

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>
allogeneic mesenchymal stem cell therapy (itMSCs)	Stemedica Cell Technologies San Diego, CA	Alzheimer's disease	Phase II www.stemedica.com
AstroStem stem cell therapy	Nature Cell Seoul, South Korea	Alzheimer's disease	Phase I/II www.stemcellbio.com

Arthritis/Musculoskeletal Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AdipoCell™ adipose-derived autologous stem cell therapy	U.S. Stem Cell Sunrise, FL	intervertebral disc degeneration	Phase II www.us-stemcell.com
CybroCell™ human dermal fibroblast-based cell therapy	SpinalCyte Houston, TX	intervertebral disc degeneration	Phase I/II www.spinalcyte.com
ECCO-50 (adipose-derived autologous stem and regenerative cell therapy)	Cytori Therapeutics San Diego, CA	osteoarthritis of the knee	Phase II completed www.cytori.com

Arthritis/Musculoskeletal Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
IDCT (allogeneic injectable discogenic cell therapy)	DiscGenics Salt Lake City, UT	lumbar degenerative disc disease	Phase I/II www.discgenics.com
JointStem mesenchymal stem cell therapy	Nature Cell Seoul, South Korea	osteoarthritis of the knee	Phase II www.stemcellbio.com
mesenchymal stem cell therapy	Medipost Seoul, South Korea	osteoarthritis	Phase I/II completed www.medipost.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
rexlemestrocel-L (MPC-300-IV) (allogeneic mesenchymal precursor stem 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 II www.tissuegene.com

Autoimmune Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AAV1-follistatin (follistatin gene therapy) ORPHAN DRUG	Milo Biotechnology Cleveland, OH	inclusion body myositis	Phase I/II completed www.milobiotechnology.com
ATA188 (allogeneic T-cell activated therapy)	Atara Biotherapeutics South San Francisco, CA	progressive multiple sclerosis, relapsing remitting multiple sclerosis	Phase I www.atarabio.com
ATA190 (autologous EBV-specific cytotoxic T lymphocyte cell therapy)	Atara Biotherapeutics South San Francisco, CA	progressive multiple sclerosis	Phase I www.atarabio.com
cemdisiran (siRNA, RNA interference)	Alnylam Pharmaceuticals Cambridge, MA	complement-mediated diseases	Phase II www.alnylam.com
FCX-013 (genetically-modified autologous fibroblast cell therapy) ORPHAN DRUG	Fibrocell Science Exton, PA Intrexon Germantown, MD	moderate to severe localized scleroderma (Fast Track)	Phase I www.fibrocell.com www.dna.com
Habeo™ autologous cell therapy ORPHAN DRUG	Cytori Therapeutics San Diego, CA	hand scleroderma	Phase III completed www.cytori.com

Bladder Disorders

Drug Name

Sponsor

Indication

Development Phase

AMDC
(autologous muscle-derived
stem cell therapy)

Cook MyoSite
Pittsburgh, PA

stress urinary incontinence

Phase III
www.cookmyosite.com

underactive bladder

Phase I/II
www.cookmyosite.com

hMaxi-K gene therapy

Urovant Sciences
Irvine, CA

overactive bladder

Phase I
www.urovant.com

Blood Disorders

Drug Name

Sponsor

Indication

Development Phase

AMT-061
(AAV5-based gene therapy)

uniQure
Lexington, MA

hemophilia B

Phase III
www.uniqure.com

AT342
(AAV8-based gene therapy)
ORPHAN DRUG

Audentes Therapeutics
San Francisco, CA

Crigler-Najjar Syndrome
(Fast Track)

Phase I/II
www.audentestx.com

BCL11a shRNA(miR)
(miRNA-embedded shRNA,
RNA interference)

Boston Children's Hospital
Boston, MA
bluebird bio
Cambridge, MA

severe sickle cell disease

Phase I
www.bluebirdbio.com

Blood Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CTX001 (gene edited hematopoietic stem cell therapy)	CRISPR Therapeutics Cambridge, MA Vertex Pharmaceuticals Boston, MA	beta thalassemia	Phase I/II www.crisprtx.com www.vrtx.com
dilanubice (NLA101) (expanded progenitor stem cell therapy)	Nohla Therapeutics Seattle, WA	chemotherapy-induced neutropenia	Phase II www.nohlatherapeutics.com
fidanacogene elaparvovec (AAV8-hFIX19 gene therapy) ORPHAN DRUG	Pfizer New York, NY Spark Therapeutics Philadelphia, PA	hemophilia B	Phase III www.pfizer.com www.sparktx.com
fitusiran (siRNA, RNA interference) ORPHAN DRUG	Alnylam Pharmaceuticals Cambridge, MA Sanofi Genzyme Cambridge, MA	hemophilia A, hemophilia B	Phase III www.alnylam.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
		severe sickle cell disease (Fast Track)	Phase I www.bluebirdbio.com

Blood Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
NiCord™ cord blood stem cell therapy	Gamida Cell Jerusalem, Israel	severe aplastic anemia	Phase I www.gamida-cell.com
OTL-103 (ex vivo autologous stem cell therapy)	Orchard Therapeutics Boston, MA	Wiskott-Aldrich syndrome	Phase III www.orchard-tx.com
OTL-300 (ex vivo lentiviral stem cell gene therapy)	Orchard Therapeutics Boston, MA	transfusion-dependent beta thalassemia	in clinical trials www.orchard-tx.com
PF-07055480 (AAV-FVIII gene therapy) ORPHAN DRUG	Pfizer New York, NY Sangamo Therapeutics Richmond, CA	hemophilia A (Fast Track)	Phase I/II www.pfizer.com
romyelocel-L (allogeneic myeloid progenitor cell therapy)	Cellerant Therapeutics San Carlos, CA	neutropenia in AML	Phase II www.cellerant.com
RP-L101 (lentivirus-based gene therapy)	Rocket Pharmaceuticals New York, NY	Fanconi's anemia	Phase I www.rocketpharma.com
RP-L102 (lentivirus-based gene therapy) ORPHAN DRUG	Rocket Pharmaceuticals New York, NY	Fanconi's anemia	Phase I www.rocketpharma.com

Blood Disorders

Drug Name

Sponsor

Indication

Development Phase

SB-FIX
(factor IX gene therapy)
ORPHAN DRUG

Sangamo Therapeutics
Richmond, CA

hemophilia B (Fast Track)

