

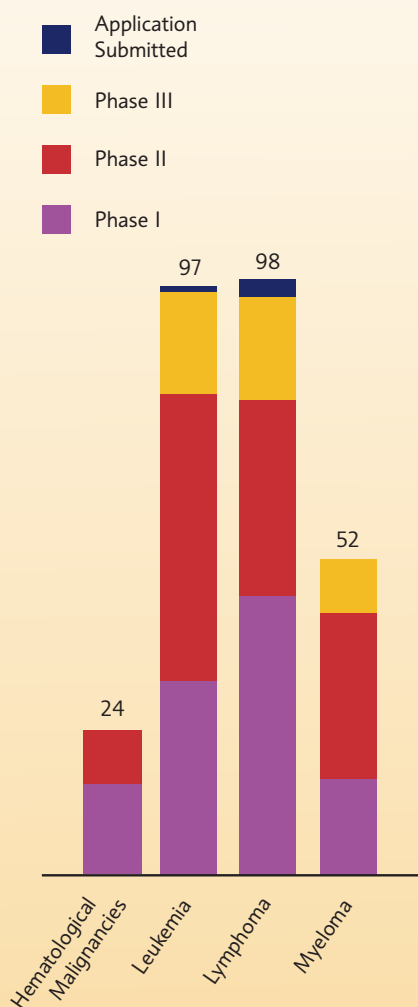
# Leukemia & Lymphoma

A Report on Cancers of the Blood

PRESENTED BY AMERICA'S BIOPHARMACEUTICAL RESEARCH COMPANIES

## More Than 240 Medicines in Development for Leukemia, Lymphoma and Other Blood Cancers

### Medicines in Development For Leading Blood Cancers



**Every 4 minutes  
a person is diagnosed  
with leukemia,  
lymphoma or  
myeloma;  
Accounting for  
9% of all cancers  
diagnosed each year**



Biopharmaceutical research companies are developing 241 medicines for blood cancers—leukemia, lymphoma and myeloma. This report lists medicines in human clinical trials or under review by the U.S. Food and Drug Administration (FDA).

The medicines in development include:

- 98 for lymphoma, including Hodgkin and non-Hodgkin lymphoma, which affect nearly 80,000 Americans each year.
- 97 for leukemia, including the four major types, which affect nearly 50,000 people in the United States each year.
- 52 for myeloma, a cancer of the plasma cells, which impacts more than 22,000 people each year in the United States.
- 24 medicines are targeting hematological malignancies, which affect bone marrow, blood and lymph nodes.

- 15 each for myeloproliferative neoplasms, such as myelofibrosis, polycythemia vera and essential thrombocythemia; and for myelodysplastic syndromes, which are diseases affecting the blood and bone marrow.

These medicines in development offer hope for greater survival for the thousands of Americans who are affected by these cancers of the blood.

Definitions for the cancers listed in this report and other terms can be found on page 27. Links to sponsor company web sites provide more information on the potential products.

For information on the value of medicines, an in-depth look at current innovation and key medical breakthroughs benefiting blood cancer patients, please see *Medicines in Development for Leukemia and Lymphoma 2013—Overview*.

## Hematological Malignancies

Product Name	Sponsor	Indication	Development Phase*
<b>Adcetris®</b> brentuximab vedotin (Orphan Drug)	Millennium Pharmaceuticals <i>Cambridge, MA</i> Seattle Genetics <i>Bothell, WA</i>	treatment of CD30-positive hematological malignancies (see also lymphoma)	Phase II <a href="http://millennium.com">millennium.com</a> <a href="http://seattlegenetics.com">seattlegenetics.com</a>
AMG 319 (PI3K-delta inhibitor)	Amgen <i>Thousand Oaks, CA</i>	hematological malignancies	Phase I <a href="http://amgen.com">amgen.com</a>
anti-CD22 antibody-drug conjugate (DCDT 2980S)	Genentech <i>South San Francisco, CA</i>	hematological malignancies (see also lymphoma)	Phase II <a href="http://gene.com">gene.com</a>
anti-CD79b antibody-drug conjugate (DCDS 4501A)	Genentech <i>South San Francisco, CA</i>	hematological malignancies (see also lymphoma)	Phase II <a href="http://gene.com">gene.com</a>
AR-42 (pan-DAC inhibitor)	Arno Therapeutics <i>Flemington, NJ</i>	hematological malignancies	Phase I/II <a href="http://arnothera.com">arnothera.com</a>
AZD1208 (PIM kinase inhibitor)	AstraZeneca <i>Wilmington, DE</i>	hematological malignancies	Phase I <a href="http://astrazeneca.com">astrazeneca.com</a>
AZD9150 (STAT3 inhibitor)	AstraZeneca <i>Wilmington, DE</i>	hematological malignancies	Phase I <a href="http://astrazeneca.com">astrazeneca.com</a>
belinostat (HDAC inhibitor)	Spectrum Pharmaceuticals <i>Henderson, NV</i>	hematological malignancies (see also lymphoma)	Phase I <a href="http://sppirx.com">sppirx.com</a>
CB3304 (noscaphine)	Cougar Biotechnology <i>Raritan, NJ</i>	hematological malignancies	Phase I <a href="http://cougarbiotechnology.com">cougarbiotechnology.com</a>
darinaparsin (ZIO-101)	ZIOPHARM Oncology <i>New York, NY</i>	hematological malignancies (see also lymphoma, myeloma)	Phase II <a href="http://ziopharm.com">ziopharm.com</a>
fostamatinib (Syk inhibitor)	AstraZeneca <i>Wilmington, DE</i> Rigel Pharmaceuticals <i>South San Francisco, CA</i>	hematological malignancies	Phase II <a href="http://astrazeneca.com">astrazeneca.com</a> <a href="http://rigel.com">rigel.com</a>
GS-9973 (Syk inhibitor)	Gilead Sciences <i>Foster City, CA</i>	B-cell hematological malignancies	Phase I <a href="http://gilead.com">gilead.com</a>

\*For more information about a specific medicine or company in the report, please click on the provided link.

## Hematological Malignancies

Product Name	Sponsor	Indication	Development Phase
<b>Jakafi®</b> ruxolitinib	Incyte <i>Wilmington, DE</i>	hematological malignancies	Phase II incyte.com
MEDI-551 (anti-CD19 mAb)	AstraZeneca <i>Wilmington, DE</i> MedImmune <i>Gaithersburg, MD</i>	hematological malignancies	Phase II astrazeneca.com medimmune.com
MLN4924 (pevonedistat)	Millennium Pharmaceuticals <i>Cambridge, MA</i>	hematological malignancies	Phase I millennium.com
MLN8237 (alisertib)	Millennium Pharmaceuticals <i>Cambridge, MA</i>	hematological malignancies (see also lymphoma)	Phase II millennium.com
MLN9708 (ixazomib) (second-generation proteasome inhibitor)	Millennium Pharmaceuticals <i>Cambridge, MA</i>	hematological malignancies (see also myeloma)	Phase I millennium.com
moxetumomab pasudotox (anti-CD22 recombinant immunotoxin)	AstraZeneca <i>Wilmington, DE</i> MedImmune <i>Gaithersburg, MD</i>	hematological malignancies	Phase I astrazeneca.com medimmune.com
nivolumab (anti-PD-1)	Bristol-Myers Squibb <i>Princeton, NJ</i>	hematological malignancies	Phase I bms.com
oprozomib	Onyx Pharmaceuticals <i>South San Francisco, CA</i>	hematological malignancies	Phase I onyx.com
RG7112 (MDM2 antagonist)	Roche <i>Nutley, NJ</i>	hematological malignancies	Phase I roche.com
RG7388 (proto oncogene protein-c mdm2 inhibitor)	Roche <i>Nutley, NJ</i>	hematological malignancies	Phase I roche.com
SAR405838 (HDM2/p53 antagonist)	Ascenta Therapeutics <i>Malvern, PA</i> Sanofi US <i>Bridgewater, NJ</i>	hematological malignancies	Phase I ascenta.com sanofi.com
SAR650984 (anti-CD38 naked mAb)	ImmunoGen <i>Waltham, MA</i> Sanofi US <i>Bridgewater, NJ</i>	hematological malignancies	Phase I immunogen.com sanofi.com

