

2016 Medicines in Development for Rare Diseases

A LIST OF ORPHAN DRUGS IN THE PIPELINE

Autoimmune Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
Actemra® tocilizumab	Genentech South San Francisco, CA	treatment of systemic sclerosis	Phase III www.gene.com
Adempas® riociguat	Bayer HealthCare Pharmaceuticals Whippany, NJ	treatment of systemic sclerosis	Phase II www.pharma.bayer.com
ARA 290	Araim Pharmaceuticals Tarrytown, NY	treatment of neuropathic pain in patients with sarcoidosis	Phase II www.ariampharma.com
ARG201 (type 1 native bovine skin collagen)	arGentis Pharmaceuticals Collierville, TN	treatment of diffuse systemic sclerosis	Phase II www.argentisrx.com
BYM338 (bimagrumab)	Novartis Pharmaceuticals East Hanover, NJ	treatment of inclusion body myositis	Phase III www.novartis.com
CCX168 (5a receptor antagonist)	ChemoCentryx Mountain View, CA	treatment of anti-neutrophil cytoplasmic auto-antibodies associated vasculitides (granulomatosis with polyangitis or Wegener's granulomatosis), microscopic polyangitis, and Churg-Strauss syndrome	Phase II www.chemocentryx.com

* This designation is issued by the FDA's Office of Orphan Products Development while the drug is still in development. The designation makes the sponsor of the drug eligible for entitlements under the Orphan Drug Act of 1983. The entitlements include seven years of marketing exclusivity following FDA approval of the drug for the designated use.

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deflazacort	Marathon Pharmaceuticals Northbrook, IL	treatment of pediatric (0 through 16 years of age) juvenile idiopathic arthritis (JIA) International League of Associations for Rheumatology (ILAR) categories excluding systemic JIA	Phase III www.marathonpharma.com
Firdapse® amifampridine	Catalyst Pharmaceuticals Coral Gables, FL	treatment of Lambert-Eaton myasthenia syndrome (Breakthrough Therapy)	application submitted www.catalystpharma.com
fostamatinib disodium (Syk kinase inhibitor)	Rigel Pharmaceuticals South San Francisco, CA	treatment of immune thrombocytopenic purpura	Phase III www.rigel.com
Gilenya® fingolimod	Novartis Pharmaceuticals East Hanover, NJ	treatment of chronic inflammatory demyelinating polyneuropathy	Phase III www.novartis.com
Hyqvia immune globulin infusion 10% (human) with recombinant human hyaluronidase	Baxalta Bannockburn, IL	treatment of chronic inflammatory demyelinating polyneuropathy	Phase III www.baxalta.com
MEDI-551 (anti-CD19 mAb)	MedImmune Gaithersburg, MD	treatment of neuromyelitis optica and neuromyelitis optica spectrum disorders	Phase II www.medimmune.com
mepolizumab	GlaxoSmithKline Research Triangle Park, NC	treatment of Churg-Strauss syndrome	Phase III www.gsk.com

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NGM282 (rhfibroblast growth factor 20)	NGM Biopharmaceuticals South San Francisco, CA	treatment of primary biliary cirrhosis (Fast Track)	Phase II www.ngmbio.com
obeticholic acid (FXR agonist)	Intercept Pharmaceuticals New York, NY	treatment of primary biliary cirrhosis (Fast Track)	application submitted www.interceptpharma.com
Otezla® apremilast	Celgene Summit, NJ	treatment of Bechet's disease	Phase III www.celgene.com
PRTX-100 (staphylococcal aureus protein A)	Protalex Florham Park, NJ	treatment of immune thrombocytopenic purpura	Phase I/II www.protalex.com
Resunab ajulemic acid	Corbus Pharmaceuticals Norwood, MA	treatment of systemic sclerosis (Fast Track)	Phase II www.corbuspharma.com
Rituxan® rituximab	Genentech South San Francisco, CA	treatment of pemphigus vulgaris	Phase III www.gene.com
SA237 (anti-IL-6R receptor mAb)	Chugai Pharma USA Berkeley Heights, NJ	treatment of neuromyelitis optica and neuromyelitis optica spectrum disorder	Phase III www.chugai-pharm.com
SHP625 (sodium bile cotransporter inhibitor)	Shire Lexington, MA	treatment of primary biliary cirrhosis	Phase II www.shire.com

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Simponi® Aria® golimumab	Janssen Biotech Horsham, PA	treatment of polyarticular juvenile idiopathic arthritis in pediatric patients (0 through 16 years of age)	Phase III www.janssenbiotech.com
Soliris® eculizumab	Alexion Pharmaceuticals New Haven, CT	treatment of myasthenia gravis	Phase III www.alexion.com
		treatment of neuromyelitis optica	Phase III www.alexion.com
teprotumumab	River Vision New York, NY	treatment of active (dynamic) phase Grave's orbitopathy	Phase II
veltuzumab subcutaneous (anti-CD20 antigen inhibitor)	Immunomedics Morris Plains, NJ	treatment of immune thrombocytopenic purpura	Phase I/II www.immunomedics.com

Blood Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
ALX-0081 (caplacizumab)	Ablynx Ghent, Belgium	treatment of thrombotic thrombocytopenic purpura	Phase III www.ablynx.com
andexanet alfa (blood coagulation stimulant)	Portola Pharmaceuticals South San Francisco, CA	for reversing the anticoagulant effect of direct or indirect factor Xa inhibitors in patients experiencing a serious uncontrolled bleeding event or who require urgent or emergent surgery	application submitted www.portola.com

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APL-2 subcutaneous	Apellis Pharmaceuticals Crestwood, KY	treatment of paroxysmal nocturnal hemoglobinuria	Phase I www.apellis.com
BAX-930 (recombinant disintegrin and metalloprotease)	Baxalta Bannockburn, IL	prevention of thrombotic thrombocytopenic purpura including its congenital, acquired idiopathic and secondary forms	Phase I www.baxalta.com
E5501 (avatrombopag)	Eisai Woodcliff Lake, NJ	treatment of idiopathic thrombocytopenic purpura	Phase III www.eisai.com
Jakafi® ruxolitinib	Incyte Wilmington, DE	treatment of essential thrombocythemia	Phase III www.incyte.com
P1101 (pegylated proline interferon alpha-2b)	PharmaEssentia Taipei, Taiwan	treatment of essential thrombocythemia	Phase III www.pharmaessentia.com
PCO371 (parathyroid hormone receptor type I agonist)	Chugai Pharma USA Berkeley Heights, NJ	treatment of hypoparathyroidism	Phase I www.chugai-pharma.com
sotatercept	Acceleron Pharma Cambridge, MA Celgene Summit, NJ	treatment of anemias associated with myelodysplastic syndrome and myelodysplastic/myeloproliferative neoplasms	Phase II www.acceleron.com www.celgene.com

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ABT-414 (EGFR ADC)	AbbVie North Chicago, IL	treatment of glioblastoma multiforme	Phase II www.abbvie.com
Ad-RTS-IL-12	Ziopharm Oncology Boston, MA	treatment of malignant glioma	Phase I www.ziopharm.com
ADXS11-001	Advaxis Princeton, NJ	treatment of HPV-positive associated anal cancer	Phase II www.advaxis.com
aglatimagene besadenovec (AdV-TK gene therapy)	Advantagene Auburndale, MA	treatment of malignant brain tumors	Phase II www.advantagene.com
AL3818 (anlotinib hydrochloride)	Advenchen Laboratories Moorpark, CA	treatment of ovarian cancer	Phase I/II www.advenchen.com
aldoxorubicin	CytRx Los Angeles, CA	treatment of soft tissue sarcoma	Phase III www.cytrx.com
		treatment of adenocarcinoma of the pancreas	Phase II www.cytrx.com
		treatment of glioblastoma multiforme	Phase II www.cytrx.com
		treatment of small cell lung cancer	Phase II www.cytrx.com

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algenpantucel-L	NewLink Genetics Ames, IA	treatment of pancreatic cancer (Fast Track)	Phase III www.linkp.com
alisertib	Millennium Pharmaceuticals Cambridge, MA	treatment of small cell lung cancer	Phase II www.millennium.com
anetumab ravtansine (anti-mesothelin ADC)	Bayer HealthCare Pharmaceuticals Whippany, NJ	treatment of mesothelioma	Phase II www.pharma.bayer.com
ANG1005 (paclitaxel trevatide)	Angiochem Montreal, Canada	treatment of glioblastoma multiforme	Phase II www.angiochem.com
		treatment of breast cancer patients with brain metastases	Phase II www.angiochem.com
antineoplaston A10/ antineoplaston AS2-2	Burzynski Research Institute Houston, TX	treatment of gliomas	Phase II www.burzynskiresearch.com
antroquinonol	Golden Biotechnology Taipei City, Taiwan	treatment of pancreatic cancer	Phase II www.goldenbiotech.com
Arenegyr NGF-hTNF	MolMed Milan, Italy	treatment of malignant pleural mesothelioma	Phase III www.molmed.com
ARQ-087	ArQule Burlington, MA	treatment of cholangiocarcinoma	Phase II www.arqule.com

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ATR-01 (ACAT1 inhibitor)	Millendo Therapeutics Ann Arbor, MI	treatment of adrenocortical carcinoma	Phase I www.millendo.com
avelumab (PF-06834635)	EMD Serono Rockland, MA Pfizer New York, NY	treatment of merkel cell carcinoma (Breakthrough Therapy) (Fast Track)	Phase II www.emdserono.com www.pfizer.com
axalimogen filolisbac	Advaxis Princeton, NJ	treatment of stage II to IV invasive cervical carcinoma	Phase II www.advaxis.com
		treatment of human papilloma virus- associated head and neck cancer	Phase II www.advaxis.com
AZD-1775 (WEE1 tyrosine kinase inhibitor)	AstraZeneca Wilmington, DE	treatment of ovarian cancer	Phase II www.astrazeneca.com
Azedra™ iobenguane I-131	Progenics Pharmaceuticals Tarrytown, NY	treatment of neuroendocrine tumors (Fast Track)	Phase II www.progenics.com
Azixa® verobulin	Immune Pharmaceuticals New York, NY	treatment of glioblastoma multiforme	Phase II www.immunepharma.com
BEL-0222 (firtecen pegol)	Belrose Pharmaceuticals Princeton, NJ	treatment of neuroblastoma	Phase I/II www.belrosepharma.com
binimetinib/MEK162	Array BioPharma Boulder, CO	treatment stage IIB-IV melanoma	Phase III www.arraybiopharma.com

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BLU-285 (exon 17 mutant KIT inhibitor)	Blueprint Medicines Cambridge, MA	treatment of gastrointestinal stromal tumors (GIST)	Phase I www.blueprintmedicines.com
BLU-554 (FGFR4 inhibitor)	Blueprint Medicines Cambridge, MA	treatment of hepatocellular cancer (HCC)	Phase I www.blueprintmedicines.com
BLZ-100	Blaze Bioscience Seattle, WA	diagnostic for the management of malignant brain tumors	Phase I www.blazebioscience.com
CA4P (fosbretabulin)	OXiGENE South San Francisco, CA	treatment of neuroendocrine tumors	Phase II www.oxigene.com
		treatment of ovarian cancer	Phase II www.oxigene.com
Cavatak™ coxsackievirus	Viralytics Sydney, Australia	treatment of stage II (T4), stage III, and stage IV melanoma	Phase II www.viralytics.com
CLR-1404I-124	Collectar Biosciences Madison, WI	diagnostic for the management of glioma	Phase II www.collectarbiosciences.com
CMB305 (G305/LV305 combination)	Immune Design Seattle, WA	treatment of soft tissue sarcoma	Phase II www.immunedesign.com
Cotara® TNT-1B mAb	Peregrine Pharmaceuticals Tustin, CA	treatment of glioblastoma multiforme and anaplastic astrocytoma	Phase II www.peregrineinc.com

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COTI-2	Critical Outcome Technologies Ontario, Canada	treatment of ovarian cancer	Phase I www.criticaloutcome.com
CPI-613	Cornerstone Pharmaceuticals Cranbury, NJ	treatment of pancreatic cancer	Phase I/II www.cornerstonepharma.com
CPP-1X (eflornithine)	Cancer Prevention Pharmaceutical Tucson, AZ	treatment of neuroblastoma	Phase II www.canprevent.com
		treatment of gastric cancer	Phase I www.canprevent.com
crenolanib	AROG Pharmaceuticals Dallas, TX	treatment of malignant glioma	Phase I www.arogpharma.com
cridanimod	Kevelt Tallinn, Estonia	treatment of progesterone receptor negative endometrial cancer in conjunction with progesterone therapy	Phase II www.kevelt.ee/en
CRLX101 (poly-CD-PEG-camptothecin)	Cerulean Pharma Waltham, MA	treatment of ovarian cancer	Phase II www.ceruleanrx.com

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CRS-207/GVAX (listeria monocytogenes)	Aduro BioTech Berkeley, CA	treatment of pancreatic cancer with GVAX	Phase II www.aduro.com
		treatment of pancreatic cancer with GVAX + antiPD-1	Phase II www.aduro.com
		treatment of mesothelioma	Phase I www.aduro.com
Cyramza® ramucirumab	Eli Lilly Indianapolis, IN	treatment of hepatocellular carcinoma	Phase III www.lilly.com
dacomitinib	Pfizer New York, NY	treatment of non-small cell lung cancer with EGFR, HER2, HER4, or DDR2 mutations	Phase III www.pfizer.com
DCC-2618 (KIT/PDGFR kinase inhibitor)	Deciphera Pharmaceuticals Waltham, MA	treatment of gastrointestinal stromal tumors (GIST)	Phase I www.deciphera.com
DCVax®-L dendritic cell immunotherapy	Northwest Biotherapeutics Bethesda, MD	treatment of primary brain malignant cancer	Phase III www.nwbio.com
defactinib (VS-6063)	Verastem Needham, NC	treatment of ovarian cancer	Phase I www.verastem.com
demcizumab	OncoMed Pharmaceuticals Redwood City, CA	treatment of pancreatic cancer	Phase II www.oncomed.com