Phase I/II
www.sangamo.com

SHP654
(AAV8-based gene therapy)
ORPHAN DRUG

Shire
Lexington, MA

hemophilia A

Phase I
www.shire.com

SPK-8011
(factor VIII gene therapy)
ORPHAN DRUG

Spark Therapeutics
Philadelphia, PA

hemophilia A

Phase I/II
www.sparktx.com

SPK-8016
(factor VIII gene therapy)
ORPHAN DRUG

Spark Therapeutics
Philadelphia, PA

hemophilia A

Phase I/II
www.sparktx.com

ST-400
(autologous hematopoietic stem/
progenitor cell therapy)

Bioverativ-Sanofi
Waltham, MA
Sangamo Therapeutics
Richmond, CA

beta thalassemia

Phase I/II
www.sangamo.com

valoctocogene roxaparvovec
(AAV-factor VIII gene therapy)
ORPHAN DRUG

BioMarin Pharmaceutical
San Rafael, CA

hemophilia A (Breakthrough Therapy)

Phase III
www.biomin.com

Cancer
Drug Name

Sponsor

Indication

Development Phase

AB-110
(hematopoietic stem and progenitor cells co-cultured and expanded with genetically-modified endothelial cells)

Angiocrine Bioscience
San Diego, CA

hematological malignancies

Phase I
www.angiocrinebioscience.com

ACTR087
(antibody-coupled T-cell receptor immunotherapy)

Unum Therapeutics
Cambridge, MA

non-Hodgkin's Lymphoma (NHL)
(+rituximab)

Phase I
www.unumrx.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

NHL (+rituximab)

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

Phase I/II
www.multivir.com

Cancer
Drug Name

Sponsor

Indication

Development Phase

Ad-RTS-hIL-12
(DNA-based in vivo gene therapy)
ORPHAN DRUG

Ziopharm Oncology
Boston, MA

recurrent glioblastoma
(combination therapy),
pediatric brain tumors

Phase I
www.ziopharm.com

ADX5-NEO immunotherapy
(patient-specific neoepitope-
based vaccine)

Advaxis
Princeton, NJ
Amgen
Thousand Oaks, CA

colorectal cancer, non-small cell lung
cancer (NSCLC), head and neck cancer

Phase I
www.advaxis.com
www.amgen.com

AFP
(alpha fetoprotein targeted
T-cell therapy)

Adaptimmune
Philadelphia, PA

liver cancer

Phase I/II
www.adaptimmune.com

aglatimagene besadenovec
(cancer suicide gene therapy)
ORPHAN DRUG

Advantagene
Auburndale, MA

intermediate/high risk localized
prostate cancer (Fast Track)

Phase III
www.advantagene.com

1L glioblastoma, localized prostate
cancer (active surveillance),
advanced non-metastatic pancreatic
cancer

Phase II
www.advantagene.com

1L glioblastoma (+PD-1),
resectable NSCLC

Phase I
www.advantagene.com

Cancer Drug Name	Sponsor	Indication	Development Phase
AlloStim T-cell therapy-device combination vaccine	Immunovative Therapies Jerusalem, Israel	metastatic colorectal cancer	Phase II www.immunovative.com
AMG 119 (CAR-T cell therapy)	Amgen Thousand Oaks, CA	small cell lung cancer (SCLC)	Phase I www.amgen.com
Andes-1537 (mitochondrial RNA; RNA interference)	Andes Biotechnologies Santiago, Chile	solid tumors	Phase I www.andesbio.com
APN401 (siRNA; RNA interference)	Apeiron Biologics Vienna, Austria	solid tumors	Phase II www.apeiron-biologics.com
APS001F (cytosine deaminase gene therapy)	Anaeropharma Science Tokyo, Japan	solid tumors	Phase I/II www.anaeropharma.co.jp
ATA520 (WT1 protein-specific T-cell therapy)	Atara Biotherapeutics South San Francisco, CA	hematologic malignancies	Phase I www.atarabio.com
AU101 (HER2-CD28 CAR-T cell therapy)	Aurora BioPharma Cambridge, MA	recurrent glioblastoma	Phase I/II www.aurora-biopharma.com
AU105 (HER2-CMV CAR-T cell therapy)	Aurora BioPharma Cambridge, MA	newly-diagnosed glioblastoma	Phase I/II www.aurora-biopharma.com

Cancer Drug Name	Sponsor	Indication	Development Phase
AV-GBM-1 (autologous dendritic cell vaccine)	AIVITA Biomedical Irvine, CA	glioblastoma, ovarian cancer	Phase II www.aivitabiomedical.com
axalimogene filolisbac (AXAL) (cancer immunotherapy vaccine) ORPHAN DRUG	Advaxis Princeton, NJ	cervical cancer (Fast Track)	Phase III www.advaxis.com
		anal cancer, head and neck cancer	Phase II www.advaxis.com
baltaleucel-T (CMD003) (EBV-targeted T-cell therapy) ORPHAN DRUG	Cell Medica London, United Kingdom	Epstein-Barr virus (EBV)-positive lymphoma (Fast Track)	Phase II www.cellmedica.com
bb2121 (BCMA CAR-T cell therapy) ORPHAN DRUG	bluebird bio Cambridge, MA Celgene Summit, NJ	relapsing/remitting multiple myeloma	Phase II www.bluebirdbio.com www.celgene.com
bb21217 (BCMA CAR-T cell therapy) ORPHAN DRUG	bluebird bio Cambridge, MA Celgene Summit, NJ	multiple myeloma	Phase I www.bluebirdbio.com www.celgene.com
BPX-201 (autologous dendritic cell vaccine)	Bellicum Pharmaceuticals Houston, TX	prostate cancer	Phase I completed www.bellicum.com

Cancer Drug Name	Sponsor	Indication	Development Phase
BPX-601 (GoCAR-T cell therapy)	Bellicum Pharmaceuticals Houston, TX	non-resectable pancreatic cancer	Phase I www.bellicum.com
BPX-701 (T-cell receptor-based therapy)	Bellicum Pharmaceuticals Houston, TX	acute myeloid leukemia (AML), myelodysplastic syndromes	Phase I www.bellicum.com
CAR CD19 2nd generation shortened manufacture (anti-CD19 CAR-T cell therapy)	Ziopharm Oncology Boston, MA	leukemia, lymphoma	Phase I www.ziopharm.com
CAR CD33 with lentivirus (anti-CD33 CAR-T cell therapy)	Ziopharm Oncology Boston, MA	AML	Phase I www.ziopharm.com
CarVAC (CAR-T cell therapy)	iCell Gene Therapeutics Stony Brook, NY	B-cell malignancies, multiple myeloma	Phase I www.icellgene.com
CD38 CAR-T (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, peritoneal metastases	Phase I www.sorrentotherapeutics.com
CLL1-CD33 cCART cell therapy (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, myelodysplastic syndromes	Phase I www.fortressbiotech.com
cobomarsen (microRNA; RNA interference)	miRagen Therapeutics Boulder, CO	cutaneous T-cell lymphoma (CTCL), chronic lymphocytic leukemia (CLL), diffuse large B-cell lymphoma (DLBCL), T-cell lymphoma/leukemia	Phase I www.miragen.com
CSTD-002-NK (adoptive natural killer cell therapy)	CytoSen Therapeutics Dallas, TX	AML	Phase I www.cytosen.com