## Leukemia

Product Name	Sponsor	Indication	Development Phase
ABT-199/GDC-0199/RG7601 (Bcl-2 inhibitor)	AbbVie North Chicago, IL Genentech South San Francisco, CA Roche Nutley, NJ	relapsed/refractory chronic lymphocytic leukemia (CLL) (see also lymphoma, myeloma)	Phase I abbvie.com gene.com roche.com
<b>Actimab-A™</b> lintuzumab Ac-225	Actinium Pharmaceuticals New York, NY	acute myeloid leukemia (AML)	Phase I/II actiniumpharmaceuticals.com
aminopterin	Syntrix Biosystems Auburn, WA	pediatric acute lymphoblastic leukemia (ALL)	Phase II syntrixbio.com
<b>Arzerra®</b> ofatumumab (Orphan Drug)	GlaxoSmithKline Rsch. Triangle Park, NC	first-line CLL, relapsed CLL (see also lymphoma)	Phase III gsk.com
AT-101 (pan Bcl-2 inhibitor)	Ascenta Therapeutics Malvern, PA	CLL (see also lymphoma)	Phase II completed ascenta.com
AT-406 (multi-IAP inhibitor)	Ascenta Therapeutics Malvern, PA Debiopharm Lausanne, Switzerland	AML	Phase I ascenta.com debiopharm.com
bafetinib	CytRx Los Angeles, CA	CLL	Phase II completed cytrx.com
BI-811283 (aurora B kinase inhibitor)	Boehringer Ingelheim Pharmaceuticals Ridgefield, CT	AML	Phase I/II boehringer-ingelheim.com
BI-836826	Boehringer Ingelheim Pharmaceuticals Ridgefield, CT	CLL	Phase I boehringer-ingelheim.com
BI-836858	Boehringer Ingelheim Pharmaceuticals Ridgefield, CT	AML	Phase I boehringer-ingelheim.com
birinapant	TetraLogic Pharmaceuticals Malvern, PA	AML (see also lymphoma)	Phase I/II tetralogicpharma.com
<b>Bismab-A™</b> lintuzumab Bi-213	Actinium Pharmaceuticals New York, NY	AML	Phase I/II actiniumpharmaceuticals.com

## Leukemia

Product Name	Sponsor	Indication	Development Phase
blinatumomab (AMG 103)	Amgen <i>Thousand Oaks, CA</i>	ALL (see also lymphoma)	Phase II amgen.com
BMS-906024 (notch inhibitor)	Bristol-Myers Squibb <i>Princeton, NJ</i>	T-cell ALL (see also lymphoma)	Phase I bms.com
BMS-936564 (anti-CXCR4)	Bristol-Myers Squibb <i>Princeton, NJ</i>	AML, CLL, diffuse large B-cell leukemia (see also lymphoma, myeloma)	Phase I bms.com
<b>Bosulif®</b> bosutinib	Pfizer <i>New York, NY</i>	first-line Ph+ chronic myeloid leukemia (CML)	Phase III pfizer.com
BP-100-1.01 (liposomal Grb-2) (Orphan Drug)	Bio-Path Holdings <i>Houston, TX</i>	ALL, AML, CML (see also myelodysplastic)	Phase I biopathholdings.com
calaspargase pegol (Orphan Drug)	Sigma-Tau Pharmaceuticals <i>Gaithersburg, MD</i>	ALL in adolescents and children	Phase III sigmatau.com
CC-292 (Btk inhibitor)	Celgene <i>Summit, NJ</i>	CLL (see also lymphoma)	Phase I celgene.com
CC-486 (nucleoside analogue)	Celgene <i>Summit, NJ</i>	post-induction AML maintenance (see also myelodysplastic)	Phase II celgene.com
<b>Ceplene®</b> histamine	EpiCept <i>Tarrytown, NY</i>	AML	Phase III epicept.com
CNDO-109 (tumor-activated natural killer cells) (Orphan Drug)	Coronado Biosciences <i>Burlington, MA</i>	AML	Phase II coronadobiosciences.com
CPX-351 (cytarabine/daunorubicin) (Orphan Drug)	Celator Pharmaceuticals <i>Princeton, NJ</i>	AML	Phase III celatorpharma.com
crenolanib (CP-868-596)	AROG Pharmaceuticals <i>Dallas, TX</i>	AML	Phase II arogpharma.com
CTL019 (CAR T-cell therapy)	Novartis Pharmaceuticals <i>East Hanover, NJ</i> University of Pennsylvania <i>Philadelphia, PA</i>	CLL	Phase II novartis.com
CWP232291	JW Pharmaceutical <i>Seoul, South Korea</i>	AML	Phase I

## Leukemia

Product Name	Sponsor	Indication	Development Phase
<b>Dacogen®</b> decitabine (Orphan Drug)	Eisai <i>Woodcliff Lake, NJ</i>	AML	application submitted eisai.com
		pediatric AML	Phase II eisai.com
DFP-10917 (cell cycle inhibitor)	Delta-Fly Pharma <i>Tokushima, Japan</i>	ALL, AML	Phase I/II
dinaciclib (SOK inhibitor) (Orphan Drug)	Merck <i>Whitehouse Station, NJ</i>	CLL	Phase III merck.com
elesclomol	Synta Pharmaceuticals <i>Lexington, MA</i>	AML	Phase I syntapharma.com
ENMD-2076 (Orphan Drug)	EntreMed <i>Rockville, MD</i>	AML (see also myeloma)	Phase I completed entremed.com
entinostat	Syndax Pharmaceuticals <i>Waltham, MA</i>	leukemia (see also lymphoma)	Phase II syndax.com
EPZ-5676 (DOT1L inhibitor)	Epizyme <i>Cambridge, MA</i>	leukemia	Phase I epizyme.com
<b>Erwinaze™</b> asparaginase <i>Erwinia chrysanthemi</i> (IV formulation)	Jazz Pharmaceuticals <i>Palo Alto, CA</i>	ALL	Phase I jazzpharma.com
forodesine (PNP inhibitor) (Orphan Drug)	BioCryst Pharmaceuticals <i>Durham, NC</i>	CLL (see also lymphoma)	Phase II biocryst.com
GNKG-168 (TLR9 agonist)	SBI Biotech <i>Tokyo, Japan</i>	CLL	Phase I sbibiotech.jp
GS-9820 (PI3K delta inhibitor)	Gilead Sciences <i>Foster City, CA</i>	CLL (see also lymphoma)	Phase I gilead.com
hTERT RNA vaccine	BioTime <i>Alameda, CA</i>	AML	Phase II biotimeinc.com
HuM195/rGel (lintuzumab-gelonin conjugate)	Targa Therapeutics <i>San Diego, CA</i>	AML, CML (see also myelodysplastic)	Phase I targatherapeutics.com
ibrutinib (Orphan Drug)	Janssen Biotech <i>Horsham, PA</i> Pharmacyclics <i>Sunnyvale, CA</i>	CLL (Fast Track) (see also lymphoma, myeloma)	Phase III janssenbiotech.com pharmacyclics.com

