

## 2021 Medicines in Development: Rare Diseases

A LIST OF ORPHAN DRUGS IN THE PIPELINE

### Autoimmune Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ABC008 (KLRG1 depleter)	Abcuro Newton, MA	inclusion body myositis	Phase I <a href="http://www.abcuro.com">www.abcuro.com</a>
ACE-1334 (TGF- $\beta$ antagonist)	Acceleron Pharma Cambridge, MA	systemic sclerosis with and without interstitial lung disease (Fast Track)	Phase I/II <a href="http://www.acceleronpharma.com">www.acceleronpharma.com</a>
alidornase alfa	SarcoMed USA Houston, TX	sarcoidosis	Phase I <a href="http://www.sarcomedusa.com">www.sarcomedusa.com</a>
AMG714 (IgG1K mAb)	Amgen Thousand Oaks, CA	refractory celiac disease	Phase II <a href="http://www.amgen.com">www.amgen.com</a>
ANX005 (complement C1 inhibitor)	Annexon South San Francisco, CA	Guillain-Barre syndrome	Phase II/III <a href="http://www.annexon.com">www.annexon.com</a>
APT-1011 (fluticasone propionate dissolving tablet)	Ellodi Pharmaceuticals Lawrenceville, NJ	eosinophilic esophagitis (Fast Track)	Phase III <a href="http://www.ellodipharma.com">www.ellodipharma.com</a>
baricitinib	Eli Lilly Indianapolis, IN	pediatric systemic lupus erythematosus	Phase III <a href="http://www.lilly.com">www.lilly.com</a>

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batoclimab (IMVT-1401) (neonatal Fc receptor antagonist)	Immunovant New York, NY	myasthenia gravis	Phase II <a href="http://www.immunovant.com">www.immunovant.com</a>
cendakimab (IL-13 inhibitor)	Bristol-Myers Squibb Princeton, NJ	eosinophilic esophagitis	Phase III <a href="http://www.bms.com">www.bms.com</a>
cilofexor (FXR agonist)	Gilead Foster City, CA	primary sclerosing cholangitis	Phase III <a href="http://www.gilead.com">www.gilead.com</a>
CLBS03 (ex-vivo expanded polyclonal regulatory T lymphocyte cell therapy)	Caladrius Biosciences Basking Ridge, NJ	recent onset type 1 diabetes (Fast Track)	Phase II <a href="http://www.caladrius.com">www.caladrius.com</a>
donisleucel (allogeneic pancreatic islet cellular therapy)	CellTrans Chicago, IL	brittle type 1 diabetes	application submitted <a href="http://www.celltrans.com">www.celltrans.com</a>
DSG3-CAART (cell therapy)	Cabaletta Bio Philadelphia, PA	pemphigus vulgaris (Fast Track)	Phase I <a href="http://www.cabalettatbio.com">www.cabalettatbio.com</a>
<b>Dupixent®</b> dupilumab	Regeneron Pharmaceuticals Tarrytown, NY Sanofi Bridgewater, NJ	bullous pemphigoid, eosinophilic esophagitis (Breakthrough Therapy)	Phase III <a href="http://www.regeneron.com">www.regeneron.com</a> <a href="http://www.sanofi.com">www.sanofi.com</a>

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efgartigimod (neonatal Fc receptor antagonist)	argenx US Boston, MA	myasthenia gravis	application submitted <a href="http://www.argenx.com">www.argenx.com</a>
		chronic inflammatory demyelinating polyneuropathy, primary immune thrombocytopenia	Phase II <a href="http://www.argenx.com">www.argenx.com</a>
EHP-101 (semi-synthetic cannabinoid)	Emerald Health Pharmaceuticals San Diego, CA	systemic sclerosis (scleroderma) (Fast Track)	Phase II <a href="http://www.emeraldpharma.life">www.emeraldpharma.life</a>
elafibranor (PPAR $\alpha$ / $\delta$ agonist)	Genfit Loos, France	primary biliary cholangitis (Breakthrough Therapy) (Fast Track)	Phase III <a href="http://www.genfit.com">www.genfit.com</a>
<b>Enspryng</b> satralizumab-mwge	Roche/Genentech South San Francisco, CA	myasthenia gravis	Phase III <a href="http://www.roche.com">www.roche.com</a>
<b>Eohilia</b> budesonide oral suspension	Takeda Deerfield, IL	eosinophilic esophagitis (Breakthrough Therapy)	application submitted <a href="http://www.takeda.com">www.takeda.com</a>
etrasimod (S1P receptor modulator)	Arena Pharmaceuticals San Diego, CA	eosinophilic esophagitis	Phase II <a href="http://www.arenapharm.com">www.arenapharm.com</a>
<b>Fasenra</b> <sup>®</sup> benralizumab	AstraZeneca Wilmington, DE	eosinophilic esophagitis, hypereosinophilic syndrome, eosinophilic granulomatosis with polyangiitis	Phase III <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>

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FCX-013 (genetically-modified autologous fibroblast cells)	Castle Creek Biosciences Exton, PA	moderate to severe localized scleroderma (Fast Track)	Phase I/II <a href="http://www.castlecreekbio.com">www.castlecreekbio.com</a>
<b>Firdapse®</b> amifampridine	Catalyst Pharmaceuticals Coral Gables, FL	myasthenia gravis	Phase III <a href="http://www.catalystpharma.com">www.catalystpharma.com</a>
<b>Hizentra®</b> immune globulin subcutaneous	CSL Behring King of Prussia, PA	dermatomyositis	Phase III <a href="http://www.cslbehring.com">www.cslbehring.com</a>
HTD1801 (lipid modulator)	HighTide Biopharma Rockville, MD	primary sclerosing cholangitis (Fast Track)	Phase II <a href="http://www.hightidetx.com">www.hightidetx.com</a>
<b>Hyqvia</b> immune globulin infusion 10% (human) with recombinant human hyaluronidase	Takeda Deerfield, IL	chronic inflammatory demyelinating polyneuropathy	Phase III <a href="http://www.takeda.com">www.takeda.com</a>
iloprost	Eicos Sciences Chevy Chase, MD	systemic sclerosis	Phase III <a href="http://www.eicossciences.com">www.eicossciences.com</a>
IMT-002 (D-methyl dopa)	IM Therapeutics Woburn, MA	type 1 diabetes	Phase I <a href="http://www.imtherapeutics.com">www.imtherapeutics.com</a>
KZR-616	Kezar Life Sciences South San Francisco, CA	dermatomyositis, polymyositis	Phase II <a href="http://www.kezarlifesciences.com">www.kezarlifesciences.com</a>

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lenabasum (ajulemic acid)	Corbus Pharmaceuticals Norwood, MA	dermatomyositis	Phase III <a href="http://www.corbuspharma.com">www.corbuspharma.com</a>
linerixibat (GSK2330672)	GlaxoSmithKline Research Triangle Park, NC	primary biliary cholangitis	Phase III <a href="http://www.gsk.com">www.gsk.com</a>
molgramostim inhalation (inhaled granulocyte-macrophage colony-stimulating factor)	Savara Pharmaceuticals Austin, TX	autoimmune pulmonary alveolar proteinosis	Phase III <a href="http://www.savarapharma.com">www.savarapharma.com</a>
nipocalimab (Fc receptor antagonist)	Janssen Research & Development Raritan, NJ	myasthenia gravis	Phase III <a href="http://www.janssen.com">www.janssen.com</a>
obexelimab (anti-CD19 mAb)	Xencor Monrovia, CA	immunoglobulin G4-related disease	Phase II <a href="http://www.xencor.com">www.xencor.com</a>
<b>Orencia</b> <sup>®</sup> abatacept	Bristol-Myers Squibb Princeton, NJ	idiopathic inflammatory myopathy	Phase III <a href="http://www.bms.com">www.bms.com</a>
parsaclisib (PI3K-delta inhibitor)	Incyte Wilmington, DE	autoimmune hemolytic anemia	Phase III <a href="http://www.incyte.com">www.incyte.com</a>
pegcetacoplan	Apellis Pharmaceuticals Waltham, MA	autoimmune hemolytic anemia	Phase II <a href="http://www.apellis.com">www.apellis.com</a>

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PF-06755347 (immunomodulation)	Pfizer New York, NY	chronic inflammatory demyelination polyneuropathy	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
PF-06823859 (IFN $\beta$ 1 fibroblast blocker)	Pfizer New York, NY	dermatomyositis	Phase II <a href="http://www.pfizer.com">www.pfizer.com</a>
PLN-74809	Pliant Therapeutics South San Francisco, CA	primary sclerosing cholangitis	Phase II <a href="http://www.pliantrx.com">www.pliantrx.com</a>
<b>REZUROCK™</b> belumosudil	Kadmon Pharmaceuticals New York, NY	systemic sclerosis	Phase II <a href="http://www.kadmon.com">www.kadmon.com</a>
rozanolixizumab	UCB Smyrna, GA	myasthenia gravis	Phase III <a href="http://www.ucb.com">www.ucb.com</a>
saroglitazar (PPAR $\alpha/\gamma$ agonist)	Zydus-Cadila Ahmedabad, India	primary biliary cholangitis (Fast Track)	Phase II <a href="http://www.zyduscadila.com">www.zyduscadila.com</a>
seladelpar (PPAR-delta agonist)	CymaBay Therapeutics Newark, CA	primary biliary cholangitis (Breakthrough Therapy)	Phase II <a href="http://www.cymabay.com">www.cymabay.com</a>
setanaxib	Genkyotex Saint-Julien-en-Genevois, France	primary biliary cholangitis	Phase II/III <a href="http://www.genkyotex.com">www.genkyotex.com</a>
Sm001 (dornase alfa)	SarcoMed USA Houston, TX	sarcoidosis	Phase I <a href="http://www.sarcomedusa.com">www.sarcomedusa.com</a>

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sutimlimab (anti-complement C1s mAb)	Sanofi Bridgewater, NJ	cold agglutinin disease (autoimmune hemolytic anemia) (Breakthrough Therapy)	application submitted <a href="http://www.sanofi.com">www.sanofi.com</a>
TAK-079 (mezagitamab)	Takeda Deerfield, IL	myasthenia gravis	Phase II <a href="http://www.takeda.com">www.takeda.com</a>
<b>Tavalisse®</b> fostamatinib	Rigel Pharmaceuticals South San Francisco, CA	autoimmune hemolytic anemia (Fast Track)	Phase III <a href="http://www.rigel.com">www.rigel.com</a>
XTMAB-16 (anti-TNF mAb)	Xentria Chicago, IL	sarcoidosis	Phase I <a href="http://www.xentria.com">www.xentria.com</a>
zilucoplan	UCB Smyrna, GA	myasthenia gravis	Phase III <a href="http://www.ucb.com">www.ucb.com</a>

## Blood Disorders

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ALXN2040 (danicopan) (factor D inhibitor)	Alexion AstraZeneca Rare Disease Boston, MA	paroxysmal nocturnal hemoglobinuria with extravascular hemolysis (Breakthrough Therapy)	Phase III <a href="http://www.alexion.com">www.alexion.com</a>
ALXN2050 (vemiropar) (factor D inhibitor)	Alexion AstraZeneca Rare Disease Boston, MA	paroxysmal nocturnal hemoglobinuria	Phase II <a href="http://www.alexion.com">www.alexion.com</a>

**Blood Disorders**

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AMT-061 (etranacogene dezaparvovec) (AAV gene therapy)	uniQure Biopharma Lexington, MA	treatment of hemophilia B	Phase III <a href="http://www.uniqure.com">www.uniqure.com</a>
ARCT-810 (mRNA therapeutic)	Arcturus Therapeutics San Diego, CA	ornithine transcarbamylase deficiency	Phase II <a href="http://www.arcturusrx.com">www.arcturusrx.com</a>
ARU-1801 (lentiviral gene therapy)	Aruvant Sciences New York, NY	sickle cell disease	Phase I/II <a href="http://www.aruvant.com">www.aruvant.com</a>
avalotcogene ontaparvovec (DTX301) (gene therapy)	Ultragenyx Pharmaceutical Novato, CA	ornithine transcarbamylase deficiency (Fast Track)	Phase II <a href="http://www.ultragenyx.com">www.ultragenyx.com</a>
BCX-9930 (oral factor D inhibitor)	BioCryst Pharmaceuticals Durham, NC	paroxysmal nocturnal hemoglobinuria (Fast Track)	Phase II <a href="http://www.biocryst.com">www.biocryst.com</a>
benralizumab	AstraZeneca Wilmington, DE	hypereosinophilic syndrome	Phase III <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
BT-200 (PEGylated aptamer)	Band Therapeutics Lexington, MA	von Willebrand disease	Phase II <a href="http://www.guardiantherapeutics.com">www.guardiantherapeutics.com</a>
concizumab	Novo Nordisk Plainsboro, NJ	hemophilia A, hemophilia B	Phase III <a href="http://www.novonordisk.com">www.novonordisk.com</a>
crovalimab (RG6107) (complement c3 inhibitor)	Roche/Chugai Berkeley Heights, NJ	paroxysmal nocturnal hemoglobinuria	Phase III <a href="http://www.roche.com">www.roche.com</a>



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CSL-889 (hemopexin replacement)	CSL Behring King of Prussia, PA	sickle cell disease	Phase I <a href="http://www.cslbehring.com">www.cslbehring.com</a>
CTX001 (autologous CRISPR/Cas9 gene-edited hematopoietic stem cell therapy)	CRISPR Therapeutics Cambridge, MA Vertex Pharmaceuticals Boston, MA	beta thalassemia (Fast Track), sickle cell anemia (Fast Track)	Phase I/II <a href="http://www.crisprtx.com">www.crisprtx.com</a> <a href="http://www.vrtx.com">www.vrtx.com</a>
dexpramipexole	Knopp Biosciences Pittsburgh, PA	hypereosinophilic syndrome	Phase II <a href="http://www.knoppbio.com">www.knoppbio.com</a>
Eclipse (EPI01) (decitabine/tetrahydrouridine)	Novo Nordisk Princeton, NJ	sickle cell disease (Fast Track)	Phase I <a href="http://mwww.novonordisk.com">mwww.novonordisk.com</a>
efanesoctocog alfa (recombinant coagulation factor VIII Fc von Willebrand Factor – XTEN fusion protein)	Sanofi Bridgewater, NJ	hemophilia A (Fast Track)	Phase III <a href="http://www.sanofi.com">www.sanofi.com</a>
etavopivat (FT-4202) (PKR activator)	Forma Therapeutics Watertown, MA	sickle cell disease (Fast Track)	Phase II/III <a href="http://www.formatherapeutics.com">www.formatherapeutics.com</a>
fidanacogene elaparvovec (coagulation factor IX gene therapy)	Pfizer New York, NY Spark Therapeutics Philadelphia, PA	hemophilia (Breakthrough Therapy)	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a> <a href="http://www.sparktx.com">www.sparktx.com</a>

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fitusiran (RNAi targeting anti-thrombin)	Sanofi Bridgewater, NJ	hemophilia A (Fast Track), hemophilia B (Fast Track) (adult and pediatric)	Phase III <a href="http://www.sanofi.com">www.sanofi.com</a>
giroctocogene fitelparvovec (coagulation factor VIII gene therapy)	Pfizer New York, NY Sangamo Therapeutics Richmond, CA	hemophilia A (Fast Track)	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
IMR-687 (tovinontrine) (PDE9 inhibitor)	Imara Cambridge, MA	sickle cell disease (homozygous HbSS or sickle- $\beta$ 0 thalassemia) (Fast Track)	Phase II <a href="http://www.imaratx.com">www.imaratx.com</a>
L-citrulline	Asklepion Pharmaceuticals Baltimore, MD	sickle cell disease	Phase I <a href="http://www.asklepionpharm.com">www.asklepionpharm.com</a>
<b>LentiGlobin</b> <sup>®</sup> beta-globin gene therapy	bluebird bio Cambridge, MA	sickle cell disease	Phase III <a href="http://www.bluebirdbio.com">www.bluebirdbio.com</a>
LNP023 (iptacopan) (CFB inhibitor)	Novartis East Hanover, NJ	paroxysmal nocturnal hemoglobinuria (Breakthrough Therapy)	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
Mim8 (bispecific antibody)	Novo Nordisk Plainsboro, NJ	hemophilia A	Phase I <a href="http://www.novonordisk.com">www.novonordisk.com</a>

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mitapivat (PKR activator)	Agios Pharmaceuticals Cambridge, MA	pyruvate kinase deficiency (Fast Track)	application submitted <a href="http://www.agios.com">www.agios.com</a>
		alpha and beta thalassemia, sickle cell disease	Phase III <a href="http://www.agios.com">www.agios.com</a>
nomacopan	Akari Therapeutics New York, NY	hematopoietic stem cell transplantation-associated thrombotic microangiopathy (pediatric) (Fast Track)	Phase III <a href="http://www.akaritx.com">www.akaritx.com</a>
<b>Nplate</b> <sup>®</sup> romiplostim	Amgen Thousand Oaks, CA	chemotherapy-induced thrombocytopenia	Phase III <a href="http://www.amgen.com">www.amgen.com</a>
olinciguat (IW-1701) (sGC agonist)	Cyclerion Therapeutics Cambridge, MA	sickle cell disease	Phase II <a href="http://www.cyclerion.com">www.cyclerion.com</a>
OPK88005 (factor VIIa-CTP)	OPKO Health Miami, FL	hemophilia A or B	Phase II <a href="http://www.opko.com">www.opko.com</a>
P1101 (pegylated proline interferon alpha-2b)	PharmaEssentia Taipei, Taiwan	essential thrombocythemia	Phase III <a href="http://www.pharmaessentia.com">www.pharmaessentia.com</a>
PF-06741086 (marstacimab) (tissue factor pathway inhibitor)	Pfizer New York, NY	hemophilia	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>

**Blood Disorders**

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PF-06755347 (immunomodulation)	Pfizer New York, NY	idiopathic thrombocytopenic purpura	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
PF-07059013 (hemoglobin beta modulator)	Pfizer New York, NY	sickle cell disease	Phase I <a href="http://www.pfizer.com">www.pfizer.com</a>
PF-07209326 (E-selectin antagonist)	Pfizer New York, NY	sickle cell disease	Phase I <a href="http://www.pfizer.com">www.pfizer.com</a>
pozelimab/cemdisiran combination (CD5 antibody/C5 siRNA)	Regeneron Pharmaceuticals Tarrytown, NY	paroxysmal nocturnal hemoglobinuria	Phase II <a href="http://www.regeneron.com">www.regeneron.com</a>
REC-994 (superoxide dismutase mimetic)	Recursion Pharmaceuticals Salt Lake City, UT	cerebral cavernous malformations	Phase I <a href="http://www.recursion.com">www.recursion.com</a>
rilzabrutinib (BTK inhibitor)	Sanofi Bridgewater, NJ	immune thrombocytopenic purpura (Fast Track)	Phase III <a href="http://www.sanofi.com">www.sanofi.com</a>
RLYB-211 (IgG mAb)	Rallybio New Haven, CT	prevention of fetal and neonatal alloimmune thrombocytopenia	Phase I <a href="http://www.rallybio.com">www.rallybio.com</a>
rofecoxib	Tremeau Pharmaceuticals Concord, MA	hemophilic arthropathy	Phase III <a href="http://www.tremeau.com">www.tremeau.com</a>
rozanolixizumab	UCB Smyrna, GA	immune thrombocytopenic purpura	Phase III <a href="http://www.ucb.com">www.ucb.com</a>

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RP-L102 (fancalen) (lentiviral gene therapy)	Rocket Pharmaceuticals Cranbury, NJ	Fanconi anemia type A patients	Phase II <a href="http://www.rocketpharma.com">www.rocketpharma.com</a>
<b>Sanguinate</b> <sup>®</sup> PEGylated carboxy hemoglobin bovine (PP-007)	Prolong Pharmaceuticals South Plainfield, NJ	vaso-occlusive crises in sickle cell disease	Phase II <a href="http://www.prolongpharma.com">www.prolongpharma.com</a>
SAR445088 (complement C1s inhibitor)	Sanofi Bridgewater, NJ	immune thrombocytopenia	Phase II <a href="http://www.sanofi.com">www.sanofi.com</a>
SAR445136 (ex-vivo ZFN gene-edited cell therapy)	Sanofi Bridgewater, NJ	sickle cell disease (Fast Track)	Phase I <a href="http://www.sanofi.com">www.sanofi.com</a>
SC411 (docosahexaenoic acid)	Micelle BioPharma Riviera Beach, FL	sickle cell disease	Phase II <a href="http://www.micellebiopharma.com">www.micellebiopharma.com</a>
SIG-001	Sigilon Therapeutics Cambridge, MA	hemophilia A	Phase I/II <a href="http://www.sigilon.com">www.sigilon.com</a>
SPK-8011 (RG6357) (gene therapy)	Spark Therapeutics Philadelphia, PA	hemophilia A (Breakthrough Therapy)	Phase III <a href="http://www.sparktx.com">www.sparktx.com</a>
SPK-8016 (RG6358) (gene therapy)	Spark Therapeutics Philadelphia, PA	hemophilia A with inhibitors to factor VIII	Phase I <a href="http://www.sparktx.com">www.sparktx.com</a>

**Blood Disorders**

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TAK-079 (mezagitamab)	Takeda Deerfield, IL	thrombotic thrombocytopenic purpura	Phase II <a href="http://www.takeda.com">www.takeda.com</a>
TAK-755 (apadamtase alfa)	Takeda Deerfield, IL	thrombotic thrombocytopenic purpura (Fast Track)	Phase III <a href="http://www.takeda.com">www.takeda.com</a>
		sickle cell disease	Phase I/II <a href="http://www.takeda.com">www.takeda.com</a>
<b>Ultomiris IV</b> ravulizumab	Alexion AstraZeneca Rare Disease Boston, MA	hematopoietic stem cell transplant- related thrombotic microangiopathy	Phase III <a href="http://www.alexion.com">www.alexion.com</a>
UX143 / BAY 2599023 (AAV8-OTC gene therapy)	Bayer Pharmaceuticals Whippany, NJ Ultragenyx Novato, CA	hemophilia A	Phase II <a href="http://www.bayer.pharma.com">www.bayer.pharma.com</a> <a href="http://www.ultragenyx.com">www.ultragenyx.com</a>
valoctocogene roxaparvovec (factor VIII gene therapy)	BioMarin Pharmaceutical Novato, CA	hemophilia A	application submitted <a href="http://www.biomarin.com">www.biomarin.com</a>
VLX-1005	Veralox Therapeutics Frederick, MD	prevention of thrombosis in heparin-induced thrombocytopenia	Phase I <a href="http://www.veralox.com">www.veralox.com</a>