Cancer Drug Name	Sponsor	Indication	Development Phase
CTL119 (BCMA CAR-T cell therapy)	Novartis East Hanover, NJ	multiple myeloma	Phase I www.novartis.com
CYAD 01 (CAR-T cell therapy)	Celyad New York, NY	colorectal cancer, ovarian cancer, bladder cancer, triple-negative breast cancer, pancreatic cancer, AML, myelodysplastic syndromes, multiple myeloma	Phase I/II www.celyad.com
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 I completed www.nwbio.com
Descartes-08 (anti-BCMA CAR-T cell therapy)	Cartesian Therapeutics Rockville, MD	multiple myeloma	Phase I www.cartesiantherapeutics.com
DFP-11207 (siRNA; RNA interference)	Delta-Fly Pharma Tokushima, Japan	solid tumors	Phase I www.delta-flypharma.com

Cancer
Drug Name

Sponsor

Indication

Development Phase

dilanubicel (NLA101)
(expanded progenitor stem cell therapy)
ORPHAN DRUG

Nohla Therapeutics
Seattle, WA

hematologic malignancies (Fast Track)

Phase I
www.nohlatherapeutics.com

ET190L1 ARTEMIS
(CAR-T cell therapy)

Eureka Therapeutics
Emeryville, CA

NHL

Phase I
www.eurekatherapeutics.com

Fate-NK100
(natural killer cell cancer immunotherapy)

Fate Therapeutics
La Jolla, CA

AML, ovarian, solid tumors
(combination mAb therapy)

Phase I
www.fatetherapeutics.com

GEN-1
(IL-12 gene therapy nanoparticle)
ORPHAN DRUG

Celsion
Lawrenceville, NJ

ovarian cancer

Phase II
www.celsion.com

GSK3377794
(NY-ESO-1 autologous engineered TCR T-cells)
ORPHAN DRUG

GlaxoSmithKline
Research Triangle Park, NC

melanoma, multiple myeloma,
NSCLC, ovarian, synovial sarcoma

Phase II
www.gsk.com

ICT-121
(dendritic cell vaccine)

ImmunoCellular Therapeutics
Calabasas, CA

recurrent glioblastoma

Phase I completed
www.imuc.com

ilixadencel
(dendritic cell vaccine)

Immunicum
Gothenburg, Sweden

renal cell carcinoma

Phase II
www.immunicum.com

Cancer Drug Name	Sponsor	Indication	Development Phase
IMA 101 (autologous endogenous T-cell therapy)	Immatics US Houston, TX	solid tumors	Phase I www.immatics.com
IMA 202 (autologous adoptive T-cell therapy)	Immatics US Houston, TX	solid tumors	Phase I www.immatics.com
inodiftagene vixteplasmid (diphtheria toxin A gene therapy)	Anchiano Therapeutics Cambridge, MA	bladder cancer (Fast Track)	Phase II www.anchiano.com
INVAC-1 (DNA-based cancer vaccine)	Invectys Paris, France	CLL	Phase II www.invectys.com
JCARH125 (anti-BCMA CAR-T cell therapy) ORPHAN DRUG	Celgene Summit, NJ Juno Therapeutics Seattle, WA	multiple myeloma	Phase I/II www.celgene.com
JNJ-68284528 (LCAR-B38M CAR-T cell therapy)	Janssen Biotech Horsham, PA	multiple myeloma	Phase I/II www.janssen.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

Cancer
Drug Name

Sponsor

Indication

Development Phase

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

KTE-X19
(CAR-T cell therapy)

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

mantle cell lymphoma

Phase II
www.kitepharma.com

acute lymphocytic leukemia (ALL)

Phase I
www.kitepharma.com

Kymriah®
tisagenlecleucel
(CAR-T cell therapy)
ORPHAN DRUG

Novartis
East Hanover, NJ

DLBCL (+pembrolizumab)

Phase I
www.novartis.com

LMAP-Vax + pp65 dendritic cell therapy
(dendritic cell vaccine)

Immunomic Therapeutics
Rockville, MD

newly-diagnosed glioblastoma

Phase I/II
www.immunomix.com

lifileucel (LN-144)
(TIL autologous cell therapy)
ORPHAN DRUG

Iovance Biotherapeutics
San Carlos, CA

malignant melanoma
(Fast Track)

Phase II
www.iovance.com

Cancer
Drug Name

Sponsor

Indication

Development Phase

<p>lisocabtagene maraleucel (JCAR017) (CAR-T cell therapy) ORPHAN DRUG</p>	<p>Celgene Summit, NJ Juno Therapeutics Seattle, WA</p>	<p>relapsed/refractory aggressive large B-cell lymphoma</p>	<p>Phase II www.celgene.com</p>
		<p>CLL</p>	<p>Phase I www.celgene.com</p>
<p>LN-145 (TIL autologous cell therapy) ORPHAN DRUG</p>	<p>Iovance Biotherapeutics San Carlos, CA</p>	<p>cervical cancer, head and neck cancer, NSCLC</p>	<p>Phase II www.iovance.com</p>
		<p>head and neck cancer (+pembrolizumab), melanoma (+pembrolizumab), NSCLC (+durvalumab)</p>	<p>Phase II www.iovance.com</p>
<p>LOAd703 (immunostimulatory gene therapy with oncolytic adenovirus therapy) ORPHAN DRUG</p>	<p>Lokon Pharma Uppsala, Sweden</p>	<p>pancreatic cancer</p>	<p>Phase I/II www.lokonpharma.com</p>
<p>MAGE-A4 (melanoma-associated antigen A4-specific T-cells)</p>	<p>Adaptimmune Philadelphia, PA</p>	<p>solid tumors</p>	<p>Phase I www.adaptimmune.com</p>
<p>MAGE-A10 (melanoma-associated antigen A10-specific T-cells)</p>	<p>Adaptimmune Philadelphia, PA</p>	<p>head and neck cancer, NSCLC, melanoma, urothelial cancer</p>	<p>Phase I/II www.adaptimmune.com</p>

