigen)	Unum Therapeutics Cambridge, MA Seattle Genetics Bothell, WA	multiple myeloma	Phase I www.unumrx.com www.seattlegenetics.com
ACTR707 (antibody-coupled T-cell receptor immunotherapy)	Unum Therapeutics Cambridge, MA	non-Hodgkin lymphoma (NHL) (+rituximab), advanced HER2+ cancers (+trastuzumab)	Phase I www.unumrx.com
Ad-p53 (adenoviral p53 gene therapy)	MultiVir Houston, TX	head and neck cancer	Phase II www.multivir.com
		liver metastases of solid tumors, recurrent head and neck cancer (+anti-PD1)	Phase I/II www.multivir.com
Ad-p53 DCV (dendritic cell vaccine)	MultiVir Houston, TX	small cell lung cancer (SCLC) (combination therapy)	Phase II www.multivir.com

Cancer

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Ad/PNP gene therapy and fludarabine (gene therapy/drug combination) ORPHAN DRUG	PNP Therapeutics Birmingham, AL	head and neck cancer	Phase I/II www.pnptherapeutics.com
Ad-RTS-hIL-12 plus veledimex (DNA-based in vivo gene therapy) ORPHAN DRUG	Ziopharm Oncology Boston, MA	glioblastoma (+cemiplimab-rwlc) (Fast Track)	Phase II www.ziopharm.com
		recurrent glioblastoma, pediatric brain tumors	Phase I www.ziopharm.com
ADP-A2AFP (alpha fetoprotein targeted T-cell therapy)	Adaptimmune Philadelphia, PA	liver cancer	Phase I www.adaptimmune.com
ADP-A2M4 (MAGE-A4 T cell therapy) ORPHAN DRUG	Adaptimmune Philadelphia, PA	myxoid round cell liposarcoma, synovial sarcoma	Phase II www.adaptimmune.com
		MAGE-A4-positive solid tumors	Phase I www.adaptimmune.com
ADP-A2M4CD8 (MAGE-A4 T cell therapy)	Adaptimmune Philadelphia, PA	MAGE-A4-positive solid tumors	Phase I www.adaptimmune.com

<u>Cancer Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ADP-A2M10 (MAGE-A10 T cell therapy)	Adaptimmune Philadelphia, PA	head and neck cancer, non-small cell lung cancer (NSCLC), melanoma, bladder cancer	Phase I www.adaptimmune.com
ADXS-NEO (patient specific neoepitope-based vaccine)	Advaxis Princeton, NJ Amgen Thousand Oaks, CA	solid tumors	Phase I www.advaxis.com www.amgen.com
ADXS-PSA (genetically-modified cancer vaccine)	Advaxis Princeton, NJ	metastatic castration-resistant prostate cancer (+pembrolizumab)	Phase I/II www.advaxis.com
aglatimagene besadenovec (cancer suicide gene therapy) ORPHAN DRUG	Candel Therapeutics Needham, MA	intermediate/high risk localized prostate cancer (Fast Track)	Phase III www.candeltx.com
		1L glioblastoma, localized prostate cancer (active surveillance), advanced non-metastatic pancreatic cancer, NSCLC (+PD-1/PD-L1)	Phase II www.candeltx.com
		1L glioblastoma (+nivolumab), resectable NSCLC	Phase I www.candeltx.com

Cancer <u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AIC100 (ICAM-1 CAR-T cell therapy) ORPHAN DRUG	AffyImmune Therapeutics Natick, MA	thyroid cancer	Phase I
ALLO-501 (CD19 CAR-T cell therapy)	Allogene Therapeutics South San Francisco, CA	large B-cell follicular lymphoma	Phase I/II www.allogene.com
ALLO-715 (BCMA CAR-T cell therapy)	Allogene Therapeutics South San Francisco, CA	multiple myeloma	Phase I/II www.allogene.com
AlloStim T-cell therapy-device combination vaccine	Immunovative Therapies Jerusalem, Israel	metastatic colorectal cancer	Phase II www.immunovative.com
AMG 119 (DLL3 CAR-T cell therapy)	Amgen Thousand Oaks, CA	SCLC	Phase I www.amgen.com
anti-HA1 T-cell therapy	ElevateBio Cambridge, MA HighPassBio Cambridge, MA	leukemia	Phase I www.elevate.bio
APN401 (autologous cellular therapy)	Apeiron Biologics Vienna, Austria	solid tumors	Phase II www.apeiron-biologics.com

Cancer Drug Name	Sponsor	Indication	Development Phase
APS001F (cytosine deaminase gene therapy)	Anaeropharma Science Tokyo, Japan	solid tumors	Phase I/II www.anaeropharma.co.jp
ASP7517 (cell therapy)	Astellas Pharma Northbrook, IL	cancer	Phase I www.astellas.com
ASP9801 (gene loading oncolytic virus therapy)	Astellas Pharma Northbrook, IL	solid tumors	Phase I www.astellas.com
ATA520 (allogeneic T cell immunotherapy)	Atara Biotherapeutics South San Francisco, CA	hematologic malignancies	Phase I completed www.atarabio.com
ATA2271 (CAR-T cell therapy)	Atara Biotherapeutics South San Francisco, CA Memorial Sloan Kettering Cancer Center New York, NY	solid tumors	Phase I www.atarabio.com
ATLCAR CD19 (CAR-T cell therapy)	Bellicum Pharmaceuticals Houston, TX UNC Lineberger Cancer Center Chapel Hill, NC	B-cell lymphoma	Phase I www.bellicum.com
ATP128 (recombinant protein vaccine)	Amal Therapeutics Geneva, Switzerland	colorectal cancer	Phase I/II www.amaltherapeutics.com

Cancer Drug Name	Sponsor	Indication	Development Phase
AU105 (HER2-CMV CAR-T cell therapy)	Aurora BioPharma New York, NY	newly-diagnosed glioblastoma	Phase I/II completed www.aurora-biopharma.com
AVA-001 (CD-19 CAR-T cell therapy)	Avalon GloboCare Freehold, NJ	B-cell malignancies	Phase I www.avalon-globocare.com
AV-GBM-1 (autologous dendritic cell vaccine)	AIVITA Biomedical Irvine, CA	glioblastoma	Phase II www.aivitabiomedical.com
AV-MEL-1 (autologous dendritic cell vaccine)	AIVITA Biomedical Irvine, CA	melanoma	Phase I www.aivitabiomedical.com
axalimogene filolisbac (AXAL) (cancer immunotherapy) ORPHAN DRUG	Advaxis Princeton, NJ	cervical cancer (Fast Track)	Phase III www.advaxis.com
		anorectal cancer	Phase II www.advaxis.com
bb21217 (BCMA CAR-T cell therapy) ORPHAN DRUG	bluebird bio Cambridge, MA Bristol-Myers Squibb Princeton, NJ	multiple myeloma	Phase I www.bluebirdbio.com www.bms.com

Cancer Drug Name	Sponsor	Indication	Development Phase
BCMA-CD19 cCAR T cells (compound CAR-T cell therapy)	iCell Gene Therapeutics Stony Brook, NY	multiple myeloma, plasmacytoid lymphoma	Phase I www.icellgene.com
BCMA-CS1 cCAR T cells (compound CAR-T cell therapy)	iCell Gene Therapeutics Stony Brook, NY	multiple myeloma	Phase I www.icellgene.com
BCMA-targeted cell therapy	Arcellx Gaithersburg, MD	multiple myeloma	Phase I www.arcellx.com
bizalimogene ralaplasmid (VGX-3100) (DNA plasmid immunotherapy)	Inovio Pharmaceuticals Plymouth Meeting, PA	cervical high grade squamous intraepithelial lesion (HSIL)	phase III www.inovio.com
		anal HSIL, vulvar HSIL	Phase II www.inovio.com
BPX-601 (GoCAR-T cell therapy)	Bellicum Pharmaceuticals Houston, TX	non-resectable pancreatic cancer, gastric cancer, prostate cancer	Phase I/II www.bellicum.com
BPX-701 (T lymphocyte cell therapy)	Bellicum Pharmaceuticals Houston, TX	acute myeloid leukemia (AML), MDS, uveal melanoma	Phase I/II www.bellicum.com
CC-97540 (CAR-T cell therapy)	Juno Therapeutics Seattle, WA	B-cell NHL	Phase I www.junotherapeutics.com