## Leukemia

Product Name	Sponsor	Indication	Development Phase
ibrutinib companion diagnostic	Abbott Laboratories <i>Abbott Park, IL</i> Janssen Biotech <i>Horsham, PA</i> Pharmacyclics <i>Sunnyvale, CA</i>	CLL (diagnosis)	in clinical trials abbott.com janssenbiotech.com pharmacyclics.com
<b>Iclusig®</b> ponatinib (Orphan Drug)	ARIAD Pharmaceuticals <i>Cambridge, MA</i>	newly-diagnosed CML	Phase III ariad.com
		AML	Phase II ariad.com
idelalisib (PI3K delta inhibitor)	Gilead Sciences <i>Foster City, CA</i>	CLL (see also lymphoma)	Phase III gilead.com
<b>Imprime PGG®</b> immunotherapeutic	Biothera <i>Eagan, MN</i>	first-line CLL (see also lymphoma)	Phase I/II biothera.com
inotuzumab ozogamicin	Pfizer <i>New York, NY</i>	ALL (see also lymphoma)	Phase III pfizer.com
<b>Iomab™-B</b> anti-CD45 mAb	Actinium Pharmaceuticals <i>New York, NY</i>	AML	Phase II actiniumpharmaceuticals.com
ISF35 (recombinant CD40 ligand immunotherapy)	Memgen <i>San Diego, CA</i>	CLL (see also lymphoma)	Phase II memgenbio.com
JVRS-100 (DNA vaccine)	Colby Pharmaceutical <i>San Jose, CA</i>	leukemia	Phase I colbypharma.com
KX01 (non-ATP competitive Src kinase inhibitor)	Kinex Pharmaceuticals <i>Buffalo, NY</i>	AML, CML	Phase I kinexpharma.com
LDE225 (erismodegib)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	CML	Phase I novartis.com
lestaurtinib (CEP-701)	Cephalon (Teva) <i>Horsham, PA</i>	AML (see also myeloproliferative)	Phase II tevapharm.com
lirilumab (anti-Kir)	Bristol-Myers Squibb <i>Princeton, NJ</i>	AML	Phase II bms.com

## Leukemia

Product Name	Sponsor	Indication	Development Phase
LOR-2040 (ribonucleotide reductase inhibitor)	Lorus Therapeutics <i>Toronto, Canada</i>	AML	Phase II lorusthera.com
LY2090314 (GSK3-beta inhibitor)	Eli Lilly <i>Indianapolis, IN</i>	AML, acute promyelocytic leukemia (APL)	Phase II lilly.com
<b>Marqibo®</b> vincristine liposomal (Orphan Drug)	Talon Therapeutics <i>South San Francisco, CA</i>	ALL in the elderly (Fast Track)	Phase III talontx.com
milatuzumab (anti-CD74 mAb) (Orphan Drug)	Immunomedics <i>Morris Plains, NJ</i>	CLL	Phase I/II immunomedics.com
MK-8242 (MDM2 inhibitor)	Merck <i>Whitehouse Station, NJ</i>	AML	Phase I merck.com
mogamulizumab (anti-CCR4 mAb) (Orphan Drug)	Kyowa Hakko Kirin <i>Tokyo, Japan</i>	adult T-cell leukemia (see also lymphoma)	Phase II kyowa-kirin.com
navitoclax (bcl-2 inhibitor)	AbbVie <i>North Chicago, IL</i>	CLL	Phase II abbvie.com
obinutuzumab (anti-CD20 mAb)	Biogen Idec <i>Weston, MA</i> Genentech <i>South San Francisco, CA</i> Roche <i>Nutley, NJ</i>	first-line CLL (see also lymphoma)	Phase III biogenidec.com gene.com roche.com
OXi4503 (Orphan Drug)	OXiGENE <i>South San Francisco, CA</i>	refractory AML	Phase I oxigene.com
perifosine	AEterna Zentaris <i>Basking Ridge, NJ</i>	CLL	Phase II aezsinc.com
PF-04449913	Pfizer <i>New York, NY</i>	AML	Phase I pfizer.com
PKC412 (midostaurin)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	AML (see also myeloproliferative)	Phase III novartis.com

## Leukemia

Product Name	Sponsor	Indication	Development Phase
PKC412 (midostaurin) companion diagnostic	Invivoscribe Technologies <i>San Diego, CA</i> Novartis Pharmaceuticals <i>East Hanover, NJ</i>	AML (diagnosis)	in clinical trials invivoscribe.com novartis.com
PLX3397	Plexxikon <i>Berkeley, CA</i>	AML (see also lymphoma)	Phase II plexxikon.com
PM01183 (cell-cycle inhibitor)	PharmaMar <i>Madrid, Spain</i>	leukemia	Phase I pharmamar.com
PR104	Proacta <i>La Jolla, CA</i>	AML	Phase I/II proacta.com
PRI-724 (CBP/ $\beta$ -catenin inhibitor)	Prism Pharma <i>Tokyo, Japan</i>	AML, CML	Phase I/II prismbiolab.com
<b>Promacta</b> <sup>®</sup> eltrombopag	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	AML (see also myelodysplastic)	Phase II gsk.com
quizartinib (FLT3 inhibitor)	Ambit Biosciences <i>San Diego, CA</i>	relapsed/refractory AML	Phase II ambitbio.com
		first-line AML (combination therapy), post-HSCT maintenance in AML	Phase I ambitbio.com
<b>Revlimid</b> <sup>®</sup> lenalidomide (Orphan Drug)	Celgene <i>Summit, NJ</i>	CLL (initial treatment and maintenance) (see also lymphoma, myelodysplastic)	Phase III celgene.com
<b>Revlimid</b> <sup>®</sup> lenalidomide and <b>Vidaza</b> <sup>®</sup> azacitidine injection (Orphan Drug)	Celgene <i>Summit, NJ</i>	AML	Phase II celgene.com
RG7356 (anti-CD44 mAb)	Roche <i>Nutley, NJ</i>	AML	Phase I roche.com
sapacitabine (Orphan Drug)	Cyclacel Pharmaceuticals <i>Berkeley Heights, NJ</i>	AML (see also myelodysplastic)	Phase III cyclacel.com
		CLL	Phase II cyclacel.com
SAR3419 (maytansin-loaded anti-CD19 mAb)	ImmunoGen <i>Waltham, MA</i> Sanofi US <i>Bridgewater, MA</i>	ALL (see also lymphoma)	Phase II immunogen.com sanofi.com