Cancer Drug Name	Sponsor	Indication	Development Phase
MB-101 (CAR-T cell therapy)	Mustang Bio New York, NY	glioblastoma	Phase I www.mustangbio.com
MB-102 (CAR-T cell therapy)	Mustang Bio New York, NY	AML, blastic plasmacytoid dendritic cell neoplasm (BPDCN)	Phase I www.mustangbio.com
MB-103 (CAR-T cell therapy)	Mustang Bio New York, NY	glioblastoma	Phase I www.mustangbio.com
MB-106 (CAR-T cell therapy)	Mustang Bio New York, NY	B-cell NHL	Phase I www.mustangbio.com
MCY-M11 (CAR-T cell therapy)	MaxCyte Gaithersburg, MD	advanced ovarian cancer, peritoneal mesothelioma	Phase I www.maxcyte.com
microencapsulated cell therapy ORPHAN DRUG	PharmaCyte Biotech Laguna Hills, CA	pancreatic cancer	Phase II www.pharmacYTE.com
MTV273 (BCMA CAR-T cell therapy)	Novartis East Hanover, NJ	multiple myeloma	Phase I www.novartis.com
multi AA T-cells (autologous cytotoxic T-cell therapy)	Marker Therapeutics Houston, TX	lymphoma, multiple myeloma	Phase I www.markertherapeutics.com

Cancer
Drug Name

Sponsor

Indication

Development Phase

nadofaragene firadenovec
(IFNA2B gene therapy)

Ferring Pharmaceuticals
Parsippany, NJ
FKD Therapies
Kuopio, Finland

superficial bladder cancer
(Fast Track) (Breakthrough Therapy)

Phase III
www.ferring.com

nalotimagene carmaleucel
(ex vivo cell therapy)
ORPHAN DRUG

MolMed
Milano, Italy

acute leukemia

Phase III
www.molmed.com

NAM-NK cells
(natural killer cell replacement)

Gamida Cell
Jerusalem, Israel

multiple myeloma, NHL

Phase I
www.gamida-cell.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, colorectal
cancer, hepatocellular carcinoma,
pancreatic cancer, squamous cell
carcinoma

Phase I/II
www.nantkwest.com

solid tumors

Phase I
www.nantkwest.com

Cancer
Drug Name

Sponsor

Indication

Development Phase

NiCord™ cord blood stem cell therapy ORPHAN DRUG	Gamida Cell Jerusalem, Israel	high-risk hematological malignancies	Phase III www.gamida-cell.com
NYESO-1 triple knockout T-cell receptor (TCR) therapeutic	Tmunity Philadelphia, PA	melanoma, multiple myeloma, synovial sarcoma	Phase I www.timunity.com
ofranergene obadenovec (targeted gene therapy) ORPHAN DRUG	VBL Therapeutics Modi'in, Israel	glioblastoma (Fast Track), recurrent platinum-resistant ovarian cancer	Phase III www.vblrx.com
Oncoprex™ gene therapy	Genprex Austin, TX	non-small cell lung cancer (NSCLC)	Phase II www.genprex.com
ovapuldencel-T (tumor stem cell specific dendritic cell vaccine)	AiVita Biomedical Irvine, CA	glioblastoma, ovarian cancer	Phase II www.aivitabiomedical.com
PACTN (autologous T-cell therapy)	PersImmune San Diego, CA	myelodysplastic syndromes	Phase I
P-BCMA-101 (CAR-T cell therapy)	Poseida Therapeutics San Diego, CA	multiple myeloma	Phase I www.poseida.com

Cancer Drug Name	Sponsor	Indication	Development Phase
pbi-shRNAEWS-FLI-Type 1 (siRNA, RNA interference)	Gradalis Houston, TX	Ewings sarcoma	Phase I www.gradalisinc.com
PEPIDH1M (dendritic cell vaccine)	Annias Immunotherapeutics Chapel Hill, NC	glioblastoma	Phase I/II www.anniasimmuno.com
PNK-007 (culture expanded natural killer cell therapy)	Celularity Warren, NJ	AML, multiple myeloma	Phase I www.celularity.com
Provenge® sipuleucel-T	Dendreon Seal Beach, CA	newly-diagnosed prostate cancer	Phase III www.dendreon.com
PSC-100 (placental-derived stem cell therapy)	Celularity Warren, NJ	hematological disorders and malignancies	Phase I www.celularity.com
PSMA CAR-T cell therapy	Tmunity Philadelphia, PA	metastatic castration-resistant prostate cancer	Phase I www.timunity.com
REIC gene therapy (AAV-based gene therapy)	Momotaro-Gene Okayama, Japan	prostate cancer	Phase I/II www.mt.gene.com

Cancer
Drug Name

Sponsor

Indication

Development Phase

rivogenlecleucel (BPX-501) (allogeneic polyclonal T-cell therapy)	Bellicum Pharmaceuticals Houston, TX	ALL (pediatric), AML (adults), hematological malignancies (adult)	Phase I/II www.bellicum.com
		hematological malignancies (pediatric)	Phase I www.bellicum.com
SGT-53 (p53 DNA gene therapy)	SynerGene Therapeutics Potomac, MD	pancreatic cancer, recurrent glioblastoma	Phase II
		solid tumors (pediatric)	Phase I
siG12D-LODER (antisense K-ras siRNA)	Silenseed Modi'in, Israel	pancreatic cancer	Phase II www.silenseed.com
SL-701 (dendritic cell vaccine) ORPHAN DRUG	Stemline Therapeutics New York, NY	glioblastoma (adults)	Phase II www.stemline.com
stapuldencel-T (DVAC/Pca) (autologous dendritic cell vaccine)	SOTIO Prague, Czech Republic	metastatic castration-resistant prostate cancer	Phase III www.sotio.com
STP705L (siRNA-based therapeutic) ORPHAN DRUG	Sirnaomics Gaithersburg, MD	liver cancer	Phase I www.sirnaomics.com

Cancer
Drug Name

Sponsor

Indication

Development Phase

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

tab-cel (tabelecleucel)
(EBV-specific T-cell therapy)
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,
nasopharyngeal carcinoma

Phase II
www.atarabio.com

TAPA-pulsed dendritic cell vaccine

Kiromic
Houston, TX

hematologic malignancies,
solid tumors

Phase I/II
www.kiromic.com

tavokinogene tetsaplasamid
(interleukin-12 gene therapy)
ORPHAN DRUG

OncoSec Medical
San Diego, CA
Merck
Kenilworth, NJ

relapsed/refractory melanoma (Fast Track)
(+Keytruda), triple negative breast
cancer (+Keytruda)