Cancer Drug Name	Sponsor	Indication	Development Phase
CD4 CAR-T cell therapy	iCell Gene Therapeutics Stony Brook, NY	T-cell leukemia, T-cell lymphoma	Phase I www.icellgene.com
CD-19-CAR-NK (natural killer cell therapy)	Takeda Deerfield, IL M.D. Anderson Cancer Clinic Houston, TX	B-cell malignancies	Phase I/II www.takeda.com
CD19-CARvac T cells (CAR-T cell therapy)	iCell Gene Therapeutics Stony Brook, NY	B-cell malignancies	Phase I www.icellgene.com
CD123-CD33 cCAR-T cells (compound CAR-T cell therapy)	iCell Gene Therapeutics Stony Brook, NY	hematological malignancies	Phase I www.icellgene.com
CD20-CD19 cCAR-T cells (compound CAR-T cell therapy)	iCell Gene Therapeutics Stony Brook, NY	B-cell malignancies	Phase I www.icellgene.com
CD38A2 (anti-CD38 CAR-T cell therapy)	Celularity Warren, NJ Sorrento Therapeutics San Diego, CA	multiple myeloma	Phase I www.sorrentotherapeutics.com

Cancer**Drug Name****Sponsor****Indication****Development Phase**

CEA CAR-T (anti-CEA CAR-T cell therapy)	Sorrento Therapeutics San Diego, CA	metastatic liver cancer	Phase II www.sorrentotherapeutics.com
		malignant ascites, pancreatic cancer	Phase I www.sorrentotherapeutics.com
CLBR001 + SWI019 (CAR-T cell therapy)	AbbVie North Chicago, IL Calibr La Jolla, CA	B-cell malignancies	Phase I www.abbvie.com
CLL1-CD33 cCAR-T cells (compound CAR-T cell therapy)	iCell Gene Therapeutics Stony Brook, NY	AML	Phase I www.icellgene.com
CMD-501 (autologous genetically-engineered CAR-NKT cell therapy)	Cell Medica Houston, TX	neuroblastoma	Phase I www.cellmedica.com
CNDO-109 (activated allogeneic natural killer cell therapy) ORPHAN DRUG	Fortress Biotech New York, NY	AML	Phase I/II completed www.fortressbiotech.com
CT053 (BCMA CAR-T cell therapy) ORPHAN DRUG	CARsgen Therapeutics Bellaire, TX	multiple myeloma	Phase I www.carsgen.com

Cancer**Drug Name****Sponsor****Indication****Development Phase**

CTL119 (huCART19)
(CD19 CAR-T cell therapy)

Novartis
East Hanover, NJ
University of Pennsylvania
Philadelphia, PA

acute lymphoid leukemia
(pediatric)

Phase II
www.novartis.com

acute lymphoblastic leukemia (ALL)

Phase I
www.novartis.com

CTX110
(allogeneic CRISPR/Cas9
gene-edited CAR-T cell therapy)

CRISPR Therapeutics
Cambridge, MA

CD19-positive malignancies

Phase I/II
www.crisprtx.com

CYAD-01
(NKG2D CAR-T cell therapy)

Celyad
New York, NY

relapsing remitting AML/MDS

Phase I
www.celyad.com

CYAD-02
(NKG2D CAR-T cell therapy)

Celyad
New York, NY

relapsing remitting AML/MDS

Phase I
www.celyad.com

CYAD-101
(NKG2D CAR-T cell therapy)

Celyad
New York, NY

metastatic colorectal cancer

Phase I
www.celyad.com

CYNK-001
(placental-derived natural killer
cell therapy)

Celularity
Warren, NJ

AML, multiple myeloma

Phase I
www.celularity.com

Cancer**Drug Name****Sponsor****Indication****Development Phase**

DCVax®-Direct autologous dendritic cell vaccine	Northwest Biotherapeutics Bethesda, MD	solid tumors	Phase I/II www.nwbio.com
DCVax®-L autologous dendritic cell vaccine ORPHAN DRUG	Northwest Biotherapeutics Bethesda, MD	newly-diagnosed glioblastoma	Phase III www.nwbio.com
DCVax®-Prostate autologous dendritic cell vaccine	Northwest Biotherapeutics Bethesda, MD	prostate cancer	Phase II completed www.nwbio.com
delolimogene mupadenorepvox (immunostimulatory gene therapy with oncolytic adenovirus therapy) ORPHAN DRUG	Lokon Pharma Uppsala, Sweden	pancreatic cancer	Phase I/II www.lokonpharma.com
Descartes-08 (anti-BCMA CAR-T cell therapy)	Cartesian Therapeutics Rockville, MD	multiple myeloma	Phase I/II www.cartesiantherapeutics.com
Descartes-11 (CAR-T cell therapy)	Cartesian Therapeutics Rockville, MD	multiple myeloma	Phase I www.cartesiantherapeutics.com
DF1001 (natural killer cell therapy)	Dragonfly Therapeutics Waltham, MA	solid tumors	Phase I/II www.dragonflytx.com

Cancer

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
dilanubicel (NLA101) (expanded progenitor stem cell therapy)	Nohla Therapeutics Seattle, WA	AML (intensive chemo)	Phase II completed www.nohlatherapeutics.com
ECT-001 (ex vivo expansion of umbilical hematopoietic stem cell therapy)	ExCellThera Montreal, Canada	high-risk hematological malignancies	Phase II www.excellthera.com
ET019002/CD19 (CAR-T cell therapy)	Eureka Therapeutics Emeryville, CA	ALL, NHL	Phase I www.eurekatherapeutics.com
ET019003/CD19 (CAR-T cell therapy)	Eureka Therapeutics Emeryville, CA	ALL, NHL	Phase I www.eurekatherapeutics.com
ET140202/AFP (CAR-T cell therapy)	Eureka Therapeutics Emeryville, CA	liver cancer	Phase I/II www.eurekatherapeutics.com
FHVH-BCMA-T (CAR-T cell therapy)	TeneoBio Newark, CA	multiple myeloma	Phase I www.teneobio.com
FT500 (allogeneic natural killer cell therapy)	Fate Therapeutics San Diego, CA	solid tumors	Phase I www.fatetherapeutics.com
FT516 (natural killer cell therapy)	Fate Therapeutics San Diego, CA	hematological malignancies	Phase I www.fatetherapeutics.com

Cancer <u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
GALE 301/302 (immunogenic peptide vaccine) ORPHAN DRUG	Sellas Life Sciences New York, NY	ovarian cancer	Phase I/II completed www.sellaslifesciences.com
GDA-201 (natural killer cell therapy)	Gamida Cell Boston, MA	multiple myeloma, NHL	Phase I www.gamida-cell.com
GEN-1 (IL-12 gene therapy nanoparticle) ORPHAN DRUG	Celsion Lawrenceville, NJ	ovarian cancer	Phase II www.celsion.com
GSK3377794 (adoptive T cell immunotherapy) ORPHAN DRUG	GlaxoSmithKline Research Triangle Park, NC	synovial sarcoma	Phase II www.gsk.com
HPN424 (PSMA targeting T-cell engager)	Harpoon Therapeutics South San Francisco, CA	prostate cancer	Phase I www.harpoontx.com
HS-110 (allogeneic tumor cell vaccine)	Heat Biologics Morrisville, NC	NSCLC	Phase II www.heatbio.com
HS-130 (allogeneic tumor cell therapy)	Heat Biologics Morrisville, NC	solid tumors	Phase I www.heatbio.com

Cancer Drug Name	Sponsor	Indication	Development Phase
HSC100 (stem cell therapy)	Tacitus New York, NY	hematological malignancies	Phase I www.tacitustherapeutics.com
huMNC2-CAR44 T cells (anti-MUC1 CAR-T cell therapy)	Minerva Biotechnologies Waltham, MA	breast cancer	Phase I www.minervabio.com
iC9/CAR.19/IL-15-transduced CB-NK cell therapy	Bellicum Pharmaceuticals Houston, TX MD Anderson Cancer Center Houston, TX	B-lymphoid malignancies	Phase I/II www.bellicum.com
ide-cel (bb2121) (BCMA CAR-T cell therapy) ORPHAN DRUG	bluebird bio Cambridge, MA Bristol-Myers Squibb Princeton, NJ	3L multiple myeloma ----- 2L multiple myeloma, 4L+ multiple myeloma	Phase III www.bluebirdbio.com Phase II www.bluebirdbio.com
IGV-001 (autologous cell vaccine) ORPHAN DRUG	Imvax Philadelphia, PA	glioblastoma	Phase I www.imvax.com
ilixadencel (dendritic cell vaccine)	Immunicum Gothenburg, Sweden	advanced cancer	Phase I/II www.immunicum.com

Cancer Drug Name	Sponsor	Indication	Development Phase
IMA 101 (endogenous T-cell therapy)	Immatics US Houston, TX	solid tumors	Phase I www.immatics.com
IMA 202 (adoptive T-cell therapy)	Immatics US Houston, TX	solid tumors	Phase I www.immatics.com
IMA 203 (adoptive T-cell therapy)	Immatics US Houston, TX	solid tumors	Phase I www.immatics.com
Imlygic® talimogene laherparepvec (genetically-modified virus)	Amgen Thousand Oaks, CA	metastatic melanoma (+pembrolizumab)	Phase III www.amgen.com
INVAC-1 (hTERT DNA-based cancer vaccine)	Invectys Paris, France	CLL	Phase II www.invectys.com
IOV-2001 (adoptive cell therapy)	Iovance Biotherapeutics San Carlos, CA	CLL, small lymphocytic lymphoma	Phase I/II www.iovance.com
ITI-1000 (LMAP-Vax+pp65 dendritic cell vaccine)	Immunomic Therapeutics Rockville, MD	newly-diagnosed glioblastoma	Phase II www.immunomix.com