## Leukemia

Product Name	Sponsor	Indication	Development Phase
SGI110 (DNMT1 inhibitor)	Astex Pharmaceuticals <i>Dublin, CA</i>	AML (see also myelodysplastic)	Phase II astx.com
SGN-CD19A (CD19 antibody-drug conjugate)	Seattle Genetics <i>Bothell, WA</i>	ALL (see also lymphoma)	Phase I seattlegenetics.com
SL-401 (recombinant fusion protein) (Orphan Drug)	Stemline Therapeutics <i>New York, NY</i>	relapsed/refractory AML, relapsed/ refractory blastic plasmacytoid dendritic cell neoplasm (see also myelodysplastic)	Phase I/II stemline.com
<b>Sprycel</b> dasatinib	Bristol-Myers Squibb <i>Princeton, NJ</i>	leukemia in children and adolescents	Phase II bms.com
tamibarotene (Orphan Drug)	CytRx <i>Los Angeles, CA</i>	acute promyelocytic leukemia (Fast Track)	Phase II cytrx.com
<b>Tasigna</b> ® nilotinib	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	ALL (adolescents and children), CML (adolescents and children)	Phase II novartis.com
TCN-P (AKT inhibitor)	Cahaba Pharmaceuticals <i>Princeton, NJ</i>	AML	Phase I cahabapharma.com
<b>Temodar</b> ® temozolomide	Merck <i>Whitehouse Station, NJ</i>	AML	Phase II completed merck.com
tetradecanoylphorbol acetate (TPA) (PD-616)	Biosuccess Biotech <i>Beverly Hills, CA</i>	AML	Phase I/II biosuccessbiotech.com
TG02 (multi-kinase inhibitor)	Tragara Pharmaceuticals <i>Carlsbad, CA</i>	acute leukemia, CLL (see also myeloma)	Phase I tragarapharma.com
TH-302 (hypoxia-targeted drug)	EMD Serono <i>Rockland, MA</i> Threshold Pharmaceuticals <i>South San Francisco, CA</i>	advanced leukemia (see also myeloma)	Phase I emdserono.com thresholdpharm.com
tosedostat	Cell Therapeutics <i>Seattle, WA</i> Chroma Therapeutics <i>Oxon, United Kingdom</i>	AML	Phase II celltherapeutics.com
<b>Treanda</b> ® bendamustine	Cephalon (Teva) <i>Frazer, PA</i>	ALL, AML (see also lymphoma, myeloma)	Phase I/II tevapharm.com
TRU-016 (anti-CD37 mAb) (Orphan Drug)	Emergent BioSolutions <i>Rockville, MD</i>	CLL	Phase II emergentbiosolutions.com

## Leukemia

Product Name	Sponsor	Indication	Development Phase
ublituximab (TG-1101) (Orphan Drug)	TG Therapeutics <i>New York, NY</i>	CLL (see also lymphoma)	Phase I/II tgtherapeutics.com
VAY736 (anti-BAFFR mAb)	MorphoSys <i>Martinsried, Germany</i> Novartis Pharmaceuticals <i>East Hanover, NJ</i>	CLL	Phase I morphosys.com novartis.com
veltuzumab (Orphan Drug)	Immunomedics <i>Morris Plains, NJ</i>	CLL (see also lymphoma)	Phase II immunomedics.com
volasertib	Boehringer Ingelheim Pharmaceuticals <i>Ridgefield, CT</i>	AML	Phase III boehringer-ingelheim.com
vosaroxin	Sunesis Pharmaceuticals <i>South San Francisco, CA</i>	AML	Phase III sunesis.com
WT1 immunotherapeutic (antigen-specific cancer immunotherapeutic/ASCI)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	AML	Phase II gsk.com
<b>XmAb® 5574/MOR208</b> (anti-CD19 mAb)	MorphoSys <i>Martinsried, Germany</i> Xenvor <i>Monrovia, CA</i>	CLL	Phase I morphosys.com xencor.com

## Lymphoma

Product Name	Sponsor	Indication	Development Phase
abexinostat (HDAC inhibitor)	Pharmacyclics <i>Sunnyvale, CA</i>	follicular lymphoma, mantle cell lymphoma	Phase II pharmacyclics.com
ABT-199/GDC-0199/RG7601 (Bcl-2 inhibitor)	AbbVie <i>North Chicago, IL</i> Genentech <i>South San Francisco, CA</i> Roche <i>Nutley, NJ</i>	non-Hodgkin lymphoma (see also leukemia, myeloma)	Phase I abbvie.com gene.com roche.com

## Lymphoma

Product Name	Sponsor	Indication	Development Phase
<b>Adcetris®</b> brentuximab vedotin (Orphan Drug)	Millennium Pharmaceuticals <i>Cambridge, MA</i> Seattle Genetics <i>Bothell, WA</i>	Hodgkin lymphoma (post-transplant relapse prevention), relapsed CD30-positive cutaneous T-cell lymphoma, first-line Hodgkin lymphoma, first-line CD30-positive mature T-cell lymphoma (see also hematological)	Phase III millennium.com seattlegenetics.com
		relapsed/refractory CD30-positive non-Hodgkin lymphoma, CD30-positive lymphoma malignancies, first-line Hodgkin lymphoma in patients 60 years and older	Phase II millennium.com seattlegenetics.com
		second-line Hodgkin lymphoma	Phase I millennium.com seattlegenetics.com
AEB071 (PKC inhibitor)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	diffuse large B-cell lymphoma	Phase I novartis.com
AEZS-112 (tubulin polymerization/ topoisomerase II inhibitor)	AEterna Zentaris <i>Basking Ridge, NJ</i>	lymphoma	Phase I completed aezsinc.com
<b>Afinitor®</b> everolimus	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	diffuse large B-cell lymphoma	Phase III novartis.com
AFM13 (bispecific tetravalent antibody) (Orphan Drug)	Affimed Therapeutics <i>Heidelberg, Germany</i>	Hodgkin lymphoma	Phase I affimed.com
anti-CD22 antibody-drug conjugate (DCDT 2980S)	Genentech <i>South San Francisco, CA</i>	diffuse large B-cell lymphoma, non-Hodgkin lymphoma (see also hematological)	Phase II gene.com
anti-CD79b antibody-drug conjugate (DCDS 4501A)	Genentech <i>South San Francisco, CA</i>	diffuse large B-cell lymphoma, non-Hodgkin lymphoma (see also hematological)	Phase II gene.com
AR-12 (PI3-K/Akt inhibitor)	Arno Therapeutics <i>Flemington, NJ</i>	lymphoma	Phase I arnothera.com