Phase II
www.oncosec.com
www.merck.com

OncoSec Medical
San Diego, CA

triple negative breast cancer

Phase I
www.oncosec.com

**Cancer
Drug Name**

Sponsor

Indication

Development Phase

TLPLDC (dendritic cell vaccine)	Cancer Insight San Antonio, TX Elios Therapeutics Austin, TX	melanoma	Phase II www.cancerinsight.com
		ovarian cancer	Phase I/I www.cancerinsight.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
		metastatic solid tumors	Phase I www.tocagen.com
TT10 (T-cell immunotherapy) ORPHAN DRUG	Tessa Therapeutics Singapore	nasopharyngeal cancer (Fast Track)	Phase III www.tessatherapeutics.com
TVI-Brain-1 cancer vaccine (personalized combination of cancer cell vaccination and killer T-cells) ORPHAN DRUG	TVAX Biomedical Lenexa, KS	astrocytoma, glioblastoma, glioma	Phase II completed www.tvaxbiomedical.com
UCART19 (CAR-T cell therapy)	Allogene South San Francisco, CA Servier Neuilly-sur-Seine, France	ALL	Phase I www.allogene.com

Cancer Drug Name	Sponsor	Indication	Development Phase
UCART123 (CAR-T cell therapy)	Cellectis New York, NY	AML, BPDCN	Phase I www.cellectis.com
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
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	Phase II www.gradalisinc.com
		gynecologic cancer (combination therapy)	Phase I www.gradalisinc.com
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)	Phase II www.gilead.com

Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ACP-01 (angiogenic cell precursor therapy)	Hemostemix Calgary, Canada	critical limb ischemia	Phase II www.hemostemix.com
allogeneic mesenchymal stem cell therapy (itMSCs)	Stemedica Cell Technologies San Diego, CA	chronic heart failure, ischemic stroke	Phase II www.stemedica.com
AMG 890 (siRNA; RNA interference)	Amgen Thousand Oaks, CA	cardiovascular disease	Phase I www.amgen.com
ASCT01 (autologous stem cell therapy)	Lifecells North Kansas City, MO	critical limb ischemia	Phase I/II completed www.lifecellsllc.com
bepnerminogene perplasmid (HGF gene therapy)	AnGes USA Bethesda, MD	critical limb ischemia (Fast Track)	Phase III www.anges.co.jp
CardiALLO™ allogenic culture expanded mesenchymal stem cells derived from bone marrow	BioCardia San Carlos, CA	ischemic heart failure	Phase I www.biocardia.com
CardiAMP™ autologous minimally processed bone marrow cells	BioCardia San Carlos, CA	chronic myocardial ischemia with refractory angina, ischemic heart failure	Phase III www.biocardia.com

Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
C-Cure® autologous stem cell therapy	Celyad New York, NY	chronic heart failure (Fast Track)	Phase III www.celyad.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	refractory angina	Phase III www.caladrius.com
		coronary microvascular dysfunction	Phase II www.caladrius.com
donaperminogene seltoplasmid (plasmid DNA vector encoding modified HGF gene therapy)	ViroMed Seoul, South Korea	peripheral artery disease	Phase III www.viomed.co.kr
Generx® alferminogene tadenovec (angiogenic gene therapy)	Angionetics San Diego, CA	refractory angina due to myocardial ischemia (Fast Track)	Phase II completed www.angiogenetics.com
inclisiran (siRNA, RNA interference) ORPHAN DRUG	Alnylam Pharmaceuticals Cambridge, MA The Medicines Company Parsippany, NJ	hypercholesterolemia	Phase III www.alnylam.com

Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
INXN-4001 (multigene therapy)	Intrexon Germantown, MD	heart failure	Phase I www.dna.com
JVS-100 (non-viral gene therapy)	Juventas Therapeutics Cleveland, OH	advanced chronic heart failure (Fast Track), peripheral artery disease (Fast Track)	Phase II www.juventasinc.com
MPC-25-IC (allogeneic autologous mesenchymal precursor cell product)	Mesoblast New York, NY	acute cardiac ischemia	Phase II completed www.mesoblast.com
MRG-110 (microRNA-92, RNA interference)	miRagen Boulder, CO Servier Neuilly-sur-Seine, France	heart failure	Phase I www.mitagen.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 completed www.us-stemcell.com

Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
PLX-PAD (emiplacel) (human placental stromal cell therapy)	Pluristem Therapeutics Haifa, Israel	critical limb ischemia (Fast Track)	Phase III www.pluristem.com
rexlemestrocel-L (MPC-150-M) (allogeneic autologous mesenchymal precursor cell product)	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 (AAV8 gene therapy) ORPHAN DRUG	REGENXBIO Rockville, MD	hyperlipoproteinemia type IIa (homozygous familial hypercholesterolemia)	Phase I/II www.regenxbio.com
RT-100 (AAV-based gene therapy)	Renova Therapeutics San Diego, CA	congestive heart failure (Fast Track)	Phase III www.renovatherapeutics.com
SERCA-2a gene therapy (AAV gene therapy)	Theragene Pharmaceuticals San Diego, CA	heart failure (Fast Track) (NYHA class III/IV disease)	Phase II www.theragenepharma.com

Crohn's Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
cenplacel-L (PDA-001) (human placental-derived stem cell therapy)	Celularity Warren, NJ	Crohn's disease	Phase II completed www.celularity.com
remestemcel-L (MSC-100-IV) (adult human 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>
CLBS03 (T-lymphocyte cell therapy) ORPHAN DRUG	Caladrius Biosciences Basking Ridge, NJ	recent onset type 1 diabetes (Fast Track)	Phase II www.caladrius.com
DV-100 (autologous dendritic cell vaccine) ORPHAN DRUG	DiaVacs La Jolla Shores, CA	type diabetes	Phase II www.diaavacs.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

Diabetes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
PEC-Encap™ encapsulated human pancreatic cell therapy	ViaCyte San Diego, C	type 1 diabetes	Phase I/II www.viacyte.com