<b>Cancer</b> <b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
186RNL (BMEDA-chelated rhenium-186 nanoliposome)	Plus Therapeutics Austin, TX	recurrent glioblastoma (Fast Track)	Phase I <a href="http://www.plustherapeutics.com">www.plustherapeutics.com</a>
2-hydroxyoleic acid	Laminar Pharma Palma, Spain	malignant glioma	Phase I/II <a href="http://www.laminarpharma.com">www.laminarpharma.com</a>
9-ING-41 (GSK-3β inhibitor)	Actuate Therapeutics Fort Worth, TX	neuroblastoma	Phase I <a href="http://www.actuatetherapeutics.com">www.actuatetherapeutics.com</a>
ABBV-011 (antibody drug conjugate)	AbbVie North Chicago, IL	small cell lung cancer	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a>
ABTL0812 (PPAR agonist)	Ability Pharmaceuticals Barcelona, Spain	pancreatic cancer	Phase I/II <a href="http://www.abilitypharma.co">www.abilitypharma.co</a>
ACT 001	Accendatech USA Houston, TX	glioblastoma multiforme	Phase I/II <a href="http://www.accendatechusa.com">www.accendatechusa.com</a>
afamitresgene autoleucel (MAGE A4-specific T cell therapy)	Adaptimmune Philadelphia, PA	soft tissue sarcoma	Phase II <a href="http://www.adaptimmune.com">www.adaptimmune.com</a>
aglatimagene besadenovec (CAN-2409) (oncolytic viral immunotherapy)	Candel Therapeutics Needham, MA	high-grade glioma (Fast Track)	Phase III <a href="http://www.candeltx.com">www.candeltx.com</a>

<b>Cancer Product Name</b>	<b>Sponsor</b>	<b>Indication</b>	<b>Development Phase</b>
AIC100 (CAR-T cell therapy)	AffyImmune Therapeutics Natick, MA	advanced refractory thyroid cancer (Fast Track)	Phase I <a href="http://www.affyimmune.com">www.affyimmune.com</a>
AK04 (cadonilimab) (PD-1/CTLA-4 bispecific antibody)	Akeso Biopharma Fremont, CA	cervical cancer (Fast Track)	Phase II <a href="http://www.akesobio.com">www.akesobio.com</a>
Aktina + haNK (activated natural killer cells)	ImmunityBio Culver City, CA	Merkel cell carcinoma	Phase II <a href="http://www.immunitybio.com">www.immunitybio.com</a>
AL101 (gamma secretase inhibitor)	Ayala Pharmaceuticals Wilmington, DE	adenoid cystic carcinoma (Fast Track)	Phase II <a href="http://www.ayalapharma.com">www.ayalapharma.com</a>
AL3818 (tyrosine kinase inhibitor)	Advenchen Laboratories Moorpark, CA	ovarian cancer, soft tissue sarcoma	Phase III <a href="http://www.advenchen.com">www.advenchen.com</a>
ALMB-0168 (connexin 43 inhibitor)	AlaMab Therapeutics Princeton, MA	osteosarcoma	Phase I <a href="http://www.alamab.com">www.alamab.com</a>
AMG910 (bispecific T-cell engager)	Amgen Thousand Oaks, CA	gastric cancer, gastroesophageal junction cancer	Phase I <a href="http://www.amgen.com">www.amgen.com</a>
annamycin liposomal	Moleculin Biotech Houston, TX	soft tissue sarcoma	Phase I/II <a href="http://www.moleculin.com">www.moleculin.com</a>
antroquinonol	Golden Biotechnology Beijing, China	pancreatic cancer	Phase I/II <a href="http://www.goldenbiotech.com">www.goldenbiotech.com</a>



<b>Cancer Product Name</b>	<b>Sponsor</b>	<b>Indication</b>	<b>Development Phase</b>
APG-1252 (pelcitoclax) (Bcl-2/NclxL inhibitor)	Ascentage Pharma Rockville, MD	small cell lung cancer	Phase I/II <a href="http://www.ascentagepharma.com">www.ascentagepharma.com</a>
APG-157 (zinflavonoid)	Aveta Biomics Burlington, MA	squamous cell carcinoma of the oral cavity	Phase II <a href="http://www.avetabiomics.com">www.avetabiomics.com</a>
ARX-788 (antibody drug conjugate)	Ambrx La Jolla, CA	gastric cancer	Phase I <a href="http://www.ambrx.com">www.ambrx.com</a>
ARY-ASP (asparaginase erythrocyte encapsulated)	Erytech Pharma Cambridge, MA	pancreatic cancer (Fast Track)	Phase III <a href="http://www.erytech.com">www.erytech.com</a>
axalimogene filolisbac (cancer vaccine)	Advaxis Monmouth Junction, NJ	cervical cancer (Fast Track)	Phase III <a href="http://www.advaxis.com">www.advaxis.com</a>
BAL-101553 (lisavanbulin)	Basilea Pharmaceutical Basel, Switzerland	malignant glioma	Phase I <a href="http://www.basilea.com">www.basilea.com</a>
belzupacap sarotalocan (AU-011) (virus-like drug conjugate)	Aura Biosciences Cambridge, MA	uveal melanoma (Fast Track)	Phase II <a href="http://www.aurabiosciences.com">www.aurabiosciences.com</a>
bemarituzumab (FPA-144) (anti-FGFR2B antibody)	Amgen Thousand Oaks, CA	gastric cancer, gastroesophageal junction cancer (Breakthrough Therapy)	Phase III <a href="http://www.amgen.com">www.amgen.com</a>

<b>Cancer Product Name</b>	<b>Sponsor</b>	<b>Indication</b>	<b>Development Phase</b>
bempegaldesleukin	Nektar Therapeutics San Francisco, CA Bristol-Myers Squibb Princeton, NJ	melanoma (Breakthrough Therapy)	Phase III <a href="http://www.nektar.com">www.nektar.com</a> <a href="http://www.bms.com">www.bms.com</a>
berubicin (type II DNA topoisomerase inhibitor)	CNS Pharmaceuticals Houston, TX	glioblastoma (Fast Track)	Phase II <a href="http://www.cnspharma.com">www.cnspharma.com</a>
bezuclastinib	Cogent Biosciences Cambridge, MA	gastrointestinal stromal tumors	Phase II <a href="http://www.cogentbio.com">www.cogentbio.com</a>
BMX-001 (manganese butoxyethyl pyridyl porphyrin)	BioMimetix Greenwood Village, CO	glioma	Phase II <a href="http://www.biomimetixpharma.com">www.biomimetixpharma.com</a>
BNT-111 (mRNA cancer vaccine)	BioNTech Mainz, Germany	stage IIB through IV melanoma	Phase II <a href="http://www.biontech.de">www.biontech.de</a>
BPM 31510 (ubidecarenone)	Berg Framingham, MA	pancreatic cancer	Phase II <a href="http://www.berghealth.com">www.berghealth.com</a>
		glioma	Phase I <a href="http://www.berghealth.com">www.berghealth.com</a>
BXQ-350 (nanovesicle containing Saposin C and dioleoylphosphatidylserine)	Bexion Pharmaceuticals Covington, KY	diffuse intrinsic pontine glioma, diffuse midline glioma, newly- diagnosed glioblastoma multiforme	Phase II <a href="http://www.bexionpharma.com">www.bexionpharma.com</a>

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camsirubicin (type II DNA topoisomerase inhibitor)	Monopar Therapeutics Wilmette, IL	soft tissue sarcoma	Phase I <a href="http://www.monopartx.com">www.monopartx.com</a>
cavrotolimod (toll-like receptor 9 agonist)	Exicure Chicago, IL	Merkel cell carcinoma (Fast Track)	Phase II <a href="http://www.exicure.com">www.exicure.com</a>
cisplatin liposomal inhaled	Eleison Pharmaceuticals Princeton, NJ	osteosarcoma	Phase II <a href="http://www.eleison-pharma.com">www.eleison-pharma.com</a>
CK-101 (olafertinib) (EGFR inhibitor)	Checkpoint Therapeutics Waltham, MA	non-small cell lung cancer	Phase III <a href="http://www.checkpointtx.com">www.checkpointtx.com</a>
CPP-1X (eflornithine)	Cancer Prevention Pharmaceuticals Tucson, AZ	gastric cancer	Phase II <a href="http://www.canprevent.com">www.canprevent.com</a>
		neuroblastoma (pediatric)	Phase I <a href="http://www.canprevent.com">www.canprevent.com</a>
crenolanib (PDGFR inhibitor)	AROG Pharmaceuticals Plano, TX	glioma	Phase II <a href="http://www.arogpharma.com">www.arogpharma.com</a>
		gastric cancer, gastroesophageal junction cancer (Fast Track)	Phase I <a href="http://www.arogpharma.com">www.arogpharma.com</a>
CS-1003	CStone Pharmaceuticals Suzhou, China	hepatocellular carcinoma	Phase III <a href="http://www.cstonepharma.com">www.cstonepharma.com</a>

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CT-041 (CAR-T immunotherapy)	CARsgen Houston, TX	gastric cancer	Phase I <a href="http://www.carsgen.com">www.carsgen.com</a>
D2C7 (recombinant immunotoxin fusion protein)	Istari Oncology Morrisville, NC	glioma	Phase I <a href="http://www.istarioncology.com">www.istarioncology.com</a>
daromun	Philogen Sovicille, Italy	melanoma	Phase III <a href="http://www.philogen.com">www.philogen.com</a>
DAY101 (raf kinase inhibitor)	Day One Biopharmaceuticals South San Francisco, CA	low grade glioma (pediatric) (Breakthrough Therapy)	Phase II <a href="http://www.dayonebio.com">www.dayonebio.com</a>
delolimogene mupadenorepvec (LOAd703) (oncolytic adenovirus)	Lokon Pharma Uppsala, Sweden	pancreatic cancer	Phase I/II <a href="http://www.lokonpharma.com">www.lokonpharma.com</a>
delta-9-tetrahydrocannabinol	Tetra Bio-Pharma Orleans, Canada	hepatocellular carcinoma	Phase I <a href="http://www.tetrabiopharma.com">www.tetrabiopharma.com</a>
derazantinib (FGFR kinase inhibitor)	Basilea Pharmaceutica Basel, Switzerland	cholangiocarcinoma	Phase II <a href="http://www.basilea.com">www.basilea.com</a>
devimistat (CPI-613) (lipoic acid analogue/ AEMD compound)	Rafael Pharmaceuticals Cranbury, NJ	biliary tract carcinoma, clear cell soft tissue sarcoma,  pancreatic cancer (Fast Track)	Phase II <a href="http://www.rafaelpharma.com">www.rafaelpharma.com</a>  Phase I <a href="http://www.rafaelpharma.com">www.rafaelpharma.com</a>

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DKN-01 (DKK1 protein inhibitor)	Leap Therapeutics Cambridge, MA	gastric cancer, gastroesophageal cancer	Phase II <a href="http://www.leaptx.com">www.leaptx.com</a>
DNA-2401 (adenovirus delta 24-RGD)	DNatrix Houston, TX	glioma (Fast Track)	Phase III <a href="http://www.dnatrix.com">www.dnatrix.com</a>
DocePLUS (docetaxel liposomal)	Plus Therapeutics Austin, TX	small cell lung cancer	Phase I <a href="http://www.plustherapeutics.com">www.plustherapeutics.com</a>
<b>DPX-Survivac</b> cancer peptide vaccine	Immunovaccine Technologies Halifax, Canada	ovarian cancer	Phase II <a href="http://www.imvaccine.com">www.imvaccine.com</a>
eflornithine oral (ornithine decarboxylase inhibitor)	Orbus Therapeutics Palo Alto, CA	anaplastic astrocytoma (Breakthrough Therapy)	Phase III <a href="http://www.orbustherapeutics.com">www.orbustherapeutics.com</a>
encequidar/oral paclitaxel	Athenex Buffalo, NY	cutaneous angiosarcoma	Phase II <a href="http://www.athenex.com">www.athenex.com</a>
envafolimab (KN035) (nanobody)	TRACON Pharmaceuticals San Diego, CA	undifferentiated pleomorphic sarcoma, myxofibrosarcoma	Phase II <a href="http://www.tracopharma.com">www.tracopharma.com</a>
enzastaurin (DB 102) (PKC- $\beta$ and PI3K/AKT inhibitor)	Denovo Biopharma San Diego, CA	glioblastoma (Fast Track)	Phase III <a href="http://www.denovobiopharma.com">www.denovobiopharma.com</a>
episcleral topotecan	3T Ophthalmics Irvine, CA	retinoblastoma	Phase I <a href="http://www.3tophthalmics.com">www.3tophthalmics.com</a>

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EQ176 (anti-PD-1 mAb)	EQRx Cambridge, MA	liver cancer	Phase III <a href="http://www.eqr.com">www.eqr.com</a>
evofosfamide (hypoxia-reversal agent)	Immunogenesis Houston, TX	pancreatic cancer (Fast Track)	Phase I <a href="http://www.immunogenesis.com">www.immunogenesis.com</a>
fimaporfin/gemcitabine (photosensitizer)	PCI Biotech Oslo, Norway	cholangiocarcinoma	Phase II <a href="http://www.pcibiotech.no">www.pcibiotech.no</a>
fisogatinib (FGFR4 inhibitor)	Blueprint Medicines Cambridge, MA	advanced hepatocellular carcinoma	Phase I <a href="http://www.blueprintmedicines.com">www.blueprintmedicines.com</a>
futibatinib (TAS-120) (FGFR inhibitor)	Taiho Oncology Princeton, NJ	cholangiocarcinoma (Breakthrough Therapy)	Phase III <a href="http://www.taihooncology.com">www.taihooncology.com</a>
G207 (ribonucleotide reductase inhibitor)	Treovir Philadelphia, PA	glioma	Phase I <a href="http://www.treovir.com">www.treovir.com</a>
gadolinium-chelated polysiloxane- based nanoparticle	NH TherAguix Meylan, France	pancreatic cancer	Phase I <a href="http://www.nhtheraguix.com">www.nhtheraguix.com</a>
gavo-cel (gavocabtagene autoleucel) (T-cell therapy)	TCR2 Therapeutics Cambridge, MA	mesothelioma, cholangiocarcinoma	Phase I/II <a href="http://www.tcr2.com">www.tcr2.com</a>
GEN-1 (IL-12 gene therapy nanoparticle)	Celsion Lawrenceville, NJ	ovarian cancer (Fast Track)	Phase II <a href="http://www.celsion.com">www.celsion.com</a>

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GLR2007 (CDK4/6 inhibitor)	Gan & Lee Pharmaceuticals Beijing, China	glioblastoma (Fast Track)	Phase I/II <a href="http://www.ganlee.com">www.ganlee.com</a>
glufosfamide (DNA synthesis inhibitor)	Eleison Pharmaceuticals Princeton, NJ	pancreatic cancer (Fast Track)	Phase III <a href="http://www.eleison-pharma.com">www.eleison-pharma.com</a>
GP-2250 (taurolidine)	Geistlich Pharma Wolhusen, Switzerland	pancreatic cancer	Phase I/II <a href="http://www.geistlich-pharma.com">www.geistlich-pharma.com</a>
GSK3377794 (letetresgene autoleucel)	GlaxoSmithKline Research Triangle Park, NC	soft tissue sarcoma	Phase III <a href="http://www.gsk.com">www.gsk.com</a>
H3B-6527 (FGFR4 inhibitor)	H3 Biomedicine (Eisai) Cambridge, MA	hepatocellular carcinoma	Phase I <a href="http://www.h3biomedicine.com">www.h3biomedicine.com</a>
<b>Hepzato™ Kit</b> melphalan hepatic delivery system	Delcath Systems New York, NY	hepatic dominant ocular melanoma (Fast Track), intrahepatic cholangiocarcinoma	Phase III <a href="http://www.delcath.com">www.delcath.com</a>
ICP-192 (gunagratinib) (FGFR inhibitor)	InnoCare Pharma Beijing, China	cholangiocarcinoma	Phase I/II <a href="http://www.innocarepharma.com">www.innocarepharma.com</a>
Ifx-Hu2.0 (immunotherapeutic vaccine)	Morphogenesis Tampa, FL	IIB to stage IV melanoma	Phase I <a href="http://www.morphogenesis.com">www.morphogenesis.com</a>
IGV-001 (autologous glioma cell therapy)	Imvax Philadelphia, PA	malignant glioma	Phase I <a href="http://www.imvax.com">www.imvax.com</a>

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<b>Imfinzi®</b> durvalumab	AstraZeneca Wilmington, DE	biliary tract cancer, hepatocellular carcinoma	Phase III <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
IMGN-632 (anti-CD123 mAb)	ImmunoGen Waltham, MA	blastic plasmacytoid dendritic cell neoplasm	Phase II <a href="http://www.immunogen.com">www.immunogen.com</a>
iniparib	TriAct Therapeutics San Francisco, CA	malignant glioma	Phase II
IRX-2 immunotherapy	Brooklyn ImmunoTherapeutics Brooklyn, NY	squamous cell carcinoma of the head and neck (Fast Track)	Phase II <a href="http://www.brooklynitx.com">www.brooklynitx.com</a>
ITIL-168 (cell therapy)	Instil Bio Dallas, TX	malignant melanoma	Phase II <a href="http://www.instilbio.com">www.instilbio.com</a>
<b>Keytruda®</b> pembrolizumab	Merck Whitehouse Station, NJ	biliary tract carcinoma	Phase III <a href="http://www.merck.com">www.merck.com</a>
<b>Lenvima®</b> lenvatinib	Eisai Woodcliff Lake, NJ	late-stage melanoma	Phase III <a href="http://www.eisai.com">www.eisai.com</a>
<b>Libtayo®</b> cemiplimab-rwlc	Regeneron Tarrytown, NY Sanofi Bridgewater, NJ	cervical cancer	application submitted <a href="http://www.regeneron.com">www.regeneron.com</a> <a href="http://www.sanofi.com">www.sanofi.com</a>



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lifileucel (TIL autologous cell therapy)	Iovance Biotherapeutics San Carlos, CA	cervical cancer (Breakthrough Therapy) (Fast Track), melanoma (Fast Track)	Phase III <a href="http://www.iovance.com">www.iovance.com</a>
<b>Lumakras™</b> sotorasib	Amgen Thousand Oaks, CA	advanced colorectal cancer	Phase II <a href="http://www.amgen.com">www.amgen.com</a>
<b>Margenza™</b> margetuximab	MacroGenics Rockville, MD	HER2-positive gastric/ gastroesophageal junction cancer	Phase III <a href="http://www.macrogenics.com">www.macrogenics.com</a>
MB102 (anti-CD123 T-cell therapy)	Mustang Bio Worcester, MA	blastic plasmacytoid dendritic cell neoplasm	Phase I/II <a href="http://www.mustangbio.com">www.mustangbio.com</a>
mecbotamab vedotin (gG1anti-AXL-antibody)	BioAtla San Diego, CA	soft tissue sarcoma	Phase II <a href="http://www.bioalta.com">www.bioalta.com</a>
milademetan	Rain Therapeutics Newark, CA	liposarcoma	Phase III <a href="http://www.rainthera.com">www.rainthera.com</a>
mirvetuximab soratansine (anti-Fra antibody-drug conjugate)	ImmunoGen Waltham, MA	ovarian cancer	Phase III <a href="http://www.immunogen.com">www.immunogen.com</a>
MN-166 (ibudilast) (neuroprotective therapeutic)	MediciNova La Jolla, CA	glioblastoma	Phase II <a href="http://www.medicinova.com">www.medicinova.com</a>
motixafortide	BioLineRx Modi'in, Israel	pancreatic cancer	Phase II <a href="http://www.biolerx.com">www.biolerx.com</a>

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MRTX-849 (KRAS G12C inhibitor)	Mirati Therapeutics San Diego, CA	KRAS G12C-positive non-small cell lung cancer (Fast Track)	Phase III <a href="http://www.mirati.com">www.mirati.com</a>
<b>Mulitkine®</b> leukocyte interleukin injection	CEL-SCI Vienna, VA	squamous cell carcinoma of the head and neck	Phase III <a href="http://www.cel-sci.com">www.cel-sci.com</a>
nadofaragene firadenovec	Trizell London, United Kingdom	malignant mesothelioma	Phase III
namodenoson (CF102) (A3AR agonist)	Can-Fite BioPharma Waltham, MA	liver cancer (Fast Track)	Phase III <a href="http://www.canfite.com">www.canfite.com</a>
NC-6300 (epirubicin micelle formulation)	NanoCarrier Tokyo, Japan	soft tissue sarcoma	Phase I/II <a href="http://www.nanocarrier.co.jp">www.nanocarrier.co.jp</a>
nemvaleukin alfa (IL-2 fusion protein)	Alkermes Waltham, MA	mucosal melanoma (Fast Track)	Phase II <a href="http://www.alkermes.com">www.alkermes.com</a>
NIS793 (anti-TGF-beta IgG2 mAb)	Novartis East Hanover, NJ	pancreatic cancer	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
nivatrotamab	Y-mAbs Therapeutics New York, NY	neuroblastoma	Phase II <a href="http://www.ymabs.com">www.ymabs.com</a>
nivolumab and Ipilimumab	Bristol-Myers Squibb Princeton, NJ	gastric cancer, gastric-esophageal junction cancer	Phase III <a href="http://www.bms.com">www.bms.com</a>