Cancer**Drug Name****Sponsor****Indication****Development Phase**

JNJ-68284528
(CAR-T cell therapy)
ORPHAN DRUG

Janssen Research & Development
Horsham, PA
Legend Biotech USA
Piscataway, NJ

multiple myeloma

Phase II
www.janssen.com

JTCR016
(CAR-T cell therapy)

Juno Therapeutics
Seattle, WA
Fred Hutchinson Cancer Center
Seattle, WA

NHL

Phase I/II
www.junotherapeutics.com

KITE-439
(T-cell receptor against HPV E7)

Gilead Sciences
Foster City, CA
Kite Pharma
Santa Monica, CA

solid tumors

Phase I
www.gilead.com

KITE-585
(BCMA CAR-T cell therapy)

Gilead Sciences
Foster City, CA
Kite Pharma
Santa Monica, CA

multiple myeloma

Phase I
www.kitepharma.com

KITE-718
(T-cell receptor against MAGE A3/A6)

Gilead Sciences
Foster City, CA
Kite Pharma
Santa Monica, CA

solid tumors

Phase I
www.kitepharma.com

Cancer

Drug Name

Sponsor

Indication

Development Phase

KTE-X19 (CAR-T cell therapy)	Gilead Sciences Foster City, CA Kite Pharma Santa Monica, CA	ALL, mantle cell lymphoma	Phase II www.kitepharma.com
		CLL	Phase I www.kitepharma.com
Kymriah [®] tisagenlecleucel ORPHAN DRUG	Novartis East Hanover, NJ	diffuse large B-cell lymphoma (DLBCL) (1st relapse), follicular lymphoma	Phase III www.novartis.com
		DLBCL (+pembrolizumab)	Phase I www.novartis.com
lifileucel (LN-144) (TIL autologous cell therapy) ORPHAN DRUG	Iovance Biotherapeutics San Carlos, CA	malignant melanoma (Fast Track), malignant melanoma (+pembrolizumab)	Phase II www.iovance.com
liso-cel (lisocabtagene maraleucel) (CAR-T cell therapy) ORPHAN DRUG	Bristol-Myers Squibb Summit, NJ Juno Therapeutics Seattle, WA	2L DLBCL	application submitted www.bms.com
		relapsed/refractory aggressive large B-cell NHL	Phase III www.bms.com
		CLL, 3L DLBCL, NHL, small lymphocytic leukemia	Phase II www.bms.com

Cancer Drug Name	Sponsor	Indication	Development Phase
LN-145 (TIL autologous cell therapy) ORPHAN DRUG	Iovance Biotherapeutics San Carlos, CA	cervical cancer (Fast Track), head and neck cancer, NSCLC	Phase II www.iovance.com
MB-101 (CAR-T cell therapy)	Mustang Bio New York, NY	glioblastoma	Phase I www.mustangbio.com
MB-102 (CAR-T cell therapy) ORPHAN DRUG	Mustang Bio New York, NY	AML, blastic plasmacytoid dendritic cell neoplasm (BPDCN), high-risk MDS	Phase I/II www.mustangbio.com
MB-103 (CAR-T cell therapy)	Mustang Bio New York, NY	glioblastoma	Phase I www.mustangbio.com
MB-104 (CAR-T cell therapy)	Mustang Bio New York, NY	multiple myeloma	Phase I www.mustangbio.com
MB-105 (CAR-T cell therapy)	Mustang Bio New York, NY	pancreatic cancer, prostate cancer	Phase I www.mustangbio.com
MB-106 (CAR-T cell therapy)	Mustang Bio New York, NY	B-cell NHL	Phase I www.mustangbio.com

Cancer**Drug Name****Sponsor****Indication****Development Phase**

MB-108
(genetically modified oncolytic virus)
ORPHAN DRUG

Mustang Bio
New York, NY
Nationwide Children's Hospital
Columbus, OH

glioblastoma

Phase I
www.mustangbio.com

MCC1 TCR
(autologous TCR engineered
T-cell therapy)

bluebird bio
Cambridge, MA

Merkel cell carcinoma

Phase I
www.bluebirdbio.com

MCY-M11
(CAR-T cell therapy)

MaxCyte
Gaithersburg, MD

advanced ovarian cancer,
peritoneal mesothelioma

Phase I
www.maxcyte.com

MEDI5395
(genetically modified attenuated
Newcastle disease virus)

AstraZeneca
Wilmington, DE

solid tumors

Phase I
www.astrazeneca.com

microencapsulated cell therapy
ORPHAN DRUG

PharmaCyte Biotech
Laguna Hills, CA

pancreatic cancer

Phase II
www.pharmacyte.com

Multi TAA
(TAA-specific T cell therapy)

Marker Therapeutics
Houston, TX

breast cancer, AML

Phase II
www.markertherapeutics.com

ALL, lymphoma, multiple myeloma,
pancreatic cancer, sarcoma

Phase I/II
www.markertherapeutics.com

Cancer

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
MV-NIS (genetically-modified attenuated measles virus)	Vyriad Rochester, MN	bladder cancer	Phase I www.vyriad.com
nadofaragene firadenovec (IFNA2B gene therapy)	FerGene Cambridge, MA	superficial bladder cancer (Fast Track) (Breakthrough Therapy)	application submitted www.fergene.com
	Trizell London, United Kingdom	malignant pleural mesothelioma	Phase III
nalotimagene carmaleucel (ex vivo cell therapy) ORPHAN DRUG	MolMed Milano, Italy	acute leukemia	Phase III www.molmed.com
NANT cancer vaccine (activated natural killer cells - haNK for infusion) ORPHAN DRUG	NantKwest Culver City, CA	merkel cell carcinoma	Phase II www.nantkwest.com
		triple negative breast cancer, pancreatic cancer, squamous cell carcinoma	Phase I www.nantkwest.com
NANT cancer vaccine (CD19.t-haNK cell therapy)	NantKwest Culver City, CA	diffuse large B-cell lymphoma (DLBCL)	Phase I www.nantkwest.com

Cancer Drug Name	Sponsor	Indication	Development Phase
NANT cancer vaccine (PD-L1.t-haNK cell therapy)	NantKwest Culver City, CA	PD-L1 high-expressing solid tumors	Phase I www.nantkwest.com
NeoTCR P1 (NeoTCR-P1 adoptive cell therapy)	PACT Pharma South San Francisco, CA	solid tumors	Phase I www.pactpharma.com
NG-350A (gene therapy)	PsiOxus Therapeutics Oxfordshire, United Kingdom	solid tumors	Phase I www.psioxus.com
NK100 (natural killer cell cancer immunotherapy)	Fate Therapeutics San Diego, CA	AML, ovarian, solid tumors (combination mAb therapy)	Phase I www.fatetherapeutics.com
NYESO-1 triple knockout T-cell receptor (TCR) therapy	Tmunity Philadelphia, PA	melanoma, synovial sarcoma	Phase I www.timunity.com
NYESO-1 T-cell receptor therapy	Kite Pharma Santa Monica, CA National Cancer Institute Bethesda, MD	NY-ESO-1 expressing cancer	Phase II www.kitepharma.com
ofranergene obadenovec (VB-111) (targeted gene therapy) ORPHAN DRUG	VBL Therapeutics Modi'in, Israel	glioblastoma (Fast Track), recurrent platinum-resistant ovarian cancer	Phase III www.vblrx.com

Cancer**Drug Name****Sponsor****Indication****Development Phase**

omidubicel
(cord blood stem cell therapy)
ORPHAN DRUG

Gamida Cell
Jerusalem, Israel

high-risk hematological malignancies

Phase III
www.gamida-cell.com

Oncoprex™
nanoparticle vector gene therapy

Genprex
Austin, TX

NSCLC (+erlotinib) (Fast Track)

Phase II
www.genprex.com

orva-cel (orvacabtagene autoleucel)
(anti-BCMA CAR-T cell therapy)
ORPHAN DRUG

Bristol-Myers Squibb
Princeton, NJ
Juno Therapeutics
Seattle, WA

multiple myeloma

Phase I/II
www.bms.com

ovapuldencel-T (AV-OVA-1)
(tumor stem cell specific
dendritic cell vaccine)

AIVITA Biomedical
Irvine, CA

ovarian cancer

Phase II
www.aivitabiomedical.com

PACTN
(adoptive T cell immunotherapy)

PersImmune
San Diego, CA

MDS

Phase I
www.persimmune.com

PBCAR0191
(CD19 CAR-T cell therapy)