Eye Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AAV-CNGB3 (AAV-based gene therapy) ORPHAN DRUG	MeiraGTX New York, NY	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	X-linked retinitis pigmentosa (Fast Track)	Phase I/II www.meiragtx.com
ACHN-CNGA3 (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>
ACHN-CNGB3 (AAV gene therapy) ORPHAN DRUG	Applied Genetic Technologies Alachua, FL	achromatopsia	Phase I/II www.agtc.com
ADVM-022 (AAV-based gene therapy)	Adverum Biotechnologies Menlo Park, CA	wet age-related macular degeneration	Phase I www.adverum.com
ASP7317 (human retinal pigment epithelial cell therapy)	Astellas Pharma Northbrook, IL	dry age-related macular degeneration, Stargardt disease	Phase II www.astellas.com
bamosiran (siRNA, RNA interference)	Sylentis Madrid, Spain	glaucoma, ocular hypertension	Phase II www.sylentis.com
BIIB-087 (XLR5) (AAV-based gene therapy) ORPHAN DRUG	Applied Genetic Technologies Alachua, FL Biogen Cambridge, MA	X-linked retinoschisis	Phase I/II www.agtc.com www.biogen.com
BIIB-088 (XLRP) (AAV-based gene therapy) ORPHAN DRUG	Applied Genetics Technologies Alachua, FL Biogen Cambridge, MA	X-linked retinitis pigmentosa	Phase I/II www.agtc.com www.biogen.com
CPCB-RPE1 (embryonic stem cell therapy)	Regenerative Patch Technologies Portola Valley, CA	dry age-related macular degeneration	Phase I/II www.regenerativepatch.com

Eye Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CPK850 (AAV8 gene therapy)	Novartis East Hanover, NJ	RLBP1-mutated retinitis pigmentosa	Phase I/II www.novartis.com
GS010 (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 combined immunodeficiency	Phase I/II www.gensight-biologics.com
HMR59 (AAV2-based gene therapy)	Hemera Biosciences Newton, MA	dry age-related macular degeneration, wet age-related macular degeneration, geographic atrophy	Phase I www.hemerabiosciences.com
jCell (retinal stem cell therapy)	jCyte Newport Beach, CA	retinitis pigmentosa	Phase II www.jcyte.com
NSR-REP1 (AAV-based gene therapy) ORPHAN DRUG	Nightstar Therapeutics Waltham, MA	choroideremia	Phase III www.nightstartx.com
NSR-RPGR (AAV-based gene therapy) ORPHAN DRUG	Nightstar Therapeutics Waltham, MA	X-linked retinitis pigmentosa	Phase I/II www.nightstartx.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	Phase III www.neurotechusa.com
		glaucoma	Phase II www.neurotechusa.com
OpRegen retinal pigment epithelial cell replacement therapy	Cell Cure Neurosciences Jerusalem, Israel	dry age-related macular degeneration (Fast Track)	Phase II www.cellcureneurosciences.com
OXB-201 (angiostatin endostatin gene therapy)	Oxford BioMedica Oxford, United Kingdom	wet age-related macular degeneration	Phase I completed www.oxfordbiomedica.co.uk
PF-655 (siRNA, RNA interference)	Quark Pharmaceuticals Newark, CA	diabetic macular edema, age-related macular degeneration	Phase II completed www.quarkpharma.com
QPI-1007 (siRNA, RNA interference) ORPHAN DRUG	Quark Pharmaceuticals Newark, CA	non-arteritic anterior ischemic optic neuropathy	Phase III www.quarkpharma.com
		acute primary angle closure glaucoma	Phase II www.quarkpharma.com
ReN-003 (human retinal stem cell therapy)	ReNeuron Bridgend, United Kingdom	retinitis pigmentosa	Phase II www.reneuron.com

Eye Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
RGX-314 (AAV8 gene therapy)	REGENXBIO Rockville, MD	wet age-related macular degeneration	Phase I 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 I/II www.sanofi.com
SPK-7001 (AAV-based gene therapy) ORPHAN DRUG	Spark Therapeutics Philadelphia, PA	X-linked choroideremia	Phase I/II www.sparktx.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AAV2/8-LSPhGAA (AAV-based gene therapy)	Actus Therapeutics Chapel Hill, NC	Pompe disease (glycogen storage disease type II)	Phase I www.actustherapeutics.com
ABO-101 (AAV-based gene therapy) ORPHAN DRUG	Abeona Therapeutics New York, NY	mucopolysaccharidosis III B (Sanfilippo syndrome type B)	Phase I/II www.abeonatherapeutics.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ABO-102 (AAV-based gene therapy) ORPHAN DRUG	Abeona Therapeutics New York, NY	mucopolysaccharidosis III A (Sanfilippo syndrome type A) (Fast Track)	Phase I/II www.abeonatherapeutics.com
AT982 (AAV-based gene therapy)	Audentes Therapeutics San Francisco, CA	Pompe disease (glycogen storage disease type II)	Phase I www.audentestx.com
AVR-RD-01 (gene therapy)	AVROBIO Cambridge, MA	Fabry's disease	Phase I/II www.avrobio.com
DTX301 (AAV8 gene therapy) ORPHAN DRUG	Ultragenyx Pharmaceutical Novato, CA	ornithine transcarbamylase deficiency (Fast Track)	Phase I www.ultragenyx.com
DTX401 (AAV8 gene therapy) ORPHAN DRUG	Ultragenyx Pharmaceutical Novato, CA	glycogen storage disease type I (Fast Track)	Phase I www.ultragenyx.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
OTL-101 (ex vivo autologous lentiviral 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

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
OTL-102 (ex vivo autologous gene therapy)	Genethon Cedex, France Orchard Therapeutics Boston, MA	X-linked chronic granulomatous disease	Phase I/II www.orchard-tx.com
RGX-111 (AAV9 gene therapy) ORPHAN DRUG	REGENXBIO Rockville, MD	mucopolysaccharidosis type I (Fast Track)	Phase I 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
SB-318 (rAAV2/6 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 (rAAV2/6 gene therapy) ORPHAN DRUG	Sangamo Therapeutics Richmond, CA	mucopolysaccharidosis II (Fast Track) (Hunter syndrome)	Phase I/II www.sangamo.com
UshStat® myosin 7A gene therapy ORPHAN DRUG	Sanofi Bridgewater, NJ	Usher syndrome 1B	Phase I/II www.sanofi.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 www.argostherapeutics.com
ALVAC-HIV (vCP2438) (gene therapy)	Sanofi Pasteur Swiftwater, PA	HIV infections	Phase II/III www.sanofipasteur.com
ARB-1467 (siRNA, RNA interference)	Arbutus Biopharma Warminster, PA	hepatitis B	Phase II www.arbutusbio.com
ARO-HBV (siRNA, RNA interference)	Arrowhead Pharmaceuticals Pasadena, CA	hepatitis B	Phase I/II www.arrowheadpharma.com
ATA230 (CMV cytotoxic T- dll therapy) 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 completed www.cslnehring.com
SB-728-T (ZFN-mediated genome editing in T-cells)	Sangamo Therapeutics Richmond, CA	HIV infections	Phase II 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 (prevention)	Phase II www.helocyte.com
V920 (recombinant Ebola vaccine)	Merck Kenilworth, NJ	Ebola infections	application submitted www.merck.com
Viralym-A™ adenovirus-specific T-cell immunotherapy ORPHAN DRUG	ViraCyte Houston, TX	adenovirus infections following hematopoietic stem cell therapy (HSCT)	Phase I www.viracyte.com
Viralym-C™ cytomegalovirus-specific T-cell immunotherapy ORPHAN DRUG	ViraCyte Houston, TX	cytomegalovirus infections following HSCT (Fast Track)	Phase I www.viracyte.com
Viralym-M™ multi-virus specific T-cell immunotherapy	ViraCyte Houston, TX	viral infections following HSCT (Adv, BK virus, CMV, EBV, HHV6)	Phase I www.viracyte.com