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NUC-1031 (acelarin) (fosgemcitabine palabenamide)	NuCana Newton, MA	biliary tract cancer	Phase III <a href="http://www.nucana.com">www.nucana.com</a>
NUV-422 (CDK inhibitor)	Nuvation Bio San Francisco, CA	malignant glioma	Phase I <a href="http://www.nuvationbio.com">www.nuvationbio.com</a>
OBP-301 (telomerase oncolytic adenovirus)	Oncolys BioPharma Edison, NJ	esophageal cancer	Phase II <a href="http://www.oncolys.com">www.oncolys.com</a>
OKN-007 (disufenton sodium)	Oblato Princeton, NJ	malignant glioma	Phase II <a href="http://www.oblatoinc.com">www.oblatoinc.com</a>
ombipepimut-S (DSP-7888) (immunotherapeutic cancer vaccine)	Sumitomo Dainippon Pharma Oncology Cambridge, MA	glioblastoma	Phase III <a href="http://www.sdponcology.com">www.sdponcology.com</a>
omburtamab (131-I-8H9 monoclonal antibody)	Y-mAbs Therapeutics New York, NY	neuroblastoma	Phase II <a href="http://www.ymabs.com">www.ymabs.com</a>
ONCT-216 (ETS oncoprotein inhibitor)	Oncternal Therapeutics San Diego, CA	Ewing sarcoma	Phase II <a href="http://www.oncternal.com">www.oncternal.com</a>
<b>Onyvide</b> <sup>®</sup> irinotecan liposome injection	Ipsen Pharmaceuticals Cambridge, MA	small cell lung cancer	Phase II/III <a href="http://www.ipsen.com">www.ipsen.com</a>
opaganib (SK2 inhibitor)	RedHill Biopharma Raleigh, NC	cholangiocarcinoma	Phase II <a href="http://www.redhillbio.com">www.redhillbio.com</a>

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<b>Opdivo®</b> nivolumab	Bristol-Myers Squibb Princeton, NJ	glioblastoma	Phase III <a href="http://www.bms.com">www.bms.com</a>
oregovomab	OncoQuest Edmonton, Canada	ovarian cancer	Phase III <a href="http://www.oncoquestinc.com">www.oncoquestinc.com</a>
OSE-2101 (cancer vaccine)	OSE Immunotherapeutics Nantes, France	non-small cell lung cancer	Phase III <a href="http://www.ose-immuno.co">www.ose-immuno.co</a>
OST-HER2 (ADXS31-164) (HER2 fusion protein)	OS Therapies Cambridge, MA	osteosarcoma (Fast Track)	Phase II <a href="http://www.ostherapies.com">www.ostherapies.com</a>
padeliporfin	Steba Biotech Luxembourg	upper tract urothelial cancer	Phase III
pamrevlumab	FibroGen San Francisco, CA	pancreatic cancer (Fast Track)	Phase III <a href="http://www.fibrogen.com">www.fibrogen.com</a>
pegargiminase (ADI-PEG-20)	Polaris Pharmaceuticals San Diego, CA	mesothelioma	Phase III <a href="http://www.polarispharma.com">www.polarispharma.com</a>
PEN-221	Tarveda Therapeutics Watertown, MA	SCLC neuroendocrine tumors	Phase II <a href="http://www.tarvedatx.com">www.tarvedatx.com</a>
polyclonal antibody stimulator (gastrin inhibitor)	Cancer Advances Durham, NC	gastric cancer, pancreatic cancer	Phase III <a href="http://www.canceradvances.com">www.canceradvances.com</a>

<b>Cancer Product Name</b>	<b>Sponsor</b>	<b>Indication</b>	<b>Development Phase</b>
pravitinib (carboxyamidotriazole orotate)	Tactical Therapeutics Dallas, TX	malignant glioma (Fast Track)	Phase II <a href="http://www.tacticaltherapeutics.com">www.tacticaltherapeutics.com</a>
PT-112	Phosplatin Therapeutics New York, NY	thymoma and thymic carcinoma	Phase II <a href="http://www.phosplatin.com">www.phosplatin.com</a>
PV-10 (rose bengal sodium)	Provectus Biopharmaceuticals Knoxville, TN	melanoma, ocular melanoma	Phase I <a href="http://www.provectusbio.com">www.provectusbio.com</a>
PVSRIPO	Istari Oncology Morrisville, NC	glioma (Breakthrough Therapy), melanoma	Phase II <a href="http://www.istarioncology.com">www.istarioncology.com</a>
racemetyrosine	Tyme Technologies Bedminster, NJ	pancreatic cancer	Phase III <a href="http://www.tymeinc.com">www.tymeinc.com</a>
regorafenib	Bayer Pharmaceuticals Whippany, NJ	glioblastoma multiforme	Phase III <a href="http://www.bayer.pharma.com">www.bayer.pharma.com</a>
REIC gene therapy (MTG201) (AAV-based gene therapy)	Momotaro-Gene Okayama, Japan	malignant pleural mesothelioma	Phase II <a href="http://www.mt-gene.com">www.mt-gene.com</a>
RenovoGEM (intra-arterial gemcitabine+ RenovoCath delivery system)	RenovoRx Los Altos, CA	pancreatic cancer	Phase III <a href="http://www.renovorx.com">www.renovorx.com</a>

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repotrectinib (TPX-0005) (ROS1/TRK inhibitor)	Turning Point Therapeutics San Diego, CA	ROS1+ advanced non-small cell lung cancer (Fast Track) (Breakthrough Therapy)	Phase II <a href="http://www.tptherapeutics.com">www.tptherapeutics.com</a>
retifanlimab	Incyte Wilmington, DE	anal cancer	application submitted <a href="http://www.incyte.com">www.incyte.com</a>
		Merkel cell carcinoma	Phase II <a href="http://www.incyte.com">www.incyte.com</a>
rivoceranib (apatinib) (tyrosine kinase inhibitor)	Elevor Therapeutics Salt Lake City, UT	gastric cancer, hepatocellular carcinoma (combination therapy)	Phase III <a href="http://www.elevortherapeutics.com">www.elevortherapeutics.com</a>
		adenoid cystic carcinoma	Phase II <a href="http://www.elevortherapeutics.com">www.elevortherapeutics.com</a>
rostaporfin (REM-001) (photosensitizer)	Kintara Therapeutics San Diego, CA	basal cell carcinoma nevus syndrome	Phase I/II <a href="http://www.kintara.com">www.kintara.com</a>
RRx-001 (CD47-SIRP $\alpha$ inhibitor)	EpicentRx La Jolla, CA	small cell lung cancer	Phase III <a href="http://www.epicentrx.com">www.epicentrx.com</a>
<b>SARTATE™</b> (targeted copper theranostic)	Clarity Pharmaceuticals Eveleigh, Australia	neuroblastoma	Phase I <a href="http://www.claritypharmaceuticals.com">www.claritypharmaceuticals.com</a>

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SBP-101 (cell division inhibitor)	Panbala Therapeutics Waconia, MN	pancreatic cancer	Phase I <a href="http://www.panbela.com">www.panbela.com</a>
silmitasertib (CX-4945) (CK2 inhibitor)	Senhwa Biosciences Taipei, Taiwan	cholangiocarcinoma	Phase II <a href="http://www.senhwabiosciences.com">www.senhwabiosciences.com</a>
sotigalimab (APX005M) (IgG1 CD40 agonistic antibody)	Apexigen San Carlos, CA	esophageal cancer, gastroesophageal junction cancer	Phase II <a href="http://www.apexigen.com">www.apexigen.com</a>
SP-2577	Salarius Pharmaceuticals Houston, TX	Ewing's sarcoma.	Phase I <a href="http://www.salariuspharma.com">www.salariuspharma.com</a>
SRF-388 (anti-IL-27 IgG1 mAb)	Surface Oncology Cambridge, MA	hepatocellular carcinoma	Phase I <a href="http://www.surfaceoncology.com">www.surfaceoncology.com</a>
STAT400 (pro-enkephalin)	Statera Biopharma Fort Collins, CO	pancreatic cancer	Phase I <a href="http://www.staterabiopharma.com">www.staterabiopharma.com</a>
STP705 (siRNA therapeutic)	Sirnaomics Gaithersburg, MD	cholangiocarcinoma, hepatocellular carcinoma	Phase I <a href="http://www.sirnaomics.com">www.sirnaomics.com</a>
surufatinib	Hutchison MediPharma Shanghai, China	pancreatic neuroendocrine tumors (Fast Track)	application submitted <a href="http://www.hutch-med.com">www.hutch-med.com</a>
synthetic survivin peptide vaccine	MimiVax Buffalo, NY	glioblastoma	Phase II <a href="http://www.mimivax.com">www.mimivax.com</a>

<b>Cancer Product Name</b>	<b>Sponsor</b>	<b>Indication</b>	<b>Development Phase</b>
TAK-252	Takeda Deerfield, IL	solid tumors	Phase I <a href="http://www.takeda.com">www.takeda.com</a>
talabostat (BXCL-701)	BioXcel Therapeutics New Haven, CT	pancreatic cancer	Phase I <a href="http://www.bioxceltherapeutics.com">www.bioxceltherapeutics.com</a>
taniraleucel	Celularity Florham Park, NY	malignant glioma (Fast Track)	Phase I <a href="http://www.celularity.com">www.celularity.com</a>
TAS-120 (futibatinib) (FGFR inhibitor)	Taiho Oncology Princeton, NJ	cholangiocarcinoma (Breakthrough Therapy)	Phase III <a href="http://www.taihooncology.com">www.taihooncology.com</a>
<b>Taverik</b> <sup>®</sup> tazemetostat	Epizyme Cambridge, MA	chordoma, mesothelioma, malignant rhabdoid tumors	Phase II <a href="http://www.epizyme.com">www.epizyme.com</a>
<b>TAVO</b> <sup>™</sup>	OncoSec Medical Pennington, NJ	melanoma	Phase III <a href="http://www.oncosec.com">www.oncosec.com</a>
TBX-3400 (autologous peripheral cell therapy)	Taiga Biotechnologies Aurora, CO	malignant melanoma Stages IIb to IV	Phase I <a href="http://www.taigabiotech.com">www.taigabiotech.com</a>
<b>Tecentriq</b> <sup>®</sup> atezolizumab	Roche/Genentech South San Francisco, CA	esophageal cancer	Phase III <a href="http://www.roche.com">www.roche.com</a>
tegavivint	Iterion Therapeutics Houston, TX	desmoid tumors	Phase I <a href="http://www.iteriontherapeutics.com">www.iteriontherapeutics.com</a>



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<b>ThermoDox®</b> doxorubicin liposomal	Celsion Lawrenceville, NJ	primary liver cancer (Fast Track)	Phase III <a href="http://www.celsion.com">www.celsion.com</a>
tiragolumab (RG6058) (anti-TIGIT mAb)	Roche/Genetech South San Francisco, CA	esophageal cancer	Phase III <a href="http://www.roche.com">www.roche.com</a>
tislelizumab (anti-PD-1 mAb)	BeiGene Cambridge, MA Novartis East Hanover, NJ	esophageal cancer	Phase III <a href="http://www.beigene.com">www.beigene.com</a> <a href="http://www.novartis.com">www.novartis.com</a>
		gastric cancer, gastric esophageal junction adenocarcinoma, hepatocellular carcinoma	Phase III <a href="http://www.beigene.com">www.beigene.com</a> <a href="http://www.novartis.com">www.novartis.com</a>
toripalimab (PD-1 inhibitor)	Coherus Biosciences Redwood City, CA	nasopharyngeal cancer (Breakthrough Therapy)	application submitted <a href="http://www.coherus.com">www.coherus.com</a>
		esophageal cancer	Phase III <a href="http://www.coherus.com">www.coherus.com</a>
		mucosal melanoma, soft tissue sarcoma	Phase I/II <a href="http://www.coherus.com">www.coherus.com</a>
tremelimumab + durvalumab	AstraZeneca Wilmington, DE	hepatocellular carcinoma	Phase III <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>

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TT-00420	TransThera Biosciences Nanjing, China	cholangiocarcinoma (Fast Track)	Phase II <a href="http://www.transtherabio.com">www.transtherabio.com</a>
tucatinib	Seagen Bothell, WA	HER2+ colorectal cancer	Phase II <a href="http://www.seagen.com">www.seagen.com</a>
UGN-102 (TLR7 agonist immunotherapy)	UroGen New York, NY	urinary bladder cancer	Phase II <a href="http://www.urogen.com">www.urogen.com</a>
unesbulin	PTC Therapeutics South Plainfield, NJ	diffuse intrinsic pontine glioma, leiomyosarcoma	Phase I <a href="http://www.ptcbio.com">www.ptcbio.com</a>
VAL-083 (dianhydrogalactitol)	Kintara Therapeutics San Diego, CA	glioblastoma (Fast Track)	Phase II <a href="http://www.kintara.com">www.kintara.com</a>
veliparib (ABT-888) (PARP inhibitor)	AbbVie North Chicago, IL	ovarian cancer	Phase III <a href="http://www.abbvie.com">www.abbvie.com</a>
vidutdimod (TLR9 agonist)	Checkmate Pharmaceuticals Cambridge, MA	melanoma	Phase II/III <a href="http://www.checkmatepharma.com">www.checkmatepharma.com</a>
<b>Vigil™</b> autologous tumor cell vaccine	Gradalis Dallas, TX	Ewing's sarcoma	Phase III <a href="http://www.gradalisinc.com">www.gradalisinc.com</a>
		melanoma, ovarian cancer	Phase I/II <a href="http://www.gradalisinc.com">www.gradalisinc.com</a>

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vorasidenib (IDH1 inhibitor)	Servier Pharmaceuticals Boston, MA	glioma	Phase III <a href="http://www.servier.com">www.servier.com</a>
YIV-906 (Chinese herbal botanical)	Yiviva New York, NY	advanced hepatocellular carcinoma	Phase II <a href="http://www.yiviva.com">www.yiviva.com</a>
zanidatamab (ZW25) (HER2 x HER2 bispecific antibody)	Zymeworks Seattle, WA	biliary tract cancer (Fast Track) (Breakthrough Therapy)	Phase III <a href="http://www.zymeworks.com">www.zymeworks.com</a>
		gastroesophageal adenocarcinoma (Fast Track)	Phase II <a href="http://www.zymeworks.com">www.zymeworks.com</a>
zenocutuzumab	Merus Utrecht, Netherlands	pancreatic cancer	Phase I/II <a href="http://www.merus.nl">www.merus.nl</a>
zolbetuximab (anti-claudin 18.2 mAb)	Astellas Pharma Northbrook, IL	gastric cancer, gastroesophageal junction adenocarcinoma	Phase III <a href="http://www.astellas.com">www.astellas.com</a>

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ADX-2191 (methotrexate intravitreal)	Aldeyra Therapeutics, Inc. Lexington, MA	primary vitreoretinal lymphoma	Phase II <a href="http://www.aldeyta.com">www.aldeyta.com</a>
AFM 13 (natural killer cell stimulant)	Affimed Therapeutics Heidelberg, Germany	peripheral T-cell lymphoma	Phase II <a href="http://www.affimed.com">www.affimed.com</a>

<b><u>Cancer, Blood Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
ALLO-715 (CAR-T cell therapy)	Allogene Therapeutics South San Francisco, CA	multiple myeloma	Phase I <a href="http://www.allogene.com">www.allogene.com</a>
ALXN2075 (cerdulatinib) (SYK/JAK inhibitor)	Alexion AstraZeneca Rare Disease Boston, MA	T-cell non-Hodgkin lymphoma	Phase I/II <a href="http://www.alexion.com">www.alexion.com</a>
AMG330 (bispecific T-cell engager)	Amgen Thousand Oaks, CA	acute myeloid leukemia	Phase I <a href="http://www.amgen.com">www.amgen.com</a>
AMG701 (bispecific T-cell engager)	Amgen Thousand Oaks, CA	multiple myeloma	Phase I <a href="http://www.amgen.com">www.amgen.com</a>
AMV564 (CD33/CD3 bispecific antibody)	Amphivena Therapeutics South San Francisco, CA	acute myeloid leukemia	Phase I <a href="http://www.amphivena.com">www.amphivena.com</a>
annamycin liposomal	Moleculin Biotech Houston, TX	acute myeloid leukemia (Fast Track)	Phase I/II <a href="http://www.moleculin.com">www.moleculin.com</a>
APG-115 (alrizomadlin) (MDM2-p53 inhibitor)	Ascentage Pharma Rockville, MD	acute myeloid leukemia, malignant melanoma (Fast Track)	Phase I/II <a href="http://www.ascentagepharma.com">www.ascentagepharma.com</a>
APG-1252 (pelcitoclax) (Bcl-2/Bcl-xl inhibitor)	Ascentage Pharma Rockville, MD	myelofibrosis	Phase I <a href="http://www.ascentagepharma.com">www.ascentagepharma.com</a>

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APG-2575 (lisaftoclax) (Bcl-2 inhibitor)	Ascentage Pharma Rockville, MD	chronic lymphocytic leukemia, multiple myeloma, Waldenström macroglobulinemia	Phase I/II <a href="http://www.ascentagepharma.com">www.ascentagepharma.com</a>
APVO436 (CD123/CD3 bispecific antibody)	Aptevo Therapeutics Seattle, WA	acute myeloid leukemia	Phase I <a href="http://www.aptevotherapeutics.com">www.aptevotherapeutics.com</a>
aspcytarabine (BST-236)	BioSight Lod, Israel	acute myeloid leukemia (Fast Track)	Phase II <a href="http://www.biosight-pharma.com">www.biosight-pharma.com</a>
AUTO1 (obe-cel)	Autolus London, United Kingdom	acute lymphoblastic leukemia	Phase I/II <a href="http://www.autolus.com">www.autolus.com</a>
bb-21217 (BCMA CAR-T cell therapy)	bluebird bio Cambridge, MA Bristol-Myers Squibb Princeton, NJ	multiple myeloma	Phase I <a href="http://www.bluebirdbio.com">www.bluebirdbio.com</a> <a href="http://www.bms.com">www.bms.com</a>
BCMA-ADC (CC-99712) (antibody drug conjugate)	Bristol-Myers Squibb Princeton, NJ Sutro Biopharma South San Francisco, CA	multiple myeloma	Phase I <a href="http://www.bms.com">www.bms.com</a>
bemcentinib (BGB-324) (RTK inhibitor)	BerGenBio Bergen, Norway	acute myeloid leukemia	Phase I/II <a href="http://www.bergenbio.com">www.bergenbio.com</a>
BNZ-1 (IL-2, IL-9, IL-15 inhibitor)	Bioniz Therapeutics Irvine, CA	cutaneous T-cell lymphoma	Phase III <a href="http://www.bioniz.com">www.bioniz.com</a>

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bomedemstat (LSD1 inhibitor)	Imago BioSciences South San Francisco, CA	myelofibrosis (Fast Track), essential thrombocythemia (Fast Track)	Phase II <a href="http://www.imagobio.com">www.imagobio.com</a>
<b>Breyanzi®</b> lisocabtagene maraleucel	Bristol-Myers Squibb Princeton, NJ	mantle cell lymphoma	Phase I <a href="http://www.bms.com">www.bms.com</a>
CART-ddBCMA (anti-B-cell maturation antigen-receptor complex T cell therapy)	Arcellx Gaithersburg, MD	multiple myeloma (Fast Track)	Phase I <a href="http://www.arcellx.com">www.arcellx.com</a>
CC-90009 (GSPT1 CELMoD)	Bristol-Myers Squibb Princeton, NJ	acute myeloid leukemia	Phase I <a href="http://www.bms.com">www.bms.com</a>
cevistamab (RG6160) (bispecific antibody)	Roche/Genentech South San Francisco, CA	multiple myeloma	Phase I <a href="http://www.roche.com">www.roche.com</a>
CFT7455 (IKZF1/3)	C4 Therapeutics Watertown, MA	multiple myeloma	Phase I/II <a href="http://www.c4therapeutics.com">www.c4therapeutics.com</a>
ciltacabtagene autoleucel (BCMA CAR-T cell therapy)	Janssen Research & Development Raritan, NJ	multiple myeloma (Breakthrough Therapy)	application submitted <a href="http://www.janssen.com">www.janssen.com</a>
CLR-131	Cellectar Biosciences Florham Park, NJ	lymphoplasmacytic lymphoma, Waldenstrom macroglobulinemia (Fast Track)	Phase III <a href="http://www.cellectar.com">www.cellectar.com</a>

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<b>Copiktra®</b> duvelisib	Secura Bio Las Vegas, NV	peripheral T-cell lymphoma (Fast Track)	Phase II <a href="http://www.securebio.com">www.securebio.com</a>
crenolanib (PDGFR inhibitor)	AROG Pharmaceuticals Plano, TX	acute myeloid leukemia	Phase III <a href="http://www.arogpharma.com">www.arogpharma.com</a>
CT053 (BCMA CAR-T cell therapy)	CARsgen Therapeutics Houston, TX	multiple myeloma (Breakthrough Therapy)	Phase I/II <a href="http://www.carsgen.com">www.carsgen.com</a>
CTX110 (allogeneic CRISPR/Cas9 gene-edited CAR-T cell therapy targeting CD19)	CRISPR Therapeutics Cambridge, MA	acute lymphoblastic leukemia	Phase I <a href="http://www.crisprtx.com">www.crisprtx.com</a>
CTX120 (allogeneic CRISPR/Cas9 gene-edited CAR-T cell therapy targeting BCMA)	CRISPR Therapeutics Cambridge, MA	multiple myeloma	Phase I <a href="http://www.crisprtx.com">www.crisprtx.com</a>
CTX130 (allogeneic CRISPR/Cas9 gene-edited CAR-T cell therapy targeting CD70)	CRISPR Therapeutics Cambridge, MA	T-cell lymphoma	Phase I <a href="http://www.crisprtx.com">www.crisprtx.com</a>
devimistat (CPI-613) (lipoic acid analogue/ AEMD compound)	Rafael Pharmaceuticals Cranbury, NJ	Burkitt's lymphoma	Phase II <a href="http://www.rafaelpharma.com">www.rafaelpharma.com</a>
disulfiram and Copper	Cantex Pharmaceuticals Weston, FL	multiple myeloma	Phase I <a href="http://www.cantex.com">www.cantex.com</a>

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DSTAT (dociparstat sodium)	Chimerix Durham, NC	acute myeloid leukemia (Fast Track)	Phase III <a href="http://www.chimerix.com">www.chimerix.com</a>
elranatamab (BCMA-CD3 bispecific antibody)	Pfizer New York, NY	multiple myeloma	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
emavusertib (CA-4948) (IRAK4 inhibitor)	Curis Lexington, MA	IRAK4L-expressing leukemia (acute myeloid leukemia/ myelodysplastic syndrome)	Phase II <a href="http://www.curis.com">www.curis.com</a>
emviodostat (PTC299) (DHODH inhibitor)	PTC Therapeutics South Plainfield, NJ	acute myeloid leukemia	Phase I <a href="http://www.ptcbio.com">www.ptcbio.com</a>
ENB003 (ETBR inhibitor)	ENB Therapeutics New York, NY	melanoma (combination therapy)	Phase I/II <a href="http://www.enbpharma.com">www.enbpharma.com</a>
entospletinib	Kronos Bio San Mateo, CA	acute myeloid leukemia	Phase III <a href="http://www.kornosbio.com">www.kornosbio.com</a>
enzastaurin (DB 102) (PKC-β and PI3K/AKT inhibitor)	Denovo Biopharma San Diego, CA	diffuse large B cell lymphoma	Phase III <a href="http://www.denovobiopharma.com">www.denovobiopharma.com</a>
eprenetapopt (APR-246) (quinuclidinone)	Aprea Boston, MA	TP53-mutant acute myeloid leukemia, myelodysplastic syndrome (Breakthrough Therapy) (Fast Track)	Phase II <a href="http://www.aprea.com">www.aprea.com</a>