## Lymphoma

Product Name	Sponsor	Indication	Development Phase
<b>Arzerra®</b> ofatumumab	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	diffuse large B-cell lymphoma, relapsed/refractory follicular lymphoma (see also leukemia)	Phase III gsk.com
ASP3026 (ALK inhibitor)	Astellas Pharma US <i>Deerfield, IL</i>	late-stage B-cell lymphoma	Phase I astellas.com
AT-101 (pan Bcl-2 inhibitor)	Ascenta Therapeutics <i>Malvern, PA</i>	non-Hodgkin lymphoma (see also leukemia)	Phase II completed ascenta.com
autologous idiotypic cancer vaccine	Bayer HealthCare Pharmaceuticals <i>Wayne, NJ</i>	follicular lymphoma	Phase I bayerpharma.com
BAY 80-6946 (PI3K inhibitor)	Bayer HealthCare Pharmaceuticals <i>Wayne, NJ</i>	non-Hodgkin lymphoma	Phase II bayerpharma.com
belinostat (HDAC inhibitor)	Spectrum Pharmaceuticals <i>Henderson, NV</i>	peripheral B-cell lymphoma (Fast Track) (see also hematological)	Phase II sppirx.com
<b>Bexxar®</b> tositumomab and iodine I-131 tositumomab	GlaxoSmithKline <i>Rsch Triangle Park, NC</i>	mantle cell lymphoma	Phase II gsk.com
BI-695500 (rituximab biosimilar)	Boehringer Ingelheim Pharmaceuticals <i>Ridgefield, CT</i>	non-Hodgkin lymphoma	Phase III boehringer-ingelheim.com
<b>BiovaxID®</b> B-cell lymphoma vaccine (Orphan Drug)	Biovest International <i>Tampa, FL</i>	indolent follicular lymphoma (Fast Track)	Phase III biovest.com
		mantle cell lymphoma	Phase II biovest.com
birinapant	TetraLogic Pharmaceuticals <i>Malvern, PA</i>	lymphoma (see also leukemia)	Phase I tetralogicpharma.com
BKM120 (PI3K inhibitor)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	relapsed/refractory non-Hodgkin lymphoma	Phase II novartis.com
blinatumomab (AMG 103)	Amgen <i>Thousand Oaks, CA</i>	non-Hodgkin lymphoma (see also leukemia)	Phase II amgen.com
BMS-906024 (notch inhibitor)	Bristol-Myers Squibb <i>Princeton, NJ</i>	T-cell lymphoblastic lymphoma (see also leukemia)	Phase I bms.com
BMS-936564 (anti-CXCR4)	Bristol-Myers Squibb <i>Princeton, NJ</i>	follicular lymphoma (see also leukemia, myeloma)	Phase I bms.com

## Lymphoma

Product Name	Sponsor	Indication	Development Phase
brentuximab vedotin companion diagnostic	Millennium Pharmaceuticals <i>Cambridge, MA</i> Seattle Genetics <i>Bothell, WA</i> Ventana Medical Systems <i>Tucson, AZ</i>	cutaneous T-cell lymphoma (diagnosis), T-cell lymphoma (diagnosis)	in clinical trials ventana.com
CC-292 (Btk inhibitor)	Celgene <i>Summit, NJ</i>	lymphoma (see also leukemia)	Phase I celgene.com
CD-5789	Galderma USA <i>Fort Worth, TX</i> M.D. Anderson Cancer Center <i>Houston, TX</i>	cutaneous T-cell lymphoma	Phase I galdermausa.com
CEP-9722 (PARP inhibitor)	Cephalon (Teva) <i>Frazer, PA</i>	mantle cell lymphoma	Phase I tevapharm.com
CGC-11047	Progen Pharmaceuticals <i>Queensland, Australia</i>	lymphoma	Phase I completed progen-pharma.com
CUDC-427 (IAP inhibitor)	Curis <i>Lexington, MA</i>	lymphoma	Phase I curis.com
CUDC-907 (HDAC/PI3K inhibitor)	Curis <i>Lexington, MA</i>	diffuse large B-cell lymphoma (see also myeloma)	Phase I curis.com
darinaparsin (ZIO-101)	ZIOPHARM Oncology <i>New York, NY</i>	non-Hodgkin lymphoma (see also hematological, myeloma)	Phase I ziopharm.com
delanzomib	Cephalon (Teva) <i>Frazer, PA</i>	non-Hodgkin lymphoma (see also myeloma)	Phase I tevapharm.com
DI-Leu16-IL2 (CD20-antigen inhibitor)	alopexx Oncology <i>Cambridge, MA</i>	non-Hodgkin lymphoma	Phase I alopexx.com
DS-3078	Daiichi Sankyo <i>Parsippany, NJ</i>	lymphoma	Phase I dsi.com
entinostat	Syndax Pharmaceuticals <i>Waltham, MA</i>	relapsed/refractory Hodgkin lymphoma (see also leukemia)	Phase II syndax.com
enzastaurin	Eli Lilly <i>Indianapolis, IN</i>	diffuse large B-cell lymphoma	Phase III lilly.com
epratuzumab (Orphan Drug)	Immunomedics <i>Morris Plains, NJ</i>	non-Hodgkin lymphoma	Phase II immunomedics.com

## Lymphoma

Product Name	Sponsor	Indication	Development Phase
epratuzumab Y-90	Immunomedics <i>Morris Plains, NJ</i>	non-Hodgkin lymphoma	Phase I/II immunomedics.com
forodesine (PNP inhibitor) (Orphan Drug)	BioCryst Pharmaceuticals <i>Durham, NC</i>	cutaneous T-cell lymphoma (see also leukemia)	Phase II biocryst.com
GDC-0425 (ChK1 inhibitor)	Genentech <i>South San Francisco, CA</i>	lymphoma	Phase I gene.com
GDC-0575 (ChK1 inhibitor)	Genentech <i>South San Francisco, CA</i>	lymphoma	Phase I gene.com
GDC-0980/RG7422 (PI3 kinase/mTOR dual inhibitor)	Genentech <i>South San Francisco, CA</i> Roche <i>Nutley, NJ</i>	non-Hodgkin lymphoma	Phase II gene.com roche.com
GS-9820 (PI3K delta inhibitor)	Gilead Sciences <i>Foster City, CA</i>	lymphoma (see also leukemia)	Phase I gilead.com
HBI-8000 (HDAC inhibitor)	HUYA Bioscience International <i>San Diego, CA</i> Quintiles <i>Durham, NC</i>	lymphoma	Phase I huyabio.com
ibrutinib (Orphan Drug)	Janssen Biotech <i>Horsham, PA</i>	mantle cell lymphoma (see also leukemia, myeloma)	Phase III janssenbiotech.com
		diffuse large B-cell lymphoma	Phase II janssenbiotech.com
		follicular lymphoma	Phase I janssenbiotech.com
idelalisib (PI3K delta inhibitor)	Gilead Sciences <i>Foster City, CA</i>	indolent non-Hodgkin lymphoma (see also leukemia)	Phase III gilead.com
IMGN529 (CD37-targeting mAb)	ImmunoGen <i>Waltham, MA</i>	non-Hodgkin lymphoma	Phase I immunogen.com
<b>Imprime PGG®</b> immunotherapeutic	Biothera <i>Eagan, MN</i>	first-line advanced indolent non-Hodgkin lymphoma (see also leukemia)	Phase II/III biothera.com
inotuzumab ozogamicin	Pfizer <i>New York, NY</i>	aggressive non-Hodgkin lymphoma (see also leukemia)	Phase III pfizer.com