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DNX-2401 (adenovirus delta 24-RGD)	DNAtrix Houston, TX	treatment of glioma (Fast Track)	Phase I www.dnatrx.com
DPX-Survivac cancer peptide vaccine	Immunovaccine Technologies Halifax, Canada	treatment of ovarian cancer	Phase I/II www.imvaccine.com
encorafenib/LGX818	Array BioPharma Boulder, CO	treatment of stage IIB-IV melanoma positive for BRAF mutation	Phase III www.arraybiopharma.com
ENMD-2076 (Aurora A/angiogenic kinase inhibitor)	CASI Pharmaceuticals Rockville, MD	treatment of hepatocellular carcinoma	Phase I www.casipharma.com
ensitiximab	Precision Biologics Dallas, TX	treatment of pancreatic cancer	Phase II www.precision-biologics.com
entrectinib	Ignyta San Diego, CA	treatment of TrkA-positive, TrkB-positive, TrkC-positive, ROS1-positive, or ALK-positive colorectal cancer	Phase II www.ignyta.com
		treatment of TrkA-positive, TrkB-positive, TrkC-positive ROS1-positive or ALK-positive non-small cell lung cancer	Phase II www.ignyta.com
		treatment of neuroblastoma	Phase I www.ignyta.com

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epacadostat	Incyte Wilmington, DE	treatment of stage IIB-IV melanoma	Phase II www.incyte.com
EPZ-6438 (tazemetostat)	Epizyme Cambridge, MA	treatment of malignant rhabdoid tumors (MRTs)	Phase II www.epizyme.com
ERC-1671	Epitopoietic Research Pasadena, CA	treatment of glioma	Phase II www.erc-usa.com
etirinotecan pegol (NKTR-102)	Nektar Therapeutics San Francisco, CA	treatment of ovarian cancer	Phase II www.nektar.com
farletuzumab (MORAb-003)	Eisai Woodcliff Lake, NJ	treatment of ovarian cancer	Phase III www.eisai.com
FPA008 (CSF1R antibody)	Five Prime Therapeutics South San Francisco, CA	treatment of pigmented villonodular synovitis and tenosynovial giant cell tumor	Phase I www.fiveprime.com
galunisertib (TGF-beta1 kinase inhibitor)	Eli Lilly Indianapolis, IN	treatment of hepatocellular carcinoma	Phase II www.lilly.com
		treatment of glioma	Phase I/II www.lilly.com

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Gedepin fludarabine phosphate	PNP Therapeutics Birmingham, LA	intratumoral treatment of anatomically accessible oral and pharyngeal cancers (lip, tongue, gum, floor of mouth, salivary gland, and other oral cavity)	Phase I completed www.pnptherapeutics.com
GEN-1	Celsion Lawrenceville, NJ	treatment of ovarian cancer	Phase I/II www.celsion.com
GL-0817 (MAGE-A3 cancer vaccine)	Gliknik Baltimore, MD	treatment of MAGE-A3 expressing head and neck squamous cell carcinoma	Phase II www.gliknik.com
glufosfomide	Eleison Pharmaceuticals St. Petersburg, FL	for treatment of pancreatic cancer (Fast Track)	Phase III www.eleison-pharma.com
GPX-150	Gem Pharmaceuticals Birmingham, AL	treatment of soft tissue sarcoma	Phase II www.gempharmaceuticals.com
GS-5745 (MMP9 mAb inhibitor)	Gilead Sciences Foster City, CA	treatment of gastric cancer	Phase III www.gilead.com
Iclusig® ponatinib	ARIAD Pharmaceuticals Cambridge, MA	treatment of gastrointestinal stromal tumors (GIST)	Phase II www.ariad.com
ICT-107 (dendritic cancer vaccine)	ImmunoCellular Therapeutics Calabasas, CA	treatment of glioblastoma or brain stem glioma	Phase III www.imuc.com

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IMA901 (peptide vaccine)	Immatics Biotechnologies Tübingen, Germany	treatment of renal cell carcinoma in HLA-A*2 positive patients	Phase III www.immatics.com
ipatasertib	Genentech South San Francisco, CA	for the treatment of gastric cancer including cancer of the gastro-esophageal junction	Phase II www.gene.com
IRX-2 immunotherapy	IRX Therapeutics New York, NY	neoadjuvant therapy in patients with squamous cell carcinoma of the head and neck (Fast Track)	Phase II www.irxtherapeutics.com
Keytruda® pembrolizumab	Merck Kenilworth, NJ	treatment of gastric cancer, including gastroesophageal junction adenocarcinoma	Phase III www.merck.com
Lenvima® lenvatinib	Eisai Woodcliff Lake, NJ	treatment of hepatocellular carcinoma	Phase III www.eisai.com
		treatment of stage IIB to stage IV melanoma	Phase II www.eisai.com
liposome entrapped paclitaxel (LEP)	Insys Therapeutics Chandler, AZ	treatment of ovarian cancer	Phase II www.insysrx.com
		treatment of gastric cancer	Phase II www.insysrx.com

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LN-144 (tumor-infiltrating lymphocytes)	Lion Biotechnologies New York, NY	treatment of malignant melanoma stages IIb to IV	Phase II www.lbio.com
Lutathera® DOTA-Tyr3 octreotide	Advanced Accelerator Applications Saint-Genis-Pouilly, France	treatment of gastro-entero-pancreatic neuroendocrine tumors	Phase III www.adacap.com
LY2606368 (Chk1 inhibitor)	Eli Lilly Indianapolis, IN	treatment of anal cancer	Phase I www.lilly.com
marizomib IV (proteasome inhibitor)	Triphase Research & Development San Diego, CA	treatment of malignant glioma	Phase I www.triphase.com
masitinib	AB Science Paris, France	treatment of gastric cancer including cancer of the gastroesophageal junction	Phase III www.ab-science.com
		treatment of malignant gastrointestinal stromal tumors	Phase III www.ab-science.com
melapuldencel-T	Caladrius Biosciences New York, NY	treatment of stage IIIB through IV metastatic melanoma	Phase III www.caladrius.com
melphalan	Delcath Systems New York, NY	treatment of patients with hepatocellular carcinoma	Phase II www.delcath.com
		treatment of cholangiocarcinoma	Phase II www.delcath.com

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MENK (met5-enkephalin)	Immune Therapeutics Orlando, FL	treatment of pancreatic cancer	Phase II www.immunetherapeutics.com
mibefradil	Cavion Charlottesville, VA	treatment of glioblastoma multiforme	Phase I www.cavionpharma.com
milciclib	Tiziana Life Sciences London, England	treatment of thymic epithelial tumors	Phase II www.tizianalifesciences.com
Minnelide™ 001 HSP70 heat shock protein	Minneamrita Therapeutics Moline, IL	treatment of gastric cancer	Phase I
mipsagargin	GenSpera San Antonio, TX	treatment of hepatocellular carcinoma	Phase II www.genspera.com
mirvetuximab soratansine (maytansinoid conjugated mAb)	ImmunoGen Waltham, MA	treatment of ovarian cancer	Phase II www.immunogen.com
MORAb-009 (amatuximab)	Eisai Woodcliff Lake, NJ	treatment of mesothelioma	Phase II www.eisai.com
MORAb-066	Eisai Woodcliff Lake, NJ	treatment of pancreatic cancer	Phase I www.eisai.com
motolimid	VentiRx Pharmaceuticals Seattle, WA	treatment of ovarian cancer (Fast Track)	Phase II www.ventirx.com

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Multikine® leukocyte interleukin injection	CEL-SCI Vienna, VA	neoadjuvant therapy in patients with squamous cell carcinoma of the head and neck	Phase III www.cel-sci.com
necuparanib	Momenta Pharmaceuticals Cambridge, MA	treatment of pancreatic cancer (Fast Track)	Phase II www.momentapharma.com
NEO100 (perillyl alcohol)	NeOnc Technologies Los Angeles, CA	treatment of glioma	Phase I/II www.neonctech.com
neuroblastoma bivalent vaccine	MabVax Therapeutics San Diego, CA	treatment of neuroblastoma	Phase I www.mabvax.com
niraparib (ADP-ribose PARP inhibitor)	TESARO Waltham, MA	treatment of ovarian cancer	Phase III www.tesaro.com
NSC-132313 (dianhydrogalactitol)	DelMar Pharmaceuticals Menlo Park, CA	treatment of malignant gliomas	Phase II www.delmarpharma.com
NVX-108 (radiosensitizer)	NuvOx Pharma Tucson, AZ	radiosensitizer to be used during radiation treatment of glioblastoma multiforme (GBM)	Phase I/II www.nuvox.com
NY-ESO T-lymphocyte cell therapy (GSK3377794)	Adaptimmune Philadelphia, PA GlaxoSmithKline Research Triangle Park, NC	treatment of soft tissue sarcoma	Phase I/II www.adaptimmune.com www.gsk.com

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olaratumab	Eli Lilly Indianapolis, IN	treatment of soft tissue sarcoma	Phase III www.lilly.com
ontuxizumab (MORAb-004)	Eisai Woodcliff Lake, NJ	treatment of soft tissue sarcoma	Phase II www.eisai.com
Opdivo® nivolumab	Bristol-Myers Squibb Princeton, NJ	treatment of small cell lung cancer	Phase III www.bms.com
		treatment of glioblastoma	Phase III www.bms.com
		treatment of hepatocellular carcinoma	Phase I www.bms.com
oregovomab	Quest PharmaTech Edmonton, Canada	treatment of epithelial ovarian cancer	Phase II www.questpharmatech.com
OSE2101	OSE Pharma Paris, France	treatment of non-small cell lung cancer in patients expressing HLA-A2	Phase III www.osepharma.com
OTL38 (diagnostic imaging enhancer)	On Target Laboratories West Lafayette, IN	detecting folate receptor alpha positive lesions in ovarian cancer patients (diagnostic for the management of ovarian cancer)	Phase II www.ontargetlaboratories.com

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pancreatic cancer cell therapy	PharmaCyte Biotech Silver Spring, MD	treatment of pancreatic cancer in combination with ifosfamide	Phase II www.pharmacYTE.com
pegargiminase (ADI-PEG20)	Polaris Pharmaceuticals San Diego, CA	treatment of hepatocellular carcinoma	Phase III www.polarispharma.com
		treatment of invasive malignant melanoma	Phase II www.polarispharma.com
		treatment of mesothelioma	Phase II www.polarispharma.com
PEG-PH20 (PEGylated recombinant human hyaluronidase PH20)	Halozyne Therapeutics San Diego, CA	treatment of pancreatic cancer (Fast Track)	Phase III www.halozyne.com
Perjeta® pertuzumab	Genentech South San Francisco, CA	treatment of gastric cancer	Phase III www.gene.com
pexastimogene devacirepvec	SillaJen Biotherapeutics San Francisco, CA	treatment of hepatocellular carcinoma	Phase III www.sillajen.com
PF-06463922 (ALK inhibitor)	Pfizer New York, NY	treatment of anaplastic lymphoma kinase (ALK)-positive or ROS1-positive or ROS1-positive non-small cell lung cancer	Phase II www.pfizer.com

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PLX3397 (pexidartinib)	Plexxikon Berkeley, CA	treatment of pigmented villonodular synovitis/giant cell tumor of the tendon sheath	Phase III www.plexxikon.com
PM1183 (lurbinectedin)	PharmaMar USA New York, NY	treatment of ovarian cancer	Phase III www.pharmamar.com
polyclonal antibody stimulator (G17DT immunogen)	Cancer Advances Durham, NC	treatment of gastric cancer	Phase III www.canceradvancesinc.com
		treatment of adenocarcinoma of the pancreas	Phase III www.canceradvancesinc.com
prolactin receptor antagonist	Oncolix Houston, TX	treatment of ovarian cancer	Phase I www.oncolixbio.com
prophage cancer vaccine	Agenus Lexington, MA	treatment of glioma	Phase II www.agenusbio.com
PV-10 (rose bengal disodium)	Provectus Pharmaceuticals Knoxville, TN	treatment of metastatic melanoma	Phase III www.pvct.com
		treatment of hepatocellular carcinoma	Phase I www.pvct.com

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Reolysin® pelareorep	Oncolytics Biotech Calgary, Canada	treatment of fallopian tube cancer	Phase II www.oncolyticsbiotech.com
		treatment of ovarian cancer	Phase II www.oncolyticsbiotech.com
		treatment of primary peritoneal cancer	Phase II www.oncolyticsbiotech.com
		treatment of pancreatic cancer	Phase I/II www.oncolyticsbiotech.com
		treatment of malignant glioma	Phase I www.oncolyticsbiotech.com
rociletinib (EGFR receptor antagonist)	Clovis Oncology Boulder, CO	treatment of non-small cell lung cancer mutations in the epidermal growth factor receptor	application submitted www.clovisoncology.com
rovalpituzumab tesirine (DLL3 protein inhibitor)	StemcentRx South San Francisco, CA	treatment of small cell lung cancer	Phase II www.stemcentrx.com
rucaparib (PARP inhibitor)	Clovis Oncology Boulder, CO	treatment of ovarian cancer	Phase III www.clovisoncology.com
RX-0201 (Akt-1 inhibitor)	Rexahn Pharmaceuticals Rockville, MD	treatment of pancreatic cancer	Phase II www.rexahn.com