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fimepinostat (HDAC/PI3K inhibitor)	Curis Lexington, MA	MYC-altered lymphoma, including diffuse large B-cell lymphoma (Fast Track)	Phase II <a href="http://www.curis.com">www.curis.com</a>
flotetuzumab (CD123 × CD3 bispecific antibody)	MacroGenics Rockville, MD	acute myeloid leukemia	Phase III <a href="http://www.macrogenics.com">www.macrogenics.com</a>
FN-1501 (CDK and FLT3 inhibitor)	Fosun Pharmaceutical Shanghai, China	acute myeloid leukemia	Phase I <a href="http://www.fosunpharma.com">www.fosunpharma.com</a>
galinpepimut-S (WT1 cancer vaccine)	Sellas Life Sciences New York, NY	acute myeloid leukemia (Fast Track)	Phase III <a href="http://www.sellalifesciences.com">www.sellalifesciences.com</a>
		malignant pleural mesothelioma (Fast Track), multiple myeloma (Fast Track)	Phase II <a href="http://www.sellalifesciences.com">www.sellalifesciences.com</a>
GBT-3550 Trike (trispesific killer engager)	GT Biopharma Beverly Hills, CA	acute myeloid leukemia	Phase I/II <a href="http://www.gtbiopharma.com">www.gtbiopharma.com</a>
GDX012 ( T lymphocyte cell therapy)	GammaDelta Therapeutics London, United Kingdom	acute myeloid leukemia	Phase I <a href="http://www.gammadeltatx.com">www.gammadeltatx.com</a>
H3B-8800 (SF3B1 protein modulator)	H3 Biomedicine (Eisai) Cambridge, MA	acute myeloid leukemia, chronic myelomonocytic leukemia	Phase I <a href="http://www.eisai.com">www.eisai.com</a>

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HM43239	Hanmi Pharmaceutical Seoul, Korea	acute myeloid leukemia	Phase I/II <a href="http://www.hanmipharm.com">www.hanmipharm.com</a>
HPN-217 (B-cell maturation antigen)	AbbVie North Chicago, IL Harpoon Therapeutics South San Francisco, CA	multiple myeloma	Phase I/II <a href="http://www.abbvie.com">www.abbvie.com</a> <a href="http://www.harpoontx.com">www.harpoontx.com</a>
HQP1351 (olverembatinib) (bcr-abl/KIT/FLT3 inhibitor)	Ascentage Pharma Rockville, MD	chronic myeloid leukemia (Fast Track)	Phase I <a href="http://www.ascentagepharma.com">www.ascentagepharma.com</a>
<b>HyBryte™</b> hypericin ointment	Soligenix Princeton, NJ	cutaneous T-cell lymphoma (Fast Track)	Phase III <a href="http://www.soligenix.com">www.soligenix.com</a>
iberdomide	Bristol-Myers Squibb Princeton, NJ	multiple myeloma	Phase II <a href="http://www.bms.com">www.bms.com</a>
ICP-022 (orelabrutinib) (BTK inhibitor)	InnoCare Pharma Beijing, China	mantle cell lymphoma (Breakthrough Therapy)	Phase I/II <a href="http://www.innocarepharma.com">www.innocarepharma.com</a>
imetelstat (telomerase inhibitor)	Geron Foster City, CA	myelodysplastic syndrome, myelofibrosis	Phase III <a href="http://www.geron.com">www.geron.com</a>
IMGN632	ImmunoGen Waltham, MA	acute myeloid leukemia	Phase I <a href="http://www.immunogen.com">www.immunogen.com</a>

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IO-202 (anti-LILRB4 IgG1 mAb)	Immune-Onc Therapeutics Palo Alto, CA	acute myeloid leukemia	Phase I <a href="http://www.immune-onc.com">www.immune-onc.com</a>
ISB-1342	Ichnos Sciences New York, NY	multiple myeloma	Phase I <a href="http://www.ichnoscience.com">www.ichnoscience.com</a>
KO539 (menin/mLL inhibitor)	Kura Oncology San Diego, CA	acute myeloid leukemia	Phase I <a href="http://www.kuraoncology.com">www.kuraoncology.com</a>
KP1237 (CD38 targeting antibody)	Biohaven Pharmaceuticals New Haven, CT	multiple myeloma	Phase I/II <a href="http://www.biohaven.com">www.biohaven.com</a>
<b>Kymriah</b> <sup>®</sup> tisagenlecleucel	Novartis East Hanover, NJ	follicular lymphoma	application submitted <a href="http://www.novartis.com">www.novartis.com</a>
<b>Kyprolis</b> <sup>®</sup> carfilzomib	Amgen Thousand Oaks, CA	pediatric acute lymphoblastic leukemia	Phase II <a href="http://www.amgen.com">www.amgen.com</a>
luxepatinib	Aptose Biosciences San Diego, CA	acute myeloid leukemia	Phase I <a href="http://www.aptose.com">www.aptose.com</a>
magrolimab (anti-CD47 antibody)	Gilead Sciences Foster City, CA	myelodysplastic syndrome Breakthrough Therapy) (Fast Track), acute myeloid leukemia (Fast Track)	Phase III <a href="http://www.gilead.com">www.gilead.com</a>

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<b>Minjuvi®</b> tafasitamab-cxix	MorphoSys Planegg, Germany	follicular lymphoma	Phase III <a href="http://www.morphosys.com">www.morphosys.com</a>
modakafusp alfa (IgG4 kappa mAb)	Takeda Deerfield, IL	multiple myeloma	Phase I/II <a href="http://www.takeda.com">www.takeda.com</a>
momelotinib	Sierra Oncology Vancouver, Canada	myelofibrosis (Fast Track)	Phase III <a href="http://www.sierraoncology.com">www.sierraoncology.com</a>
mosunetuzumab (RG7828) (bispecific antibody)	Roche/Genentech South San Francisco, CA	follicular lymphoma (Breakthrough Therapy)	Phase III <a href="http://www.roche.com">www.roche.com</a>
MT-101	Myeloid Therapeutics Cambridge, MA	T-cell lymphoma	Phase I <a href="http://www.myeloidtx.com">www.myeloidtx.com</a>
naloxone topical (opioid mu receptor antagonist)	Elorac Vernon Hills, IL	pruritus in cutaneous T-cell lymphoma (Fast Track)	Phase III <a href="http://www.eloracpharma.com">www.eloracpharma.com</a>
nanatinostat and valganciclovir	Viracta Therapeutics South San Francisco, CA	T-cell lymphoma (Fast Track)	Phase II <a href="http://www.viracta.com">www.viracta.com</a>
naratuximab emtansine	Debiopharm International Lausanne, Switzerland	diffuse large B-cell lymphoma	Phase II completed <a href="http://www.debiopharm.com">www.debiopharm.com</a>

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navitoclax	AbbVie North Chicago, IL	myelofibrosis	Phase III <a href="http://www.abbvie.com">www.abbvie.com</a>
navtemadlin	Kartos Therapeutics Redwood City, CA	myelofibrosis	Phase III <a href="http://www.kartosthera.com">www.kartosthera.com</a>
		Merkel cell lymphoma	Phase II <a href="http://www.kartosthera.com">www.kartosthera.com</a>
NT-17	NeoImmuneTech Rockville, MD	idiopathic CD4+ lymphocytopenia	Phase I <a href="http://www.neoimmunetech.com">www.neoimmunetech.com</a>
odronextamab (CD20 x CD3 bispecific mAb)	Regeneron Pharmaceuticals Tarrytown, NY	diffuse large -cell lymphoma, follicular lymphoma	Phase I <a href="http://www.regenreon.com">www.regenreon.com</a>
olutasidemib (IDH1 inhibitor)	Forma Therapeutics Watertown, MA	acute myeloid leukemia	Phase I/II <a href="http://www.formatherapeutics.com">www.formatherapeutics.com</a>
Oxi4503 (combretastatin A1 phosphate)	Oncotelic Therapeutics Agoura Hills, CA	acute myeloid leukemia (Fast Track)	Phase II <a href="http://www.oncotelic.com">www.oncotelic.com</a>
parsaclisib (PI3K-delta inhibitor)	Incyte Wilmington, DE	extranodal marginal zone lymphoma, nodal marginal zone lymphoma, follicular lymphoma, splenic marginal zone lymphoma, mantle cell lymphoma	Phase III <a href="http://www.incyte.com">www.incyte.com</a>

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P-BCMA-ALL01	Poseida Therapeutics San Diego, CA	multiple myeloma	Phase I <a href="http://www.poseida.com">www.poseida.com</a>
PBCAR0191 (CD19-directed CAR-T cell therapy)	Precision BioSciences Durham, NC	acute lymphoblastic leukemia, mantle cell lymphoma	Phase I/II <a href="http://www.precisionbiosciences.com">www.precisionbiosciences.com</a>
PBCAR20A (CD20 CAR-T therapy)	Precision BioSciences Durham, NC	mantle cell lymphoma	Phase I/II <a href="http://www.precisionbiosciences.com">www.precisionbiosciences.com</a>
PBCAR269A	Precision BioSciences Durham ,NC	multiple myeloma	Phase I <a href="http://www.precisionbiosciences.com">www.precisionbiosciences.com</a>
pelabresib	Constellation Pharmaceuticals Cambridge, MA	myelofibrosis (Fast Track)	Phase III <a href="http://www.constellationpharma.com">www.constellationpharma.com</a>
pemigatinib	Incyte Wilmington, DE	myeloid/lymphoid neoplasms with eosinophilia	Phase II <a href="http://www.incyte.com">www.incyte.com</a>
pirtobrutinib	Eli Lilly Indianapolis, IN	mantle cell lymphoma	Phase III <a href="http://www.lilly.com">www.lilly.com</a>
		chronic lymphocytic leukemia	Phase I <a href="http://www.lilly.com">www.lilly.com</a>
prexigebersen (PB1001) (liposomal Grb-2)	Bio-Path Houston, TX	acute myeloid leukemia	Phase II <a href="http://www.bio-pathholdings.com">www.bio-pathholdings.com</a>

<b><u>Cancer, Blood Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
PRGN-3006	Precigen Germantown, MD	acute myeloid leukemia	Phase I <a href="http://www.precigen.com">www.precigen.com</a>
PT-112	Phosplatin Therapeutics New York, NY	multiple myeloma	Phase I <a href="http://www.phosplatin.com">www.phosplatin.com</a>
PTG-300 (hepcidin mimetic peptide)	Protagonist Therapeutics Newark, CA	polycythemia vera	Phase II <a href="http://www.protagonist-inc.com">www.protagonist-inc.com</a>
PTX-200 (tricitabine phosphate)	Prescient Therapeutics South Melbourne, Australia	acute myeloid leukemia	Phase I/II <a href="http://www.ptxtherapeutics.com">www.ptxtherapeutics.com</a>
quizartinib (FLT3 inhibitor)	Daiichi Sankyo Basking Ridge, NJ	acute myeloid leukemia (Breakthrough Therapy) (Fast Track)	Phase III <a href="http://www.dsi.com">www.dsi.com</a>
RAPA-201 (rapamycin-resistant T-cell therapy)	Rapa Therapeutics Rockville, MD	multiple myeloma	Phase II
<b>Reblozyl®</b> luspatercept	Acceleron Cambridge, MA	myelofibrosis	Phase II <a href="http://www.acceleronpharma.com">www.acceleronpharma.com</a>
RG6354 (rhPTX-2 for injection)	Roche/Genentech South San Francisco, CA	myelofibrosis	Phase II <a href="http://www.roche.com">www.roche.com</a>
SEL24/MEN1703 (PIM/FLT3 inhibitor)	Menarini Ricerche Florence, Italy	acute myeloid leukemia	Phase I/II <a href="http://www.menarini.com">www.menarini.com</a>

<b><u>Cancer, Blood Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
SNDX-5613 (menin-mixed lineage leukemia inhibitor)	Syndax Pharmaceuticals Waltham, MA	acute myeloid leukemia	Phase I/II <a href="http://www.syndax.com">www.syndax.com</a>
STRO-001 (CD-74 antibody drug conjugate)	Sutro Biopharma South San Francisco, CA	multiple myeloma	Phase I <a href="http://www.sutrobio.com">www.sutrobio.com</a>
sugemalimab (CS1001) (anti-PD-L1 antibody)	EQRx Cambridge, MA	extranodal natural killer T-cell lymphoma	Phase II <a href="http://www.eqr.com">www.eqr.com</a>
SY-2101 (apoptosis stimulant)	Syros Pharmaceuticals Cambridge, MA	acute promyelocytic leukemia	Phase II <a href="http://www.syros.com">www.syros.com</a>
talquetamab (bispecific antibody)	Janssen Research & Development Raritan, NJ	multiple myeloma	Phase II <a href="http://www.janssen.com">www.janssen.com</a>
tamibarotene (RAR-alpha agonist)	Syros Pharmaceuticals Cambridge, MA	acute myeloid leukemia	Phase II <a href="http://www.syros.com">www.syros.com</a>
TC-110 (T-cell therapy)	TCR2 Therapeutics Cambridge, MA	acute lymphoblastic leukemia	Phase I/II <a href="http://www.tcr2.com">www.tcr2.com</a>
teclistamab (bispecific antibody)	Janssen Research & Development Raritan, NJ	multiple myeloma (Breakthrough Therapy)	Phase III <a href="http://www.janssen.com">www.janssen.com</a>



<b><u>Cancer, Blood Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
tinostamustine hydrochloride	Mundipharma Cambridge, United Kingdom	multiple myeloma	Phase I <a href="http://www.mundipharma.com">www.mundipharma.com</a>
TNB-383B (BCMA-targeting immunotherapeutic)	AbbVie North Chicago, IL	multiple myeloma	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a>
tolinapant	Astex Pharmaceuticals Pleasanton, MA	T-cell lymphoma	Phase I/II <a href="http://www.astx.com">www.astx.com</a>
ublrituximab (rituximab biobetter)	TG Therapeutics New York, NY	chronic lymphocytic leukemia	application submitted <a href="http://www.tgtherapeutics.com">www.tgtherapeutics.com</a>
umbralisib	TG Therapeutics New York, NY	chronic lymphocytic leukemia	Phase III <a href="http://www.tgtherapeutics.com">www.tgtherapeutics.com</a>
<b>Venclexta</b> <sup>®</sup> venetoclax	AbbVie North Chicago, IL	multiple myeloma, myelodysplastic syndromes	Phase III <a href="http://www.abbvie.com">www.abbvie.com</a>
vodobatinib	Sun Pharma Advanced Research Mumbai, India	chronic myeloid leukemia	Phase I/II <a href="http://www.sparc.life">www.sparc.life</a>
vosaroxin	DeNovo Biopharma San Diego, CA	acute myeloid leukemia (Fast Track)	Phase II <a href="http://www.denovobiopharma.com">www.denovobiopharma.com</a>

<b>Cancer, Blood</b> <b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
zandelisib (oral PI3Kδ Inhibitor)	MEI Pharma San Diego, CA	follicular lymphoma (Fast Track)	Phase III <a href="http://www.meipharma.com">www.meipharma.com</a>
zanubrutinib	BeiGene USA Cambridge, MA	chronic lymphocytic leukemia	Phase III <a href="http://www.beigene.com">www.beigene.com</a>
zelavespib	Samus Therapeutics Boston, MA	myelofibrosis	Phase I <a href="http://www.samustherapeutics.com">www.samustherapeutics.com</a>
zelenoleucel (MT-401) (-cell therapy)	Marker Therapeutics Houston, TX	acute myeloid leukemia	Phase II <a href="http://www.markertherapeutics.com">www.markertherapeutics.com</a>
zilovertamab (ROR1 mAb)	Oncternal Therapeutics San Diego, CA	chronic lymphocytic leukemia, mantle-cell lymphoma	Phase II <a href="http://www.oncternal.com">www.oncternal.com</a>
<b>Zynlonta®</b> loncastuximab tesirine	ADC Therapeutics New Providence, NJ	mantle cell lymphoma	Phase I/II <a href="http://www.adctherapeutics.com">www.adctherapeutics.com</a>
<b>Cardiovascular Diseases</b> <b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
AER-901 (imatinib inhalation)	Aerami Therapeutics Durham, NC	pulmonary arterial hypertension	Phase I/II <a href="http://www.aerami.com">www.aerami.com</a>
aficamten (cardiac myosin inhibitor)	Cytokinetics South San Francisco, CA	hypertrophic cardiomyopathy	Phase II <a href="http://www.cytokinetics.com">www.cytokinetics.com</a>

## Cardiovascular Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AV-101 (imatinib dry powder inhalation)	Aerovate Therapeutics Boston, MA	pulmonary arterial hypertension	Phase I <a href="http://www.aerovatetx.com">www.aerovatetx.com</a>
BIIB093 (glibenclamide IV) (SUR1-TRPM4 channel inhibitor)	Biogen Cambridge, MA	severe cerebral edema in patients with acute ischemic stroke (Fast Track)	Phase III <a href="http://www.biogen.com">www.biogen.com</a>
brilaroxazine (RP5063) (serotonin and dopamine agonist)	Reviva Pharmaceuticals Cupertino, CA	pulmonary arterial hypertension	Phase I <a href="http://www.revivapharma.com">www.revivapharma.com</a>
clazosentan (endothelin receptor antagonist)	Idorsia Pharmaceuticals Allschwil, Switzerland	cerebral vasospasm associated with aneurysmal subarachnoid hemorrhage	Phase III <a href="http://www.idorsia.com">www.idorsia.com</a>
imatinib mesylate	Tenax Therapeutics Morrisville, NC	pulmonary arterial hypertension	Phase I <a href="http://www.tenaxthera.com">www.tenaxthera.com</a>
inclisiran (KJX839) (siRNA)	Novartis East Hanover, NJ	homozygous familial hypercholesterolemia	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
<b>INOpulse</b> nitric oxide inhalation	Bellerophon Therapeutics Warren, NJ	pulmonary arterial hypertension	Phase III <a href="http://www.bellerophon.com">www.bellerophon.com</a>
L-citrulline intravenous	Asklepion Pharmaceuticals Baltimore, MD	prevention of clinical sequelae of acute lung injury induced by cardiopulmonary bypass in pediatric patients	Phase III <a href="http://www.asklepionpharm.com">www.asklepionpharm.com</a>

## Cardiovascular Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
mavacamten (cardiac myosin inhibitor)	MyoKardia South San Francisco, CA Bristol-Myers Squibb Princeton, NJ	obstructive hypertrophic cardiomyopathy (Breakthrough Therapy)	application submitted <a href="http://www.bms.com">www.bms.com</a>
<b>Opsumit®</b> macitentan	Janssen Research & Development Raritan, NJ	chronic thromboembolic pulmonary hypertension	Phase III <a href="http://www.janssen.com">www.janssen.com</a>
pemziviaptadil (PB-1046) (VPAC2 selective agonist)	PhaseBio Pharmaceuticals Malvern, PA	pulmonary arterial hypertension	Phase II <a href="http://www.phasebio.com">www.phasebio.com</a>
PF-07265803 (p38 mitogen-activated protein kinase antagonist)	Pfizer New York, NY	dilated cardiomyopathy due to lamin A/C gene mutation	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
ralinepag (prostacyclin receptor agonist)	United Therapeutics Silver Spring, MD	pulmonary arterial hypertension	Phase III <a href="http://www.unither.com">www.unither.com</a>
rexlemestrocel (mesenchymal precursor cell therapy)	Mesoblast New York, NY	prevention of post-LVAD implantation mucosal bleeding	Phase III <a href="http://www.mesoblast.com">www.mesoblast.com</a>
rodatristat ethyl (tryptophan hydroxylase inhibitor)	Altavant Sciences Cary, NC	pulmonary arterial hypertension	Phase II <a href="http://www.altavant.com">www.altavant.com</a>
RT234-PAH (vardenafil)	Respira Therapeutics Albuquerque, NM	pulmonary arterial hypertension	Phase II <a href="http://www.respiratherapeutics.com">www.respiratherapeutics.com</a>

## Cardiovascular Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
sotatercept (activin inhibitor)	Acceleron Pharma Cambridge, MA	pulmonary arterial hypertension (Breakthrough Therapy)	Phase III <a href="http://www.acceleron.com">www.acceleron.com</a>
tacrolimus	VIVUS Campbell, CA	pulmonary arterial hypertension	Phase II <a href="http://www.vivus.com">www.vivus.com</a>
<b>Trevyent</b> treprostinil patch pump	United Therapeutics Silver Spring, MD	pulmonary arterial hypertension	application submitted <a href="http://www.unither.com">www.unither.com</a>
<b>Uptravi</b> <sup>®</sup> selexipag	Janssen Research & Development Raritan, NJ	thromboembolic pulmonary hypertension	Phase III <a href="http://www.janssen.com">www.janssen.com</a>
vonapanitase (rh type 1 elatase)	Protara Therapeutics New York, NY	peripheral arterial disease (Breakthrough Therapy) (Fast Track)	Phase I completed <a href="http://www.protaratx.com">www.protaratx.com</a>

## Gastrointestinal Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AK001 (anti-Siglec-8 mAb)	Allakos Redwood City, CA	eosinophilic gastritis, eosinophilic gastroenteritis	Phase III <a href="http://www.allakos.com">www.allakos.com</a>
<b>Alofisel</b> darvadstrocel	Takeda Deerfield, IL	perianal fistulas in Crohn's disease	Phase III <a href="http://www.takeda.com">www.takeda.com</a>