## Lymphoma

Product Name	Sponsor	Indication	Development Phase
interleukin-12 gene therapy	OncoSec Medical <i>San Diego, CA</i>	cutaneous T-cell lymphoma	Phase II oncosec.com
ISF35 (recombinant CD40 ligand immunotherapy)	Memgen <i>San Diego, CA</i>	non-Hodgkin lymphoma (see also leukemia)	Phase II memgenbio.com
<b>Istodax®</b> romidepsin	Celgene <i>Summit, NJ</i>	newly-diagnosed peripheral T-cell lymphoma	Phase III celgene.com
JNJ-26481585 (HDAC inhibitor)	Janssen Research & Development <i>Raritan, NJ</i>	cutaneous T-cell lymphoma	Phase II janssenrnd.com
JNJ-40346527 (FMS inhibitor)	Janssen Research & Development <i>Raritan, NJ</i>	Hodgkin lymphoma	Phase I/II janssenrnd.com
JNJ-42756493 (FGFR inhibitor)	Janssen Research & Development <i>Raritan, NJ</i>	lymphoma	Phase I janssenrnd.com
LEE011 (CDK-4/6 inhibitor)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	lymphoma	Phase I novartis.com
LY3039478	Eli Lilly <i>Indianapolis, IN</i>	lymphoma	Phase I lilly.com
marizomib	Nereus Pharmaceuticals <i>San Diego, CA</i>	lymphoma (see also myeloma)	Phase I
mechlorethamine gel (Orphan Drug)	Ceptaris Therapeutics <i>Malvern, PA</i>	mycosis fungoides (cutaneous T-cell lymphoma)	application submitted ceptaris.com
MK-8808	Merck <i>Whitehouse Station, NJ</i>	follicular lymphoma	Phase I merck.com
MLN8237 (alisertib)	Millennium Pharmaceuticals <i>Cambridge, MA</i>	relapsed/refractory peripheral T-cell lymphoma (see also hematological)	Phase III millennium.com
mocetinostat (MGCD0103) (HDAC inhibitor) (Orphan Drug)	MethylGene <i>Montreal, Canada</i>	diffuse large B-cell lymphoma, follicular lymphoma	Phase II methylgene.com
mogamulizumab (anti-CCR4 mAb) (Orphan Drug)	Kyowa Hakko Kirin <i>Tokyo, Japan</i>	cutaneous T-cell lymphoma (see also leukemia)	Phase III kyowa-kirin.com
		adult T-cell lymphoma, peripheral T-cell lymphoma	Phase II kyowa-kirin.com

## Lymphoma

Product Name	Sponsor	Indication	Development Phase
obinutuzumab (anti-CD20 mAb)	Biogen Idec <i>Weston, MA</i> Genentech <i>South San Francisco, CA</i> Roche <i>Nutley, NJ</i>	diffuse large B-cell lymphoma, first-line indolent non-Hodgkin lymphoma, refractory indolent non-Hodgkin lymphoma (see also leukemia)	Phase III biogenidec.com gene.com roche.com
ocaratuzumab (AME-133v)	MENTRIK Biotech <i>Dallas, TX</i>	non-Hodgkin lymphoma	Phase II mentrik.com
ON 013105 (cyclin D1 modulator)	Onconova Therapeutics <i>Newtown, PA</i>	refractory lymphoma	Phase I onconova.com
ONO 4059 (Btk inhibitor)	Ono Pharma USA <i>Trenton, NJ</i>	B-cell lymphoma	Phase I ono.co.jp
pacritinib	Cell Therapeutics <i>Seattle, WA</i>	relapsed lymphoma (see also myeloproliferative)	Phase II completed celltherapeutics.com
palbociclib (PD-0332991) (CDK-4/6 kinase inhibitor)	Pfizer <i>New York, NY</i>	mantle cell lymphoma (see also myeloma)	Phase I completed pfizer.com
PF-05082566 (CD137 antigen agonist)	Pfizer <i>New York, NY</i>	non-Hodgkin lymphoma	Phase I pfizer.com
pidilizumab (CT-011)	CureTech <i>Yavne, Israel</i>	diffuse large B-cell lymphoma	Phase II curetechbio.com
<b>Pixuvri®</b> pixantrone	Cell Therapeutics <i>Seattle, WA</i>	diffuse large B-cell lymphoma, follicular lymphoma	Phase III celltherapeutics.com
PLX3397	Plexxikon <i>Berkeley, CA</i>	Hodgkin lymphoma (see also leukemia)	Phase II plexxikon.com
PNT2258 (bcl-2 oncogene inhibitor)	ProNAi Therapeutics <i>Plymouth, MI</i>	B-cell lymphoma	Phase II pronai.com
<b>Revlimid®</b> lenalidomide	Celgene <i>Summit, NJ</i>	relapsed/refractory mantle-cell lymphoma (see also leukemia, myelodysplastic)	application submitted celgene.com
		diffuse large B-cell lymphoma (maintenance), follicular lymphoma	Phase III celgene.com
		relapsed/refractory diffuse large B-cell lymphoma	Phase II celgene.com

## Lymphoma

Product Name	Sponsor	Indication	Development Phase
RG7602	Genentech <i>South San Francisco, CA</i> Roche <i>Nutley, NJ</i>	lymphoma	Phase I gene.com roche.com
SAR3419 (maytansin-loaded anti-CD19 mAb)	ImmunoGen <i>Waltham, MA</i> Sanofi US <i>Bridgewater, NJ</i>	non-Hodgkin lymphoma (see also leukemia)	Phase II immunogen.com sanofi.com
SAR245409 (XL765) (PI3K/mTOR inhibitor)	Exelixis <i>South San Francisco, CA</i> Sanofi US <i>Bridgewater, NJ</i>	non-Hodgkin lymphoma	Phase II exelixis.com sanofi.com
sepantronium bromide (YM155)	Astellas Pharma US <i>Northbrook, IL</i>	non-Hodgkin lymphoma	Phase II astellas.com
SGN-75	Seattle Genetics <i>Bothell, WA</i>	non-Hodgkin lymphoma	Phase I completed seattlegenetics.com
SGN-CD19A (CD19 antibody-drug conjugate)	Seattle Genetics <i>Bothell, WA</i>	non-Hodgkin lymphoma (see also leukemia)	Phase I seattlegenetics.com
SHP-141 (topical HDAC inhibitor)	Leukemia & Lymphoma Society <i>White Plains, NY</i> Shape Pharmaceuticals <i>Cambridge, MA</i>	cutaneous T-cell lymphoma	Phase I shapepharma.com
SNS01-T (nano-encapsulated gene therapy) (Orphan Drug)	Senesco Technologies <i>Bridgewater, NJ</i>	diffuse large B-cell lymphoma, mantle cell lymphoma (see also myeloma)	Phase I/II senesco.com
SNX-5422 (Hsp90 inhibitor)	Esanex <i>Indianapolis, IN</i>	non-Hodgkin lymphoma	Phase I
TKM-PLK1 (RNAi therapeutic)	Tekmira Pharmaceuticals <i>Burnaby, Canada</i>	lymphoma	Phase I tekmirapharm.com
trametinib (GSK1120212)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	lymphoma	Phase II gsk.com
<b>Treanda®</b> bendamustine (Orphan Drug)	Cephalon (Teva) <i>Frazer, PA</i>	first-line indolent B-cell non-Hodgkin lymphoma (see also leukemia, myeloma)	application submitted tevapharm.com
		mantle cell lymphoma	Phase II tevapharm.com