Kidney Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
DCR-PHXC (siRNA, RNA interference) ORPAHN DRUG	Dicerna Pharmaceuticals Cambridge, MA	primary hyperoxaluria	Phase I www.dicerna.com
lumasiran (siRNA, RNA interference) ORPHAN DRUG	Alnylam Pharmaceuticals Cambridge, MA	primary hyperoxaluria type 1	Phase III www.alnylam.com
Neo-Kidney Augment autologous cultured kidney tissue cell therapy	RegenMedTX Winston-Salem, NC	chronic kidney disease and type 2 diabetes mellitus	Phase II www.regenmedtx.com
QPI-1002 (siRNA, RNA interference)	Quark Pharmaceuticals Newark, NJ	acute kidney injury following cardiac surgery	Phase II www.quarkpharma.com
rexlemestrocel-L (MPC-300-IV) (allogeneic mesenchymal precursor stem cell therapy)	Mesoblast New York, NY	diabetic nephropathy in type 2 diabetes	Phase II www.mesoblast.com
RG-012 (SAR339375) (miRNA-21, RNA interference) ORPHAN DRUG	Regulus Therapeutics San Diego, CA Sanofi Genzyme Cambridge, MA	Alport syndrome	Phase II www.regulusrx.com
RGLS4326 (microRNA-17, RNA interference)	Regulus Therapeutics San Diego, CA	autosomal polycystic kidney disease	Phase I www.regulusrx.com

Liver Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ARO-AAT (siRNA, RNA interference) ORPHAN DRUG	Arrowhead Pharmaceuticals Pasadena, CA	alpha-1 antitrypsin deficiency	Phase I www.arrowheadpharma.com
BMS-986263 (siRNA, RNA interference)	Bristol-Myers Squibb Princeton, NJ	advanced hepatic fibrosis (Fast Track)	Phase II www.bms.com
givosiran (siRNA, RNA interference) ORPHAN DRUG	Alnylam Pharmaceuticals Cambridge, MA	acute hepatic porphyria (Breakthrough Therapy)	Phase III www.alnylam.com

Muscular Dystrophy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AAV1-follistatin (follistatin gene therapy) ORPHAN DRUG	Milo Biotechnology Cleveland, OH	Becker muscular dystrophy, Duchenne muscular dystrophy,	Phase I/II completed www.milobiotechnology.com
CAP-1002 (allogeneic cardiosphere-derived cell therapy) ORPHAN DRUG	Capricor Therapeutics Beverly Hills, CA	Duchenne muscular dystrophy	Phase II www.capricor.com

Muscular Dystrophy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
GALGT2 gene therapy	Nationwide Children's Hospital Columbus, OH Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy	Phase I/II www.sarepta.com
micro-dystrophin gene therapy	Nationwide Children's Hospital Columbus, OH Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy	Phase I/II www.sarepta.com
MYO-101 (AAV-Rh74 gene therapy) ORPHAN DRUG	Myonex Therapeutics New Albany, OH Sarepta Therapeutics Cambridge, MA	limb girdle muscular dystrophy (type 2E)	Phase I/II www.myonexustx.com
MYO-102 (AAV-Rh74 gene therapy)	Myonex Therapeutics New Albany, OH Sarepta Therapeutics Cambridge, MA	limb girdle muscular dystrophy (type 2D)	Phase I/II www.myonexustx.com
MYO-201 (AAV-Rh74 gene therapy)	Myonex Therapeutics New Albany, OH Sarepta Therapeutics Cambridge, MA	limb girdle muscular dystrophy (type 2B)	Phase I www.myonexustx.com

Muscular Dystrophy

Drug Name

Sponsor

Indication

Development Phase

PF-06939926
(rAAV gene therapy)
ORPHAN DRUG

Pfizer
New York, NY

Duchenne muscular dystrophy

Phase I
www.pfizer.com

SGT-001
(AAV-based gene therapy)
ORPHAN DRUG

Solid Biosciences
Cambridge, MA

Duchenne muscular dystrophy
(Fast Track)

Phase I/II
www.solidbio.com

Neurologic Disorders

Drug Name

Sponsor

Indication

Development Phase

allogeneic mesenchymal
stem cell therapy (itMSCs)

Stemedica Cell Technologies
San Diego, CA

traumatic brain injury

Phase II
www.stemedica.com

AST-OPC1
(human stem cell therapy)
ORPHAN DRUG

Asterias Biotherapeutics
Fremont, CA

spinal cord injury (subacute)

Phase I/II
www.asteriasbiotherapeutics.com

AT132
(AAV8-based gene therapy)
ORPHAN DRUG

Audentes Therapeutics
San Francisco, CA

X-linked myotubular myopathy
(Fast Track)

Phase I/II
www.audentestx.com

Neurologic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AVXS-101 (onasemnogene abeparvovec) (gene therapy) ORPHAN DRUG	AveXis Bannockburn, IL	spinal muscular atrophy (SMN1) (Fast Track) (Breakthrough Therapy)	application submitted www.avexis.com
		spinal muscular atrophy (SMN2)	Phase I www.avexis.com
CEVA101 (autologous bone marrow mononuclear cell therapy)	Cellvation New York, NY	traumatic brain injury	Phase II www.cellvation.com
CLN6 gene therapy	Amicus Therapeutics Cranbury, NJ	CLN6 neuronal ceroid lipofuscinosis (Batten disease)	Phase I/II www.amicusrx.com
donaperminogene seltoplasmid (plasmid DNA vector encoding modified hepatocyte growth factor gene therapy) ORPHAN DRUG	ViroMed Seoul, South Korea	diabetic peripheral neuropathy	Phase III www.viromed.co.kr
		amyotrophic lateral sclerosis (ALS) (Fast Track)	Phase I/II 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