## Gastrointestinal Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
apraglutide	VectivBio Basel, Switzerland	short bowel syndrome with intestinal failure	Phase III <a href="http://www.vectivbio.com">www.vectivbio.com</a>
EXE-346 (live biotherapeutic)	ExeGi Pharma Rockville, MD	pouchitis	Phase II <a href="http://www.exegipharma.com">www.exegipharma.com</a>
glepaglutide (GLP-2 stimulant)	Zealand Pharma US Marlborough, MA	short bowel syndrome	Phase III <a href="http://www.zealandpharma.com">www.zealandpharma.com</a>
mirikizumab (LY3074828) (IL-23 antibody)	Eli Lilly Indianapolis, IN	pediatric Crohn's disease, pediatric ulcerative colitis	Phase III <a href="http://www.lilly.com">www.lilly.com</a>
RHB-104	RedHill Biopharma Raleigh, NC	Crohn's disease	Phase III <a href="http://www.redhillbio.com">www.redhillbio.com</a>
SGX203 (beclometasone dipropionate)	Soligenix Princeton, NJ	pediatric Crohn's disease (Fast Track)	Phase II <a href="http://www.soligenix.com">www.soligenix.com</a>
<b>Simponi</b> <sup>®</sup> golimumab	Janssen Research & Development Raritan, NJ	pediatric ulcerative colitis	Phase III <a href="http://www.janssen.com">www.janssen.com</a>
<b>Stelara</b> <sup>®</sup> ustekinumab	Janssen Research & Development Raritan, NJ	pediatric Crohn's disease, pediatric ulcerative colitis	Phase III <a href="http://www.janssen.com">www.janssen.com</a>
STP-206	Leadiant Gaithersburg, MD	necrotizing enterocolitis in premature infants	Phase I completed <a href="http://www.leadiant.com">www.leadiant.com</a>

**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
4D-110 (gene therapy)	4D Molecular Therapeutics Emeryville, CA	choroideremia	Phase I <a href="http://www.4dmoleculartherapeutics.com">www.4dmoleculartherapeutics.com</a>
4D-310 (gene therapy)	4D Molecular Therapeutics Emeryville, CA	Treatment of Fabry Disease	Phase I <a href="http://www.4moleculartherapeutics.com">www.4moleculartherapeutics.com</a>
AAV-CNGA3 (gene therapy)	MeiraGTx New York, NY	achromatopsia (CNGA3 disease) (Fast Track)	Phase I/II <a href="http://www.meiragtx.com">www.meiragtx.com</a>
AAV-CNGB3 (gene therapy)	MeiraGTx New York, NY	achromatopsia (CNGB3 disease) (Fast Track)	Phase I/II <a href="http://www.meiragtx.com">www.meiragtx.com</a>
AAV-RPE65 (gene therapy)	MeiraGTx New York, NY	RPE65 deficiency (Leber's congenital amaurosis)	Phase I/II <a href="http://www.meiragtx.com">www.meiragtx.com</a>
AAV-RPGR (gene therapy)	MeiraGTx New York, NY	x-linked retinitis pigmentosa (Fast Track)	Phase I/II <a href="http://www.meiragtx.com">www.meiragtx.com</a>
ABO-101 (gene therapy)	Abeona Therapeutics New York, NY	Sanfilippo syndrome type B (MPS IIIB) (Fast Track)	Phase I/II <a href="http://www.abeonatherapeutics.com">www.abeonatherapeutics.com</a>
ABO-102 (gene therapy)	Abeona Therapeutics New York, NY	Sanfilippo syndrome type A (MPS IIIA) (Fast Track)	Phase I/II <a href="http://www.abeonatherapeutics.com">www.abeonatherapeutics.com</a>
ACHM-1 (gene therapy)	Applied Genetic Technologies Alachua, FL	achromatopsia (CNGB3 disease)	Phase I/II <a href="http://www.agtc.com">www.agtc.com</a>

**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
ACHM-2 (gene therapy)	Applied Genetic Technologies Alachua, FL	achromatopsia (CNGA3 disease)	Phase I/II <a href="http://www.agtc.com">www.agtc.com</a>
acoramidis (AG10) (prealbumin modulator)	Eidos Therapeutics Boston, MA	transthyretin-related amyloidosis (hereditary)	Phase III <a href="http://www.eidostx.com">www.eidostx.com</a>
ACTUS-101 (AAV gene therapy)	Asklepios Biopharmaceutics Baltimore, MD Bayer Pharmaceuticals Whippany, NJ	Pompe disease	Phase I/II <a href="http://www.asklepiospharm.com">www.asklepiospharm.com</a>
adrabetadex (cholesterol modulator)	Mandos Health Los Angeles, CA	Niemann-Pick disease type C (Breakthrough Therapy)	Phase III <a href="http://www.mandoshealth.com">www.mandoshealth.com</a>
ADX-2191 (methotrexate intravitreal)	Aldeyra Therapeutics Lexington, MA	retinitis pigmentosa	Phase I <a href="http://www.aldeyra.com">www.aldeyra.com</a>
AGLE-177 (enzyme replacement therapy)	Aeglea Biotherapeutics Austin, TX	homocystinuria	Phase I/II <a href="http://www.aeglea.com">www.aeglea.com</a>
AK002 (anti-Siglec-8 mAb)	Allakos Redwood City, CA	eosinophilic esophagitis	Phase II/III <a href="http://www.allakos.com">www.allakos.com</a>
ALK-001 (retinol deuterated)	Alkeus Pharmaceuticals Boston, MA	Stargardt's disease	Phase II <a href="http://www.alkeuspharma.com">www.alkeuspharma.com</a>



**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
alvelestat (leucocyte elastase inhibitor)	Mereo Biopharma London, United Kingdom	alpha-1 antitrypsin deficiency	Phase II <a href="http://www.mereobiopharma.com">www.mereobiopharma.com</a>
ALXN1840 (bis-choline tathrethiomolybdate)	Alexion AstraZeneca Gene Therapy Boston, MA	Wilson disease (hepatolenticular degeneration) (Fast Track)	Phase III <a href="http://www.alexion.com">www.alexion.com</a>
ANAVEX®2-73 (blarcamesine) (muscarinic receptor agonist/ sigma-1 receptor agonist)	Anavex Life Sciences New York, NY	Rett syndrome (Fast Track)	Phase II <a href="http://www.anavex.com">www.anavex.com</a>
apitegromab (myostatin inhibitor)	Scholar Rock Cambridge, MA	spinal muscular atrophy (Fast Track)	Phase II <a href="http://www.scholarrock.com">www.scholarrock.com</a>
arimoclolol (heat shock protein stimulant)	Orphazyme Copenhagen, Denmark	Niemann-Pick disease type C (Breakthrough Therapy) (Fast Track)	application submitted <a href="http://www.orphazyme.com">www.orphazyme.com</a>
ARO-AAT (RNA interference)	Arrowhead Pharmaceuticals Pasadena, CA	alpha-1 antitrypsin deficiency (Breakthrough Therapy) (Fast Track)	Phase II/III <a href="http://www.arrowheadpharma.com">www.arrowheadpharma.com</a>
ARO-APOC3 (apoC-III inhibitor)	Arrowhead Pharmaceuticals Pasadena, CA	familial chylomicronemia syndrome	Phase II <a href="http://www.arrowheadpharma.com">www.arrowheadpharma.com</a>
ASP7317 (retinal pigment epithelium cell therapy)	Astellas Pharma US Northbrook, IL	Stargardt disease	Phase I <a href="http://www.astellas.com">www.astellas.com</a>

**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
AT-007 (gavorestat) (aldehyde reductase inhibitor)	Applied Therapeutics New York, NY	galactosemia (Fast Track)	Phase II/III <a href="http://www.appliedtherapeutics.com">www.appliedtherapeutics.com</a>
		phosphomannomutase 2 deficiency (congenital disorder of glycosylation)	Phase I <a href="http://www.appliedtherapeutics.com">www.appliedtherapeutics.com</a>
AT132 (AAV8-based gene therapy)	Astellas Gene Therapies San Francisco, CA	X-linked myotubular myopathy	Phase II <a href="http://www.astellasgenetherapies.com">www.astellasgenetherapies.com</a>
AT845 (AAV-based gene therapy)	Astellas Gene Therapies San Francisco, CA	Pompe disease	Phase I <a href="http://www.astellasgenetherapies.com">www.astellasgenetherapies.com</a>
AT-GTX-501 gene therapy	Amicus Therapeutics Philadelphia, PA	CLN6 Batten disease (Fast Track) (neuronal ceroid lipofuscinoses)	Phase I/II <a href="http://www.amicusrx.com">www.amicusrx.com</a>
AT-GTX-502 gene therapy	Amicus Therapeutics Philadelphia, PA	CLN3 Batten disease (Fast Track) (neuronal ceroid lipofuscinoses)	Phase I/II <a href="http://www.amicusrx.com">www.amicusrx.com</a>
avexitide	Eiger Biopharmaceuticals Palo Alto, CA	congenital hyperinsulinism (Breakthrough Therapy)	Phase II <a href="http://www.eigerbio.com">www.eigerbio.com</a>
AVR-RD-01 (lentiviral gene therapy)	AVROBIO Cambridge, MA	Fabry's disease	Phase II <a href="http://www.avrobio.com">www.avrobio.com</a>
AVR-RD-02 (lentiviral gene therapy)	AVROBIO Cambridge, MA	Gaucher's disease	Phase I/II <a href="http://www.avrobio.com">www.avrobio.com</a>

**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
AVR-RD-04 (lentiviral gene therapy)	AVROBIO Cambridge, MA	cystinosis (Fast Track)	Phase I/II <a href="http://www.avrobio.com">www.avrobio.com</a>
AVTX-801 (D-Galactose replacement)	Avalo Therapeutics Wayne, PA	phosphoglucomutase 1 deficiency (congenital disorder of glycosylation) (Fast Track)	Phase II <a href="http://www.avalotx.com">www.avalotx.com</a>
AVTX-802 (D-mannose replacement)	Avalo Therapeutics Wayne, PA	mannose phosphate isomerase deficiency (congenital disorder of glycosylation) (Fast Track)	Phase II <a href="http://www.avalotx.com">www.avalotx.com</a>
AVTX-803 (L-fucose replacement)	Avalo Therapeutics Wayne, PA	leukocyte adhesion deficiency type II (SLC35C1-CDG) (Fast Track)	Phase I <a href="http://www.avalotx.com">www.avalotx.com</a>
AVXS-201 (OAV201) (MECP2 gene therapy)	Novartis East Hanover, NJ	Rett syndrome	Phase I <a href="http://www.novartis.com">www.novartis.com</a>
AXO-AAV-GM1 (AAV9-based gene therapy)	Sio Gene Therapies New York, NY	GM1 gangliosidosis (Fast Track)	Phase I/II <a href="http://www.sioctx.com">www.sioctx.com</a>
AXO-AAV-GM2 (AAVrh8-based gene therapy)	Sio Gene Therapies New York, NY	Tay-Sachs/Sandoff disease (GM2 gangliosidosis) (Fast Track)	Phase I/II <a href="http://www.sioctx.com">www.sioctx.com</a>

**Genetic Diseases**

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
bardoxolone methyl (Nrf2 activator)	Reata Pharmaceuticals Plano, TX	chronic kidney disease caused by Alport syndrome	application submitted <a href="http://www.reatapharma.com">www.reatapharma.com</a>
		autosomal dominant polycystic kidney disease	Phase III <a href="http://www.reatapharma.com">www.reatapharma.com</a>
BBP-631 (AAV5 gene therapy)	Adrenas Therapeutics Raleigh, NC	congenital adrenal hyperplasia (Fast Track)	Phase I/II <a href="http://www.adrenastx.com">www.adrenastx.com</a>
BBP-671 (PanK activator)	CoA Therapeutics San Francisco, CA	propionic acidemia	Phase I <a href="http://www.coatherapeutics.com">www.coatherapeutics.com</a>
BBP-711 (oxalate inhibitor)	Cantero Therapeutics San Francisco, CA	primary hyperoxaluria type 1	Phase I <a href="http://www.canterrotx.com">www.canterrotx.com</a>
BBP-812 (AAV9 gene therapy)	Aspa Therapeutics Palo Alto, CA	Canavan Disease (Fast Track)	Phase I/II <a href="http://www.aspatx.com">www.aspatx.com</a>
belcesiran (RNA interference)	Dicerna Pharmaceuticals Lexington, MA	alpha 1-antitrypsin deficiency	Phase II <a href="http://www.dicerna.com">www.dicerna.com</a>
BIIB112 (cotoretigene toliparvovec) (AAV RPGR gene therapy)	Biogen Cambridge, MA	x-linked retinitis pigmentosa (Fast Track)	Phase II <a href="http://www.biogen.com">www.biogen.com</a>
BIO-017 (TRPV1 agonist/GPR55 modulator)	Biom Therapeutics Sarasota, FL	Angelman syndrome	Phase I <a href="http://www.biomtherapeutics.com">www.biomtherapeutics.com</a>

**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
BMN-307 (AAV5-phenylalanine hydroxylase gene therapy)	BioMarin Pharmaceutical Novato, CA	phenylketonuria (Fast Track)	Phase I <a href="http://www.biomarin.com">www.biomarin.com</a>
branaplam (LMIO70) (SMN2 RNA splicing modulator)	Novartis East Hanover, NJ	spinal muscular atrophy	Phase I/II <a href="http://www.novartis.com">www.novartis.com</a>
BSN175 (D tagatose)	Biospherics.net Bethesda, MD	Prader-Willi syndrome	Phase III <a href="http://www.biospherics.net">www.biospherics.net</a>
cannabidiol	Radius Pharmaceuticals Boston, MA	Prader Willi syndrome	Phase II <a href="http://www.radiuspharm.com">www.radiuspharm.com</a>
CAP-1002 (cardiac-derived cell therapy)	Capricor Therapeutics Beverly Hills, CA	Duchenne muscular dystrophy	Phase II <a href="http://www.capricor.com">www.capricor.com</a>
carisbamate	SK Life Sciences Paramus, NJ	Lennox-Gastaut syndrome	Phase I/II <a href="http://www.sklifesciencesinc.com">www.sklifesciencesinc.com</a>
cipaglucosidase alfa (AT-GAA) (enzyme replacement therapy)	Amicus Therapeutics Philadelphia, PA	glycogen storage disease type II (Pompe disease) (Breakthrough Therapy)	application submitted <a href="http://www.amicusrx.com">www.amicusrx.com</a>
CPP-1X/sul (eflornithine/sulindac)	Cancer Prevention Pharmaceuticals Tucson, AZ	familial adenomatous polyposis (Fast Track)	Phase III <a href="http://www.canprevent.com">www.canprevent.com</a>

**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
crinecerfont (CRF-1 antagonist)	Neurocrine Biosciences San Diego, CA	congenital adrenal hyperplasia (adult and pediatric)	Phase III <a href="http://www.neurocrine.com">www.neurocrine.com</a>
CSL312 (garadacimab) (factor XIIIa inhibitor)	CSL Behring King of Prussia, PA	hereditary angioedema	Phase III <a href="http://www.cslobehring.com">www.cslobehring.com</a>
CUTX-101 (copper histidine subcutaneous)	Cyprium Therapeutics New York, NY	Menkes kinky hair syndrome (Breakthrough Therapy) (Fast Track)	Phase III <a href="http://www.cyriumtx.com">www.cyriumtx.com</a>
dasiglucagon	Zealand Pharm Marlborough, MA	hypoglycemia in congenital hyperinsulinism	Phase II/III <a href="http://www.zealandpharma.com">www.zealandpharma.com</a>
DCCR (diazoxide choline controlled release)	Soleno Therapeutics Redwood City, CA	Prader-Willi syndrome (Fast Track)	Phase III <a href="http://www.soleno.life">www.soleno.life</a>
dersimelagon (MT-7117) (melanocortin type 1 receptor agonist)	Mitsubishi Tanabe Pharma Jersey City, NJ	erythropoietic protoporphyria (Fast Track)	Phase III <a href="http://www.mt-pharma.co.jp">www.mt-pharma.co.jp</a>
DNL310 (iduronate sulfatase replacement therapy)	Denali Therapeutics South San Francisco, CA	mucopolysaccharidosis II (Hunter syndrome) (Fast Track)	Phase I/II <a href="http://www.denalitherapeutics.com">www.denalitherapeutics.com</a>
DS-1211 (TNAP inhibitor)	Daiichi Sankyo Basking Ridge, NJ	pseudoxanthoma elasticum	Phase I <a href="http://www.dsi.com">www.dsi.com</a>

## Genetic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
DTX401 (AAV8-GloPhase gene therapy)	Ultragenyx Pharmaceutical Novato, CA	glycogen storage disease type Ia (von Gierke Disease)	Phase II <a href="http://www.ultragenyx.com">www.ultragenyx.com</a>
DYN101 (RNA interference)	Dynacure Jenkintown, PA	congenital structural myopathies	Phase I/II <a href="http://www.dynacure.com">www.dynacure.com</a>
E2023 (serotonin receptor agonist)	Eisai Woodcliff Lake, NJ	Dravet syndrome	Phase III <a href="http://www.eisai.com">www.eisai.com</a>
EB 002 (epicatechin) (mitochondrial protein modulator)	Epirium Bio San Diego, CA	Becker muscular dystrophy	Phase I <a href="http://www.epirium.com">www.epirium.com</a>
EDIT-101	Editas Medicine Cambridge, MA	CEP290 gene mutations (Leber congenital amaurosis)	Phase I/II <a href="http://www.editasmedicine.com">www.editasmedicine.com</a>
<b>Edsivo™</b> celiprolol	Acer Therapeutics Newton, MA	Ehlers-Danlos syndrome	Phase II <a href="http://www.acertx.com">www.acertx.com</a>
elamipretide (mitochondria-targeting amide)	Stealth Biotherapeutics Needham, MA	Barth syndrome (Fast Track)	application submitted <a href="http://www.stealthbt.com">www.stealthbt.com</a>
		Leber's hereditary optic neuropathy (Fast Track), nDNA-related primary mitochondrial disease (Fast Track)	Phase II <a href="http://www.stealthbt.com">www.stealthbt.com</a>
ELX-02 (ribosomal protein modulator)	Eloxx Pharmaceuticals Watertown, MA	cystic fibrosis (Fast Track)	Phase II <a href="http://www.eloxxpharma.com">www.eloxxpharma.com</a>

## Genetic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
emixustat (retinoid isomerohydrolase inhibitor)	Kubota Vision Seattle, WA	Stargardt disease	Phase III <a href="http://www.kubotavision.com">www.kubotavision.com</a>
EPX-100 (clemizole) (serotonin receptor modulator)	Epygenix Therapeutics Paramus, NJ	Dravet syndrome  Lennox-Gastaut syndrome	Phase II <a href="http://www.epygenix.com">www.epygenix.com</a>  Phase I <a href="http://www.epygenix.com">www.epygenix.com</a>
EPX-200 (lorcaserin) (serotonin receptor agonist)	Epygenix Therapeutics Paramus, NJ	Dravet syndrome, Lennox-Gastaut syndrome	Phase I <a href="http://www.epygenix.com">www.epygenix.com</a>
EPX-300 (serotonin receptor modulator)	Epygenix Therapeutics Paramus, NJ	Dravet syndrome	Phase I <a href="http://www.epygenix.com">www.epygenix.com</a>
eRAPA (encapsulated rapamycin)	Emtora Biosciences San Antonio, TX	familial adenomatous polyposis	Phase II <a href="http://www.emtorabio.com">www.emtorabio.com</a>
FBX-101 (AAV gene therapy)	Forge Biologics Columbus, OH	Krabbe disease (Fast Track) (globoid cell leukodystrophy)	Phase I/II <a href="http://www.forgebiologics.com">www.forgebiologics.com</a>
<b>Fintepla</b> <sup>®</sup> fenfluramine	Zogenix Emeryville, CA	Lennox-Gastaut syndrome	application submitted <a href="http://www.zogenix.com">www.zogenix.com</a>
fordadistrogene movaparvovec (minidystrophin gene therapy)	Pfizer New York, NY	Duchenne muscular dystrophy (Fast Track)	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>



## Genetic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Fycopia</b> <sup>®</sup> perapanel	Eisai Woodcliff Lake, NJ	Lennox-Gastaut syndrome	Phase III <a href="http://www.eisai.com">www.eisai.com</a>
ganaxolone (GABA-1 receptor agonist)	Marinus Pharmaceuticals Radnor, PA	CDKL5 deficiency disorder	application submitted <a href="http://www.marinuspharma.com">www.marinuspharma.com</a>
		tuberous sclerosis	Phase II <a href="http://www.marinuspharma.com">www.marinuspharma.com</a>
garetosmab (activin-A modulator)	Regeneron Pharmaceuticals Tarrytown, NY	fibrodysplasia ossificans progressiva	Phase II <a href="http://www.regeneron.com">www.regeneron.com</a>
gavorestat	Applied Therapeutics New York, NY	phosphomannomutase 2 deficiency	Phase I <a href="http://www.appliedtherapeutics.com">www.appliedtherapeutics.com</a>
givinostat (histone deacetylase inhibitor)	Italfarmaco Milan, Italy	Duchenne muscular dystrophy (Fast Track)	Phase III <a href="http://www.italfarmaco.com">www.italfarmaco.com</a>
GS030 (AAV gene therapy)	GenSight Biologics New York, NY	Leber's hereditary neuropathy	Phase III <a href="http://www.gensight-biologics.com">www.gensight-biologics.com</a>
		retinitis pigmentosa (Fast Track)	Phase I/II <a href="http://www.gensight-biologics.com">www.gensight-biologics.com</a>