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SBP-101 (cell division inhibitor)	Sun BioPharma Waconia, MN	treatment of pancreatic cancer	Phase I www.sunbiopharma.com
seviprotimut-L (polyvalent melanoma vaccine)	Polynoma San Diego, CA	treatment of stage IIB to stage IV melanoma	Phase III www.polynoma.com
SL-401 (dendritic cell inhibitor)	Stemline Therapeutics New York, NY	treatment of blastic plasmacytoid dendritic cell neoplasm	Phase I/II www.stemline.com
Somakit-TATE® gallium-68 DOTATATE	Advanced Accelerator Applications Saint-Genis-Pouilly, France	diagnostic for the management of gastro-entero-pancreatic neuroendocrine tumors	application submitted www.adacap.com
SomaKit-TOC® gallium (68Ga) edotreotide	Advanced Accelerator Applications Saint-Genis-Pouilly, France University of Iowa Iowa City, IA	diagnostic for the clinical management of neuroendocrine tumors	Phase II www.adacap.com
Somatuline® lanreotide acetate	Ipsen Biopharmaceuticals Basking Ridge, NJ	treatment of symptoms associated with carcinoid syndrome	Phase III www.ipсен.com
sonidegib (LDE225)	Novartis Pharmaceuticals East Hanover, NJ	treatment of medulloblastoma	Phase II www.novartis.com
SOR-C13 (TRPV6 protein inhibitor)	Soricimed Biopharma Moncton, Canada	treatment of ovarian cancer	Phase I www.soricimed.com

Cancer <u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
Stivarga® regorafenib	Bayer HealthCare Pharmaceuticals Wayne, NJ	treatment of hepatocellular carcinoma	Phase III www.pharma.bayer.com
STM 434 (actinin inhibitor)	Atara Biotherapeutics South San Francisco, CA	treatment of ovarian cancer	Phase I www.atarabio.com
Tafinlar™ + Mekinist® dabrafenib and trametinib	Novartis Pharmaceuticals East Hanover, NJ	treatment of patients with BRAF mutation positive non-small cell lung	Phase II www.novartis.com
tarextumab (notch 2/3 mAb)	OncoMed Pharmaceuticals Redwood City, CA GlaxoSmithKline Research Triangle Park, NC	treatment of pancreatic cancer	Phase II www.oncomed.com www.gsk.com
		treatment of small cell lung cancer	Phase II www.oncomed.com www.gsk.com
telatinib (VEGFR inhibitor)	Eddingpharm Westlake Village, CA	treatment of gastric cancer	Phase II www.eddingpharm.com
telotristat etiprate	Lexicon Pharmaceuticals The Woodlands, TX	management of symptoms of carcinoid syndrome associated with carcinoid tumor (Fast Track)	application submitted www.lexpharma.com
TEN-010 (BET inhibitor)	Roche Basel, Switzerland	treatment of nuclear protein in testis (NUT) midline carcinoma	Phase I www.roche.com

Cancer

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
ThermoDox® lyso-thermosensitive liposomal doxorubicin	Celsion Lawrenceville, NJ	treatment of hepatocellular carcinoma (Fast Track)	Phase III www.celsion.com
tivantinib (MET inhibitor)	ArQule Burlington, MA Daiichi Sankyo Parsippany, NJ	treatment of hepatocellular carcinoma	Phase III www.dsi.com
Toca 511 + Toca FC	Tocagen San Diego, CA	treatment of glioblastoma multiforme	Phase II/III www.tocagen.com
tozaride (Rhenium 188 P2045 somatostatin analog)	Andarix Pharmaceuticals Somerville, NJ	treatment of small cell lung cancer	Phase I www.andarix.com
TPI 287 (abeotaxane inhibitor)	Cortice Biosciences New York, NY	treatment of gliomas	Phase II www.corticebiosciences.com
TPIV200 (multi-epitope folate receptor alpha peptide vaccine with GM-CSF adjuvant)	Tapimmune Jacksonville, FL	treatment of ovarian cancer	Phase II www.tapimmune.com

Cancer Product Name	Sponsor	Official FDA Designation*	Development Status
trans sodium crocetinate (TSC)	Diffusion Pharmaceuticals Charlottesville, VA	treatment of glioblastoma in conjunction with radiotherapy	Phase II www.diffusionpharma.com
		treatment of brain metastasis	Phase I www.diffusionpharma.com
TRC105 (ENG protein inhibitor)	Tracon Pharmaceuticals San Diego, CA	treatment of soft tissue sarcoma	Phase II www.traconpharma.com
tremelimumab (CTLA-4 mAb)	MedImmune Gaithersburg, MD	treatment of malignant mesothelioma (Fast Track)	Phase III www.medimmune.com
VB-111	Vascular Biogenics Or Yehuda, Israel	treatment of malignant glioma (Fast Track)	Phase III www.vblrx.com
VB4-847	Viventia Biotech Winnipeg, Canada	treatment of Ep-CAM-positive squamous cell carcinoma of the head and neck (Fast Track)	Phase II www.viventia.com
veliparib	AbbVie North Chicago, IL	treatment of epithelial ovarian cancer in combination with DNA-damaging agents	Phase III www.abbvie.com
		treatment of malignant melanoma stages IIB through IV	Phase II www.abbvie.com

Cancer <u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
vemurafenib	Genentech South San Francisco, CA	treatment of anaplastic thyroid carcinoma and advanced papillary thyroid cancer whose tumors harbor a BRAF V600 mutation	Phase II www.gene.com
Vesimune™ (TLR7 agonist immunotherapy)	UroGen New York, NY	treatment of carcinoma in situ (CIS) of the urinary bladder	Phase I www.urogen.com
Vigil™ autologous tumor cell vaccine	Gradalis Dallas, TX	treatment of ovarian cancer	Phase III www.gradalisinc.com
		treatment of Ewing's sarcoma	Phase II www.gradalisinc.com
		treatment of stage IIB to IV melanoma	Phase I www.gradalisinc.com
VS-5584 (PI3K/mTORC1/2 inhibitor)	Verastem Needham, MA	treatment of malignant mesothelioma	Phase I www.verastem.com
VS-6063 (defactinib)	Verastem Needham, MA	treatment of mesothelioma	Phase I/II www.verastem.com
VT-122 (propranolol/etodolac)	Vicus Therapeutics Morristown, NJ	treatment of hepatocellular carcinoma	Phase II www.vicusrx.com
		treatment of pancreatic cancer	Phase I www.vicusrx.com

Cancer <u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
WT1 (galinpepimut-S)	SELLAS Life Sciences New York, NY	treatment of mesothelioma	Phase II www.sellaslifesciences.com

Cancer, Blood <u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
ABT-199 (venetoclax)	AbbVie North Chicago, IL	treatment of acute myeloid leukemia	Phase II www.abbvie.com
		treatment of diffuse large B-cell lymphoma	Phase I/II www.abbvie.com
Actimab-A 225Ac-lintuzumab	Actinium Pharmaceuticals New York, NY	treatment of acute myelogenous leukemia	Phase I/II www.actiniumpharma.com
Adcetris® brentuximab vedotin	Seattle Genetics Bothell, WA	treatment of mycosis fungoides B-cell lymphoma	Phase III www.seattlegenetics.com
		treatment of patients with peripheral T-cell lymphoma, not otherwise specified (Fast Track)	Phase III www.seattlegenetics.com
		treatment of patients with diffuse large B-cell lymphoma	Phase II www.seattlegenetics.com

Cancer, Blood
Product Name

Sponsor

Official FDA Designation*

Development Status

Afinitor® everolimus	Novartis Pharmaceuticals East Hanover, NJ	treatment of diffuse large B-cell lymphoma	application submitted www.novartis.com
AFM13	Affimed Therapeutics Heidelberg, Germany Merck Kenilworth, NJ	treatment of Hodgkin lymphoma	Phase II www.affimed.com www.merck.com
AG-120 (IDH1 mutant inhibitor)	Agios Pharmaceuticals Cambridge, MA Celgene Summit, NJ	treatment of acute myeloid leukemia (AML)	Phase II www.agios.com www.celgene.com
AG-221 (IDH2 inhibitor)	Agios Cambridge, MA Celgene Summit, NJ	treatment of acute myelogenous leukemia	Phase III www.agios.com www.celgene.com
alvocidib	Tolero Pharmaceuticals Lehi, UT	treatment of acute myeloid leukemia	Phase II www.toleropharma.com
anti-CD45 mAb 131I-BC8	Actinium Pharmaceuticals New York, NY	treatment of acute myeloid leukemia (AML)	Phase II www.actiniumpharma.com
BGB324	BerGenBio Bergen, Norway	treatment of acute myeloid leukemia	Phase I www.bergenbio.com
BI-505 (Axl RTK inhibitor)	BioInvent International Lund, Sweden	treatment of multiple myeloma	Phase II www.bioinvent.com

Cancer, Blood
Product Name

Sponsor

Official FDA Designation*

Development Status

BL-8040 (CXCR4 chemokine receptor)	BioLineRx Modi'in, Israel	treatment of acute myeloid leukemia	Phase II www.biolinerx.com
Bosulif® bosutinib	Pfizer New York, NY	treatment of chronic myelogenous leukemia (1st-line)	Phase III www.pfizer.com
BP1001 (liposomal Grb-2)	Bio-Path Holdings Houston, TX	treatment of acute myeloid leukemia	Phase II www.bio-pathholdings.com
BT-062 (indatuximab ravtansine)	Biotest Dreieich, Germany	treatment of multiple myeloma	Phase II www.biotest.com
CB-5083 (CDC48/p97 ATPase inhibitor)	Cleave Biosciences Burlingame, CA	treatment of multiple myeloma	Phase I www.cleavebio.com
CLR 131 radiopharmaceutical	Cellectar Biosciences Madison, WI	treatment of multiple myeloma	Phase I www.cellectorbiosciences.com
CMD-003 (autologous Epstein-Barr virus specific T-cells)	Cell Medica London, England	treatment of Epstein-Barr virus positive non-Hodgkin lymphomas	Phase II www.cellmedica.co.uk
CNDO-109 (activated allogeneic natural killer cells)	Fortress Biotech Burlington, MA	treatment of acute myeloid leukemia	Phase I www.fortressbiotech.com

Cancer, Blood <u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
CPI-613	Cornerstone Pharmaceuticals Cranbury, NJ	treatment of acute myeloid leukemia	Phase II www.cornerstonepharma.com
		treatment of myelodysplastic syndrome	Phase II www.cornerstonepharma.com
CPX-351 (cytarabine:daunorubicin)	Celator Pharmaceuticals Ewing, NJ	treatment of acute myeloid leukemia (Fast Track)	Phase III www.celatorpharma.com
crenolanib	AROG Pharmaceuticals Dallas, TX	treatment of acute myelogenous leukemia	Phase II www.arogpharma.com
CUDC-907	Curis Lexington, MA	treatment of diffuse large B-cell lymphoma (DLBCL)	Phase II www.curis.com
Darzalex® daratumumab	Janssen Research and Development Raritan, NJ	treatment of diffuse large B-cell lymphoma	Phase II www.janssenrnd.com
		treatment of follicular lymphoma	Phase II www.janssenrnd.com
		treatment of mantle cell lymphoma	Phase II www.janssenrnd.com

Cancer, Blood <u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
duvelisib	Infinity Pharmaceuticals Cambridge, MA	treatment of follicular lymphoma	Phase III www.infi.com
		treatment of chronic lymphocytic leukemia and small lymphocytic lymphoma	Phase III www.infi.com
EBV-CTL (allogeneic Epstein-Barr virus cytotoxic T lymphocytes)	ATARA Biotherapeutics South San Francisco, CA	treatment of Epstein-Barr virus-positive post-transplant lymphoproliferative disorders (Breakthrough Therapy)	Phase II www.atarabio.com
epratuzumab	Immunomedics Morris Plains, NJ	treatment of acute lymphoblastic leukemia	Phase III www.immunomedics.com
fenretinide intravenous	CerRx Lubbock, TX	treatment of peripheral T-cell lymphoma	Phase II www.cerrx.com
filanesib	Array BioPharma Boulder, CO	treatment of multiple myeloma	Phase II www.arraybiopharma.com
Gazyva® obinutuzumab	Genentech South San Francisco, CA	treatment of diffuse large B cell lymphoma	Phase III www.gene.com
		treatment of follicular lymphoma	Phase II www.gene.com

Cancer, Blood
Product Name

Sponsor

Official FDA Designation*

Development Status

GMI-1271
(E-selectin antagonist)

GlycoMimetics
Rockville, MD

treatment of acute myeloid leukemia
(AML)

Phase I/II
www.glycomimetics.com

Imbruvica®
ibrutinib

Janssen Biotech
Horsham, PA
Pharmacyclics
Sunnyvale, CA

treatment of diffuse large B-cell lymphoma

Phase III
www.janssenbiotech.com
www.pharmacyclics.com

treatment of follicular lymphoma

Phase III
www.janssenbiotech.com
www.pharmacyclics.com

treatment of patients with extranodal
marginal zone lymphoma (mucosa
associated lymphoid tissue [MALT
type] lymphoma)

Phase III
www.janssenbiotech.com
www.pharmacyclics.com

treatment of nodal marginal zone
lymphoma

Phase III
www.janssenbiotech.com
www.pharmacyclics.com

treatment of splenic marginal zone
lymphoma

Phase III
www.janssenbiotech.com
www.pharmacyclics.com

treatment of multiple myeloma

Phase II
www.janssenbiotech.com
www.pharmacyclics.com

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Product Name

Sponsor

Official FDA Designation*

Development Status

imetelstat (telomerase inhibitor)	Janssen Research & Development Raritan, NJ	treatment of myelodysplastic syndrome	Phase II/III www.janssenrnd.com
		treatment of myelofibrosis	Phase II www.janssenrnd.com
IMO-8400 (TLR antagonist)	Idera Pharmaceuticals Cambridge, MA	treatment of diffuse large B-cell lymphoma	Phase I/II www.iderapharma.com
		treatment of Waldenstrom's macroglobulinemia	Phase I/II www.iderapharma.com
inotuzumab ozogamicin	Pfizer New York, NY	treatment of B-cell acute lymphoblastic leukemia (Breakthrough Therapy)	Phase III www.pfizer.com
isatuximab	Sanofi US Bridgewater, NJ	treatment of multiple myeloma	Phase II www.sanofi.com
JCAR015	Juno Therapeutics Seattle, WA	treatment of acute lymphoblastic leukemia (ALL)	Phase II www.junotherapeutics.com
JNJ-56022473	Janssen Research & Development Raritan, NJ	treatment of acute myeloid leukemia	Phase II www.janssenrnd.com