Precision Biosciences
Durham, NC

B-cell ALL, NHL

Phase I/II
www.precisionbiosciences.com

P-BCMA-101
(CAR-T cell therapy)
ORPHAN DRUG

Poseida Therapeutics
San Diego, CA

multiple myeloma

Phase II
www.poseida.com

Cancer Drug Name	Sponsor	Indication	Development Phase
PEPIDH1M (dendritic cell vaccine)	Annias Immunotherapeutics Chapel Hill, NC	malignant glioma, recurrent medulloblastoma	Phase I www.anniasimmuno.com
Pexa-Vec (pexastimogene devacirepvec) (genetically-engineered strain of oncolytic vaccinia poxvirus) ORPHAN DRUG	SillaJen Biotherapeutics Busan, South Korea	liver cancer	Phase III www.sillajen.com
		renal cell carcinoma (+cemiplimab-rwlc)	Phase I www.sillajen.com
PRGN-3005 (CAR-T cell therapy)	PGEN Therapeutics Germantown, MD	ovarian cancer	Phase I www.precigen.com
PRGN-3006 (CAR-T cell therapy) ORPHAN DRUG	PGEN Therapeutics Germantown, MD	AML, MDS	Phase I www.precigen.com
Provenge® sipuleucel-T	Dendreon Seal Beach, CA	newly-diagnosed prostate cancer	Phase III www.dendreon.com
PSMA CAR-T cell therapy	Tmunity Philadelphia, PA	metastatic castration-resistant prostate cancer	Phase I www.timunity.com
PTG-01 (CAR-T cell therapy)	Protheragen Ronkonkoma, NY	ALL	Phase I www.protheragen.com

Cancer**Drug Name****Sponsor****Indication****Development Phase**REIC gene therapy (MTG201)
(AAV-based gene therapy)Momotaro-Gene
Okayama, Japan

malignant pleural mesothelioma

Phase II

www.mt-gene.com

ORPHAN DRUG

prostate cancer

Phase I/II

www.mt-gene.com**RIVO-CEL™**

rivotiglecleucel (BPX-501)

Bellicum Pharmaceuticals
Houston, TX

AML (12 years and older), MDS

Phase II/III

www.bellicum.com

ALL (pediatric), leukemia, lymphoma

Phase I/II

www.bellicum.comhematological malignancies
(pediatric)

Phase I

www.bellicum.comrQNestin 34.5
(genetically-modified oncolytic HSV)Candel Therapeutics
Needham, MA

recurrent glioblastoma

Phase I

www.candeltx.comseviprotimut-L
(cell line vaccine)Polynoma
San Diego, CA

malignant melanoma

Phase III

www.polynoma.com

ORPHAN DRUG

Cancer**Drug Name****Sponsor****Indication****Development Phase**

SGT-53
(p53 DNA gene therapy)

SynerGene Therapeutics
Potomac, MD

pancreatic cancer

Phase II

solid tumors (pediatric)

Phase I

SL-701
(dendritic cell vaccine)

ORPHAN DRUG

Stemline Therapeutics
New York, NY

glioblastoma (adults)

Phase I/II completed

www.stemline.com

spanlecortemlocel (MGTA-456)
(stem cell therapy)

Magenta Therapeutics
Cambridge, MA

hematological malignancies

Phase II

www.magentatx.com

stapuldencel-T (DVAC/Pca)
(autologous dendritic cell vaccine)

SOTIO
Prague, Czech Republic

metastatic castration-resistant
prostate cancer

Phase III

www.sotio.com

SV-BR-1-GM cancer vaccine
(allogeneic genetically-engineered
cell vaccine)

BriaCell Therapeutics
Berkeley, CA

breast cancer
(combination therapy)

Phase I/II

www.briacell.com

SVF-CAL1
(autologous stem cell-mediated
immunotherapy via an oncolytic
virus)

Calidi Biotherapeutics
San Diego, CA

solid tumors

Phase I

www.calidibio.com

Cancer
Drug Name

Sponsor

Indication

Development Phase

tab-cel (tabelecleucel) (EBV-specific T cell immunotherapy) ORPHAN DRUG	Atara Biotherapeutics South San Francisco, CA	EBV-associated post transplant lymphoproliferative disorder (EBV+PTLD) following hematopoietic cell transplant, EBV+PTLD following solid organ transplant	Phase III www.atarabio.com
		1L EBV+PTLD, EBV-associated cancer	Phase II www.atarabio.com
		nasopharyngeal carcinoma	Phase I/II www.atarabio.com
TAVO (tavokinogene tetsaplasmid) (interleukin-12 gene therapy) ORPHAN DRUG	OncoSec Medical San Diego, CA Merck Kenilworth, NJ	relapsed/refractory melanoma (+pembrolizumab) (Fast Track)	Phase III www.oncosec.com www.merck.com
		triple negative breast cancer (+pembrolizumab)	Phase II www.oncosec.com www.merck.com
TBX-3400 (cell-based immunotherapy) ORPHAN DRUG	Taiga Biotechnologies Aurora, CO	malignant melanoma	Phase I www.taigabio.com

Cancer Drug Name	Sponsor	Indication	Development Phase
TC-210 (T cell therapy) ORPHAN DRUG	TCR2 Therapeutics Cambridge, MA	cholangiocarcinoma, mesothelioma, NSCLC, ovarian cancer	Phase I/II www.tcr2.com
TCR (T cell receptor gene therapy)	Ziopharm Oncology Boston, MA	solid tumors	Phase I www.ziopharm.com
TLPLDC (dendritic cell vaccine)	Cancer Insight San Antonio, TX Elios Therapeutics Austin, TX	melanoma	Phase II www.cancerinsight.com
		ovarian cancer	Phase I/II www.cancerinsight.com
TnMUC-1 CAR-T cell therapy	Tmunity Philadelphia, PA	advanced TnMUC-positive solid tumors	Phase I www.tmunity.com
Toca 511 + Toca FC (vocimagene amiretrorepvec- flucytosine gene therapy) ORPHAN DRUG	Tocagen San Diego, CA	recurrent high grade glioma (Fast Track) (Breakthrough Therapy)	Phase III www.tocagen.com
		newly diagnosed glioblastoma	Phase II www.tocagen.com
		bladder cancer, metastatic solid tumors	Phase I www.tocagen.com

Cancer Drug Name	Sponsor	Indication	Development Phase
TRQ-1501 (T cell therapy)	Torque Therapeutics Cambridge, MA	hematologic malignancies (Fast Track), solid tumors (Fast Track)	Phase I/II www.torquetx.com
TT10 (rovaleucel) (T-cell immunotherapy) ORPHAN DRUG	Tessa Therapeutics Singapore	nasopharyngeal cancer (Fast Track)	Phase III www.tessatherapeutics.com
TT12 (T lymphocyte cell therapy)	Tessa Therapeutics Singapore Baylor College of Medicine Waco, TX	cervical cancer	Phase I www.tessatherapeutics.com
UCART19 (CAR-T cell therapy)	Allogene South San Francisco, CA Servier Neuilly-sur-Seine, France	ALL	Phase I www.allogene.com
UCART22 (CAR-T cell therapy)	Cellectis New York, NY	B-cell ALL	Phase I www.cellectis.com
UCART123 (CAR-T cell therapy)	Cellectis New York, NY	relapsing/remitting AML	Phase I www.cellectis.com
UCARTCS1 (CAR-T cell therapy)	Cellectis New York, NY	multiple myeloma	Phase I www.cellectis.com

Cancer
Drug Name

Sponsor

Indication

Development Phase

VAC2 (dendritic cell vaccine)	Lineage Cell Therapeutics Carlsbad, CA	NSCLC	Phase I www.lineagecell.com
Vigil™ genetically-modified autologous tumor cell vaccine and bi-shRNAfurin	Gradalis Dallas, TX	Ewing's sarcoma (first relapse)	Phase III www.gradalisinc.com
		1L maintenance ovarian cancer, melanoma	Phase II www.gradalisinc.com
		solid tumors	Phase I www.gradalisinc.com
Voyager-V1 (genetically-modified vesicular stomatitis virus)	Vyriad Rochester, MN	head and neck cancer, NSCLC (+pembrolizumab)	Phase II www.vyriad.com
		metastatic colorectal cancer (+avelumab)	Phase II www.vyriad.com
		solid tumors	Phase I www.vyriad.com

Cancer

Drug Name

Sponsor

Indication

Development Phase

Yescarta®
axicabtagene ciloleucel
ORPHAN DRUG

Gilead Sciences
Foster City, CA
Kite Pharma
Santa Monica, CA

2L DLBCL

Phase III

www.gilead.com

indolent NHL, DLBCL (+PD-L1),
1L DLBCL, DLBCL (+rituximab or
lenalidomide)

Phase II

www.gilead.com

DLBCL (+4-1BB)