**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
GTX-102 (RNA interference)	GeneTx Biotherapeutics Sarasota, FL Ultragenyx Pharmaceutical Novato, CA	Angelman syndrome (Fast Track)	Phase I <a href="http://www.genetx.bio.com">www.genetx.bio.com</a> <a href="http://www.ultragenyx.com">www.ultragenyx.com</a>
hLB-101	LogicBio Therapeutics Lexington, MA	methylmalonic acidemia (Fast Track)	Phase I/II <a href="http://www.logicbio.com">www.logicbio.com</a>
HMI-102 (gene therapy)	Homology Medicines Bedford, MA	phenylketonuria (Fast Track)	Phase II <a href="http://www.homologymedicines.com">www.homologymedicines.com</a>
HST5040 (dimethylbutanoic acid)	HemoShear Therapeutics Charlottesville, VA	methylmalonic acidemia (Fast Track), propionic acidemia (Fast Track)	Phase II <a href="http://www.hemoshear.com">www.hemoshear.com</a>
IB1001 (N-acetyl-L-leucine)	IntraBio Oxfordshire, United Kingdom	Sandoff disease (Fast Track), Tay-Sachs disease (Fast Track), Neimann-Pick type C (Fast Track)	Phase II <a href="http://www.intrabio.com">www.intrabio.com</a>
<b>Imcivree™</b> setmelanotide	Rhythm Pharmaceuticals Boston, MA	Alström syndrome, Bardet-Biedl syndrome	Phase III <a href="http://www.rhythmtx.com">www.rhythmtx.com</a>
INCB0929	Incyte Wilmington, DE	fibrodysplasia ossificans progressiva	Phase II <a href="http://www.incyte.com">www.incyte.com</a>
INZ-701 (enzyme replacement therapy)	Inozyme Pharma Boston, MA	ABCC6 deficiency, ENPP1 deficiency	Phase I/II <a href="http://www.inozyme.com">www.inozyme.com</a>

## Genetic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ION357/QR1123 (antisense oligonucleotide)	ProQR Therapeutics Leiden, Netherlands Ionis Pharmaceuticals Carlsbad, CA	retinitis pigmentosa	Phase II/III <a href="http://www.ionis.com">www.ionis.com</a>
ION373 (GFAP inhibitor, RNA interference)	Ionis Pharmaceuticals Carlsbad, CA	Alexander disease (leukodystrophy)	Phase III <a href="http://www.ionis.com">www.ionis.com</a>
isaralgagene civaparvivec	Sangamo Therapeutics Brisbane, CA	Fabry Disease	Phase I/II <a href="http://www.sangamo.com">www.sangamo.com</a>
KB105 (inetagugene geperpavec gene therapy)	Krystal Biotech Pittsburgh, PA	TGM-1-deficient autosomal recessive congenital ichthyosis (Fast Track)	Phase I/II <a href="http://www.krystalbio.com">www.krystalbio.com</a>
LB101 (chenodeoxycholic acid)	Leadiant Biosciences Gaithersburg, MD	cerebrotendinous xanthomatosis	Phase III <a href="http://www.leadiant.com">www.leadiant.com</a>
leniolisib	Novartis East Hanover, NJ Pharming Leiden, Netherlands	activated PI3Kdelta syndrome, PASLI disease	Phase II/III <a href="http://www.novartis.com">www.novartis.com</a>
<b>Lenti-D™</b> elivaldogene autotemcel	bluebird bio Cambridge, MA	cerebral adrenoleucodystrophy (adolescents, children, infants, neonates) (Breakthrough Therapy) (Fast Track)	Phase III <a href="http://www.bluebirdbio.com">www.bluebirdbio.com</a>
leriglitazone (MIN-102) (PPAR gamma agonist)	Minoryx Therapeutics Barcelona, Spain	cerebral X-linked adrenoleuco- dystrophy (Fast Track)	Phase II/III <a href="http://www.minoryx.com">www.minoryx.com</a>

**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
LM-030 (kallikrein-7/elastase-2 inhibitor)	LifeMax Laboratories Redwood City, CA	Netherton Syndrome (Fast Track)	Phase II/III <a href="http://www.lifemaxlabs.com">www.lifemaxlabs.com</a>
LNP023 (iptacopan) (CFB inhibitor)	Novartis East Hanover, NJ	C3 glomerulopathy	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
lucerastat	Idorsia Pharmaceuticals Allschwil, Switzerland	Fabry disease	Phase III <a href="http://www.idorsia.com">www.idorsia.com</a>
LV-101 (carbetocin intranasal)	Levo Therapeutics Skokie, IL	hyperphagia and behavior associated with Prader-Willi syndrome (Fast Track)	Phase III <a href="http://www.levotx.com">www.levotx.com</a>
LX1004 (AAV-mediated gene therapy)	LEXEO Therapeutics New York, NY	CLN2 Batten disease (neuronal ceroid lipofuscinoses)	Phase I/II <a href="http://www.lexeotx.com">www.lexeotx.com</a>
LYS-GM-101 (AAV gene therapy)	Lysogene Neuilly-sur-Seine, France	GM1 gangliosidosis (Fast Track)	Phase I/II <a href="http://www.lysogene.com">www.lysogene.com</a>
LYS-SAF 302 (AAV-mediated gene therapy)	Lysogene Neuilly-sur-Seine, France Sarepta Therapeutics Cambridge, MA	mucopolysaccharidosis type IIIa	Phase II/III <a href="http://www.sarepta.com">www.sarepta.com</a>
ManNAc (N-acetyl-D-mannosamine)	Leadiant Gaithersburg, MD	hereditary inclusion body myopathy type 3	Phase II <a href="http://www.leadiant.com">www.leadiant.com</a>

**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
mavorixafor	X4 Pharmaceuticals Boston, MA	WHIM syndrome (warts, hypogammaglobulinemia, immunodeficiency and myelokathexis syndrome) (Breakthrough Therapy) (Fast Track)	Phase III <a href="http://www.x4pharma.com">www.x4pharma.com</a>
MCO-010	NanoScope Technologies Bedford, TX	retinitis pigmentosa	Phase II <a href="http://www.nanotherapeutics.com">www.nanotherapeutics.com</a>
miransertib (MK-7075) (c-AKT inhibitor)	Merck Kenilworth, NJ	Proteus syndrome	Phase II <a href="http://www.merck.com">www.merck.com</a>
mirdametinib (MEK 1/2 inhibitor)	SpringWorks Therapeutics Stamford, CT	neurofibromatosis 1 (Fast Track)	Phase II <a href="http://www.springworkstx.com">www.springworkstx.com</a>
mRNA-3705 (modified mRNA)	Moderna Therapeutics Cambridge, MA	methylalonic acidemia	Phase I <a href="http://www.modernatx.com">www.modernatx.com</a>
mRNA-3745 (modified mRNA)	Moderna Cambridge, MA	glycogen storage disease type 1a	Phase I <a href="http://www.modernatx.com">www.modernatx.com</a>
mRNA-3927 (modified mRNA)	Moderna Therapeutics Cambridge, MA	propionic acidemia	Phase I <a href="http://www.modernatx.com">www.modernatx.com</a>
MT1621	Zogenix Emeryville, CA	thymidine kinase 2 deficiency	Phase III <a href="http://www.zogenix.com">www.zogenix.com</a>

## Genetic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
NBI-921352 (Nav1.6 voltage-gated sodium channel inhibitor)	Neurocrine Biosciences San Diego, CA	SCN8A-DEE epilepsy	Phase II <a href="http://www.neurocrine.com">www.neurocrine.com</a>
nedosiran (RNA oligonucleotide)	Dicerna Pharmaceuticals Lexington, MA	primary hyperoxaluria (Breakthrough Therapy)	Phase III <a href="http://www.dicerna.com">www.dicerna.com</a>
NFX-179 topical gel (MEK inhibitor)	NFlection Therapeutics Wayne, PA	neurofibromatosis 1	Phase II <a href="http://www.nflectionrx.com">www.nflectionrx.com</a>
NT-501 (ciliary neurotrophic growth factor)	Neurotech USA Cumberland, RI	macular telangiectasia	Phase III <a href="http://www.neurotechusa.com">www.neurotechusa.com</a>
odevixibat	Albireo Pharma Boston, MA	Alagille syndrome	Phase III <a href="http://www.albireopharma.com">www.albireopharma.com</a>
olipudase alfa	Sanofi Bridgewater, NJ	acid sphingomyelinase deficiency in Neimann-Pick disease	application submitted <a href="http://www.sanofi.com">www.sanofi.com</a>
pabinafusp alfa	PCR Pharmaceuticals Hyogo, Japan Takeda Deerfield, IL	mucopolysaccharidosis II (Hunter syndrome)	Phase III <a href="http://www.takeda.com">www.takeda.com</a>
palovarotene	Ipsen Cambridge, MA	fibrodysplasia ossificans progressiva (Fast Track)	Phase III <a href="http://www.ipсен.com">www.ipсен.com</a>

**Genetic Diseases**

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
pamrevlumab	FibroGen San Francisco, CA	Duchenne muscular dystrophy (Fast Track)	Phase III <a href="http://www.fibrogen.com">www.fibrogen.com</a>
PBA-003 (cavosonstat)	Path BioAnalytics Chapel Hill, NC	cystic fibrosis (Fast Track)	Phase II <a href="http://www.pathbioanalytics.com">www.pathbioanalytics.com</a>
PBGM01 (AAV gene therapy)	Passage Bio Philadelphia, PA	GM1 gangliosidosis	Phase I/II <a href="http://www.passagebio.com">www.passagebio.com</a>
PBKR-03	Passage Bio Philadelphia, PA	globoid cell leukodystrophy (Krabbe disease)	Phase I/II <a href="http://www.passagebio.com">www.passagebio.com</a>
pegcetacoplan	Apellis Pharmaceuticals Waltham, MA	C3 glomerulopathy	Phase II <a href="http://www.apellis.com">www.apellis.com</a>
pegzilarginase (pegylated human recombinant arginase)	Aeglea Biotherapeutics Austin, TX	arginase 1 deficiency (hyperargininemia) (Breakthrough Therapy) (Fast Track)	Phase III <a href="http://www.aeglea.com">www.aeglea.com</a>
<b>Piqray</b> <sup>®</sup> alpelisib (BYL719)	Novartis East Hanover, NJ	PIK3CA-related overgrowth spectrum (Breakthrough Therapy)	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
PLX-200 (TFEB upregulator)	Polaryx Therapeutics Paramus, NJ	CLN3 Batten disease (Fast Track) (neuronal ceroid lipofuscinoses)	Phase II <a href="http://www.polarityx.com">www.polarityx.com</a>

**Genetic Diseases**

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
pozelimab (C5 antibody)	Regeneron Pharmaceuticals Tarrytown, NY	CD55-deficient protein-losing enteropathy	Phase II <a href="http://www.regeneron.com">www.regeneron.com</a>
PR001 (AAV9 gene therapy)	Prevail Therapeutics New York, NY Lilly Indianapolis, IN	Gaucher disease (Fast Track)	Phase I/II <a href="http://www.prevailtherapeutics.com">www.prevailtherapeutics.com</a>
progerinin	PRG S&T Busan, South Korea	progeria (Werner syndrome) Hutchinson-Gilford progeria syndrome	Phase I <a href="http://www.prgst.com">www.prgst.com</a>
<b>Prolia</b> <sup>®</sup> denosumab	Amgen Thousand Oaks, CA	osteogenesis imperfecta (pediatric)	Phase III <a href="http://www.amgen.com">www.amgen.com</a>
PTC923 (sepiapterin) (mixed function oxygenase modulator)	PTC Therapeutics South Plainfield, NJ	phenylketonuria	Phase III <a href="http://www.ptcbio.com">www.ptcbio.com</a>
PTC-AADC (eladocagene exuparvovec)	PTC Therapeutics South Plainfield, NJ	aromatic L-amino acid decarboxylase deficiency	Phase II <a href="http://www.ptcbio.com">www.ptcbio.com</a>
PXT3003 (baclofen/naltrexone/sorbitol)	Pharnext Paris, France	Charcot-Marie-Tooth disease (Fast Track)	Phase III <a href="http://www.pharnext.com">www.pharnext.com</a>
QR-421a (antisense oligonucleotide)	ProQR Therapeutics Leiden, Netherlands	retinitis pigmentosa	Phase II <a href="http://www.proqr.com">www.proqr.com</a>



## Genetic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
REC-2282 (HDAC inhibitor)	Recursion Pharmaceuticals Salt Lake City, UT	NF2-mutation meningioma (Fast Track)	Phase I <a href="http://www.recursion.com">www.recursion.com</a>
recifercept (FGFR3 decoy)	Pfizer New York, NY	achondroplasia	Phase II <a href="http://www.pfizer.com">www.pfizer.com</a>
reloxaliase (bacillus subtilis oxalate decarboxylase)	Allena Pharmaceuticals Newton, MA	primary hyperoxaluria	Phase III <a href="http://www.allenapharma.com">www.allenapharma.com</a>
REN001 (PPAR agonist)	Reneo Pharmaceuticals Irvine, CA	primary mitochondrial myopathy (Fast Track)	Phase II/III <a href="http://www.reneopharma.com">www.reneopharma.com</a>
		fatty acid oxidation disorders	Phase I <a href="http://www.reneopharma.com">www.reneopharma.com</a>
ribitol (BBP-418) (glycosylatin substrate)	ML BioSolutions Charlotte, NC	limb-girdle muscular dystrophy (Fast Track)	Phase II <a href="http://www.mlbiosolutions.com">www.mlbiosolutions.com</a>
romosozumab	Amgen Thousand Oaks, CA	osteogenesis imperfecta	Phase I <a href="http://www.amgen.com">www.amgen.com</a>
RP-A501	Rocket Pharmaceuticals Cranbury, NJ	Danon disease (Fast Track)	Phase I <a href="http://www.rocketpharma.com">www.rocketpharma.com</a>
RP-L201 (stem cell therapy)	Rocket Pharmaceuticals Cranbury, NJ	leukocyte adhesion deficiency type I (Fast Track)	Phase II <a href="http://www.rocketpharma.com">www.rocketpharma.com</a>

**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
RP-L401	Rocket Pharmaceuticals Cranbury, NJ	infantile malignant osteopetrosis (Fast Track)	Phase I <a href="http://www.rocketpharma.com">www.rocketpharma.com</a>
RZ358 (IgG2 mab)	Rezolute Redwood City, CA	congenital hyperinsulinism	Phase II <a href="http://www.rezolutebio.com">www.rezolutebio.com</a>
S48168 (ARM-210) (ryanodine receptor calcium release channel modulator)	ARMGO Pharma Tarrytown, NY	congenital structural RYR1-related myopathies	Phase I <a href="http://www.armgo.com">www.armgo.com</a>
SAR339375 (RG-012) (miRNA-21)	Regulus Therapeutics San Diego, CA Sanofi Bridgewater, NJ	Alport syndrome	Phase II <a href="http://www.sanofi.com">www.sanofi.com</a>
sepfarsen (QR-110) (antisense oligonucleotide)	ProQR Therapeutics Leiden, Netherlands	Leber's congenital amaurosis (Fast Track)	Phase II/III <a href="http://www.proqr.com">www.proqr.com</a>
SGT-001 (AAV gene therapy)	Solid Biosciences Cambridge, MA	Duchenne muscular dystrophy (Fast Track)	Phase I/II <a href="http://www.solidbio.com">www.solidbio.com</a>
SLS-005 (trehalose) (protein aggregation inhibitor)	Seelos New York, NY	oculopharyngeal muscular dystrophy, Sanfilippo syndrome	Phase II <a href="http://www.seelos.com">www.seelos.com</a>
soticlestat (cholesterol 24S-hydroxylase inhibitor)	Takeda Deerfield, IL	Dravet syndrome, Lennox-Gastaut syndrome	Phase III <a href="http://www.takeda.com">www.takeda.com</a>

**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
SPK-3006 (RG6359) (AAV-based gene therapy)	Spark Therapeutics Philadelphia, PA	Pompe disease	Phase II <a href="http://www.sparktx.com">www.sparktx.com</a>
SPK-7001 (RG6367) (gene therapy)	Spark Therapeutics Philadelphia, PA	choroideremia	Phase II <a href="http://www.sparktx.com">www.sparktx.com</a>
SRP-5051	Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy	Phase II <a href="http://www.sarepta.com">www.sarepta.com</a>
SRP-6004 (dysferlin)	Sarepta Therapeutics Cambridge, MA	limb girdle muscular dystrophy 2B	Phase I <a href="http://www.sarepta.com">www.sarepta.com</a>
SRP-9001 (delandistrogene moxeparvovec)	Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy (Fast Track)	Phase III <a href="http://www.sarepta.com">www.sarepta.com</a>
SRP-9003 (bidridistrogene xeboparvovec)	Sarepta Therapeutics Cambridge, MA	limb girdle muscular dystrophy 2E	Phase I/II <a href="http://www.sarepta.com">www.sarepta.com</a>
SRP-9004 (AAV-Rh74 gene therapy)	Sarepta Therapeutics Cambridge, MA	limb girdle muscular dystrophy 2D	Phase I/II <a href="http://www.sarepta.com">www.sarepta.com</a>
SYNB-1934 (probiotic bacterium)	Synlogic Cambridge, MA	phenylketonuria	Phase II <a href="http://www.synlogictx.com">www.synlogictx.com</a>
TAK-611 (cerebrosidase replacement)	Takeda Deerfield, IL	metachromatic leukodystrophy	Phase II <a href="http://www.takeda.com">www.takeda.com</a>

**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
TAK-999	Takeda Deerfield, IL	alpha-1 antitrypsin deficiency (Breakthrough Therapy) (Fast Track)	Phase II/III <a href="http://www.takeda.com">www.takeda.com</a>
<b>Tavneos™</b> avacopan	ChemoCentryx San Carlos, CA	C3 glomerulopathy	Phase II <a href="http://www.chemocentryx.com">www.chemocentryx.com</a>
tideglusib	AMO Pharma Durham, NC	myotonic dystrophy type 1	Phase II/III <a href="http://www.amo-pharma.com">www.amo-pharma.com</a>
tildacerfont (CRF-receptor 1)	Spruce Biosciences Dale City, CA	congenital adrenal hyperplasia	Phase II <a href="http://www.sprucebiosciences.com">www.sprucebiosciences.com</a>
tinlarebant (LBS-008)	Belite Bio San Diego, CA	Stargardt disease	Phase I completed <a href="http://www.belitebio.com">www.belitebio.com</a>
tiratricol	Rare Thyroid Therapeutics (Egetis) Stockholm, Sweden	monocarboxylate 8 transporter deficiency (Fast Track) (Allan-Herndon-Dudley-Syndrome)	Phase II/III <a href="http://www.egetis.com">www.egetis.com</a>
<b>Trappsol® Cyclo™</b> hydroxypropyl betadex	Cyclo Therapeutics Alachua, FL	Niemann-Pick disease type C (Fast Track)	Phase III <a href="http://www.cyclotherapeutics.com">www.cyclotherapeutics.com</a>
<b>Truseltiq™</b> low dose infigratinib	QED Therapeutics Brisbane, CA	achondroplasia	Phase II <a href="http://www.qedtx.com">www.qedtx.com</a>

**Genetic Diseases**

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
TSHA-101 (AAV9-based gene therapy)	Taysha Gene Therapies Dallas, TX	GM2 gangliosidosis	Phase I/II <a href="http://www.tayshagtx.com">www.tayshagtx.com</a>
TSHA-1218 (AAV9-based gene therapy)	Taysha Gene Therapies Dallas, TX	CLN1 disease	Phase I/II <a href="http://www.tayshatx.com">www.tayshatx.com</a>
TXA127 injectable (angiotensin 1 7)	Constant Therapeutics Boston, MA	Duchenne muscular dystrophy (Fast Track)	Phase I <a href="http://www.constanttherapeutics.com">www.constanttherapeutics.com</a>
UX143 (setrusumab) (anti-sclerostin mAb)	Ultragenyx Pharmaceutical Novato, CA	osteogenesis imperfecta	Phase II <a href="http://www.ultragenyx.co">www.ultragenyx.co</a>
UX701 (AAV9-ATP7B gene therapy)	Ultragenyx Pharmaceutical Novato, CA	Wilson's disease	Phase I <a href="http://www.ultragenyx.com">www.ultragenyx.com</a>
VBP15 (vamorolone)	ReveraGen Biopharma Rockville, MD Santhera Pharmaceuticals Burlington, MA	Duchenne muscular dystrophy (Fast Track)	Phase III <a href="http://www.reveragen.com">www.reveragen.com</a> <a href="http://www.santhera.com">www.santhera.com</a>
venglustat malate (oral GCS inhibitor)	Sanofi Bridgewater, NJ	GM2 gangliosidosis	Phase III <a href="http://www.sanofi.com">www.sanofi.com</a>
		Fabry disease (Fast Track), Gaucher disease type 3	Phase II <a href="http://www.sanofi.com">www.sanofi.com</a>

**Genetic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
verdiperstat	Biohaven Pharmaceuticals New Haven, CT	multiple system atrophy (Fast Track)	Phase III <a href="http://www.biohaven.com">www.biohaven.com</a>
VK-0214 (TR beta agonist)	Viking Therapeutics San Diego, CA	X-linked adrenoleucodystrophy	Phase I <a href="http://www.vikingtherapeutics.com">www.vikingtherapeutics.com</a>
XLRP	Applied Genetic Technologies Alachua, FL	retinitis pigmentosa	Phase II/III <a href="http://www.agtc.com">www.agtc.com</a>
XP-0863 (diazepam subcutaneous)	XERIS Pharmaceuticals Chicago, IL	Dravet syndrome	Phase I <a href="http://www.exrispharma.com">www.exrispharma.com</a>
VTX-801 (AAV gene therapy)	Vivet Therapeutics Paris, France	Wilson's disease (Fast Track)	Phase I/II <a href="http://www.vivet-therapeutics.com">www.vivet-therapeutics.com</a>
VX-121 + tezacaftor + deutivacaftor (once-daily treatment)	Vertex Pharmaceuticals Boston, MA	cystic fibrosis (Fast Track)	Phase III <a href="http://www.vrtx.com">www.vrtx.com</a>
zatolmilast	Tetra Discovery Partners Grand Rapids, MI	fragile X Syndrome	Phase II <a href="http://www.tetratherapeutics.com">www.tetratherapeutics.com</a>
<b>Zegalogue</b> <sup>®</sup> dasiglucagon	Zealand Pharma Boston, MA	congenital hyperinsulinism	Phase III <a href="http://www.zealandpharma.com">www.zealandpharma.com</a>
zunsemetinib (ATI-450)	Aclaris Therapeutics Wayne, PA	cryopyrin-associated periodic syndrome	Phase II <a href="http://www.aclaristx.com">www.aclaristx.com</a>

## Genetic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Zygel® cannabidiol transdermal gel	Zynerba Pharmaceuticals Devon, PA	fragile X syndrome	Phase III <a href="http://www.zynerba.com">www.zynerba.com</a>
		22q deletion syndrome (DiGeorge syndrome)	Phase II <a href="http://www.zynerba.com">www.zynerba.com</a>