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Development Status

Keytruda® pembrolizumab	Merck Kenilworth, NJ	treatment of multiple myeloma	Phase III www.merck.com
		treatment of primary mediastinal B cell lymphoma	Phase II www.merck.com
		treatment of Hodgkin lymphoma	Phase II www.merck.com
luspatercept (recombinant fusion protein)	Acceleron Pharma Cambridge, MA Celgene Summit, NJ	treatment of myelodysplastic syndromes	Phase III www.acceleronpharma.com www.celgene.com
marizomib IV (proteasome inhibitor)	Triphase Research and Development Toronto, Canada	treatment of multiple myeloma	Phase I www.triphaseco.com
melphalan flufenamide	Oncopeptides Stockholm, Sweden	treatment of plasma cell myeloma, also referred to as multiple myeloma	Phase I/II www.oncopeptides.se
midostaurin (PKC412)	Novartis Pharmaceuticals East Hanover, NJ	treatment of acute myeloid leukemia	Phase III www.novartis.com
MLN4924 (pevonedistat)	Millennium Pharmaceuticals Cambridge, MA	treatment of acute myelogenous leukemia	Phase II www.millennium.com
		treatment of myelodysplastic syndrome	Phase II www.millennium.com

Cancer, Blood <u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
mocetinostat	Mirati Therapeutics San Diego, CA	treatment of diffuse large B-cell lymphoma	Phase I/II www.mirati.com
mogamulizumab	Kyowa Kirin USA Princeton, NJ	treatment of patients with cutaneous T-cell lymphoma	Phase III www.kyowa-kirin.com
		treatment of adult T-cell leukemia/ lymphoma (ATLL)	Phase II www.kyowa-kirin.com
momelotinib	Gilead Sciences Foster City, CA	treatment of myelofibrosis	Phase III www.gilead.com
MOR208 (anti-CD19 Fc mAb)	MorphoSys Planegg, Germany	treatment of diffuse large B-cell lymphoma (Fast Track)	Phase II www.morphosys.com
		treatment of patients with chronic lymphocytic leukemia/small lymphocytic lymphoma	Phase II www.morphosys.com
moxetumomab pasudotox	MedImmune Gaithersburg, MD	treatment of hairy cell leukemia	Phase III www.medimmune.com

Cancer, Blood
Product Name

Sponsor

Official FDA Designation*

Development Status

NiCord® stem/progenitor cell therapy	Gamida Cell Jerusalem, Israel	treatment of acute lymphoblastic leukemia	Phase I/II www.gamida-cell.com
		treatment of myelodysplastic syndrome	Phase I/II www.gamida-cell.com
		treatment of Hodgkin lymphoma	Phase I/II www.gamida-cell.com
Opdivo® nivolumab phalan	Bristol-Myers Squibb Princeton NJ	treatment of Hodgkin lymphoma (Breakthrough Therapy)	Phase II www.bms.com
oprozomib	Amgen Thousand Oaks, CA	treatment of Waldenstrom's macroglobulinemia	Phase I/II www.amgen.com
		treatment of multiple myeloma	Phase I www.amgen.com
otlertuzumab (anti-CD37 mAb)	Emergent BioSolutions Rockville, MD	treatment of chronic lymphocytic leukemia	Phase II www.emergentbiosolutions.com
Oxi4503	OxiGene South San Francisco, CA	treatment of acute myelogenous leukemia	Phase I/II www.oxigene.com
P1101 (pegylated proline interferon alpha-2b)	PharmaEssentia, Corporation Taipei, Taiwan	treatment of myelofibrosis	Phase II www.pharmaessentia.com

Cancer, Blood <u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
pinometostat (EPZ-5676) (DOT1L inhibitor)	Celgene Summit, NJ Epizyme Cambridge, MA	treatment of acute myeloid leukemia	Phase I www.celgene.com www.epizyme.com
		treatment of acute lymphoblastic leukemia (ALL)	Phase I www.celgene.com www.epizyme.com
plitidepsin	PharmaMar USA New York, NY	treatment of multiple myeloma	Phase III www.pharmamar.com
PNT2258 (rosomidnar)	ProNAi Therapeutics Vancouver, Canada	treatment of diffuse large B-cell lymphoma	Phase II www.pronai.com
pracinostat (HDAC inhibitor)	MEI Pharma San Diego, CA	treatment of acute myeloid leukemia	Phase II www.meipharma.com
PRM-151 (rhPTX-2 for injection)	Promedior Lexington, MA	treatment of myelofibrosis (Fast Track)	Phase II www.promedior.com
PVX-410 (multi-peptide cancer vaccine)	OncoPep North Andover, MA	treatment of multiple myeloma	Phase I/II www.oncopep.com
quizartinib (FLT3 inhibitor)	Ambit Biosciences San Diego, CA Daiichi Sankyo Parsippany, NJ	treatment of acute myeloid leukemia (Fast Track)	Phase III www.ambitbio.com www.dsi.com

Cancer, Blood
Product Name

Sponsor

Official FDA Designation*

Development Status

Resimmune™ A-dmDT390-bisFv (UCHT1)	Angimmune Rockville, MD	treatment of cutaneous T-cell lymphoma	Phase II www.angimmune.com
Revlimid® lenalidomide	Celgene Summit, NJ	treatment of chronic lymphocytic leukemia	Phase III www.celgene.com
		treatment of diffuse large B-cell lymphoma	Phase III www.celgene.com
		treatment of follicular lymphoma	Phase III www.celgene.com
rigosertib	Onconova Therapeutics Newtown, PA	treatment of myelodysplastic syndromes	Phase III www.onconova.com
sapacitabine	Cyclacel Berkeley Heights, NJ	treatment of acute myelogenous leukemia	Phase III www.cyclacel.com
		treatment of myelodysplastic syndromes	Phase II www.cyclacel.com

Cancer, Blood
Product Name

Sponsor

Official FDA Designation*

Development Status

selinexor (nuclear export inhibitor)	Karyopharm Therapeutics Newton, MA	treatment of multiple myeloma	Phase II www.karyopharm.com
		treatment of diffuse large B-cell lymphoma	Phase II www.karyopharm.com
		treatment of acute myeloid leukemia	Phase II www.karopharm.com
SGI-110 (DNMT inhibitor)	Astex Pharmaceuticals Pleasanton, CA	treatment of acute myeloid leukemia	Phase III www.astx.com
SGN-CD33A (vadastuximab talirine)	Seattle Genetics Bothell, WA	treatment of acute myeloid leukemia	Phase I/II www.seattlegenetics.com
SGX301	Soligenix Princeton, NJ	treatment of cutaneous T-cell lymphoma (Fast Track)	Phase III www.soligenix.com
SHAPE (HDAC inhibitor)	TetraLogic Pharmaceuticals Malvern, PA	treatment of cutaneous T-cell lymphoma	Phase II www.tetralogicpharma.com
SL-401 (dendritic cell inhibitor)	Stemline Therapeutics New York, NY	treatment of acute myeloid leukemia	Phase I/II www.stemline.com
Sylvant ® siltuximab	Janssen Research & Development Raritan, NJ	treatment of multiple myeloma	Phase II www.janssenrnd.com

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Sponsor

Official FDA Designation*

Development Status

tisagenlecleucel-T	Novartis Pharmaceuticals East Hanover, NJ	treatment of diffuse large B-cell lymphoma	Phase II www.novartis.com
		for the treatment of acute lymphoblastic leukemia	Phase II www.novartis.com
tosedostat	Cell Therapeutics Seattle, WA	treatment of acute myeloid leukemia	Phase II www.celltherapeutics.com
ublituximab	TG Therapeutics New York, NY	treatment of chronic lymphocytic leukemia	Phase III www.tgtherapeutics.com
		treatment of nodal marginal zone lymphoma	Phase I/II www.tgtherapeutics.com
		treatment of extranodal marginal zone lymphoma (mucosa-associated lymphatic tissue, MALT)	Phase I/II www.tgtherapeutics.com
ulocuplumab (CXCR4 receptor antagonist)	Bristol-Myers Squibb Princeton, NJ	treatment of acute myeloid leukemia	Phase I www.bms.com
VLX1570	VivoLux Ashburn, VA	treatment of multiple myeloma	Phase I www.vivolux.com
volasertib	Boehringer Ingelheim Pharmaceuticals Ridgefield, CT	treatment of acute myeloid leukemia (Breakthrough Therapy)	Phase III www.boehringer-ingelheim.com

Cancer, Blood

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
vosaroxin (SNS-595)	Sunesis Pharmaceuticals South San Francisco, CA	treatment of acute myeloid leukemia (Fast Track)	Phase I/II www.sunesis.com
WT1 (galenpepimut-S) peptides)	SELLAS Life Sciences New York, NY	treatment of acute myeloid leukemia	Phase II www.sellaslifesciences.com
Zydelig® idelalisib	Gilead Sciences Foster City, CA	treatment of extranodal marginal zone lymphoma	Phase II www.gilead.com
		treatment of splenic marginal zone lymphoma	Phase II www.gilead.com
		treatment of nodal marginal zone lymphoma	Phase II www.gilead.com
		treatment of lymphoplasmacytic lymphoma with or without Waldenstrom's macroglobulinemia	Phase II www.gilead.com

Cardiovascular Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
ARRY-797 (p38 mitogen-activated kinase inhibitor)	Array BioPharma Boulder, CO	treatment of dilated cardiomyopathy, including dilated cardiomyopathy secondary to lamin A/C gene mutations	Phase II www.arraybiopharma.com

Cardiovascular Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
bardoxolone methyl (BARD)	Reata Pharmaceuticals Irving, TX	treatment of pulmonary arterial hypertension	Phase II www.reatapharma.com
esuberaprost	Lung Biotechnology Silver Spring, MD	treatment of pulmonary arterial hypertension	Phase III www.lungbiotechnology.com
GS-4997 (ASK1 inhibitor)	Gilead Sciences Foster City, CA	treatment pulmonary arterial hypertension	Phase II www.gilead.com
INOpulse® nitric oxide	Bellerophon Therapeutics Warren, NJ	treatment of pulmonary arterial hypertension	Phase III www.bellerophon.com
ixmyelocel-T	Vericel Cambridge, MA	treatment of dilated cardiomyopathy	Phase III www.vcel.com
PB1046	PhaseBio Pharmaceuticals Malvern, PA	treatment of cardiomyopathy associated with dystrophinopathies; Duchenne muscular dystrophy (DMD), Becker muscular dystrophy BMD), and X-linked dilated cardiomyopathy (XL-dCMP)	Phase I www.phasebio.com
plasmin (human)	Grifols Therapeutics Los Angeles, CA	treatment of acute peripheral arterial occlusion	Phase II www.grifols.com
ralinepag	Arena Pharmaceuticals San Diego, CA	treatment of pulmonary arterial hypertension	Phase II www.arenapharma.com

Cardiovascular Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
SPI-026 (tacrolimus)	Selten Pharma Saratoga, CA	treatment of pulmonary arterial hypertension	Phase II www.seltenpharma.com
Trevynt® treprostinil sodium solution infusion pump	SteadyMed Therapeutics San Ramon, CA	treatment of pulmonary arterial hypertension	in clinical trials www.steadymed.com
Vyndaqel® tafamidis meglumine	Pfizer New York, NY	treatment of symptomatic transthyretin (TTR) amyloid cardiomyopathy	Phase III www.pfizer.com

Digestive Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
ABT-308/RPC-4046 (IL-13 receptor antagonist)	AbbVie North Chicago, IL Receptos San Diego, CA	treatment of eosinophilic esophagitis	Phase II www.abbvie.com www.receptos.com
alicaforsen	Atlantic Healthcare Essex, England	treatment of pouchitis (Fast Track)	Phase III www.atlantichc.com
Humira® adalimumab	AbbVie North Chicago, IL	treatment of pediatric patients with ulcerative colitis	Phase III www.abbvie.com
Lialda® mesalamine controlled release	Shire Lexington, MA	treatment of ulcerative colitis in pediatric patients (revised indication 10/21/2010)	Phase III www.shire.com

Digestive Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
metronidazole 10% ointment	SLA Pharma Leistal, Switzerland	topical treatment of active perianal Crohn's disease	Phase III www.slapharma.com
omeprazole-lansoprazole with buffer	Effexus Pharmaceuticals Leawood, KS	treatment of esophageal ulcers	Phase I
SGX203 (beclomethasone)	Soligenix Princeton, NJ	treatment of pediatric patients with Crohn disease (Fast Track)	Phase II www.soligenix.com
SHP621 (budesonide)	Shire Lexington, MA	treatment of patients with eosinophilic esophagitis	Phase II www.shire.com
Simponi® golimumab	Janssen Biotech Horsham, PA	treatment of pediatric ulcerative colitis	Phase I www.janssenbiotech.com
STP-206	Sigma-Tau Pharmaceuticals Gaithersburg, MD	prevention of necrotizing enterocolitis (NEC) in premature infants with very low birth weight less than or equal to 1500 grams	Phase II www.sigmatau.com

Eye Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
human plasminogen	Kedrion Barga, Italy	treatment of ligneous conjunctivitis (Fast Track)	Phase II/III www.kedrion.com
Humira® adalimumab	AbbVie North Chicago, IL	treatment of non-infectious intermediate, posterior, or pan-uveitis, or chronic non-infectious anterior uveitis	application submitted www.abbvie.com

Eye Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
Mitosol® mitomycin	Mobius Therapeutics St. Louis, MO	prevention of recurrence of pterygium after its surgical excision	application submitted www.mobiustherapeutics.com
QPI-1007 (siRNA oligonucleotide)	Quark Pharmaceuticals Fremont, CA	treatment of ischemic optic neuropathy	Phase III www.quarkpharma.com
recombinant human nerve growth factor (rhNGF)	Dompe Milan, Italy	Treatment of neurotrophic keratitis	Phase II www.dompe.com
riboflavin ophthalmic solution ultraviolet-A (UVA) irradiation	Avedro Waltham, MA	treatment of corneal ectasia following refractive surgery	application submitted www.avedro.com
		treatment of keratoconus	application submitted www.avedro.com
SHP607 (mecasermin rinfabate)	Shire Lexington, MA	prevention of retinopathy of prematurity in premature infants born at risk for the disease (Fast Track)	Phase II www.shire.com
sirolimus ophthalmic (DE-109)	Santen Emeryville, CA	treatment of chronic/refractory anterior noninfectious uveitis, noninfectious intermediate uveitis, noninfectious panuveitis and non-infectious, uveitis affecting the posterior of the eye (NICUPS)	Phase III www.santen.com
thymosin beta 4 (TB4) (RGN-259)	ReGenTree Princeton, NJ	treatment of patients with neurotrophic keratopathy	Phase III www.regentrellc.com