Phase I

www.gilead.com

YTB323
(CAR-T cell therapy)

Novartis
East Hanover, NJ

ALL, DLBCL,
small lymphocytic lymphoma

Phase I

www.novartis.com

Cardiovascular Disease

Drug Name

Sponsor

Indication

Development Phase

AAV-hTERT
(AAV-based gene therapy)

Libella Gene Therapeutics
Manhattan, KS

critical limb ischemia

Phase I

www.libellagenetherapeutics.com

ACP-01
(angiogenic cell precursor therapy)

Hemostemix
Calgary, Canada

critical limb ischemia

Phase II

www.hemostemix.com

Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
allogeneic mesenchymal stem cell therapy (itMSCs)	Stemedica Cell Technologies San Diego, CA CardioCell San Diego, CA	chronic heart failure	Phase II www.stemedica.com
	Stemedica San Diego, CA	ischemic stroke	Phase II www.stemedica.com
ASCT01 (autologous stem cell therapy)	Lifecells North Kansas City, MO	critical limb ischemia	Phase II completed www.lifecellsllc.com
BCDA-01 (CardiAMP cell therapy)	BioCardia San Carlos, CA	ischemic heart failure	Phase III www.biocardia.com
BCDA-02 (CardiAMP cell therapy)	BioCardia San Carlos, CA	chronic myocardial ischemia with refractory angina	Phase III www.biocardia.com
CLBS12 (CD34+ stem cell therapy)	Caladrius Biosciences Basking Ridge, NJ	critical limb ischemia	Phase II www.caladrius.com
CLBS14 (CD34+ stem cell therapy)	Caladrius Biosciences Basking Ridge, NJ	no option refractory disabling angina	Phase III www.caladrius.com

Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CLBS16 (CD34+ stem cell therapy)	Caladrius Biosciences Basking Ridge, NJ	coronary microvascular dysfunction	Phase II www.caladrius.com
Collatogene [®] bepermingene perplasmid (HGF gene therapy)	AnGes USA Bethesda, MD	critical limb ischemia (Fast Track)	Phase III www.anges.co.jp
Generx [®] alferminogene tadenovec (angiogenic gene therapy)	Angionetics San Diego, CA Gene Biotherapeutics San Diego, CA	refractory angina due to myocardial ischemia (Fast Track)	Phase II completed www.angionetics.com
INXN-4001 (non-viral plasmid-based gene therapy)	Triple-Gene Germantown, MD	heart failure	Phase I www.3gtx.com
MultiStem [®] allogeneic stem cell therapy	Athersys Cleveland, OH	ischemic stroke (Fast Track)	Phase III www.athersys.com
		acute myocardial infarction	Phase II www.athersys.com
MyoCell [®] adipose-derived autologous stem cell therapy	U.S. Stem Cell Sunrise, FL	severe heart damage in congestive heart failure	Phase II/III www.us-stemcell.com

Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
NAN-101 (AAV-based gene therapy)	Asklepios BioPharmaceutical Research Triangle Park, NC	heart failure	Phase I www.askbio.com
NSI-566 (spinal cord-derived neural stem cell therapy)	Seneca Biopharma Germantown, MD	ischemic stroke	Phase II/III www.senecabio.com
PLX-PAD (emiplacel) (human placental stromal cell therapy)	Pluristem Therapeutics Haifa, Israel	critical limb ischemia (Fast Track)	Phase III www.pluristem.com
ReN-001 (neural stem cell therapy)	ReNeuron Bridgend, United Kingdom	ischemic stroke	Phase II www.reneuron.com
rexlemestrocel-L (Revascor) (mesenchymal precursor cell therapy) ORPHAN DRUG	Mesoblast New York, NY	advanced heart failure (class 3)	Phase III www.mesoblast.com
		end-stage heart failure (class 4)	Phase II www.mesoblast.com
RGX-501 (AAV-based gene therapy) ORPHAN DRUG	REGENXBIO Rockville, MD	hyperlipoproteinemia type IIa (homozygous familial hypercholesterolemia)	Phase I/II www.regenxbio.com

Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
RT-100 (AAV-based gene therapy)	Renova Therapeutics San Diego, CA	congestive heart failure (Fast Track)	Phase II www.renovatherapeutics.com

Crohn's Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Alofisel® darvadstrocel (stem cell therapy) ORPHAN DRUG	Takeda Deerfield, IL	perianal fistulas in Crohn's disease	Phase III www.takeda.com
remestemcel-L (mesenchymal stem cell therapy)	Mesoblast New York, NY	Crohn's disease (Fast Track)	Phase III www.mesoblast.com

Diabetes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AG019 (genetically modified <i>Lactococcus lactis</i> expressing autoantigen human proinsulin and interleukin-10)	ActoBio Therapeutics Ghent, Belgium Precigen Germantown, MD	type 1 diabetes	Phase I/II www.actobio.com www.precigen.com
AVT001 (autologous dendritic cell therapy)	Avotres New York, NY	type 1 diabetes	Phase I/II

Diabetes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CLBS03 (T-regulatory cell therapy) ORPHAN DRUG	Caladrius Biosciences Basking Ridge, NJ	recent onset type 1 diabetes (Fast Track)	Phase II www.caladrius.com
donislecel (allogeneic islets of Langerhans cell therapy)	CellTrans Chicago, IL	type 1 diabetes	Phase III www.celltransinc.com
islet cell replacement therapy	Sernova London, Canada	type 1 diabetes	Phase I/II www.sernova.com
PEC-Direct™ pancreatic progenitor stem cell therapy	ViaCyte San Diego, CA	type 1 diabetes	Phase I/II www.viacyte.com
PEC-Encap™ encapsulated human pancreatic cell therapy	ViaCyte San Diego, C	type 1 diabetes	Phase I/II www.viacyte.com
TOL-3021 (DNA plasmid immunotherapeutic vaccine)	Tolerion South San Francisco, CA	type 1 diabetes	Phase II www.tolerion.bio

Eye Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AAV-CNGA3 (AAV-based gene therapy) ORPHAN DRUG	MeiraGTx New York, NY Janssen Research & Development Raritan, NJ	achromatopsia (color vision defects)	Phase I/II www.meiragtx.com
AAV-CNGB3 (AAV-based gene therapy) ORPHAN DRUG	MeiraGTx New York, NY Janssen Research & Development Raritan, NJ	achromatopsia (color vision defects)	Phase I/II www.meiragtx.com
AAV-RPE65 (AAV-based gene therapy) ORPHAN DRUG	MeiraGTx New York, NY	Leber's congenital amaurosis	Phase I/II www.meiragtx.com
AAV-RPGR (AAV-based gene therapy) ORPHAN DRUG	MeiraGTx New York, NY Janssen Research & Development Raritan, NJ	X-linked retinitis pigmentosa (Fast Track)	Phase I/II www.meiragtx.com
ACHM-CNGA3 (AAV-based gene therapy) ORPHAN DRUG	Applied Genetic Technologies Alachua, FL	achromatopsia	Phase I/II www.agtc.com
ACHM-CNGB3 (AAV gene therapy) ORPHAN DRUG	Applied Genetic Technologies Alachua, FL	achromatopsia	Phase I/II www.agtc.com

Eye Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ADVM-022 (AAV-based gene therapy)	Adverum Biotechnologies Menlo Park, CA	wet age-related macular degeneration (Fast Track), neovascular age-related macular degeneration	Phase I www.adverum.com
AGN-151587 (EDIT-101) (CRISPR gene editing)	Allergan Madison, NJ Editas Medicine Cambridge, MA	Leber's congenital amaurosis	Phase I/II www.allergan.com www.editasmedicine.com
ASP7317 (human retinal pigment epithelial cell therapy) ORPHAN DRUG	Astellas Pharma Northbrook, IL	dry age-related macular degeneration, Stargardt disease	Phase II www.astellas.com
BIIB111 (timerpigene emparvovec) (AAV-based gene therapy) ORPHAN DRUG	Biogen Cambridge, MA	x-linked choroideremia	Phase III www.biogen.com
BIIB112 (NSR-RPGR) (AAV-based gene therapy) ORPHAN DRUG	Biogen Cambridge, MA	x-linked retinitis pigmentosa (Fast Track)	Phase II/III www.biogen.com
CAM-101 (allogeneic human platelet lysate cell therapy)	Cambium Medical Technologies Atlanta, GA	dry eyes secondary to graft-versus-host disease (GVHD)	Phase I/II www.cambiumbio.com