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/II www.internationalstemcell.com
Lenti-D™ elivaldogene tavalentivec (stem cell therapy) ORPHAN DRUG	bluebird bio Cambridge, MA	cerebral adrenoleukodystrophy	Phase II/III www.bluebirdbio.com
LYS-SAF 301 (AAV-based gene therapy) ORPHAN DRUG	Lysogene Cambridge, MA Sarepta Therapeutics Cambridge, MA	mucopolysaccharidosis III	Phase II www.lysogene.com www.sarepta.com
neurotrophin-3 gene therapy	Sarepta Therapeutics Cambridge, MA Nationwide Children's Hospital Columbus, OH	Charcot-Marie-Tooth disease	Phase I/II www.sarepta.com
NSI-566 (spinal cord-derived neural stem cell therapy) ORPHAN DRUG	Neuralstem Germantown, MD	ALS	Phase II www.neuralstem.com
		chronic spinal cord injury	Phase I www.neuralstem.com

Neurologic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
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
OTL-200 (ex vivo stem cell gene therapy) ORPHAN DRUG	Orchard Therapeutics Boston, MA	metachromatic leukodystrophy (Breakthrough Therapy)	Phase III www.orchard-tx.com
PDA-002 (human placental-derived adherent cell therapy)	Celularity Warren, NJ	diabetic peripheral neuropathy	Phase II www.celularity.com
rexlemestrocel-L (MPC-06-ID) (allogeneic autologous mesenchymal precursor stem 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	chronic motor deficit due to stroke, 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	polyglutamine 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
WVE-120101 (allele-specific gene silencing; RNA interference) ORPHAN DRUG	WaVe Life Sciences Cambridge, MA	Huntington's disease	Phase I/II www.wavelifesciences.com
WVE-120102 (allele-specific gene silencing; RNA interference)	WaVe Life Sciences Cambridge, MA	Huntington's disease	Phase I/II www.wavelifesciences.com

Respiratory

<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	Phase II www.athersys.com
Pneumostem [®] mesenchymal stem cell therapy ORPHAN DRUG	Medipost Seoul, South Korea	bronchopulmonary dysplasia (pediatric)	Phase I/II www.medipost.com

Skin			
<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	cutaneous photoaging	Phase I completed www.stemedica.com
EB-101 (autologous ex vivo gene-corrected cell therapy) ORPHAN DRUG	Abeona Therapeutics New York, NY	recessive dystrophic epidermolysis bullosa	Phase I/II completed www.abeonatherapeutics.com
FCX-007 (genetically-modified autologous human dermal fibroblast cell therapy) ORPHAN DRUG	Fibrocell Science Exton, PA Intrexon Germantown, MD	recessive dystrophic epidermolysis bullosa (Fast Track)	Phase I/II www.fibrocell.com www.dna.com
KB103 (modified HSV-1 gene therapy) ORPHAN DRUG	Krystal Biotech Pittsburgh, PA	dystrophic epidermolysis bullosa (Fast Track)	Phase I/II www.krystalbio.com
MRG-110 (microRNA-92, RNA interference)	miRagen Boulder, CO Servier Neuilly-sur-Seine, France	incisional complications	Phase I www.mitagen.com
PDA-002 (human placental-derived adherent cell therapy)	Celularity Warren, NJ	diabetic foot ulcers	Phase II www.celularity.com

Skin

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
remlarsen (microRNA-29b, RNA interference)	miRagen Therapeutics Boulder, CO	cutaneous fibrosis	Phase II www.miragen.com
STP705 (siRNA-based therapeutic)	Sirnaomics Gaithersburg, MD	hypertrophic scars	Phase II www.sirnaomics.com

Transplantation

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ATIR101 (T-cell replacement therapy) ORPHAN DRUG	Kiadis Pharma Amsterdam, The Netherlands	graft-versus-host disease (GVHD) (Fast Track)	Phase III www.kiadis.com
CK0801 (allogeneic cord blood-derived regulatory T-cell therapy)	Cellenkos Houston, TX	GVHD	Phase I www.cellenkosinc.com
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.medeortherapeutics.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.medeortherapeutics.com

Transplantation

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
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.medeortherapeutics.com
MGTA-456 (allogeneic ex vivo stem cell therapy)	Magenta Therapeutics Cambridge, MA	in patients with inherited metabolic disorders undergoing HSCT	Phase II www.magentatx.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
		solid organ transplant support	Phase I www.athersys.com
NJA-730 (siRNA, RNA interference)	NapaJen Pharma Burlingame, CA	GVHD	Phase I www.napajen.com
PLX-R18 (placenta-derived allogenic stem cell therapy) ORPHAN DRUG	Pluristem Therapeutics Haifa, Israel	GVHD	Phase I www.pluristem.com
ProTmune™ ex vivo programmed hematopoietic cellular immunotherapy ORPHAN DRUG	Fate Therapeutics San Diego, CA	GVHD (Fast Track)	Phase II www.fatetherapeutics.com

Transplantation

Drug Name

Sponsor

Indication

Development Phase

QPI-1002
(siRNA, RNA interference)
ORPHAN DRUG

Quark Pharmaceuticals
Newark, NJ

delayed graft function (Fast Track)

Phase III
www.quarkpharma.com

remestemcel-L (MSC-100-IV)
(adult human mesenchymal stem
cell therapy)
ORPHAN DRUG

Mesoblast
New York, NY

acute GVHD (Fast Track)

Phase III
www.mesoblast.com

Other

Drug Name

Sponsor

Indication

Development Phase

AAV-AQP1
(AAV-based gene therapy)

MeiraGTx
New York, NY

radiation-induced xerostomia

Phase I/II
www.meiragtx.com

ALN-TTRsc02
(siRNA, RNA interference)
ORPHAN DRUG

Alnylam Pharmaceuticals
Cambridge, MA

ATTR amyloidosis

Phase I
www.alnylam.com

AMDC
(autologous muscle-derived
stem cell therapy)

Cook MyoSite
Pittsburgh, PA

fecal incontinence

Phase I/II
www.cookmyosite.com

CGF166
(recombinant AAV5 gene therapy)

Novartis
East Hanover, NJ

unilateral or bilateral severe-to-
profound hearing loss

Phase I/II
www.novartis.com

Other

Drug Name

Sponsor

Indication

Development Phase

LMSC (mesenchymal stem cell therapy)	Longeveron Miami, FL	aging frailty	Phase II www.longeveron.com
		immunosenescence in aging frailty	Phase I/II www.longeveron.com
rivogenlecleucel (BPX-501) (allogeneic polyclonal T-cell therapy) ORPHAN DRUG	Bellicum Pharmaceuticals Houston, TX	immunodeficiency disorders	Phase I/II www.bellicum.com

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 November 28, 2018**. 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).

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.

Glossary

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.