## Lymphoma

Product Name	Sponsor	Indication	Development Phase
ublituximab (TG-1101)	TG Therapeutics New York, NY	non-Hodgkin lymphoma (see also leukemia)	Phase I/II tgtherapeutics.com
UNBS 5162	Drais Pharmaceuticals Bridgewater, NJ	lymphoma	Phase I draispharma.com
urelumab (anti-CD137 mAb)	Bristol-Myers Squibb Princeton, NJ	relapsed/refractory B-cell non-Hodgkin lymphoma	Phase I bms.com
<b>Velcade®</b> bortezomib	Millennium Pharmaceuticals Cambridge, MA	first-line mantle cell lymphoma	Phase III millennium.com
		diffuse large B-cell lymphoma	Phase II millennium.com
veltuzumab	Immunomedics Morris Plains, NJ	non-Hodgkin lymphoma (see also leukemia)	Phase II immunomedics.com
veltuzumab/epiratuzumab Y-90	Immunomedics Morris Plains, NJ	diffuse large B-cell lymphoma	Phase I immunomedics.com
vinorelbine liposomal	Talon Therapeutics South San Francisco, CA	non-Hodgkin lymphoma	Phase I talontx.com
<b>XmAb®2513</b> anti-CD30 mAb	Xencor Monrovia, CA	Hodgkin lymphoma	Phase I xencor.com
zanolimumab	Emergent BioSolutions Rockville, MD	peripheral T-cell lymphoma	Phase II emergentbiosolutions.com
<b>Zevalin®</b> ibritumomab tiuxetan	Spectrum Pharmaceuticals Henderson, NV	diffuse large B-cell lymphoma	Phase III sppirx.com
		mantle cell lymphoma	Phase II sppirx.com

## Myelodysplastic Syndromes

Product Name	Sponsor	Indication	Development Phase
ACE-536 (TGF-beta superfamily protein inhibitor) (Orphan Drug)	Acceleron Pharma Cambridge, MA Celgene Summit, NJ	anemia in myelodysplastic syndromes	Phase I acceleronpharma.com celgene.com
<b>Aranesp®</b> darbepoetin alfa	Amgen Thousand Oaks, CA	myelodysplastic syndromes	Phase III amgen.com

## Myelodysplastic Syndromes

Product Name	Sponsor	Indication	Development Phase
ARRY-614 (p38/TIE2 inhibitor)	Array BioPharma <i>Boulder, CO</i>	myelodysplastic syndromes	Phase I arraybiopharma.com
BP-100-1.01 (liposomal Grb-2)	Bio-Path Holdings <i>Houston, TX</i>	myelodysplastic syndromes (see also leukemia)	Phase I biopathholdings.com
CC-486 (nucleoside analogue)	Celgene <i>Summit, NJ</i>	lower-risk myelodysplastic syndromes (see also leukemia)	Phase II celgene.com
HuM195/rGel (lintuzumab-gelonin conjugate)	Targa Therapeutics <i>San Diego, CA</i>	myelodysplastic syndromes (see also leukemia)	Phase I targatherapeutics.com
pracinostat (HDAC inhibitor)	MEI Pharma <i>San Diego, CA</i>	myelodysplastic syndromes (see also myeloproliferative)	Phase II meipharma.com
<b>Promacta</b> <sup>®</sup> eltrombopag	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	myelodysplastic syndromes (see also leukemia)	Phase II gsk.com
<b>Revlimid</b> <sup>®</sup> lenalidomide (Orphan Drug)	Celgene <i>Summit, NJ</i>	myelodysplastic syndromes (non-deletion 5q) (see also leukemia, lymphoma, myeloma)	Phase III celgene.com
rigosertib (Plk1 inhibitor/c-akt inhibitor) (Orphan Drug)	Onconova Therapeutics <i>Newtown, PA</i>	myelodysplastic syndromes	Phase III onconova.com
sapacitabine (Orphan Drug)	Cyclacel Pharmaceuticals <i>Berkeley Heights, NJ</i>	myelodysplastic syndromes (see also leukemia)	Phase II cyclacel.com
SGI110 (DNMT1 inhibitor)	Astex Pharmaceuticals <i>Dublin, CA</i>	myelodysplastic syndromes (see also leukemia)	Phase II astx.com
SL-401 (recombinant fusion protein) (Orphan Drug)	Stemline Therapeutics <i>New York, NY</i>	myelodysplastic syndromes (see also leukemia)	Phase I/II stemline.com
sotatercept (ACE-011)	Acceleron Pharma <i>Cambridge, MA</i> Celgene <i>Summit, NJ</i>	anemia in myelodysplastic syndromes	Phase II acceleronpharma.com celgene.com
<b>Telintra</b> <sup>®</sup> ezatiostat (Orphan Drug)	Telik <i>Palo Alto, CA</i>	myelodysplastic syndromes	Phase II telik.com

## Myeloma

Product Name	Sponsor	Indication	Development Phase
ABT-199/GDC-0199/RG7601 (Bcl-2 inhibitor)	AbbVie <i>North Chicago, IL</i> Genentech <i>South San Francisco, CA</i> Roche <i>Nutley, NJ</i>	myeloma (see also leukemia, lymphoma)	Phase I abbvie.com gene.com roche.com
ACY-1215 (HDAC inhibitor)	Acetylon Pharmaceuticals <i>Boston, MA</i>	myeloma	Phase I/II acetylon.com
ALT-801 (TCR/IL-2 fusion protein)	Altor BioScience <i>Miramar, FL</i>	myeloma	Phase I/II altorbioscience.com
antibody-drug conjugate (RG7598)	Genentech <i>South San Francisco, CA</i>	myeloma	Phase I gene.com
<b>Aplidin®</b> plitidepsin	PharmaMar <i>Madrid, Spain</i>	myeloma	Phase III pharmamar.com
ARRY-520 (KSP inhibitor)	Array BioPharma <i>Boulder, CO</i>	myeloma	Phase II arraybiopharma.com
AT7519 (CDK inhibitor)	Astex Pharmaceuticals <i>Dublin, CA</i>	myeloma	Phase II astx.com
AT9283 (aurora/JAK 2 inhibitor)	Astex Pharmaceuticals <i>Dublin, CA</i>	myeloma	Phase II astx.com
AUY922 (Hsp90 inhibitor)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	myeloma	Phase I/II novartis.com
BHQ880	Morphosys <i>Martinsried, Germany</i> Novartis Pharmaceuticals <i>East Hanover, NJ</i>	myeloma	Phase II morphosys.com novartis.com
BI-505 (ICAM-1-targeting mAb) (Orphan Drug)	BioInvent International <i>Lund, Sweden</i>	myeloma	Phase I bioinvent.com
BIW-8962 (anti-GM2 mAb)	BioWa <i>La Jolla, CA</i> Kyowa Hakko Kirin <i>Tokyo, Japan</i>	myeloma	Phase I/II biowa.com kyowa-kirin.com