## Growth Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ibutamoren mesylate	Lumos Pharma Ames, IA	growth hormone deficiency	Phase II <a href="http://www.lumos-pharma.com">www.lumos-pharma.com</a>
paltusotine	Crinetics Pharmaceuticals San Diego, CA	acromegaly	Phase III <a href="http://www.crinetics.com">www.crinetics.com</a>
PF-06836922 (somatrogen) (human growth hormone agonist)	Pfizer New York, NY	pediatric growth hormone deficiency	application submitted <a href="http://www.pfizer.com">www.pfizer.com</a>
		adult growth hormone deficiency	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
Somatrogen® human growth hormone	OPKO Health Miami, FL	growth hormone deficiency	application submitted <a href="http://www.opko.com">www.opko.com</a>

## Growth Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
TransConCNP	Ascendis Pharma North Brunswick, NJ	achondroplasia	Phase II <a href="http://www.ascendiapharma.com">www.ascendiapharma.com</a>
TSX-002 oral testosterone	TesoRx Pharma Pomona, CA	constitutional delay of growth and puberty in boys	Phase II <a href="http://www.tesorx.com">www.tesorx.com</a>

## Infectious Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
adimlecleucel	Atara Biotherapeutics South San Francisco, CA	cytomegalovirus viremia	Phase II <a href="http://www.atarabio.com">www.atarabio.com</a>
AR-101 mAb (Aerumab) (lipopolysaccharide inhibitor)	Aridis Pharmaceuticals Los Gatos, CA	hospital-acquired bacterial pneumonia (HABP)/ventilator-acquired bacterial pneumonia (VABP) caused by multi-drug resistant <i>Pseudomonas</i> <i>aeruginosa</i> LPS serotype 011	Phase II <a href="http://www.aridispharma.com">www.aridispharma.com</a>
AR-301 mAb (tosataxumab) (bacterial toxin inhibitor)	Aridis Pharmaceuticals San Jose, CA	hospital-acquired pneumonia and ventilator-associated pneumonia due to <i>Staphylococcus aureus</i> (Fast Track)	Phase III <a href="http://www.aridispharma.com">www.aridispharma.com</a>
AR-501 (Panaecin) (gallium citrate inhaled)	Aridis Pharmaceuticals San Jose, CA	cystic fibrosis-associated respiratory tract infections (Fast Track)	Phase II <a href="http://www.aridispharma.com">www.aridispharma.com</a>



## Infectious Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AR-501 mAb (Aerucin) (bacterial growth inhibitor)	Aridis Pharmaceuticals Los Gatos, CA	antibiotic-resistant gram-negative and gram-positive infections in cystic fibrosis, including <i>Pseudomonas aeruginosa</i> (Fast Track)	Phase II <a href="http://www.aridispharma.com">www.aridispharma.com</a>
AV7909 (pre-formulated anthrax vaccine)	Emergent BioSolutions Gaithersburg, MD	post-exposure prevention of anthrax (Fast Track)	Phase III <a href="http://www.emergentbiosolutions.com">www.emergentbiosolutions.com</a>
BSN389 (aphidicolin)	Biospherics.net Beltsville, MD	Ebola virus infections	Phase I <a href="http://www.biospherics.net">www.biospherics.net</a>
bulevirtide (lipopeptide)	MYR Pharma Burgwedel, Germany	hepatitis D	Phase III <a href="http://www.myr-pharma.com">www.myr-pharma.com</a>
cipargamin (KAE609) (PfATP4 inhibitor)	Novartis East Hanover, NJ	malaria	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
DAS181 (virus internalization inhibitor)	Ansun Biopharma San Diego, CA	parainfluenza virus infections (Fast Track) (Breakthrough Therapy)	Phase III <a href="http://www.ansunbiopharma.com">www.ansunbiopharma.com</a>
F901318 (olorofim) (dihydroorotate dehydrogenase inhibitor)	F2G Manchester, England	mycoses (Breakthrough Therapy)	Phase II <a href="http://www.f2g.com">www.f2g.com</a>
fosmanogepix (Gwt1 inhibitor)	Pfizer New York, NY	invasive fungal infections caused by aspergillosis and rare molds	Phase II <a href="http://www.pfizer.com">www.pfizer.com</a>

## Infectious Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Hepcludex®</b> bulevirtide	Gilead Sciences Foster City, CA	hepatitis D (Breakthrough Therapy)	Phase II <a href="http://www.gilead.com">www.gilead.com</a>
ibrexafungerp (glucan synthase inhibitor)	SCYNEXIS Jersey City, NJ	invasive aspergillosis (Fast Track)	Phase I/II <a href="http://www.scynexis.com">www.scynexis.com</a>
lonafarnib (farnesyltransferase inhibitor)	Eiger BioPharmaceuticals Palo Alto, CA	hepatitis D infections (+ritonavir) (Breakthrough Therapy) (Fast Track)	Phase III <a href="http://www.eigerbio.com">www.eigerbio.com</a>
maribavir (TAK-620) (ganciclovir kinase inhibitor)	Takeda Deerfield, IL	CMV infections in transplantation (Breakthrough Therapy) (Fast Track)	application submitted <a href="http://www.takeda.com">www.takeda.com</a>
MAT2203 (oral enochleated amphotericin B)	Matinas BioPharma Bedminster, NJ	cryptococcosis (Fast Track)	Phase I/II <a href="http://www.matinasbiopharma.com">www.matinasbiopharma.com</a>
MAT2501 (oral enochleated amikacin)	Matinas BioPharma Nanotechnologies Bridgewater, NJ	nontuberculous mycobacterium infections	Phase I <a href="http://www.matinasbiopharma.com">www.matinasbiopharma.com</a>
omadacycline	Paratek Pharmaceuticals Boston, MA	nontuberculous mycobacteria infections	Phase II <a href="http://www.paratekpharma.com">www.paratekpharma.com</a>
peginterferon lambda (IL-29 receptor agonist)	Eiger BioPharmaceuticals Palo Alto, CA	hepatitis D (Breakthrough Therapy) (Fast Track)	Phase III <a href="http://www.eigerbio.com">www.eigerbio.com</a>
<b>PhageBank™</b> bacteriophage therapeutics	Adaptive Phage Therapeutics Gaithersburg, MD	prosthetic joint infections	Phase I/II <a href="http://www.aphage.com">www.aphage.com</a>

## Infectious Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
PLG0206 (engineered cationic antibiotic peptide)	Peptilogics Pittsburgh, PA	prosthetic joint infections	Phase II <a href="http://www.peptilogics.com">www.peptilogics.com</a>
<b>Pulmazole</b> itraconazole inhaled	Pulmatrix Lexington, MA	aspergillosis (Fast Track)	Phase II <a href="http://www.pulmatrix.com">www.pulmatrix.com</a>
RBX2660 (enema formulation) (microbiota restoration therapy)	Rebiotix Roseville, MN	prevention of recurrent <i>Clostridioides difficile</i> infections (Breakthrough Therapy) (Fast Track)	Phase III <a href="http://www.rebiotix.com">www.rebiotix.com</a>
reltecimod (AB-103) (CD28 co-stimulatory receptor antagonist)	Atox Bio Chapel Hill, NC	necrotizing soft tissue infections (Fast Track)	application submitted <a href="http://www.atoxbio.com">www.atoxbio.com</a>
<b>Remune</b> <sup>®</sup> GP120-depleted HIV-1 vaccine	Immune Response BioPharma Atlantic City, NJ	HIV-1 infections (pediatric)	Phase I/II <a href="http://www.immuneresponsebio.com">www.immuneresponsebio.com</a>
rezafungin (long-acting echinocandin and glucan synthase inhibitor)	Cidara Therapeutics San Diego, CA	fungal infections caused by <i>Candida spp.</i> , including, candidemia and invasive candidiasis (Fast Track)	Phase III <a href="http://www.cidara.com">www.cidara.com</a>
RHB-204 (clarithromycin/clofazimine/rifabutin)	RedHill BioPharma Raleigh, NC	nontuberculous mycobacterium infection (Fast Track)	Phase III <a href="http://www.redhillbio.com">www.redhillbio.com</a>
SER-109 (microbiome therapeutic)	Seres Therapeutics Cambridge, MA	prevention of recurrent <i>Clostridioides difficile</i> infections (Breakthrough Therapy)	Phase III <a href="http://www.serestherapeutics.com">www.serestherapeutics.com</a>

## Infectious Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
SPR720 (DNA gyrase inhibitor)	Spero Therapeutics Cambridge, MA	nontuberculous mycobacterial infections (Fast Track)	Phase II <a href="http://www.sperotherapeutics.com">www.sperotherapeutics.com</a>
suramin	PaxMedica Tarrytown, NY	African trypanosomiasis	Phase III <a href="http://www.paxmedica.com">www.paxmedica.com</a>
SVT-15652 (clotrimazole otic solution)	Laboratorios SALVAT Barcelona, Spain	fungal otitis externa (otomycosis)	Phase III <a href="http://www.svt.com">www.svt.com</a>
telacebec (Q203) (bacterial growth inhibitor)	Qurient Therapeutics Seongnam-si, South Korea	<i>Mycobacterium tuberculosis</i> (Fast Track)	Phase II completed <a href="http://www.qurient.com">www.qurient.com</a>
TNP-2092 (dual-acting antibacterial)	TenNor Therapeutics Suzhou, China	prosthetic joint infections (Fast Track)	Phase I <a href="http://www.tennorrx.com">www.tennorrx.com</a>
VE303 (microbiome therapeutic)	Vedanta Biosciences Cambridge, MA	high-risk recurrent <i>Clostridioides</i> <i>difficile</i> infections (high-risk)	Phase II <a href="http://www.vedantabio.com">www.vedantabio.com</a>
VT-1598 (tetrazole antifungal)	Mycovia Pharmaceuticals Durham, NC NQP 1598 Grand Cayman	coccidioidomycosis (Fast Track)	Phase I <a href="http://www.mycovia.com">www.mycovia.com</a>

## Neurologic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AL001 (SORT1 inhibitor)	Alector South San Francisco, CA GlaxoSmithKline Research Triangle Park, NC	frontotemporal dementia with granulin mutation (Fast Track)	Phase III <a href="http://www.alector.com">www.alector.com</a>
		frontotemporal dementia with a C9orf72 mutation	Phase II <a href="http://www.alector.com">www.alector.com</a>
ALMB-0166 (connexin 43 inhibitor)	AlaMab Therapeutics Princeton, NJ	spinal cord injury	Phase I <a href="http://www.alamab.com">www.alamab.com</a>
ALS 001 (Treg cell therapy)	Coya Therapeutics Houston, TX	amyotrophic lateral sclerosis (ALS)	Phase II <a href="http://www.coyatherapeutics.com">www.coyatherapeutics.com</a>
AMT-130 (AAV5 gene therapy)	uniQure Biopharma Lexington, MA	Huntington's disease (Fast Track)	Phase I/II <a href="http://www.uniqure.com">www.uniqure.com</a>
AMX0035 (sodium phenylbutyrate/ tauroursodeoxycholic acid)	Amylyx Pharmaceuticals Cambridge, MA	ALS	Phase III <a href="http://www.amylyx.com">www.amylyx.com</a>
ANAVEX®2-73 (blarcamesine) (muscarinic receptor agonist/ sigma-1 receptor agonist)	Anavex Life Sciences New York, NY	infantile spasms	Phase I <a href="http://www.anavex.com">www.anavex.com</a>
ANAVEX® 3-71 (AF710B) (muscarinic receptor agonist/ sigma-1 receptor agonist)	Anavex Life Sciences New York, NY	frontotemporal dementia	Phase I <a href="http://www.anavex.com">www.anavex.com</a>

**Neurologic Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
ANX005 (complement C1 inhibitor)	Annexon South San Francisco, CA	Huntington's disease	Phase II <a href="http://www.annexon.com">www.annexon.com</a>
AT-1501 (CD40 ligand inhibitor)	Eledon Pharmaceuticals Irvine, CA	ALS	Phase II <a href="http://www.eledon.com">www.eledon.com</a>
BBP-671 (PanK activator)	CoA Therapeutics San Francisco, CA	pantothenate kinase-associated neurodegeneration	Phase I <a href="http://www.coatherapeutics.com">www.coatherapeutics.com</a>
BIIB067 (tofersen) (SOD1 inhibitor)	Biogen Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	ALS	Phase III <a href="http://www.biogen.com">www.biogen.com</a>
BIIB093	Biogen Cambridge, MA	severe cerebral edema in acute ischemic stroke	Phase III <a href="http://www.biogen.com">www.biogen.com</a>
brivaracetam	UCB Smyrna, GA	juvenile absence epilepsy, childhood absence epilepsy	Phase III <a href="http://www.ucb.com">www.ucb.com</a>
CNM-Au8 (faceted gold nanocrystals)	Cleane Nanomedicine Salt Lake City, UT	ALS	Phase III <a href="http://www.clece.com">www.clece.com</a>
CORT113176	Corcept Therapeutics Menlo Park, CA	ALS	Phase I <a href="http://www.corcept.com">www.corcept.com</a>

## Neurologic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CTI-1601 (frataxin protein replacement)	Larimar Therapeutics Bala Cynwyd, PA	Friedreich's ataxia	Phase I <a href="http://www.larimartx.com">www.larimartx.com</a>
dipraglurant	Addex Therapeutics San Francisco, CA	levodopa-induced dyskinesia in Parkinson's disease	Phase II/III <a href="http://www.addextherapeutics.com">www.addextherapeutics.com</a>
donaperminogene seltoplasmid (gene therapy)	Helixmith Seoul, South Korea	ALS (Fast Track)	Phase II <a href="http://www.helixmith.com">www.helixmith.com</a>
ecopipam (dopamine D1 receptor antagonist)	Emalex Biosciences Chicago, IL	Tourette's syndrome (Fast Track)	Phase III <a href="http://www.emalexbiosciences.com">www.emalexbiosciences.com</a>
elezanumab (RGMA protein inhibitor)	AbbVie North Chicago, IL	spinal cord injury (Fast Track)	Phase II <a href="http://www.abbvie.com">www.abbvie.com</a>
<b>Ery-Dex</b> dexamethasone erythrocyte encapsulated	EryDel Bresso, Italy	ataxia telangiectasia	Phase III <a href="http://www.erydel.com">www.erydel.com</a>
fasudil (rho kinase inhibitor)	Woolsey Pharmaceuticals New York, NY	progressive supranuclear palsy, corticobasal degeneration	Phase II <a href="http://www.woolseypharma.com">www.woolseypharma.com</a>
ganaxolone (GABA-1 receptor agonist)	Marinus Pharmaceuticals Radnor, PA	refractory status epilepticus	Phase II <a href="http://www.marinuspharma.com">www.marinuspharma.com</a>

## Neurologic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
GM604 (pleiotropic peptide)	Genervon Biopharmaceuticals Pasadena, CA	ALS (Fast Track)	Phase II <a href="http://www.genervon.com">www.genervon.com</a>
IB1001 (N-acetyl-L-leucine)	IntraBio Oxfordshire, United Kingdom	ataxia telangiectasia	Phase II <a href="http://www.intrabio.com">www.intrabio.com</a>
JOT101 (trans-resveratrol oral)	Jupiter Neurosciences Palm Beach Gardens, FL	Friedreich's ataxia	Phase II <a href="http://www.jupiterneurosciences.com">www.jupiterneurosciences.com</a>
<b>Libervant™</b> diazepam buccal soluble film	Aquestive Therapeutics Warren, NJ	breakthrough seizures, cluster seizures (FastTrack)	application submitted <a href="http://www.aquestive.com">www.aquestive.com</a>
masitinib (tyrosine kinase inhibitor)	AB Science Paris, France	ALS	Phase III <a href="http://www.ab-science.com">www.ab-science.com</a>
mazindol	NLS-O Pharma Stans, Switzerland	narcolepsy	Phase II <a href="http://www.nlspharma.com">www.nlspharma.com</a>
MN-166 (ibudilast) (neuroprotective therapeutic)	MediciNova La Jolla, CA	ALS (Fast Track)	Phase III <a href="http://www.medicinova.com">www.medicinova.com</a>
MP101 (brain penetrant mitochondrial uncoupler)	Mitochon Pharmaceuticals Blue Bell, PA	Huntington's disease	Phase I <a href="http://www.mitochonpharma.com">www.mitochonpharma.com</a>



## Neurologic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>NeuroVax™</b> T cell receptor peptide vaccine	Immune Response BioPharma Atlantic City, NJ	multiple sclerosis (Fast Track)	Phase II/III <a href="http://www.immuneresponsebio.com">www.immuneresponsebio.com</a>
NPT520-34 (neuroinflammation)	Neuropore Therapies San Diego, CA	ALS	Phase I <a href="http://www.neuropore.com">www.neuropore.com</a>
<b>NurOwn®</b> neurotrophic factor-producing mesenchymal stem cell therapy	BrainStorm Cell Therapeutics New York, NY	ALS (Fast Track)	Phase III <a href="http://www.brainstorm-cell.com">www.brainstorm-cell.com</a>
omaveloxolone (RTA-408) (Nrf2 stimulant)	Reata Pharmaceuticals Plano, TX	Friedreich's ataxia	Phase III <a href="http://www.reatapharma.com">www.reatapharma.com</a>
OPC1 (oligodendrocyte progenitor stem cell therapy)	Lineage Cell Therapeutics Carlsbad, CA	spinal cord injury	Phase III <a href="http://www.lineagecell.com">www.lineagecell.com</a>
PBFT-02	Passage Bio Philadelphia, PA	frontotemporal dementia (Fast Track)	Phase I/II <a href="http://www.passagebio.com">www.passagebio.com</a>
pepinemab (anti-SEMA4D mAb)	Vaccinex Rochester, NY	Huntington's disease (Fast Track)	Phase II completed <a href="http://www.vaccinex.com">www.vaccinex.com</a>
PR-006	Prevail Therapeutics New York, NY	frontotemporal dementia (Fast Track)	Phase I/II <a href="http://www.prevailtherapeutics.com">www.prevailtherapeutics.com</a>

## Neurologic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
PRAX-562	Praxis Precision Medicines Boston, MA	epileptic encephalopathy, SCN2A developmental	Phase I <a href="http://www.praxismedicines.com">www.praxismedicines.com</a>
pridopidine (sigma-1 receptor agonist)	Prilenia Therapeutics Naarden, Netherlands	ALS	Phase II/III <a href="http://www.prilenia.com">www.prilenia.com</a>
		Huntington's disease (Fast Track)	Phase II <a href="http://www.prilenia.com">www.prilenia.com</a>
PrimeC (celecoxib/ciprofloxacin FDC)	NeuroSense Therapeutics Herzeliyz, Israel	ALS	Phase I <a href="http://www.neurosense-tx.com">www.neurosense-tx.com</a>
reboxetine	Axsome Therapeutics New York, NY	narcolepsy	Phase III <a href="http://www.axsome.com">www.axsome.com</a>
reldesemtiv (troponin stimulant)	Cytokinetics South San Francisco, CA	ALS	Phase III <a href="http://www.cytokinetics.com">www.cytokinetics.com</a>
RG6042 (tominersen) (RNA interference)	Ionis Pharmaceuticals Carlsbad, CA Roche/Genentech South San Francisco, CA	Huntington's disease	Phase III <a href="http://www.roche.com">www.roche.com</a>
RNS60 (intracellular signaling pathway activator)	Revalesio Tacoma, WA	ALS (Fast Track)	Phase II <a href="http://www.revalesio.com">www.revalesio.com</a>

## Neurologic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
RT001 (lipid peroxidation inhibitor)	Retrotope Los Altos, CA	Friedreich's ataxia (Fast Track)	Phase III <a href="http://www.retrotope.com">www.retrotope.com</a>
		progressive supranuclear palsy	Phase II <a href="http://www.retrotope.com">www.retrotope.com</a>
		infantile neuroaxonal dystrophy	Phase II/III <a href="http://www.retrotope.com">www.retrotope.com</a>
SLS-005 (trehalose) (protein aggregation inhibitor)	Seelos Therapeutics New York, NY	ALS	Phase II <a href="http://www.seelostherapeutics.com">www.seelostherapeutics.com</a>
sodium oxybate extended-release oral suspension	Avadel Pharmaceuticals Dublin, Ireland	narcolepsy	application submitted <a href="http://www.avadel.com">www.avadel.com</a>
SRX-246 (vasopressin 1a receptor antagonist)	Azevan Pharmaceuticals Bethlehem, PA	Huntington's disease	Phase II <a href="http://www.azevan.com">www.azevan.com</a>
TAK-925 (danavorexton) (orexin receptor type 2 agonist)	Takeda Deerfield, IL	narcolepsy	Phase I <a href="http://www.takeda.com">www.takeda.com</a>
troriluzole (BHV4157) (glutamate modulator)	Biohaven Pharmaceuticals New Haven, CT	spinocerebellar ataxia (Fast Track)	Phase III <a href="http://www.biohavenpharma.com">www.biohavenpharma.com</a>
TSHA-120 (AAV9-based gene therapy)	Taysha Gene Therapies Dallas, TX	giant axonal neuropathy	Phase I/II <a href="http://www.tayshagtx.com">www.tayshagtx.com</a>

## Neurologic Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
vatiquinone (NQO1 modulator)	PTC Therapeutics South Plainfield, NJ	Friedreich's ataxia (Fast Track)	Phase III <a href="http://www.ptcbio.com">www.ptcbio.com</a>
		mitochondrial epilepsy	Phase II/III <a href="http://www.ptcbio.com">www.ptcbio.com</a>
XEN-496 (ezogabine)	Xenon Pharmaceuticals Burnaby, Canada	KCNQ2 epileptic encephalopathy (Fast Track)	Phase III <a href="http://www.xenon-pharma.com">www.xenon-pharma.com</a>
XP-0863 (diazepam subcutaneous)	XERIS Pharmaceuticals Chicago, IL	acute repetitive seizures (Fast Track)	Phase I <a href="http://www.exrispharma.com">www.exrispharma.com</a>

## Respiratory Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>AeroFact</b> SF-RI-1 bovine surfactant for inhalation	Aerogen Pharma San Mateo, CA	respiratory distress syndrome (Fast Track)	Phase II <a href="http://www.aerogenpharma.com">www.aerogenpharma.com</a>
ARD-3150 (liposomal ciprofloxacin inhalation)	Aradigm Hayward, CA	non-cystic fibrosis bronchiectasis (Fast Track)	application submitted <a href="http://www.aradigm.com">www.aradigm.com</a>
ARV-1801 (protein synthesis inhibitor)	Arrevus Raleigh, NC	pulmonary exacerbations cystic fibrosis	Phase II <a href="http://www.arrevus.com">www.arrevus.com</a>