Eye Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
Translarna® ataluren	PTC Therapeutics South Plainfield, NJ	treatment of aniridia	Phase I www.ptcbio.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
AAV1-FS344 (gene therapy)	Milo Biotechnology Cleveland, OH	treatment of Duchenne's and Becker's muscular dystrophy	Phase I/II www.milobiotechnology.com
AAV2-REP1 (gene therapy)	NightstaRx London, England	treatment of choroideremia	Phase II www.nightstarx.com
AAV5-hFIXco	uniQure Lexington, MA	treatment of hemophilia B	Phase I/II www.uniqure.com
acamprosate	Confluence Pharmaceuticals Indianapolis, IN	treatment of fragile X syndrome	Phase III www.confluencepharma.com
ACER-001 (reformulated NaPBA)	Acer Therapeutics Cambridge, MA	treatment of maple syrup urine disease	Phase II www.acertx.com
ACER-002 (celiprolol)	Acer Therapeutics Cambridge, MA	treatment of Ehlers-Danlos syndrome	Phase III www.acertx.com
Actimmune® interferon gamma-1b	Horizon Pharma Lake Forest, IL	treatment of Friedreich's ataxia (Fast Track)	Phase III www.horizonpharma.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
AEM-28 (apolipoprotein E mimetic peptide)	Lipimetix Development Natick, MA	treatment of homozygous familial hypercholesterolemia	Phase II www.lipimetix.com
AG-348 (PK(r) activator)	Agios Pharmaceuticals Cambridge, MA	treatment of pyruvate kinase deficiency	Phase II www.agios.com
AGT-181 (IDUA-HIRMAb fusion protein)	ArmaGen Technologies Calabasas, CA	treatment of mucopolysaccharidosis type 1 (MPS)	Phase I/II www.armagen.com
AGT-182/SHP631 (HIRMAb-IDS)	ArmaGen Technologies Calabasas, CA Shire Lexington, MA	treatment of mucopolysaccharidosis type II (Hunter Syndrome)	Phase I www.armagen.com www.shire.com
ALK-001 (retinol deuterated)	Alkeus Pharmaceuticals Boston, MA	treatment of Stargardt's disease	Phase II www.alkeuspharma.com
ALN-AT3SC (fitusiran)	Alnylam Pharmaceuticals Cambridge, MA	treatment of hemophilia A	Phase I www.alnylam.com
		treatment of hemophilia B	Phase I www.alnylam.com
alpha1-proteinase inhibitor (alpha1-HC)	Grifols Therapeutics Los Angeles, CA	treatment of cystic fibrosis	Phase II www.grifols.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
ALXN-1101 (cPMP replacement therapy)	Alexion Pharmaceuticals New Haven, CT	treatment of molybdenum cofactor deficiency type A (MoCD)	Phase II/III www.alexion.com
ARC-AAT (RNA interference)	Arrowhead Research Pasadena, CA	treatment of alpha-1 antitrypsin deficiency	Phase I www.arrowheadresearch.com
ARQ092 (c-AKT inhibitor)	ArQule Burlington, MA	treatment of Proteus syndrome	Phase I www.arqule.com
ARM210 (ryanodone receptor calcium release channel modulator)	ARMGO Pharma Tarrytown, NY	treatment of patients with Duchenne muscular dystrophy	Phase I www.armgo.com
ATYR 1940 (recombinant human histidyl tRNA synthetase)	aTyr Pharma San Diego, CA	treatment of facioscapulohumeral muscular dystrophy	Phase I/II www.atyrpharma.com
avoralstat (kallikrein/bradykinin inhibitor)	BioCryst Pharmaceuticals Durham, NC	prevention of acute attacks of angioedema in individuals with hereditary angioedema	Phase II/III www.biocryst.com
BAX335 (factor IX stimulant)	Baxalta Bannockburn, IL	treatment of hemophilia B	Phase I/II www.baxalta.com
BEL-0218 (ambroxol)	Belrose Pharma Princeton, NJ Zywie Princeton, NJ	treatment of Gaucher disease	Phase I www.belrosepharma.com www.zywiebio.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
BMN044 (exon 44 specific oligonucleotide)	BioMarin Pharmaceuticals San Rafael, CA	treatment of Duchenne muscular dystrophy in patients who have a mutation correctable by skipping of exon 44 of the dystrophin gene	Phase II www.biomarin.com
BMN045 (exon 45 specific oligonucleotide)	BioMarin Pharmaceutical San Rafael, CA	treatment of Duchenne muscular dystrophy patients bearing mutations that can be corrected by skipping exon 45	Phase II www.biomarin.com
BMN053 (exon 53 specific oligonucleotide)	BioMarin Pharmaceutical San Rafael, CA	treatment of Duchenne muscular dystrophy patients bearing mutations that can be corrected by skipping exon 55	Phase II www.biomarin.com
BMN190 (rhTPP1)	BioMarin Pharmaceutical San Rafael, CA	treatment of neuronal ceroid lipofuscinosis type 2	Phase II www.biomarin.com
BMN701 (reveglucosidase alfa)	BioMarin Pharmaceutical San Rafael, CA	treatment of Pompe disease	Phase III www.biomarin.com
BMS-986089 (anti-myostatin adnectin-IgG2)	Bristol-Myers Squibb Princeton, NJ	treatment of Duchenne muscular dystrophy	Phase I/II www.bms.com
cannabidiol	GW Pharma Salisbury, England	treatment of Dravet syndrome (Fast Track)	Phase III www.gwpharm.com
cannabidiol	Insys Therapeutics Phoenix, AZ	treatment of Dravet syndrome	Phase III www.insysrx.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
CAP-1002 (allogeneic cardiosphere-derived cells)	Capricor Therapeutics Beverly Hills, CA	treatment of Duchenne muscular dystrophy	Phase I/II www.capricor.com
Carbaglu® carglumic acid	Orphan Europe Paris, France	treatment of organic acidemias	in clinical trials www.orphan-europe.com
CAT-1004 (NF-Kappa B inhibitor)	Catabasis Pharmaceuticals Cambridge, MA	treatment of Duchenne muscular dystrophy	Phase II www.catabasis.com
CEQ508 (RNA interference)	Marina Biotech Bothell, WA	treatment of familial adenomatous polyposis (Fast Track)	Phase I/II www.marinabio.com
chenodiol	Sigma-Tau Pharmaceuticals Gaithersburg, MD	treatment of cerebrotendinous xanthomatosis	application submitted www.sigmatau.com
CPP-1X/sul (eflornithine plus sulindac)	Cancer Prevention Pharmaceuticals Tucson, AZ	treatment of familial adenomatous polyposis	Phase III www.canprevent.com
CTX-4430 (acebilustat)	Celtaxsys Atlanta, GA	treatment of cystic fibrosis	Phase II www.celtaxsys.com
deflazacort	Marathon Pharmaceuticals Northbrook, IL	treatment of Duchenne muscular dystrophy	Phase III www.marathonpharma.com
diazoxide choline controlled release	Essentialis Carlsbad, CA	treatment of Prader-Willi syndrome	Phase I www.essentialistherapeutics.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
drisapersen	BioMarin Pharmaceutical Novato, CA	treatment of Duchenne muscular dystrophy (Breakthrough Therapy) (Fast Track)	application submitted www.biomarin.com
DTX101 (gene transference)	Dimension Therapeutics Cambridge, MA	treatment of hemophilia B	Phase I/II www.dimensiontx.com
EDI200	Edimer Pharmaceuticals Cambridge, MA	treatment of X-linked hypohidrotic ectodermal dysplasia (Fast Track)	Phase II www.edimerpharma.com
eleclazine	Gilead Sciences Foster City, CA	treatment of congenital long QT syndrome	Phase III www.gilead.com
EPI-743 (vatiquinone)	Edison Pharmaceuticals Mountain View, CA	treatment of Rett syndrome	Phase II www.edisonpharma.com
		treatment of inherited mitochondrial respiratory chain diseases	Phase II www.edisonpharma.com
		treatment of Leigh syndrome	Phase II www.edisonpharma.com
EryDex erythrocyte-encapsulated dexamethasone	EryDel Urbino, Italy	treatment of ataxia-telangiectasia	Phase I www.erydel.com
eteplirsen/AVI-4658 (exon 51)	Sarepta Therapeutics Cambridge, MA	treatment of Duchenne muscular dystrophy (Fast Track)	Phase III www.sareptatherapeutics.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
evinacumab (ANGPTL3 protein inhibitor)	Regeneron Pharmaceuticals Tarrytown, NY	treat of homozygous familial hypercholesterolemia	Phase II www.regeneron.com
EZN-2279 (recombinant adenosine deaminase)	Sigma-Tau Pharmaceuticals Gaithersburg, MD	treatment of adenosine deaminase deficiency in patients with severe combined immunodeficiency	Phase III www.sigmatau.com
Firdapse® amifampridine	Catalyst Pharmaceuticals Coral Gables, FL	treatment of congenital myasthenic syndromes	application submitted www.catalystpharma.com
GBT440 (hemoglobin modulator)	Global Blood Therapeutics South San Francisco, CA	treatment of sickle cell disease (SCD)	Phase II www.globalbloodtx.com
glucagon	Biodel Danbury, CT	prevention of hypoglycemia in the congenital hyperinsulinism population	Phase I www.biodel.com
G-Pen™ glucagon injection	Xeris Pharmaceuticals Austin, TX	prevention of chronic, severe hypoglycemia related to congenital hyperinsulinism	Phase II www.xerispharma.com
GS010	Gen Sight Biologics Paris, France	treatment of leber hereditary optic neuropathy	Phase III www.gensight-biologics.com
GSK2696273 (ex-vivo stem cell gene therapy)	GlaxoSmithKline Research Triangle Park, NC	treatment of severe combined immuno- deficiency due to adenosine deaminase deficiency	Phase II/III www.gsk.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
GSK2696274 (ex-vivo stem cell gene therapy)	GlaxoSmithKline Research Triangle Park, NC	treatment of metachromatic leukodystrophy	Phase II www.gsk.com
GSK2696275 (ex-vivo stem cell gene therapy)	GlaxoSmithKline Research Triangle Park, NC	treatment of Wiskott Aldrich syndrome	Phase II www.gsk.com
GZ402666 (neoGAA)	Genzyme Cambridge, MA	treatment of Pompe disease	Phase I www.genzyme.com
GZ402671 (oral GCS inhibitor)	Genzyme Cambridge, MA	treatment of Fabry's disease	Phase II www.genzyme.com
		treatment of Gaucher disease	Phase I www.genzyme.com
HT-100 (halofuginone hydrobromide)	Akashi Therapeutics Cambridge, MA	treatment of Duchenne muscular dystrophy (Fast Track)	Phase II www.halotherapeutics.com
human retinal progenitor cells (hRPC)	jCyte Newport Beach, CA	treatment of retinitis pigmentosa	Phase I/II www.jcyte.com
hydrocortisone modified release capsules	Diurnal Cardiff, Wales	Treatment of congenital adrenal hyperplasia	Phase II www.diurnal.co.uk

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
Ilaris® canakinumab	Novartis Pharmaceuticals East Hanover, NJ	treatment of familial Mediterranean fever	Phase III www.novartis.com
		treatment of hyperimmunoglobulinemia D and periodic fever syndrome	Phase II www.novartis.com
		treatment of TNF-receptor associated periodic syndrome (TRAPS)	Phase II www.novartis.com
IONIS-DMPK-2.5RX	Biogen Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	treatment of myotonic dystrophy Type I	Phase II www.ionispharma.com www.biogen.com
IONIS-HTRx (antisense oligonucleotide)	Ionis Pharmaceuticals Carlsbad, CA Roche Basel, Switzerland	treatment of Huntington's disease	Phase II www.ionispharma.com www.roche.com
IONIS-TTRx (antisense oligonucleotide)	GlaxoSmithKline Research Triangle Park, NC Ionis Pharmaceuticals Carlsbad, CA	treatment of familial amyloid polyneuropathy (Fast Track)	Phase III www.gsk.com www.ionispharma.com
KRN23	Kyowa Kirin USA Princeton, NJ Ultragenyx Pharmaceutical Novato, CA	treatment of X-linked hypophosphatemia (formerly known as vitamin D-resistant rickets)	Phase III www.kyowa-kirin.com www.ultragenyx.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
L-glutamine	Emmaus Medical Torrance, CA	treatment of sickle cell disease (Fast Track)	Phase III www.emmausmedical.com
LCQ908 (pradigastat)	Novartis Pharmaceuticals East Hanover, NJ	treatment of hypertriglyceridemia in the setting of type I hyperlipoproteinemia, also known as familial chylomicronemia syndrome	Phase III www.novartis.com
Lenti-D™ gene therapy	bluebird bio Cambridge, MA	treatment of adrenoleukodystrophy	Phase II/III www.bluebirdbio.com
LentiGlobin® gene therapy	bluebird bio Cambridge, MA	treatment of B-thalassemia major and intermedia	Phase I/II www.bluebirdbio.com
		for the treatment of sickle cell disease	Phase I www.bluebirdbio.com
lucerastat	Actelion Pharmaceuticals South San Francisco, CA	treatment of Fabry disease	Phase I/II www.actelion.com
luspatercept (ACE-536)	Accelaron Pharma Cambridge, MA Celgene Summit, NJ	treatment of B-thalassemia (Fast Track)	Phase III www.accelaronpharma.com www.celgene.com
MA09-hRPE cells	Astellas Pharma US Northbrook, IL Ocata Therapeutics Marlborough, MA	treatment of Stargardt's macular dystrophy	Phase I/II www.astellas.us