Eye Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CPCB-RPE1 (embryonic stem cell therapy)	Regenerative Patch Technologies Portola Valley, CA	dry age-related macular degeneration	Phase I/II www.regenerativepatch.com
CPK850 (AAV-based gene therapy)	Novartis East Hanover, NJ	RLBP1-mutated retinitis pigmentosa	Phase I/II www.novartis.com
GS010 (lenadogene nolparvovec) (AAV-based gene therapy) ORPHAN DRUG	GenSight Biologics New York, NY	Leber's hereditary optic atrophy	Phase III www.gensight-biologics.com
GS030 (AAV-based gene therapy) ORPHAN DRUG	GenSight Biologics New York, NY	retinitis pigmentosa	Phase I/II www.gensight-biologics.com
HMR59 (AAV-based gene therapy)	Hemera Biosciences Newton, MA	dry age-related macular degeneration, wet age-related macular degeneration	Phase I www.hemerabiosciences.com
jCell (retinal stem cell therapy)	jCyte Newport Beach, CA	retinitis pigmentosa	Phase II www.jcyte.com

Eye Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
NT-501 ECT (encapsulated cell therapy) ORPHAN DRUG	Neurotech USA Cumberland, RI	macular telangiectasia type 2 (Fast Track)	Phase III www.neurotechusa.com
		glaucoma	Phase II www.neurotechusa.com
OpRegen® retinal pigment epithelial cell replacement therapy	Lineage Cell Therapeutics Carlsbad, CA	dry age-related macular degeneration (Fast Track)	Phase II www.lineagecelltherapeutics.com
ReN-003 (human retinal stem cell therapy) ORPHAN DRUG	ReNeuron Bridgend, United Kingdom	retinitis pigmentosa (Fast Track)	Phase I/II www.reneuron.com
RGX-314 (AAV-based gene therapy)	REGENXBIO Rockville, MD	wet age-related macular degeneration	Phase I/II www.regenxbio.com
RST-001 (green algae-derived photoreceptor gene therapy) ORPHAN DRUG	Allergan Madison, NJ	retinitis pigmentosa	Phase I/II www.allergan.com
SAR422459 (ABCA4 gene therapy) ORPHAN DRUG	Sanofi Bridgewater, NJ	Stargardt disease	Phase II www.sanofi.com

Eye Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
SPK-7001 (AAV-based gene therapy) ORPHAN DRUG	Spark Therapeutics Philadelphia, PA	X-linked choroideremia	Phase I/II www.sparktx.com
XLRP (AAV-based gene therapy) ORPHAN DRUG	Applied Genetics Technologies Alachua, FL	X-linked retinitis pigmentosa	Phase I/II www.agtc.com
XLRS (AAV-based gene therapy) ORPHAN DRUG	Applied Genetics Technologies Alachua, FL	X-linked retinoschisis	Phase I/II www.agtc.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ABO-101 (AAV-based gene therapy) ORPHAN DRUG	Abeona Therapeutics New York, NY	mucopolysaccharidosis IIIB (Sanfilippo syndrome type B) (Fast Track)	Phase I/II www.abeonatherapeutics.com
ABO-102 (AAV-based gene therapy) ORPHAN DRUG	Abeona Therapeutics New York, NY	mucopolysaccharidosis IIIA (Sanfilippo syndrome type A) (Fast Track)	Phase I/II www.abeonatherapeutics.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Actus-101 (AAV-based gene therapy)	Asklepios BioPharmaceutical Research Triangle Park, NC Actus Therapeutics Chapel Hill, NC	glycogen storage disease type II (Pompe disease)	Phase I/II www.askbio.com
AT132 (resamirigene bilarvovec) (AAV-based gene therapy) ORPHAN DRUG	Audentes Therapeutics San Francisco, CA	X-linked myotubular myopathy (Fast Track)	Phase I/II www.audentestx.com
AT845 (AAV-based gene therapy)	Audentes Therapeutics San Francisco, CA	glycogen storage disease type II (Pompe disease)	Phase I/II www.audentestx.com
AT-GTX-501 (CLN6 gene therapy) ORPHAN DRUG	Amicus Therapeutics Cranbury, NJ	CLN6 neuronal ceroid lipofuscinosis (Batten disease)	Phase I/II www.amicusrx.com
AT-GTX-502 (CLN3 gene therapy) ORPHAN DRUG	Amicus Therapeutics Cranbury, NJ	CLN3 neuronal ceroid lipofuscinosis (Batten disease)	Phase I/II www.amicusrx.com
AVR-RD-01 (lentiviral gene therapy) ORPHAN DRUG	AVROBIO Cambridge, MA	Fabry's disease	Phase II www.avrobio.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AVR-RD-02 (gene therapy) ORPHAN DRUG	AVROBIO Cambridge, MA	Gaucher's disease	Phase I/II www.avrobio.com
AVR-RD-04 (lentiviral gene therapy)	AVROBIO Cambridge, MA	cystinosis	Phase I/II www.avrobio.com
AVXS-201 (MECP2 gene replacement therapy)	AveXis (Novartis) Bannockburn, IL	Rett syndrome	Phase I www.avexis.com
AXO-AAV-GM1 (AAV-based gene therapy) ORPHAN DRUG	Axovant Gene Therapies New York, NY	GM1 gangliosidosis	Phase I/II www.axovant.com
AXO-AAV-GM2 (AAV-based gene therapy)	Axovant Gene Therapies New York, NY	GM2 gangliosidosis (Tay-Sachs disease, Sandhoff disease)	Phase I/II www.axovant.com
DTX301 (AAV-based gene therapy) ORPHAN DRUG	Ultragenyx Pharmaceutical Novato, CA	ornithine transcarbamylase deficiency (Fast Track)	Phase II www.ultragenyx.com
DTX401 (AAV-based gene therapy) ORPHAN DRUG	Ultragenyx Pharmaceutical Novato, CA	glycogen storage disease type I (Fast Track)	Phase I www.ultragenyx.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
HMI-102 (AAV-based gene therapy) ORPHAN DRUG	Homology Medicines Bedford, MA	phenylketonuria (Fast Track)	Phase I/II www.homologymedicines.com
LYS-SAF302 (AAV-based gene therapy) ORPHAN DRUG	Lysogene Cambridge, MA Sarepta Therapeutics Cambridge, MA	mucopolysaccharidosis IIIA	Phase II/III www.lysogene.com www.sarepta.com
MB-107 (ex vivo lentiviral gene therapy)	Mustang Bio New York, NY	X-linked severe combined immunodeficiency (SCID)	Phase I/II www.mustangbio.com
neurotrophin 3 (AAV-based gene therapy)	Sarepta Therapeutics Cranbury, NJ Nationwide Children's Hospital Columbus, OH	Charcot-Marie-Tooth neuropathy type 1A	Phase I/II www.sarepta.com
OTL-101 (simoladagene autotemcel) (ex vivo autologous gene therapy) ORPHAN DRUG	Orchard Therapeutics Boston, MA	adenosine deaminase severe combined immunodeficiency (ADA-SCID) (Fast Track) (Breakthrough Therapy)	Phase III www.orchard-tx.com
OTL-102 (ex vivo autologous gene therapy) ORPHAN DRUG	Genethon Cedex, France Orchard Therapeutics Boston, MA	X-linked chronic granulomatous disease	Phase I/II www.orchard-tx.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
OTL-103 (ex vivo stem cell therapy) ORPHAN DRUG	Orchard Therapeutics Boston, MA	Wiskott-Aldrich syndrome	Phase II www.orchard-tx.com
OTL-201 (ex vivo stem cell therapy) ORPHAN DRUG	Orchard Therapeutics Boston, MA	mucopolysaccharidosis type IIIA	Phase I/II www.orchard-tx.com
OTL-203 (ex vivo stem cell therapy)	Orchard Therapeutics Boston, MA	mucopolysaccharidosis type I	Phase I/II www.orchard-tx.com
RGX-111 (AAV-based gene therapy) ORPHAN DRUG	REGENXBIO Rockville, MD	mucopolysaccharidosis type I (Fast Track)	Phase I/II www.regenxbio.com
RGX-121 (AAV9 gene therapy) ORPHAN DRUG	REGENXBIO Rockville, MD	mucopolysaccharidosis type II (Hunter syndrome) (Fast Track)	Phase I/II www.regenxbio.com
RP-A501 (AAV9-based gene therapy) ORPHAN DRUG	Rocket Pharmaceuticals New York, NY	glycogen storage disease type II (Danon disease) (Fast Track)	Phase I www.rocketpharma.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
RP-L201 (lentiviral-based gene therapy) ORPHAN DRUG	Rocket Pharmaceuticals New York, NY	leukocyte adhesion deficiency-1 (Fast Track)	Phase I/II www.rocketpharma.com
SB-318 (AAV-based gene therapy) ORPHAN DRUG	Sangamo Therapeutics Richmond, CA	mucopolysaccharidosis I (Fast Track) (Scheie, Hurler-Scheie and Hurler syndromes)	Phase I/II www.sangamo.com
SB-913 (AAV-based gene therapy) ORPHAN DRUG	Sangamo Therapeutics Richmond, CA	mucopolysaccharidosis II (Fast Track) (Hunter syndrome)	Phase I/II www.sangamo.com
spanlecortemlocel (MGTA-456) (stem cell therapy)	Magenta Therapeutics Cambridge, MA	in patients with inherited metabolic disorders undergoing HSCT	Phase II www.magentatx.com
SPK-3006 (gene therapy)	Spark Therapeutics Philadelphia, PA	Pompe disease	Phase I/II www.sparktx.com
ST-920 (AAV-based gene therapy) ORPHAN DRUG	Sangamo Therapeutics Richmond, CA	Fabry's disease	Phase I/II www.sangamo.com
Zolgensma [®] onasemnogene abeparvovec-xioi	AveXis (Novartis) Bannockburn, IL	spinal muscular atrophy (SMN2), spinal muscular atrophy (SMN3)	Phase I www.avexis.com

Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AGS-004 (autologous dendritic cell vaccine)	Argos Therapeutics Durham, NC University of North Carolina Chapel Hill, NC	HIV infections	Phase I/II www.argostherapeutics.com
ATA230 (adimlecleucel) (allogeneic T cell immunotherapy) ORPHAN DRUG	Atara Biotherapeutics South San Francisco, CA	cytomegalovirus infections	Phase II www.atarabio.com
CAL-1 (genetically-modified stem progenitor cell therapy)	CSL Behring King of Prussia, PA	HIV-1 infections	Phase I/II www.cslnehring.com
SB-728-T (ZFN-mediated genome editing in T-cells)	Sangamo Therapeutics Richmond, CA	HIV infections	Phase I www.sangamo.com
SB-728-HSPC (ZFN-modified progenitor stem cells)	Sangamo Therapeutics Richmond, CA	HIV infections	Phase I www.sangamo.com
SB-728-mR-T (ZFN-mediated genome editing in T-cells)	Sangamo Therapeutics Richmond, CA	HIV infections	Phase I www.sangamo.com

Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
StealthVector® HGTV43™ antisense HIV-1 therapy	Enzo Therapeutics New York, NY	HIV-1 infections	Phase II www.enzo.com
Triplex cytomegalovirus gene MVA virus vaccine	HeloCyte New York, NY	cytomegalovirus infections in patients undergoing hematopoietic stem cell transplantation and solid organ transplantation (prevention)	Phase II www.helocyte.com
Viralym-M (ALVR105) multi-virus specific T-cell immunotherapy	AlloVir Houston, TX	viral infections following HSCT (BKV, CMV, EBV, CV, HHV6)	Phase II www.allovir.com
VXA G2.4 NS (recombinant vaccine)	Vaxart South San Francisco, CA	norovirus infections	Phase I www.vaxart.com

Kidney Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ReACT (autologous cultured kidney tissue cell therapy)	inRegen New York, NY	delay of diabetic kidney disease	Phase II www.inregen.com
		prevention of diabetic kidney disease, renal failure due to congenital anomalies	Phase I www.inregan.com

Kidney Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
rexlemestrocel-L (MPC-300-IV) (mesenchymal precursor cell therapy)	Mesoblast New York, NY	diabetic nephropathy in type 2 diabetes	Phase II www.mesoblast.com

Muscular Dystrophy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CAP-1002 (allogeneic cardiosphere-derived cell therapy) ORPHAN DRUG	Capricor Therapeutics Beverly Hills, CA	Duchenne muscular dystrophy	Phase II www.capricor.com
GALGT2 gene therapy	Sarepta Therapeutics Cambridge, MA Nationwide Children's Hospital Columbus, OH	Duchenne muscular dystrophy	Phase I/II www.sarepta.com
MYO-201 (AAV-based gene therapy)	Sarepta Therapeutics Cambridge, MA	limb girdle muscular dystrophy (type 2B)	Phase I www.sarepta.com
PF-06939926 (AAV-based gene therapy) ORPHAN DRUG	Pfizer New York, NY	Duchenne muscular dystrophy	Phase I www.pfizer.com

Muscular Dystrophy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
SGT-001 (AAV-based gene therapy) ORPHAN DRUG	Solid Biosciences Cambridge, MA	Duchenne muscular dystrophy (Fast Track)	Phase I/II www.solidbio.com
SRP-9001 (micro-dystrophin gene therapy)	Sarepta Therapeutics Cambridge, MA Nationwide Children's Hospital Columbus, OH	Duchenne muscular dystrophy	Phase II www.sarepta.com
SRP-9003 (AAV-based gene therapy) ORPHAN DRUG	Sarepta Therapeutics Cambridge, MA	limb girdle muscular dystrophy (type 2E)	Phase I/II www.sarepta.com
SRP-9004 (MYO-102) (AAV-based gene therapy) ORPHAN DRUG	Sarepta Therapeutics Cambridge, MA	limb girdle muscular dystrophy (type 2D)	Phase I/II www.sarepta.com

Neurologic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AAV-GAD (AAV-based gene therapy)	MeiraGTx New York, NY	Parkinson's disease (Fast Track)	Phase I/II www.meiragtx.com
AAV2-GDNF gene therapy (AAV-based gene therapy)	Brain Neurotherapy Bio Oakland, CA	Parkinson's disease	Phase I www.brainneubio.com

Neurologic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AMT-130 (AAV5-based gene therapy) ORPHAN DRUG	uniQure Lexington, MA	Huntington's disease (Fast Track)	Phase I/II www.uniquire.com
AXO-LENTI-PD (gene therapy)	Axovant Gene Therapies New York, NY	Parkinson's disease	Phase I/II www.axovant.com
CEVA101 (autologous bone marrow mononuclear cell therapy)	Cellvation New York, NY	traumatic brain injury	Phase II www.cellvation.com
donaperminogene seltoplasmid (plasmid DNA vector encoding modified HGF gene therapy) ORPHAN DRUG	Helixmith San Diego, CA	diabetic peripheral neuropathy	Phase III www.viromed.co.kr
		amyotrophic lateral sclerosis (ALS) (Fast Track)	Phase I completed www.viromed.co.kr
GT-AADC (AAV-based gene therapy) ORPHAN DRUG	PTC Therapeutics South Plainfield, NJ	aromatic L-amino acid decarboxylase (AADC) deficiency	Phase II www.ptcbio.com
HB-adMSC (autologous adipose-derived mesenchymal stem cell therapy)	Hope Biosciences Sugar Land, TX	traumatic brain injury	Phase I/II www.hope.bio

Neurologic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ISC-hpNSC (human parthenogenetic stem cell therapy)	International Stem Cell Carlsbad, CA	Parkinson's disease	Phase I www.internationalstemcell.com
KLS-2031 (AAV-based gene therapy)	Kolon Life Science Seoul, South Korea	neuropathic pain from lumbo-sacral radiculopathy (sciatica)	Phase I/II www.kolonls.co.kr
Lenti-D™ elivaldogene tavalentivec (gene therapy) ORPHAN DRUG	bluebird bio Cambridge, MA	cerebral adrenoleukodystrophy (up to 17 years of age)	Phase III www.bluebirdbio.com
NSI-566 (spinal cord-derived neural stem cell therapy) ORPHAN DRUG	Seneca Biopharma Germantown, MD	ALS	Phase II www.senecabio.com
		chronic spinal cord injury	Phase I www.senecabio.com
NurOwn® neurotrophic factor-producing mesenchymal stem cell therapy ORPHAN DRUG	BrainStorm Cell Therapeutics New York, NY	ALS (Fast Track)	Phase III www.brainstorm-cell.com
OK99 (neural allogeneic stem cell therapy)	Celavie Biosciences Oxnard, CA	Parkinson's disease	Phase I www.celavie.com

Neurologic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
OPC1 (human stem cell therapy) ORPHAN DRUG	Lineage Cell Technologies Carlsbad, CA	spinal cord injury (subacute)	Phase I/II www.asteriasbiotherapeutics.com
OTL-200 (ex vivo stem cell therapy) ORPHAN DRUG	Orchard Therapeutics Boston, MA	metachromatic leukodystrophy	Phase II www.orchard-tx.com
PR001 (gene therapy)	Prevail Therapeutics New York, NY	Parkinson's disease (Fast Track)	Phase I/II www.prevailtherapeutics.com
rexlemestrocel-L (MPC-06-ID) (mesenchymal precursor cell therapy)	Mesoblast New York, NY	chronic low back pain	Phase III www.mesoblast.com
SB623 (allogeneic mesenchymal stem cell therapy)	SanBio Mountain View, CA Sunovion Pharmaceuticals Marlborough, MA	chronic motor deficit due to stroke	Phase II www.san-bio.com www.sunovion.com
	SanBio Mountain View, CA	traumatic brain injury	Phase II www.san-bio.com
SB-IB02 (adipose-derived allogeneic mesenchymal stem cell therapy) ORPHAN DRUG	Steminent Biotherapeutics San Diego, CA	spinocerebellar ataxia	Phase II www.steminent.com

Neurologic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
VY-AADC (AAV-based gene therapy)	Voyager Therapeutics Cambridge, MA	Parkinson's disease (Fast Track)	Phase II www.voyagertherapeutics.com