**Respiratory Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
brilaroxazine (RP5063) (serotonin and dopamine agonist)	Reviva Pharmaceuticals Cupertino, CA	idiopathic pulmonary fibrosis	Phase I <a href="http://www.revivapharma.com">www.revivapharma.com</a>
cudetaxestat (autotaxin Inhibitor)	Blade Therapeutics South San Francisco, CA	idiopathic pulmonary fibrosis	Phase I <a href="http://www.blademed.com">www.blademed.com</a>
<b>Esbriet®</b> pirfenidone	Roche/Genentech South San Francisco, CA	interstitial lung disease	application submitted <a href="http://www.roche.com">www.roche.com</a>
GB0139 (inhaled galectin 3 inhibitor)	Galecto Copenhagen, Denmark	idiopathic pulmonary fibrosis	Phase II <a href="http://www.galecto.com">www.galecto.com</a>
IM-156	ImmunoMet Therapeutics Houston, TX	idiopathic pulmonary fibrosis (Fast Track)	Phase I <a href="http://www.immunomet.com">www.immunomet.com</a>
inhaled nitric oxide/INOpulse®	Bellerophon Therapeutics Warren, NJ	idiopathic pulmonary fibrosis	Phase III <a href="http://www.bellerophon.com">www.bellerophon.com</a>
INO-3107	Inovio Pharmaceuticals Plymouth Meeting, PA	recurrent respiratory papillomatosis	Phase II <a href="http://www.inovio.com">www.inovio.com</a>
liposomal cyclosporin A	Zambon Cambridge, MA	bronchiolitis obliterans (Fast Track)	Phase III <a href="http://www.zambonpharma.com">www.zambonpharma.com</a>
LTI-03	Lung Therapeutics Austin, TX	idiopathic pulmonary fibrosis	Phase I <a href="http://www.lungtx.com">www.lungtx.com</a>

## Respiratory Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
MN-001 (tipelukast) (oral anti-inflammatory)	MediciNova la Jolla, CA	idiopathic pulmonary fibrosis (Fast Track)	Phase II <a href="http://www.medicinova.com">www.medicinova.com</a>
PLN-74809	Pliant Therapeutics South San Francisco, CA	idiopathic pulmonary fibrosis	Phase II <a href="http://www.pliantrx.com">www.pliantrx.com</a>
PRGN-2012	Precigen Germantown, MD	recurrent respiratory papillomatosis	Phase I <a href="http://www.precigen.com">www.precigen.com</a>
RG6354 (pentraxin-2/serumamyloid P)	Roche/Genentech South San Francisco, CA	idiopathic pulmonary fibrosis	Phase II <a href="http://www.roche.com">www.roche.com</a>
<b>Teton</b> inhaled treprostinil	United Therapeutics Silver Spring, MD	idiopathic pulmonary fibrosis	Phase III <a href="http://www.unither.com">www.unither.com</a>

## Skin Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
allo-APZ2-EB (cell replacement therapy)	RHEACELL Heidelberg, Germany	recessive dystrophic epidermolysis bullosa	Phase I/II <a href="http://www.rheacell.com">www.rheacell.com</a>
APZ-2	RHEACELL Heidelberg, Germany	epidermolysis bullosa	Phase I/II <a href="http://www.rheacell.com">www.rheacell.com</a>

## Skin Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
beremagene geperpavec (B-VEC) (gene therapy)	Krystal Biotech Pittsburgh, PA	epidermolysis bullosa (Fast Track)	Phase III <a href="http://www.krystalbio.com">www.krystalbio.com</a>
BPM 31510 (ubidecarenone)	Berg Framingham, MA	epidermolysis bullosa	Phase I <a href="http://www.berghealth.com">www.berghealth.com</a>
DSG3-CAART (DSG3 CAAR-T cell therapy)	Cabaletta Bio Philadelphia, PA	pemphigus vulgaris (Fast Track)	Phase I <a href="http://www.cabalettabio.com">www.cabalettabio.com</a>
EB-101 (collagen type VII replacement)	Abeona Therapeutics New York, NY	recessive dystrophic epidermolysis bullosa (Breakthrough Therapy) (Fast Track)	Phase III <a href="http://www.abeonatherapeutics.com">www.abeonatherapeutics.com</a>
FCX-007 (dabocemagene autoficel) (type VII collagen)	Castle Creek Biosciences Exton, PA	epidermolysis bullosa (Fast Track)	Phase III <a href="http://www.castlecreekbio.com">www.castlecreekbio.com</a>
<b>Filsuvez</b> <sup>®</sup> birch triterpenes	Amryt Pharma Boston, MA	epidermolysis bullosa (Fast Track)	application submitted <a href="http://www.amrytpharma.com">www.amrytpharma.com</a>
imsidolimab	AnaptysBio San Diego, CA	generalized pustular psoriasis	Phase III <a href="http://www.anaptysbio.com">www.anaptysbio.com</a>
MT-7117	Mitsubishi Tanabe Pharma Jersey City, NJ	cutaneous variants of porphyria (Fast Track)	Phase III <a href="http://www.mt-pharma.co.jp">www.mt-pharma.co.jp</a>

**Skin Disorders**  
**Product Name**

**Sponsor**

**Indication**

**Development Phase**

<b>NexoBrid</b> bromelain topical	MediWound Yavne, Israel	eschar removal of deep partial and full thickness burns	application submitted <a href="http://www.mediwound.com">www.mediwound.com</a>
nomacopan	Akari Therapeutics New York, NY	bullous pemphigoid (Fast Track)	Phase III <a href="http://www.akaritx.com">www.akaritx.com</a>
PTR-01 (BBP-589) (recombinant collagen)	Phoenix Tissue Repair Boston, MA	recessive dystrophic epidermolysis bullosa (Fast Track)	Phase II <a href="http://www.phoenixtissuerepair.com">www.phoenixtissuerepair.com</a>
<b>QTORIN™</b> rapamycin 3.9%	Palvella Therapeutics Wayne, PA	pachyonychia congenita (Fast Track)	Phase III <a href="http://www.palvellatx.com">www.palvellatx.com</a>
RGN-137 topical (thymosin beta-4)	Lenus Therapeutics Princeton, NJ	epidermolysis bullosa	Phase II <a href="http://www.gtreetbnt.com">www.gtreetbnt.com</a>
spesolimab	Boehringer Ingelheim Pharmaceuticals Ridgefield, CT	generalized pustular psoriasis	Phase II <a href="http://www.boehringer-ingelheim.com">www.boehringer-ingelheim.com</a>
STP705 (siRNA therapeutic)	Sirnaomics Gaithersburg, MD	keloids	Phase II <a href="http://www.sirnaomics.com">www.sirnaomics.com</a>
TMB-001 (isotretinoin topical)	Timber Pharmaceuticals Woodcliff Lake, NJ	congenital ichthyosiform erythroderma	Phase II <a href="http://www.patagoniarx.com">www.patagoniarx.com</a>
<b>XENO-Skin™</b> (genetically engineered skin graft)	XenoTherapeutics Boston, MA	deep full-thickness burn injury	Phase I/II <a href="http://www.xenotherapeutics.com">www.xenotherapeutics.com</a>



<b><u>Transplantation Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
AB-205-S (stem cell therapy)	Angiocrine Bioscience San Diego, CA	prevention of morbidity and mortality associated with hematopoietic stem cell transplantation	Phase I/II <a href="http://www.angiocrinebioscience.com">www.angiocrinebioscience.com</a>
AMG592	Amgen Thousand Oaks, CA	graft-versus-host-disease	Phase I <a href="http://www.amgen.com">www.amgen.com</a>
ANG-3777 (HGF mimetic)	Angion Biomedica Uniondale, NY	improve renal function and prevent delayed graft function in renal transplantaion (Fast Track)	Phase III <a href="http://www.angion.com">www.angion.com</a>
axatilimab (SNDX-6352) (CSF-1R mAb)	Syndax Pharmaceuticals Waltham, MA	graft-versus-host disease	Phase II <a href="http://www.syndax.com">www.syndax.com</a>
clazakizumab	CSL Behring King of Prussia, PA	chronic antibody-mediated rejection in kidney transplant recipients	Phase III <a href="http://www.cslbehring.com">www.cslbehring.com</a>
F-652 (rhIL022 dimer)	Evive Biotech Bridgewater, NJ	graft-versus-host disease	Phase II <a href="http://www.evivebiotech.com">www.evivebiotech.com</a>
FCR001 (allogeneic cell therapy)	Talaris Therapeutics Wellesley, MA	renal transplant rejection	Phase III <a href="http://www.talaristx.com">www.talaristx.com</a>
human Alpha1-proteinase inhibitor	CSL Behring King of Prussia, PA	graft versus host disease	Phase II/III <a href="http://www.cslbehring.com">www.cslbehring.com</a>

<u>Transplantation Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
itacitinib	Incyte Wilmington, DE	graft versus host disease	Phase I/II <a href="http://www.incyte.com">www.incyte.com</a>
itolizumab	Equillum, Inc. San Diego, CA	acute graft versus host disease	Phase II <a href="http://www.equilibio.com">www.equilibio.com</a>
JSP191 (anti-CD117 mAb)	Jasper Therapeutics Redwood City, CA	hematopoietic stem cell transplantation	Phase I <a href="http://www.jaspertherapeutics.com">www.jaspertherapeutics.com</a>
leronlimab (anti-CDR6 mAb)	CytoDyn Vancouver, WA	prevention of graft versus host disease	Phase II <a href="http://www.cytodyn.com">www.cytodyn.com</a>
MDR101	Medeor Therapeutics South San Francisco, CA	prevention of graft rejection following solid organ transplantation	Phase III <a href="http://www.medeortx.com">www.medeortx.com</a>
MGTA-145 (truncated CXC motif chemokine 2)	Magenta Therapeutics Cambridge, MA	mobilization of hematopoietic stem cells to the peripheral blood for collection and subsequent transplant	Phase II <a href="http://www.magentatx.com">www.magentatx.com</a>
<b>Multistem</b> <sup>®</sup> allogeneic stem cell therapy	Athersys Cleveland, OH	graft versus host disease	Phase II <a href="http://www.athersys.com">www.athersys.com</a>
narsoplimab (MASP-2 inhibitor)	Omeros Seattle, WA	hematopoietic stem-cell transplant associated thrombotic microangiopathy	Phase III <a href="http://www.omeros.com">www.omeros.com</a>

<b><u>Transplantation Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
neihulizumab	AltruBio Redwood City, CA	acute graft-versus-host disease (Fast Track)	Phase I <a href="http://www.altrubio.com">www.altrubio.com</a>
PLX-R18 (ex-vivo expanded stromal cells)	Pluristem Haifa, Israel	treatment graft failure and incomplete hematopoietic recovery, following hematopoietic cell transplantation	Phase I <a href="http://www.pluristem.com">www.pluristem.com</a>
Pro-Ocular (PG-101) (progesterone)	GLIA Boston, MA	ocular graft versus host disease	Phase II <a href="http://www.gliacell.com">www.gliacell.com</a>
RGI-2001 (alpha galactosylceramide liposomal)	Regimmune Taipei, Taiwan	graft-versus-host disease	Phase II <a href="http://www.regimmune.com">www.regimmune.com</a>
rivo-cel (allogeneic polydonal T-cell therapy)	Bellicum Pharmaceuticals Houston, TX	immunodeficiency and graft versus host disease after hematopoietic stem cell transplantation	Phase I/II completed <a href="http://www.bellicum.com">www.bellicum.com</a>
siplizumab	ITB-MED New York, NY	prevention of solid organ transplant rejection (Fast Track)	Phase II <a href="http://www.itb-med.com">www.itb-med.com</a>
TFFTAC-LAC (tacrolimus)	TFF Pharmaceuticals Austin, TX	prevention of lung allograft rejection	Phase I <a href="http://www.tfpharma.com">www.tfpharma.com</a>

**Other Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
AAV-AQP1 (gene therapy)	MeiraGTx New York, NY	radiation-induced xerostomia	Phase I <a href="http://www.meiragtx.com">www.meiragtx.com</a>
ABBV-4083	AbbVie North Chicago, IL	onchocerciasis	Phase II <a href="http://www.abbvie.com">www.abbvie.com</a>
ACER-001	Acer Therapeutics Cambridge, MA	maple syrup urine disease	Phase I <a href="http://www.acertx.com">www.acertx.com</a>
avexitide	Eiger Biopharmaceuticals Palo Alto, CA	post-bariatric hypoglycemia (Breakthrough Therapy)	Phase II <a href="http://www.eigerbio.com">www.eigerbio.com</a>
AVTX-006 (dual mTOR inhibitor)	Avalo Therapeutics Wayne, PA	complex lymphatic malformations	Phase I <a href="http://www.avalotx.com">www.avalotx.com</a>
BIO-300 (genistein nanoparticle)	Humanetics Minneapolis, MN	acute radiation injury (Fast Track)	Phase II <a href="http://www.humaneticscorp.com">www.humaneticscorp.com</a>
birtamimab (immunoglobulin G1)	Prothena Biosciences South San Francisco, CA	amyloid light-chain amyloidosis (Fast Track)	Phase III <a href="http://www.prothena.com">www.prothena.com</a>
budesonide controlled release	Calliditas Therapeutics Stockholm, Sweden	IgA nephropathy	application submitted <a href="http://www.calliditas.se">www.calliditas.se</a>
BV201 (terlipressin)	BioVie Santa Monica, CA	ascites not due to cancer	Phase II <a href="http://www.bioviepharma.com">www.bioviepharma.com</a>

**Other Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
CAEL-101 (fibril-reactive mAb)	Alexion AstraZeneca Gene Therapy Cambridge, MA Caelum Biosciences Bordertown, NJ	amyloid light-chain amyloidosis (Fast Track)	Phase III <a href="http://www.alexion.com">www.alexion.com</a>
choline chlorine IV (phospholipid substrate replacement)	Protara Therapeutics New York, NY	intestinal failure associated liver disease (Fast Track)	Phase II <a href="http://www.protaratx.com">www.protaratx.com</a>
dehydrated alcohol injection	Eton Pharmaceuticals Deer Park, IL	methanol poisoning	application submitted <a href="http://www.etonpharma.com">www.etonpharma.com</a>
encaleret sulfate (Ca sensing receptor antagonist)	Calcilytix Therapeutics San Francisco, CA	autosomal dominant hypocalcemia type 1 (Fast Track)	Phase II <a href="http://www.calcilytix.com">www.calcilytix.com</a>
etelcalcetide	Amgen Thousand Oaks, CA	pediatric hyperparathyroidism	Phase III <a href="http://www.amgen.com">www.amgen.com</a>
<b>Eylea</b> <sup>®</sup> aflibercept	Regeneron Pharmaceuticals Tarrytown, NY Sanofi Bridgewater, NJ	retinopathy of prematurity	Phase III <a href="http://www.regeneron.com">www.regeneron.com</a> <a href="http://www.sanofi.com">www.sanofi.com</a>
<b>Gazyva</b> <sup>®</sup> obinutuzumab	Roche/Genetech South San Francisco, CA	membranous nephropathy	Phase III <a href="http://www.roche.com">www.roche.com</a>
GC4419 (avasopasem) (superoxide dismutase mimetic)	Galera Therapeutics Malvern, PA	oral mucositis	Phase III <a href="http://www.galeratx.com">www.galeratx.com</a>

## Other Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
glucagon (ready-to-use)	Xeris Pharmaceuticals Chicago, IL	hyperinsulinemic hypoglycemia	Phase II <a href="http://www.xerispharma.com">www.xerispharma.com</a>
<b>Granexin® Gel</b>	FirstString Research Mount Pleasant, SC	cutaneous radiation injury due to radiation disaster	Phase III <a href="http://www.firststringresearch.com">www.firststringresearch.com</a>
IVMED-80 (copper sulfate)	iVeena Drug Delivery Systems Salt Lake City, UT	keratoconus	Phase I/II <a href="http://www.iveenamed.com">www.iveenamed.com</a>
levoketoconazole (adrenal steroidogenesis inhibitor)	Strongbridge Biopharma Trevose, PA	Cushing syndrome	Phase III <a href="http://www.strongbridge.com">www.strongbridge.com</a>
LPCN 1107 (hydroxyprogesterone)	Lipocine Salt Lake City, UT	prevention of recurrent preterm labor	Phase II <a href="http://www.lipocine.com">www.lipocine.com</a>
maralixibat	Mirum Pharmaceuticals Foster City, CA	progressive familial intrahepatic cholestasis	Phase III <a href="http://www.mirumpharma.com">www.mirumpharma.com</a>
		biliary atresia	Phase II <a href="http://www.mirumpharma.com">www.mirumpharma.com</a>
masitinib (tyrosine kinase inhibitor)	AB Science Paris, France	mastocytosis	Phase III <a href="http://www.ab-science.com">www.ab-science.com</a>
mavrilimumab	Kiniksa Pharmaceuticals Lexington, MA	giant cell arteritis	Phase II <a href="http://www.kiniksa.com">www.kiniksa.com</a>

**Other Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
methotrexate	Aldeyra Therapeutics Lexington, MA	proliferative vitreoretinopathy (Fast Track)	Phase III <a href="http://www.aldetra.com">www.aldetra.com</a>
NaBen (benzoate)	SyneuRx Taipei City, Taiwan	pediatric schizophrenia	Phase II/III <a href="http://www.syneurx.com">www.syneurx.com</a>
narsoplimab	Omeros Seattle, WA	IgA nephropathy (Breakthrough Therapy)	Phase III <a href="http://www.omeros.com">www.omeros.com</a>
Nexagon (antisense oligonucleotide)	OcuNexus Therapeutics San Diego, CA	corneal persistent epithelial injuries	Phase II <a href="http://www.ocunexus.com">www.ocunexus.com</a>
nirogacestat (gamma secretase inhibitor)	SpringWorks Stamford, CT	desmoid tumor (aggressive fibromatosis)	Phase III <a href="http://www.springworkstx.com">www.springworkstx.com</a>
NTLA-2001 (CRISPR/ Cas9-based gene therapy)	Intellia Therapeutics Cambridge, MA Regeneron Tarrytown, NY	transthyretin amyloidosis	Phase I <a href="http://www.intelliatx.com">www.intelliatx.com</a>
PA101B (sodium cromoglicate inhalant)	Respivant Sciences San Diego, CA	mastocytosis	Phase II
PCS-499 deuterated S-lisofylline	Processa Pharmaceuticals Hanover, MD	necrobiosis lipoidica	Phase II <a href="http://www.processapharmaceuticals.com">www.processapharmaceuticals.com</a>

**Other Diseases**

<b><u>Product Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
PLX-R18 (ex-vivo stromal cell therapy)	Pluristem Haifa, Israel	acute radiation syndrome	Phase II <a href="http://www.pluristem.com">www.pluristem.com</a>
posoleucel (ALVR105) (HLA-matched multi virus T-cell therapy)	AlloVir Waltham, MA	virus-associated hemorrhagic cystitis	Phase III <a href="http://www.allovir.com">www.allovir.com</a>
<b>QTORIN™</b> rapamycin 3.9%	Palvella Therapeutics Wayne, PA	microcystic lymphatic malformations	Phase II <a href="http://www.palvellatx.com">www.palvellatx.com</a>
relacorilant	Corcept Therapeutics Menlo Park, CA	endogenous Cushing syndrome	Phase III <a href="http://www.corcept.com">www.corcept.com</a>
RGN-259 eye drops (thymosin beta-4)	ReGenRx Rockville, MD	neurotrophic keratopathy	Phase III <a href="http://www.regenrx.com">www.regenrx.com</a>
<b>RiVax®</b> (ricin vaccine)	Soligenix Princeton, NJ	prevention of ricin poisoning (Fast Track)	Phase I <a href="http://www.soligenix.com">www.soligenix.com</a>
RZL-012 (lipolysis modulator)	Raziel Therapeutics Rehovot, Israel	Dercum's disease (adiposis dolorosa)	Phase II <a href="http://www.raziel-therapy.com">www.raziel-therapy.com</a>
SGX201 (beclomethasone)	Soligenix Princeton, NJ	radiation enteritis	Phase I <a href="http://www.soligenix.com">www.soligenix.com</a>



## Other Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
sirolimus ophthalmic	Santen Emeryville, CA	noninfectious uveitis	Phase III <a href="http://www.santen.com">www.santen.com</a>
SNF472 (hexasodium phytate)	Sanifit Therapeutics Palma, Spain	calciophylaxis	Phase III <a href="http://www.sanifit.com">www.sanifit.com</a>
sodium thiosulfate (STS)	Fennec Pharmaceuticals Durham, NC	platinum-induced ototoxicity (pediatric)	application submitted <a href="http://www.fennecpharma.com">www.fennecpharma.com</a>
sparsentan	Traverse Therapeutics San Diego, CA	IgA nephropathy	Phase III <a href="http://www.traverse.com">www.traverse.com</a>
		----- focal segmental glomerulosclerosis	Phase II <a href="http://www.traverse.com">www.traverse.com</a>
STAT-600 (entolimod) (TLR5 agonist)	Statera BioPharma Fort Collins, CO	acute radiation syndrome (Fast Track)	Phase I <a href="http://www.staterabiopharma.com">www.staterabiopharma.com</a>
tadekinig alfa (IL-18 inhibitor)	AB2 Bio Lausanne, Switzerland	hemophagocytic lymphohistiocytosis (Breakthrough Therapy)	Phase III <a href="http://www.ab2bio.com">www.ab2bio.com</a>
TARA-002 (picibanil biosimilar)	Protara Therapeutics New York, NY	lymphatic malformations	Phase II <a href="http://www.protaratx.com">www.protaratx.com</a>
varespladib	Ophirex Corte Madera, CA	snakebite	Phase II <a href="http://www.ophirex.com">www.ophirex.com</a>

## Other Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
vutrisiran	Alnylam Pharmaceuticals Cambridge, MA	ATTR amyloidosis (Fast Track)	application submitted <a href="http://www.alnylam.com">www.alnylam.com</a>

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 30, 2021.** The medicines in this listing include medicines being developed by U.S.-based companies conducting trials in the United States and 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](http://www.clinicaltrials.gov). The entire series of *Medicines in Development* is available on PhRMA's website: [www.phrma.org](http://www.phrma.org).

## Definitions

**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.