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
MBX-8025 (PPAR-delta agonist)	CymaBay Therapeutics Newark, CA	treatment of homozygous familial hypercholesterolemia (HoFH)	Phase II www.cymbabay.com
metadoxine	Alcobra Tel Aviv, Israel	treatment of fragile X syndrome (Fast Track)	Phase II www.alcobrapharma.com
migalastat (AT1001)	Amicus Therapeutics Cranbury, NJ	treatment of Fabry disease	Phase III www.amicusrx.com
MOD-5014 (long-acting recombinant factor VIIa-CTP4)	OPKO Biologics Nes Ziona, Israel	treatment and prophylaxis of bleeding episodes in patients with hemophilia A or B with inhibitors to factor VIII or factor IX	Phase II www.opkobiologics.com
MST188 (poloxamer 188, purified)	Mast Therapeutics San Diego, CA	treatment of sickle cell disease (this includes the treatment and prevention of complications of sickle cell disease)	Phase III www.masttherapeutics.com
N91115 (S-nitrosoglutathione)	Nivalis Therapeutics Boulder, CO	treatment of cystic fibrosis	Phase II www.nivalis.com
N-acetyl-D-mannosamine (ManNAc)	Escala Therapeutics New York, NY	treatment of hereditary inclusion body myopathy type 3	Phase II www.escalabio.com
NKTT120 (IgG1k mAb)	NKT Therapeutics Waltham, MA	treatment of sickle cell disease (Fast Track)	Phase I www.nktrx.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
NLX-101	Neurolinx San Diego, CA	treatment of Rett syndrome	Phase I www.neurolinx.com
NNZ-2566 (trafnetide)	Neuren Pharmaceuticals Camberwell, Australia	treatment of fragile X syndrome (Fast Track)	Phase II www.neurenpharma.com
		treatment of Rett syndrome (Fast Track)	Phase II www.neurenpharma.com
nonocog beta pegol (40K PEGylated recombinant factor IX)	Novo Nordisk Princeton, NJ	routine prophylactic administration for prevention of bleeding in patients with hemophilia B (Christmas disease)	Phase III www.novonordisk.com
NT-501 (ciliary neurotrophic growth factor)	Neurotech USA Cumberland, RI	treatment of retinitis pigmentosa (Fast Track)	Phase II/III www.neurotechusa.com
		treatment of macular telangiectasia type 2 (MacTel)	Phase II www.neurotechusa.com
nusinersen (IONIS-SMNRX)	Biogen Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	treatment of spinal muscular atrophy (Fast Track)	Phase III www.biogen.com www.ionispharma.com
olipudase alfa (rhASM)	Genzyme Cambridge, MA	treatment of acid sphingomyelinase deficiency (Niemann-Pick disease)	Phase II www.genzyme.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
omigapil	Santhera Pharmaceuticals Leistal, Switzerland	treatment of congenital muscular dystrophy	Phase I www.santhera.com
OMS824	Omeros Seattle, WA	treatment of Huntington's disease	Phase II www.omeros.com
palovarotene	Clementia Pharmaceuticals Montreal, Canada	treatment of fibrodysplasia ossificans progressiva (Fast Track)	Phase II www.clementiapharma.com
PAT-001 (isotretinoin)	Patagonia Pharmaceuticals Woodcliff Lake, NJ	treatment of congenital ichthyosis	Phase I www.patagoniarx.com
patisiran (ALN-TTR02) (siRNA inhibitor targeting TTR)	Alnylam Pharmaceuticals Cambridge, MA Genzyme Cambridge, MA	treatment of familial amyloidotic polyneuropathy (Fast Track)	Phase III www.alnylam.com www.genzyme.com
PBT2 (chelating agent)	Prana Biotechnology Parkville, Australia	treatment of Huntington's disease	Phase II www.pranabio.com
pegvaliase (PEG-PAL)	BioMarin Pharmaceutical San Rafael, CA	treatment of hyperphenylalaninemia	Phase III www.biomarin.com
PF-02545920 (PDE10 inhibitor)	Pfizer New York, NY	treatment of Huntington's disease	Phase II www.pfizer.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
PF-04447943	Pfizer New York, NY	treatment of sickle cell disease	Phase I www.pfizer.com
PF-06252616 (myostatin inhibitor)	Pfizer New York, NY	treatment of Duchenne muscular dystrophy (Fast Track)	Phase II www.pfizer.com
plasminogen intravenous	ProMetic Biotherapeutics Quebec, Canada	treatment of hypoplasminogenemia, or type I plasminogen deficiency	Phase II/III www.prometic.com
pridopidine (TV-7820)	Teva Pharmaceutical North Wales, PA	treatment of Huntington's disease	Phase II/III www.tevapharm.com
QLT091001	QLT Vancouver, Canada	treatment of Leber congenital amaurosis (LCA) due to inherited mutations in RPE65 (encoding the protein retinal pigment epithelial protein 65) or LRAT (encoding the enzyme lecithin:retinol acyltransferase) genes	Phase I www.qltinc.com
		treatment of retinitis pigmentosa	Phase I www.qltinc.com
QR-010 (antisense oligonucleotide)	ProQR Therapeutics Palo Alto, CA	treatment of cystic fibrosis	Phase I/II www.proqr.com
rAAV1-CB-hAAT (gene therapy)	Applied Genetic Technologies Alachua, FL	treatment of alpha1-antitrypsin deficiency	Phase II www.agtc.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
rAAV2-CB-hRPE65 (gene therapy)	Applied Genetic Technologies Alachua, FL	treatment of type II Leber's congenital amaurosis	Phase I/II www.agtc.com
Raxone® idebenone	Santhera Pharmaceuticals Leistal, Switzerland	treatment of Duchenne muscular dystrophy (Fast Track)	Phase III www.santhera.com
RE-024 (pantothenate phosphate)	Retrophin San Diego, CA	treatment of pantothenate kinase associated neurodegeneration (PKAN) (Fast Track)	Phase I www.retrophin.com
Resunab ajulemic acid	Corbus Pharmaceuticals Norwood, MA	treatment of cystic fibrosis (Fast Track)	Phase II www.corbuspharma.com
RG6013 (emicizumab)	Chugai Pharma Berkeley Heights, NJ	treatment of hemophilia A	Phase III www.chugai-pharma.com
RG7800 (SMN2 gene-targeted therapy)	PTC Therapeutics South Plainfield, NJ Roche Basel, Switzerland	treatment of spinal muscular atrophy	Phase I/II www.ptcbio.com www.roche.com
rivipansel (GMI-1070) (pan-selectin antagonist)	Pfizer New York, NY	treatment of vaso-occlusive crisis in patients with sickle cell disease (Fast Track)	Phase III www.pfizer.com
RP103 (cysteamine)	Raptor Therapeutics Novato, CA	treatment of Huntington's disease	Phase II/III www.raptorpharma.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
RST100 (non-replicating recombinant adeno-associated virus vector containing a fragment of the gene encoding channel rhodopsin-2 protein)	RetroSense Therapeutics Ann Arbor, MI	treatment of retinitis pigmentosa	Phase I/II www.retro-sense.com
Samsca® tolvaptan	Otsuka Pharmaceuticals Rockville, MD	treatment of autosomal dominant polycystic kidney disease (Fast Track)	application submitted www.otsuka.com
Sanguinate™ pegylated carboxyhemoglobin bovine	Prolong Pharmaceuticals South Plainfield, NJ	treatment of the comorbidities associated with sickle cell disease	Phase II www.prolongpharma.com
SAR339375 (anti-miR21 RNA)	Regulus Therapeutics San Diego, CA Sanofi US Bridgewater, NJ	treatment of Alport syndrome	Phase I www.regulusrx.com www.sanofi.com
SAR422459 (ABCA4 gene therapy)	Sanofi US Bridgewater, NJ	treatment of Stargardt disease	Phase I www.sanofi.com
SBC-103 (rhNAGLU)	Alexion Pharmaceuticals New Haven, CT	treatment of mucopolysaccharidosis IIIB (Sanfilippo B syndrome) (Fast Track)	Phase I/II www.alexion.com
Scenesse® afamelanotide	Clinuvel Melbourne, Australia	treatment of erythropoietic porphyrias	Phase III www.clinuvel.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
SD-101 (allantoin)	Amicus Therapeutics Cranbury, NJ	treatment of skin blistering and erosions associated with inherited epidermolysis bullosa (Breakthrough Therapy)	Phase II www.amicusrx.com
SD-809 (VMAT2 inhibitor)	Auspex Pharmaceuticals La Jolla, CA	treatment of Huntington's disease	application submitted www.auspexpharma.com
SelG1 (humanized IgG2 antibody)	Selexys Pharmaceuticals Oklahoma City, OK	treatment of vaso-occlusive crisis in patients with sickle cell disease	Phase II www.selexys.com
setmelanotide (MC4R agonist)	Rhythm Metabolics Boston, MA	treatment of Prader-Willi syndrome	Phase II www.rhythmtx.com
SHP610 (sulfamidase)	Shire Lexington, MA	tor treatment of Sanfilippo syndrome (MPS IIIA)	Phase II www.shire.com
SHP611 (arylsulfatase A)	Shire Lexington, MA	treatment of metachromatic leukodystrophy	Phase I/II www.shire.com
SHP625 (sodium bile cotransporter inhibitor)	Shire Lexington, MA	treatment of alagille syndrome	Phase II www.shire.com
		treatment of progressive familial intrahepatic cholestasis	Phase II www.shire.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
Spiriva® Handihaler® tiotropium bromide	Boehringer Ingelheim Pharmaceuticals Ridgefield, CT	to improve pulmonary function in conjunction with standard therapy in the management of patients with cystic fibrosis	Phase III www.boehringer-ingelheim.com
SPK-CHM (gene therapy)	Spark Therapeutics Philadelphia, PA	treatment of choroideremia due to mutations in the human choroideremia gene (CHM)	Phase I/II www.sparkstx.com
SPK-FIX (blood coagulation factor)	Spark Therapeutics Philadelphia, PA	treatment of hemophilia B	Phase I/II www.sparktx.com
SPK-RPE65 (gene transference)	Spark Therapeutics Philadelphia, PA	treatment of retinitis pigmentosa due to autosomal recessive RPE65 gene mutations	Phase III www.sparktx.com
SRP-4053 (exon 53)	Sarepta Therapeutics Cambridge, MA	treatment of Duchenne muscular dystrophy in patients with a deletion, duplication or frame shift mutation correctable by skipping of exon 50 of the dystrophin gene to allow restoration of the reading frame (including mutations within exons 51, 51-53, or 51-55)	Phase II www.sareptatherapeutics.com
TD-101 (small interfering RNA)	TransDerm Santa Cruz, CA	treatment of pachyonychia congenita	Phase II www.transdermnc.com
Thiolanox® Nitric oxide	Novoteris Garden Grove, CA	treatment of cystic fibrosis	Phase I www.novoteris.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
Translarna ataluren	PTC Therapeutics South Plainfield, NJ	treatment of cystic fibrosis (Fast Track)	Phase III www.ptcbio.com
		treatment of mucopolysaccharidosis type I	Phase I www.ptcbio.com
trehalose (protein aggregation inhibitor)	BioBlast Pharma New Haven, CT	treatment of spinal cerebellar ataxia type 3 (also known as SCA3 or Machado Joseph disease)	Phase II www.bioblast-pharma.com
		treatment of oculopharyngeal muscular dystrophy (Fast Track)	Phase II www.bioblast-pharma.com
TXA127 (angiotensin 1-7)	Tarix Orphan Cambridge, MA	treatment of Duchenne muscular dystrophy muscular dystrophy (Fast Track)	Phase II www.tarixorphan.com
		treatment of LAMA2-related muscular dystrophy (MDC1A)	Phase I www.tarixorphan.com
UshStat® MT07A gene therapy	Sanofi US Bridgewater, NJ	treatment of retinitis pigmentosa associated with Usher syndrome 1B gene defect	Phase I www.sanofi.com
UX001 (aceneuramic acid)	Ultragenyx Pharmaceutical Novato, CA	treatment of hereditary inclusion body myopathy	Phase III www.iltragenyx.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
UX003 (recombinant human beta-glucuronidase)	Ultragenyx Pharmaceutical Novato, CA	treatment of mucopolysaccharidosis VII (MPS VII, Sly Syndrome) (Fast Track)	Phase III www.ultragenyx.com
UX007 (triheptanoin)	Ultragenyx Pharmaceutical Novato, CA	treatment of glucose transporter type-1 deficiency syndrome	Phase II www.ultragenyx.com
		treatment of fatty acid oxidation disorders	Phase II www.ultragenyx.com
vatiquinone	Edison Pharmaceuticals Mountain View, CA	treatment of Friedreich's ataxia	Phase II www.edisonpharma.com
VBP15	ReveraGen Biopharma Rockville, MD	treatment of Duchenne muscular dystrophy	Phase I www.reveragen.com
volanesorsen	Akcea Therapeutics Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	treatment of familial chylomicronemia syndrome	Phase I www.akceatx.com www.ionispharma.com
VTS-270	Vtesse Gaithersburg, MD	treatment of Niemann Pick disease, type C	Phase II/III www.vtessepharma.com
VX-661	Vertex Pharmaceuticals Boston, MA	treatment of cystic fibrosis	Phase III www.vrtx.com

Genetic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
Vyndaqel® tafamidis meglumine	Pfizer New York, NY	treatment of familial amyloid polyneuropathy (Fast Track)	application submitted www.pfizer.com
WTX-101 (bis-choline tetrathiomolybdate)	Wilson Therapeutics Westford, MA	treatment of Wilson's disease	Phase II www.wilsontherapeutics.com
Xenbilox® chenodeoxycholic acid	Sigma-Tau Pharmaceuticals Gaithersburg, MD	treatment of cerebrotendinous xanthomatosis	application submitted www.sigmatau.com
XOMA 358 (IgG2 mAb)	XOMA Berkeley, CA	treatment of congenital hyperinsulinism	Phase II www.xoma.com
ZX-008 (fenfluramine low-dose)	Zogenix Emeryville, CA	for the treatment of Dravet syndrome (Fast Track)	Phase III www.zogenix.com