Respiratory Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
MultiStem® allogeneic stem cell therapy	Athersys Cleveland, OH	acute respiratory distress syndrome (Fast Track)	Phase II www.athersys.com

Pneumostem® mesenchymal stem cell therapy ORPHAN DRUG	Medipost America Rockville, MD	bronchopulmonary dysplasia (premature infants)	Phase I/II completed www.medi-post.com
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Skin

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ALLO-ASC-DFU (adipose stem cell therapy)	Anterogen Seoul, South Korea	diabetic foot ulcers	Phase II www.anterogen.com

allogeneic mesenchymal stem cell therapy (itMSCs)	Stemedica Cell Technologies San Diego, CA	cutaneous photoaging	Phase II www.stemedica.com
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Skin

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
donaperminogene seltoplasmid (plasmid DNA vector encoding modified HGF gene therapy)	Helixmith San Diego, CA	diabetic foot ulcers	Phase III www.viomed.co.kr
EB-101 (autologous ex vivo gene-corrected cell therapy) ORPHAN DRUG	Abeona Therapeutics New York, NY	recessive dystrophic epidermolysis bullosa (Breakthrough Therapy)	Phase III www.abeonatherapeutics.com
ESS (autologous engineered skin replacement) ORPHAN DRUG	Amarantus BioScience New York, NY Emerald Organic Products New Haven, CT	burns	Phase II www.amarantus.com www.emeraldorganic.com
ExpressGraft™ genetically-modified anti-infective skin replacement therapeutic	Mallinckrodt St. Louis, MO	diabetic foot ulcers	Phase I www.mallinckrodt.com
FCX-007 (fibroblast gene therapy) ORPHAN DRUG	Castle Creek Pharmaceuticals Parsippany, NJ Fibrocell Science Exton, PA	recessive dystrophic epidermolysis bullosa (Fast Track)	Phase III www.castlecreekpharma.com www.fibrocell.com
KB103 (bercolagene telpavec) (modified HSV-1 gene therapy) ORPHAN DRUG	Krystal Biotech Pittsburgh, PA	dystrophic epidermolysis bullosa (Fast Track)	Phase II www.krystalbio.com

Transplantation

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
MDR-101 (transplant kidney donor-derived stem cells engineered to create hematopoietic cells)	Medeor Therapeutics South San Francisco, CA	renal transplant rejection (HLA-matched living donor)	Phase III www.medeorthapeutics.com
MDR-102 (transplant kidney donor-derived stem cells engineered to create hematopoietic cells)	Medeor Therapeutics South San Francisco, CA	renal transplant rejection (HLA-mismatched living donor)	Phase II www.medeorthapeutics.com
MDR-103 (transplant kidney donor-derived stem cells engineered to create hematopoietic cells)	Medeor Therapeutics South San Francisco, CA	renal transplant rejection (HLA-matched living donor, delayed therapy)	Phase II www.medeorthapeutics.com
MultiStem® allogeneic stem cell therapy ORPHAN DRUG	Athersys Cleveland, OH	prevention of graft versus host disease in HSCT (Fast Track)	Phase II www.athersys.com
PLX-R18 (placenta-derived allogenic stem cell therapy) ORPHAN DRUG	Pluristem Therapeutics Haifa, Israel	incomplete hematopoietic recovery following hematopoietic cell transplantation	Phase I www.pluristem.com

Transplantation

Drug Name

Sponsor

Indication

Development Phase

ProTmune™

ex vivo programmed hematopoietic cellular immunotherapy

ORPHAN DRUG

Fate Therapeutics
San Diego, CA

GVHD in hematopoietic cell transplantation (Fast Track)

Phase II
www.fatetherapeutics.com

remestemcel-L
(mesenchymal stem cell therapy)

ORPHAN DRUG

Mesoblast
New York, NY

pediatric steroid refractory acute GVHD (Fast Track)

Phase III
www.mesoblast.com

Other

Drug Name

Sponsor

Indication

Development Phase

AAV-AQP1
(AAV-based gene therapy)

ORPHAN DRUG

MeiraGTx
New York, NY

radiation-induced xerostomia

Phase I/II
www.meiragtx.com

AAV-hTERT
(AAV-based gene therapy)

Libella Gene Therapeutics
Manhattan, KS

aging

Phase I
www.libellagenetherapeutics.com

AB-205
(genetically-engineered human cells derived from human umbilical vein tissue)

Angiocrine Bioscience
San Diego, CA

anal fistulas

Phase I/I
www.angiocrinebioscience.com

Other**Drug Name****Sponsor****Indication****Development Phase**

AG013 (genetically modified <i>Lactococcus lactis</i> expressing TFF1)	ActoBio Therapeutics Ghent, Belgium Precigen Germantown, MD	oral mucositis	Phase II www.actobio.com www.precigen.com
AMDC (autologous muscle-derived stem cell therapy)	Cook MyoSite Pittsburgh, PA	fecal incontinence	Phase I/II www.cookmysite.com
		tongue dysphagia	Phase I www.cookmysite.com
Cellgram™ autologous mesenchymal stem cell therapy	Pharmicell Seoul, South Korea	liver cirrhosis	Phase I www.pharmicell.com
CGF166 (AAV-based gene therapy)	Novartis East Hanover, NJ Precigen Germantown, MD	unilateral or bilateral severe-to-profound hearing loss	Phase I/II www.novartis.com www.precigen.com
LMSC (mesenchymal stem cell therapy)	Longeveron Miami, FL	aging frailty, metabolic syndrome	Phase II www.longeveron.com
		immunosenescence in aging frailty (weakening of immune system)	Phase I/II www.longeveron.com

Other

Drug Name

Sponsor

Indication

Development Phase

RTX-134
(genetically-engineered
allogeneic cell therapy)

Rubius Therapeutics
Cambridge, MA

phenylketonuria

Phase I
www.rubiustx.com

TARA-002
(cellular therapy)

ArTara Therapeutics
New York, NY

lymphangioma

Phase III
www.artaratx.com

ORPHAN DRUG

The content of this report has been obtained through public, government and industry sources, and the Springer "Adis Insight" database based on the latest information. Report current **as of February 13, 2020**. The medicines in this listing include medicines being developed by U.S.-based companies conducting trials in the United States abroad, PhRMA-member companies conducting trials in the United States and abroad, and foreign companies conducting clinical trials in the United States. Some products may not be in active clinical trials. The information may not be comprehensive. For more, specific information about a particular product, contact the individual company directly or go to www.clinicaltrials.gov. The entire series of *Medicines in Development* is available on PhRMA's website: www.phrma.org.

Glossary

Application Submitted—An application for marketing has been submitted by the company to the U.S. Food and Drug Administration (FDA).

Breakthrough Therapy—Upon request by a sponsor, the FDA can grant this designation to expedite the development and review of a drug or biologic intended, alone or in combination with one or more other drugs, to treat a serious or life threatening disease or condition and preliminary clinical evidence indicates that it may demonstrate substantial improvement over existing therapies on one or more clinically-significant endpoints, such as substantial treatment effects observed early in clinical development. If a drug or biologic is designated as a Breakthrough Therapy, the FDA will expedite the development and review. With this designation, all Fast Track features convey to the medicine.

Fast Track—Upon request by a sponsor, the FDA can grant this designation to facilitate the development and expedite the review of a drug or biologic to treat a serious condition and fill an unmet medical need. When considering a biopharmaceutical company's request for Fast Track designation for an investigational drug or biologic, the FDA evaluates whether it will affect factors such as survival, day-to-day functioning, or the likelihood that the disease, if left untreated, will progress from a less severe condition to a more serious one, and whether a condition can be adequately addressed by available therapy. With Fast Track designation,

early and frequent communication between the FDA and the biopharmaceutical company is encouraged throughout the entire drug development and review process to help to quickly resolve any questions or issues that arise, potentially leading to an earlier approval and access by patients.

Orphan Designation—Upon request by a sponsor, the FDA can grant special status (“orphan status”) to a drug or biologic to treat a rare disease or condition. In order to receive an orphan designation, a qualifying drug or biologic must be intended for the treatment, diagnosis, or prevention of a rare disease or condition that affects usually fewer than 200,000 people in the United States.

Phase I—Researchers test the investigational drug or biologic in a small group of people, usually between 20 and 100 healthy adult volunteers, to evaluate its initial safety and tolerability profile, determine a safe dosage range, and identify potential side effects.

Phase II—The investigational drug or biologic is given to volunteer patients, usually between 100 and 500, to determine whether it is effective, identify an optimal dose, and to further evaluate its short-term safety.

Phase III—The investigational drug or biologic is given to a larger, more diverse patient population, often involving between 1,000 and 5,000 patients (but sometimes many more), to generate statistically significant evidence to confirm its safety and effectiveness. Phase III studies are the longest studies and usually take place in multiple sites around the world.