Growth Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
BMN111 (recombinant CNP)	BioMarin Pharmaceutical San Rafael, CA	treatment of achondroplasia	Phase II www.biomin.com
Debio8206 triptorelin pamoate)(Debiopharm Lausanne, Switzerland	treatment of central precocious puberty	Phase III www.debiopharm.com
Macrilen® macimorelin acetate	Aeterna Zentaris South San Francisco, CA	diagnosis of growth hormone deficiency	Phase III www.aezsinc.com

Growth Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
octreotide (oral)	Chiasma Waltham, MA	for the oral treatment of acromegaly	application submitted www.chiasmapharma.com
PF-06836922 (MOD-4023) (HGH agonist)	OPKO Biologics Miami, FL Pfizer New York, NY	treatment of growth hormone deficiency (adults)	Phase III www.pfizer.com
		treatment of growth hormone deficiency (pediatric)	Phase II www.pfizer.com
somavaratan (fusion protein/rhGH)	Versartis Menlo Park, CA	treatment of growth hormone deficiency	Phase III www.versartis.com
TV-1106 (albutropin)	Teva Pharmaceuticals North Wales, PA	treatment of growth hormone deficiency	Phase III www.tevapharm.com

Infectious Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
AB103 (CD28 antigen inhibitor)	Atox Bio Ness Ziona, Israel	treatment of necrotizing soft tissue infections (NSTI) (Fast Track)	Phase III www.atoxbio.com
AB569 (bacterial growth modulator)	Arch Biopartners Toronto, Canada	treatment of <i>Pseudomonas aeruginosa</i> pulmonary infections in patients with with cystic fibrosis	Phase I www.archbiopartners.com

Infectious Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
AeroVanc™ vancomycin hydrochloride (inhalation)	Savara Pharmaceuticals Austin, TX	treatment of persistent methicillin-resistant <i>S. aureus</i> lung infection in patients with cystic fibrosis	Phase II www.savarapharma.com
anti-Ebola mAb	LeafBio San Diego, CA Mapp Biopharmaceutical San Diego, CA	treatment of Ebola virus infection	Phase I/II www.leafbio.com
AriKayce™ liposomal amikacin	Insmed Bridgewater, NJ	treatment infections caused by non-tuberculous mycobacteria (Fast Track)	Phase III www.insmed.com
		treatment of bronchiectasis in patients with <i>Pseudomonas aeruginosa</i> or other susceptible microbial pathogens	Phase II www.insmed.com
ASP0113 (cytomegalovirus DNA vaccine)	Astellas Pharma US Northbrook, IL	prevention of clinically significant cytomegalovirus (CMV) viremia, CMV disease and associated complications in at-risk hematopoietic cell transplant populations	Phase III www.astellas.us
		prevention of clinically significant cytomegalovirus (CMV) viremia, CMV disease and associated complications in solid transplant populations	Phase II www.astellas.us

Infectious Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
AVI-7288 (antisense oligonucleotide)	Sarepta Therapeutics Cambridge, MA	prophylaxis for patients following documented or suspected exposure to marburg virus	Phase I www.sarepta.com
AVI-7537 (antisense oligonucleotide)	Sarepta Therapeutics Cambridge, MA	prophylaxis for patients following documented or suspected exposure to ebolavirus	Phase I www.sarepta.com
CD101 IV (long-acting echinocandin)	Cidara Therapeutics San Diego, CA	treatment of candidemia and invasive candidiasis infections caused by susceptible Candida spp. (Fast Track)	Phase I www.cidara.com
CEM-102 (sodium fusidate)	Cempra Pharmaceuticals Chapel Hill, NC	treatment of patients with prosthetic joint infections	Phase II/III www.cempra.com
clofazimine	Novartis Pharmaceuticals East Hanover, NJ	treatment of active tuberculosis	Phase II/III www.novartis.com
Civacir® hepatitis C virus immune globulin	Biotest Pharmaceuticals Boca Raton, FL	prophylaxis of hepatitis C infection in liver transplant recipients	Phase III www.biotest.com
delamanid (OPC-67683)	Otsuka Pharmaceutical Rockville, MD	treatment of pulmonary tuberculosis	Phase III www.otsuka.com
Dificid® fidaxomicin	Merck Kenilworth, NJ	treatment of pediatric <i>Clostridium difficile</i> infection	Phase III www.merck.com

Infectious Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
GS-5734 (Nuc inhibitor)	Gilead Sciences Foster City, CA	treatment of Ebola virus disease	Phase I www.gilead.com
letermovir (NK-8228)	Merck Kenilworth, NJ	prevention of human cytomegalovirus viremia and disease in at risk populations (Fast Track)	Phase III www.merck.com
MP-376 (levofloxacin inhalation)	Raptor Pharmaceutical Novato, CA	treatment of pulmonary infections due to <i>Pseudomonas aeruginosa</i> and other bacteria in patients with cystic fibrosis	Phase III www.raptorpharma.com
nifurtimox	Bayer HealthCare Pharmaceuticals Whippany, NJ	treatment of Chagas disease (American Trypanosomiasis) caused by <i>T. cruzi</i>	Phase III www.pharma.bayer.com
pleconaril	AntiVirus Therapeutics Princeton Junction, NJ	treatment of symptomatic entoviral infection in the neonate	Phase II
Q203	Qurient Gyeonggi-do, South Korea	treatment of active tuberculosis (TB)	Phase I www.qurient.com
RBX2660 (fecal microbiota)	Rebiotx Roseville, MN	treatment of recurrent <i>Clostridium difficile</i> infection (<i>Clostridium difficile</i> gastrointestinal disease) (Fast Track)	Phase II www.rebiotx.com
REMUNE HIV vaccine	Immune Response BioPharma Atlantic City, NJ	treatment of pediatric HIV/AIDS (age through 16 years)	Phase II www.immuneresponsebiopharma.com

Infectious Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
Sarasar® lonafarnib	Eiger Biopharmaceuticals Palo Alto, CA	treatment of hepatitis delta virus (HDV) infection	Phase II www.eigerbio.com
SER-109 (bacteria replacement)	Seres Health Cambridge, MA	treatment of recurrent <i>Clostridium difficile</i> infection (CDI) (Breakthrough Therapy)	Phase II www.serestherapeutics.com
SHP620 (maribavir)	Shire Lexington, MA	treatment of clinically significant cytomegalovirus viremia and disease in at-risk patients	Phase II www.shire.com
SQ109 (ethylenediamine)	Sequella Rockville, MD	treatment of tuberculosis	Phase II www.sequella.com
tafenoquine	GlaxoSmithKline Research Triangle Park, NC	treatment of malaria	Phase III www.gsk.com
tecovirimat	SIGA Technologies New York, NY	treatment of orthopoxvirus infections (Fast Track)	Phase III www.siga.com
TMB-355 ibalizumab	TaiMed Biologics Taipei City, Taiwan	treatment of HIV-1 infection in treatment experienced adult patients with documented multi-antiretroviral class resistance and evidence of HIV-1 replication despite ongoing antiretroviral therapy (Fast Track)	application submitted www.taimedbiologics.com

Infectious Diseases

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
ViekiraPak™ ombitasvir/paritaprevir/ritonavir	AbbVie North Chicago, IL	treatment of pediatric patients with chronic hepatitis C virus infection	Phase III www.abbvie.com
VT-1129 (selective inhibitor of fungal lanosterol demethylase)	Viamet Pharmaceuticals Durham, NC	treatment of cryptococcal meningitis	Phase I www.viamet.com

Neurologic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
ABBV-8E12/C2N-8E12 (recombinant human anti-tau antibody)	AbbVie North Chicago, IL C2N Diagnostics St. Louis, MO	treatment of progressive supranuclear palsy	Phase I www.abbvie.com www.c2ndiagnostics.com
ADS-5102 (amantadine ER)	Adamas Pharmaceuticals Emeryville, CA	treatment of levodopa-induced dyskinesia	Phase III www.adamaspharma.com
Amiket® amitriptyline and ketamine	Immune Pharmaceuticals New York, NY	treatment of postherpetic neuralgia	Phase II www.immunepharmaceuticals.com
AST-OPC1 (oligodendrocyte progenitor stem cell therapy)	Asterias Biotherapeutics Fremont, CA	treatment of acute spinal cord injury	Phase I/II www.asteriasbiotherapeutics.com
AVXS-101 (adeno-associated virus)	AveXis Bannockburn, IL	treatment of spinal muscular atrophy (Fast Track)	Phase I www.avexis.com

Neurologic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
BMS-986168 (anti-eTau IgG4 mAb)	Bristol-Myers Squibb Princeton, NJ	treatment of progressive supranuclear palsy	Phase I www.bms.com
bupivacaine transdermal	Impax Laboratories Hayward, CA	relief of persistent pain associated with postherpetic neuralgia	Phase II www.impaxlabs.com
cannabidiol	GW Pharma Salisbury, England	treatment of Lennox-Gastaut syndrome	Phase III www.gwpharm.com
cannabidiol	Insys Therapeutics, Inc. Phoenix, AZ	treatment of Lennox-Gastaut syndrome	Phase III www.insysrx.com
		treatment of infantile spasms	Phase II www.insysrx.com
carbon dioxide intranasal	Capnia Redwood City, CA	treatment of trigeminal neuralgia	Phase II www.capnia.com
Cirara™ glibenclamide	Remedy Pharmaceuticals New York, NY	treatment of acute spinal cord injury	Phase II www.remedypharmaceuticals.com
CPP-115 (GABA-AT inhibitor)	Catalyst Pharmaceuticals Coral Gables, FL	treatment of infantile spasms	Phase I www.catalystpharma.com
dipraglurant	Addex Therapeutics Geneva, Switzerland	treatment of levodopa-induced dyskinesias	Phase II www.addextherapeutics.com

Neurologic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
ecopipam hydrochloride	Psyadon Pharmaceuticals Germantown, MD	treatment of Tourette's syndrome in children 0-16 years old	Phase II www.psyadonrx.com
eltoprazine	Amarantus BioScience San Francisco, CA	treatment of levodopa-induced dyskinesia associated with Parkinson's disease	Phase II www.amarantus.com
ganaxolone (GABA-A receptor agonist)	Marinus Pharmaceuticals Radnor, PA	treatment of protocadherin 19 (PCDH19) female epilepsy	Phase II www.marinuspharma.com
GM06 (oligopeptide)	Genervon Biopharmaceuticals Pasadena, CA	treatment of amyotrophic lateral sclerosis (Fast Track)	Phase II www.genervon.com
H.P. Acthar® Gel repository corticotropin injection	Mallinckrodt Pharmaceuticals St. Louis, MO	treatment of amyotrophic lateral sclerosis	Phase II www.mallinckrodt.com
inhaled xenon gas	Neuroprotexon Orchard Park, NY	to improve neurological outcome in hospitalized cardiac arrest patients	Phase II www.neuroprotexon.com
intravenous carbamazepine	Lundbeck Deerfield, IL	treatment of epilepsy patients who cannot take anything by mouth (NPO)	application submitted www.lundbeck.com
IONIS-SOD111RX	Biogen Cambridge, MA Ionis Pharmaceuticals Carlsbad, CA	treatment of amyotrophic lateral sclerosis	Phase II www.ionispharma.com www.biogen.com

Neurologic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
JZP-110 (phenylalanine derivative)	Jazz Pharmaceuticals Dublin, Ireland	treatment of narcolepsy	Phase III www.jazzpharma.com
masitinib	AB Science Paris, France	treatment of amyotrophic lateral sclerosis	Phase III www.ab-science.com
NurOwn™ mesenchymal stromal cells secreting neurotrophic factors	BrainStorm Cell Therapeutics Hackensack, NJ	treatment of amyotrophic lateral sclerosis (Fast Track)	Phase II www.brainstormcell.com
midazolam intranasal spray	Upsher-Smith Laboratories Maple Grove, MN	rescue treatment of seizures in patients who who require control of intermittent bouts of of increased seizure activity (e.g. acute repetitive seizures, seizure clusters) (Fast Track)	Phase III www.upsher-smith.com
NP001 (sodium chlorite)	Neuraltus Pharmaceuticals Palo Alto, CA	for slowing the progression of amyotrophic lateral sclerosis (Fast Track)	Phase II www.neuraltus.com
NRL-1 diazepam (intranasal)	Neurelis Pharmaceuticals San Diego, CA	management of acute repetitive seizures	Phase III www.neurelis.com
NSI-566 (stem cell therapy)	Neuralstem Germantown, MD	treatment of amyotrophic lateral sclerosis	Phase II www.neuralstem.com
OS-320 (amantadine)	Osmotica Pharmaceutical Marietta, GA	treatment of levodopa-induced dyskinesia	Phase III www.osmotica.com

Neurologic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
Plumiaz™ diazepam (intranasal)	Acorda Therapeutics Ardsley, NY	management of patients with acute repetitive seizures	Phase III www.acorda.com
SAGE-547 (allopregnanolone)	Sage Therapeutics Cambridge, MA	treatment of status epilepticus	Phase III www.sagerx.com
SD-809 (VMAT2 inhibitor)	Auspex Pharmaceuticals La Jolla, CA	treatment of Tourette syndrome in the pediatric population (defined as 0 through 16 years of age)	Phase I www.auspexpharma.com
tirasemtiv	Cytokinetics South San Francisco, CA	treatment of amyotrophic lateral sclerosis (ALS) (Fast Track)	Phase III www.cytokinetics.com
topiramate injection	CURx Pharmaceuticals San Diego, CA	treatment of partial onset or primary generalized tonic-clonic seizures for hospitalized epilepsy patients or epilepsy patients being treated in an emergency care setting who are unable to take oral topiramate	Phase I www.curxpharma.com
TPI 287 (abeotaxane inhibitor)	Cortice Biosciences New York, NY	treatment of progressive supranuclear palsy	Phase I www.corticebiosciences.com
VM202 (hepatocyte growth factor-X7)	ViroMed Seoul, South Korea	treatment of amyotrophic lateral sclerosis	Phase II www.viromed.co.kr

Neurologic Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
VX-210 (recombinant fusion protein)	Vertex Pharmaceuticals Boston, MA	treatment of acute spinal cord injury (Fast Track)	Phase II www.vrtx.com
zoledronic acid	Axsome Therapeutics New York, NY	treatment of complex regional pain syndrome (CRPS) (Fast Track)	Phase III www.axsome.com

Respiratory Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
ARD-3150 (ciprofloxacin inhalation)	Aradigm Hayward, CA	the management of bronchiectasis	Phase III www.aradigm.com
BG00011 (STX-100)	Biogen Cambridge, MA	treatment of idiopathic pulmonary fibrosis	Phase II www.biogen.com
CC-90001	Celgene Summit, NJ	treatment of idiopathic pulmonary fibrosis	Phase I www.celgene.com
Cipro-DPI ciprofloxacin dry powder inhaler	Bayer HealthCare Pharmaceuticals Whippany, NJ	treatment of non-cystic fibrosis bronchiectasis	Phase III www.pharma.bayer.com
FG-3019 (pamrevlumab)	FibroGen San Francisco, CA	treatment of idiopathic pulmonary fibrosis	Phase II www.fibrogen.com
LAM-001 (mTOR inhibitor inhaled)	LAM Therapeutics Guilford, CT	treatment of lymphangioleiomyomatosis	Phase I www.lamtherapeutics.com

Respiratory Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
lebrikizumab	Genentech South San Francisco, CA	treatment of idiopathic pulmonary fibrosis	Phase II www.gene.com
MN-001 (tipelukast)	MediciNova La Jolla, CA	treatment of idiopathic pulmonary fibrosis (Fast Track)	Phase II www.medicinova.com
Pneumostem® human umbilical cord blood- derived mesenchymal stem cells	Medipost America Rockville, MD	prevention of bronchopulmonary dysplasia	Phase I/II www.medipostamerica.com
PRM-151 (rhPentraxin-2/rhSerum Amyloid P)	Promedior Lexington, MA	treatment of idiopathic pulmonary fibrosis	Phase II www.promedior.com
SAR156597 (IL4/IL13 bi-specific Ab)	Sanofi US Bridgewater, NJ	treatment of idiopathic pulmonary fibrosis	Phase II www.sanofi.com

Transplantation

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
alpha-1 proteinase inhibitor (AAT)	Baxalta Bannockburn, IL Kamada Ness Ziona, Israel	treatment of graft versus host disease	Phase I/II www.baxalta.com
anti-T-lymphocyte immune globulin	Neovii Biotech Munich, Germany	prevention of graft versus host disease (GVHD)	Phase III www.neoviibiotech.com

Transplantation Product Name	Sponsor	Official FDA Designation*	Development Status
BPX-501 (donor T cells + CaspaCIDE safgety switch)	Bellicum Pharmaceuticals Houston, TX	replacement T-cell therapy for the treatment of immunodeficiency and graft versus host disease after allogeneic hematopoietic stem cell transplant	Phase I/II www.bellicum.com
emricasan	Conatus Pharmaceuticals San Diego, CA	treatment of liver transplant recipients with reestablished fibrosis to delay the progression to cirrhosis and end stage liver disease	Phase II www.conatuspharma.com
FCR001	Novartis Pharmaceuticals East Hanover, NJ RegeneRx Biopharmaceuticals Louisville, KY	prevention of organ rejection in living donor kidney transplant recipients	Phase II www.novartis.com www.regenerx.com
FT1050 (Prohema)	Fate Therapeutics San Diego, CA	enhancement of stem cell engraftment through ex vivo treatment of human allogeneic hematopoietic stem cells (treatment of neutropenia, thrombocytopenia, lymphopenia, and anemia)	Phase II www.fatetherapeutics.com
Multistem® (allogeneic stem cell therapy)	Athersys Cleveland, OH	prophylaxis of graft vs host disease (Fast Track)	Phase I www.athersys.com
QPI-1002 (siRNA oligonucleotide)	Quark Pharmaceuticals Fremont, CA	prophylaxis of delayed graft function in renal transplant patients	Phase III www.quarkpharma.com

Transplantation

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
refanalin (BB3) (HGF mimetic)	Angion Biomedica Uniondale, NY	to improve renal function and prevent delayed graft function following renal transplantation (Fast Track)	Phase III www.angion.com
reparixin IL18A/B receptor antagonist)	Dompe Milan, Italy	prevention of graft loss in pancreatic islet transplantation	Phase III www.dompe.com
RGI-2001 (liposomal galactosylceramide)	REGIMMUNE San Francisco, CA	prevention of graft-versus-host disease	Phase II www.regimmune.com
Soliris® eculizumab	Alexion Pharmaceuticals New Haven, CT	prevention of delayed graft function after renal transplantation	Phase III www.alexion.com
TZ101 (human recombinant enzyme)	TargaZyme Carlsbad, CA	to increase the activity of regulatory T-cells (Tregs) used in the prevention of graft versus host disease in patients undergoing poietic stem cell transplantation	Phase III www.targazyme.com

Other Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
alpha1-proteinase inhibitor (human) modified process	Grifols Therapeutics Los Angeles, CA	treatment of type 1 diabetes mellitus patients with residual beta-cell function	Phase II www.grifolsusa.com
Ampligen® rintatolimod	Hemispherx Biopharma Philadelphia, PA	treatment of chronic fatigue syndrome	application submitted www.hemispherx.net

Other Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
BAF312 (siponimod)	Novartis Pharmaceuticals East Hanover, NJ	treatment of polymyositis	Phase II www.novartis.com
BIO 300 (radioprotective)	Humanetics Minneapolis, MN	prevention of acute radiation syndrome	Phase I www.humaneticscorp.com
BLU-285 (exon 17 mutant KIT inhibitor)	Blueprint Medicines Cambridge, MA	treatment of mastocytosis	Phase I www.blueprintmedicines.com
CCX168 (complement 5a receptor antagonist)	ChemoCentryx Mountain View, CA	treatment of atypical hemolytic uremic syndrome	Phase II www.chemocentryx.com
CN-105 (apolipoprotein E agonist)	CereNova Durham, NC	treatment of intracerebral hemorrhage	Phase I www.cerenova.com
COR-003	Stonebridge Biopharma Trevose, PA	treatment of endogenous Cushing's syndrome	Phase III www.stonebridgebio.com
Debrase® bromelain topical	MediWound Yvane, Israel	debridement of acute, deep dermal burns in hospitalized patients	Phase III www.mediwound.com
Diamyd® recombinant human glutamic acid decarboxylase 65KDa isoform	Diamyd Medical Stockholm, Sweden	treatment of Type I diabetes with residual beta cell function	Phase III www.diamyd.com
DigiBind® digitoxin immune fab (ovine)	Glenveigh Medical Chattanooga, TN	treatment of severe preeclampsia and eclampsia (Fast Track)	Phase II www.glenveigh.com

Other Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
DV-100 (dendritic cell therapy)	DiaVacs Edgewater, NJ	treatment of type 1 diabetes mellitus patients with residual beta cell function	Phase II www.diaavacs.com
EG-1962 (intraventricular nimodipine)	Edge Therapeutics Berkeley Heights, NJ	treatment of subarachnoid hemorrhage	Phase II www.edgetherapeutics.com
ELAD® immortalized human liver cells/ extracorporeal liver assist device	Vital Therapies San Diego, CA	treatment of fulminant hepatic failure (acute liver failure)	Phase III www.vitaltherapies.com
entolimod	Cleveland BioLabs Buffalo, NY	prevention of death following a potentially lethal dose of total body irradiation during or after a radiation disaster (Fast Track)	Phase I www.cbiolabs.com
EX-404	EffRx Pharmaceuticals Freienbark, Switzerland	treatment of pediatric polycystic ovary syndrome	Phase II/III www.effrx.com
EX-RAD™ recilisib sodium	Onconova Therapeutics Newtown, PA	prevention of acute radiation syndrome	Phase I www.onconova.com
GC4419 (superoxide dismutase mimetic)	Galera Therapeutics Malvern, PA	prevention of radiation- or chemotherapy- induced oral mucositis in cancer patients (Fast Track)	Phase II www.galeratx.com
GSK2398852 + GSK2315698 (serum amyloid P component [SAP] mAb + SAP depleter (CPHPC)	GlaxoSmithKline Research Triangle Park, NC	treatment of AL amyloidosis	Phase II www.gsk.com

Other Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
ixazomib (MLN9708)	Millennium Pharmaceuticals Cambridge, MA	treatment of systemic light chain (AL) amyloidosis	Phase III www.millennium.com
Kiacta™ eprodiate	Auven Therapeutics New York, NY	treatment of secondary amyloidosis	Phase III www.auventx.com
LCI699 (osilodrostat)	Novartis Pharmaceuticals East Hanover, NJ	treatment of Cushing's disease	Phase III www.novartis.com
LG631 (stem cell gene therapy)	Opus Bio Greenwich, CT	for bone marrow protection in the treatment of glioblastoma multiforme	Phase I www.opusbio.com
LPCN 1107 (hydroxyprogesterone)	Lipocine Salt Lake City, UT	prevention of preterm birth in women with a singleton pregnancy	Phase I www.lipocine.com
midostaurin (PKC412)	Novartis Pharmaceuticals East Hanover, NJ	treatment of mastocytosis	Phase I www.novartis.com
NaBen® benzoate	SyneuRx Taipei City, Taiwan	treatment of pediatric schizophrenia	Phase II/III www.syneurx.com
naloxone topical	Elorac Vernon Hills, IL	topical treatment of pruritus associated with mycosis fungoides (Fast Track)	Phase III www.eloracpharma.com
NEOD001 (anti-IgG1 anti-serum amyloid A mAb)	Prothena Therapeutics South San Francisco, CA	treatment of AA amyloidosis and AL amyloidosis (Fast Track)	Phase III www.prothena.com

Other Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
NI-0501 (mAb)	NovImmune Geneva, Switzerland	treatment of hemophagocytic lymphohistiocytosis	Phase II www.novimmune.com
NT-100 (rhGCSF)	Nora Therapeutics Palo Alto, CA	prevention of implantation failure	Phase II www.noratherapeutics.com
obeticholic acid (FXR agonist)	Intercept Pharmaceuticals New York, NY	treatment of primary sclerosing cholangitis (Fast Track)	Phase II www.interceptpharma.com
OCT-002 (L-ornithine phenylacetate)	Ocera Therapeutics Palo Alto, CA	treatment of hyperammonemia and resultant hepatic encephalopathy (HE) in patients with acute liver failure or acute on chronic liver disease (Fast Track)	Phase II www.ocerainc.com
OMS-721 (MASP-2)	Omeros Seattle, WA	prevention (inhibition) of complement- mediated thrombotic microangiopathy (Fast Track)	Phase II www.omeros.com
ONO-01210 (recilisib sodium)	Onconova Therapeutics Newton, MA	treatment of acute radiation syndrome	Phase I www.onconova.com
OrbeShield® beclomethasone dispropionate	Soligenix Princeton, NJ	treatment of acute radiation syndrome (Fast Track)	Phase I www.soligenix.com
Oxabact® oxalobacter formigenes	OxThera Stockholm, Sweden	treatment of primary hyperoxaluria	Phase II/III www.oxthera.com

Other Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
PA101B (cromolyn sodium for inhalation)	Patara Pharma San Diego, CA	treatment of mastocytosis	Phase II www.patarapharma.com
PF-05230907 (pan-HER inhibitor)	Pfizer New York, NY	treatment of intracerebral hemorrhage	Phase I www.pfizer.com
revusiran (RNA interference)	Alnylam Pharmaceuticals Cambridge, MA Genzyme Cambridge, MA	treatment of transthyretin amyloidosis	Phase III www.alnylam.com www.genzyme.com
RiVax™ ricin vaccine	Soligenix Princeton, NJ	prevention of ricin intoxication	Phase I www.soligenix.com
SHP625 (sodium bile cotransporter inhibitor)	Shire Lexington, MA	treatment of primary sclerosing cholangitis	Phase II www.shire.com
silibinin	Meda Pharmaceuticals Somerset, NJ	treatment of amatoxin poisoning, which includes the prevention and treatment of amatoxin induced hepatic failure	Phase II/III www.medapharma.us
simtuzumab	Gilead Sciences Foster City, CA	treatment of primary sclerosing cholangitis	Phase II www.gilead.com

Other Disorders

<u>Product Name</u>	<u>Sponsor</u>	<u>Official FDA Designation*</u>	<u>Development Status</u>
skin tissue replacement therapy	Stratatech Madison, WI	treatment of hospitalized patients with complex skin defects resulting from partial and full thickness skin burns requiring excision and grafting	Phase I/II www.stratatechcorp.com
sodium thiosulfate (STS)	Fennec Pharma Research Triangle Park, NC	prevention of platinum-induced ototoxicity in pediatric patients	Phase III www.fenecpharma.com
sparsentan	Retrophin San Diego, CA	treatment of focal segmental glomerulosclerosis	Phase II/III www.retrophin.com
Systebryl™ PTI-111	ProteoTech Kirkland, WA	treatment of AL amyloidosis	Phase I
TV-45070 (funapide)	Teva Pharmaceuticals North Wales, PA	treatment of erythromelalgia (Fast Track)	Phase II www.tevapharm.com
vonapanitase (PRT-201)	Proteon Therapeutics Waltham, MA	prevention of arteriovenous fistula and arteriovenous graft failure in patients with chronic kidney disease (CKD) who are receiving hemodialysis or preparing for hemodialysis (Fast Track)	Phase III www.proteontherapeutics.com

The content of this report has been obtained through public, government and industry sources, and the Springer "Adis Insight" database based on the latest information. **Report current as of April 18, 2016.** 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. The entire series of *Medicines in Development* is available on PhRMA's website, 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 fewer than 200,000 people in the United States, or that affects more than 200,000 persons but is not expected to recover the costs of its development and marketing.

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.