## Myeloma

Product Name	Sponsor	Indication	Development Phase
BMS-936564 (CXCR4 receptor antagonist)	Bristol-Myers Squibb <i>Princeton, NJ</i>	relapsed/refractory myeloma (see also leukemia, lymphoma)	Phase I bms.com
BT-062 (anti-CD138 mAb)	Biotest Pharmaceuticals <i>Boca Raton, FL</i> ImmunoGen <i>Waltham, MA</i>	myeloma	Phase I/II biotest.com immunogen.com
CUDC-907 (HDAC/PI3K inhibitor)	Curis <i>Lexington, MA</i>	myeloma (see also lymphoma)	Phase I curis.com
CX-4945 (casein kinase II inhibitor)	Cylene Pharmaceuticals <i>San Diego, CA</i>	myeloma	Phase I
daratumumab	Janssen Biotech <i>Horsham, PA</i>	myeloma (Fast Track)	Phase I/II janssenbiotech.com
darinaparsin (ZIO-101)	ZIOPHARM Oncology <i>New York, NY</i>	myeloma (see also hematological, lymphoma)	Phase II ziopharm.com
delanzomib	Cephalon (Teva) <i>Frazer, PA</i>	myeloma (see also lymphoma)	Phase II tevapharm.com
DKN-01 (DKK2 protein inhibitor)	Dekkun <i>Cambridge, MA</i>	myeloma	Phase I
elotuzumab	AbbVie <i>North Chicago, IL</i> Bristol-Myers Squibb <i>Princeton, NJ</i>	myeloma	Phase III abbvie.com bms.com
ENMD-2076 (Orphan Drug)	EntreMed <i>Rockville, MD</i>	myeloma (see also leukemia)	Phase I completed entremed.com
GL-0817 (immune modulator)	Gliknik <i>Baltimore, MD</i>	myeloma	Phase II gliknik.com
GSK2110183 (AKT protein kinase inhibitor)	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	myeloma	Phase I gsk.com
ibrutinib (Orphan Drug)	Janssen Biotech <i>Horsham, PA</i>	myeloma (see also leukemia, lymphoma)	Phase II janssenbiotech.com
imetelstat (telomerase inhibitor)	Geron <i>Menlo Park, CA</i>	myeloma (see also myeloproliferative)	Phase II geron.com

## Myeloma

Product Name	Sponsor	Indication	Development Phase
IMGN901 (lorvotuzumab mertansine)	ImmunoGen <i>Waltham, MA</i>	myeloma	Phase I immunogen.com
KW-2478 (HSP90 inhibitor)	Kyowa Hakko Kirin <i>Tokyo, Japan</i>	myeloma	Phase I/II kyowa-kirin.com
<b>Kyprolis®</b> carfilzomib (Orphan Drug)	Onyx Pharmaceuticals <i>South San Francisco, CA</i>	relapsed/refractory myeloma (monotherapy), relapsed myeloma (combination therapy)	Phase III onyx.com
LBH589 (panobinostat)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	myeloma	Phase III novartis.com
LGH447 (proto-oncogene pim-1 inhibitor)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	myeloma	Phase I novartis.com
mapatumumab	GlaxoSmithKline <i>Rsch. Triangle Park, NC</i>	myeloma	Phase II gsk.com
marizomib	Nereus Pharmaceuticals <i>San Diego, CA</i>	myeloma (see also lymphoma)	Phase I
melfalan intravenous (captisol-enabled) (Orphan Drug)	Ligand Pharmaceuticals <i>La Jolla, CA</i> Spectrum Pharmaceuticals <i>Henderson, NV</i>	myeloma	Phase II/III ligand.com sppirx.com
MFGR-1877S (FGF-3 receptor antagonist)	Genentech <i>South San Francisco, CA</i>	myeloma	Phase I completed gene.com
milatuzumab-doxorubicin conjugate (anti-CD74 mAb)	Immunomedics <i>Morris Plains, NJ</i>	myeloma	Phase I/II immunomedics.com
MLN0128 (TORC1/TORC2 inhibitor)	Millennium Pharmaceuticals <i>Cambridge, MA</i>	myeloma	Phase I millennium.com
MLN9708 (ixazomib) (second-generation proteasome inhibitor)	Millennium Pharmaceuticals <i>Cambridge, MA</i>	relapsed/refractory myeloma (see also hematological)	Phase III millennium.com

## Myeloma

Product Name	Sponsor	Indication	Development Phase
monoclonal antibody (DFRF4539A)	Genentech <i>South San Francisco, CA</i>	myeloma	Phase I gene.com
palbociclib (PD-0332991) (CDK-4/6 kinase inhibitor)	Pfizer <i>New York, NY</i>	myeloma (see also lymphoma)	Phase I/II pfizer.com
PRLX-93936	Prolexys Pharmaceuticals <i>Salt Lake City, UT</i>	myeloma	Phase I/II
PVX-410 (therapeutic cancer vaccine)	OncoPep <i>North Andover, MA</i>	smoldering myeloma	Phase I/II oncopep.com
<b>Revlimid®</b> lenalidomide	Celgene <i>Summit, NJ</i>	myeloma (newly-diagnosed and maintenance) (see also leukemia, lymphoma, myelodysplastic)	Phase III celgene.com
siltuximab (anti-IL6 mAb)	Janssen Biotech <i>Horsham, PA</i>	myeloma	Phase II janssenbiotech.com
SNS01-T (nano-encapsulated gene therapy) (Orphan Drug)	Senesco Technologies <i>Bridgewater, NJ</i>	myeloma (see also lymphoma)	Phase I/II senesco.com
tabalumab (BAFF antibody)	Eli Lilly <i>Indianapolis, IN</i>	myeloma	Phase II/III lilly.com
TG02 (multi-kinase inhibitor)	Tragara Pharmaceuticals <i>Carlsbad, CA</i>	myeloma (see also leukemia)	Phase I tragarapharma.com
TH-302 (hypoxia-targeted drug)	EMD Serono <i>Rockland, MA</i> Threshold Pharmaceuticals <i>South San Francisco, CA</i>	myeloma (see also leukemia)	Phase I/II emdserono.com thresholdpharm.com
TKI258 (FGFR3 inhibitor)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	myeloma	Phase II novartis.com
<b>Treanda®</b> bendamustine	Cephalon (Teva) <i>Frazer, PA</i>	myeloma (see also leukemia, lymphoma)	Phase I/II tevapharm.com
<b>Zelboraf®</b> vemurafenib	Roche <i>Nutley, NJ</i>	myeloma (BRAF V600 mutation-positive)	Phase II roche.com
<b>Zolinza®</b> vorinostat	Merck <i>Whitehouse Station, NJ</i>	myeloma	Phase III merck.com

## Myeloproliferative Neoplasms

Product Name	Sponsor	Indication	Development Phase
BMS-911543 (JAK2 inhibitor)	Bristol-Myers Squibb <i>Princeton, NJ</i>	myelofibrosis	Phase I/II bms.com
imetelstat (telomerase inhibitor)	Geron <i>Menlo Park, CA</i>	essential thrombocythemia (see also myeloma)	Phase II geron.com
INCB39110 (JAK1 inhibitor)	InCyte <i>Wilmington, DE</i>	myelofibrosis	Phase II incyte.com
<b>Jakafi®</b> ruxolitinib	Incyte <i>Wilmington, DE</i>	polycythemia vera (see also hematological)	Phase III incyte.com
		essential thrombocythemia	Phase II incyte.com
lestaurtinib (CEP-701)	Cephalon (Teva) <i>Horsham, PA</i>	essential thrombocytosis, polycythemia vera (see also leukemia)	Phase II tevapharm.com
LY2784544 (JAK2 inhibitor)	Eli Lilly <i>Indianapolis, IN</i>	myeloproliferative disorders	Phase II lilly.com
mometinib (JAK inhibitor) (Orphan Drug)	Gilead Sciences <i>Foster City, CA</i>	myelofibrosis	Phase II gilead.com
NS-018 (JAK2 inhibitor)	Nippon Shinyaku <i>Tokyo, Japan</i>	myelofibrosis	Phase I/II nippon-shinyaku.co.jp
pacritinib (JAK2/FLT3 inhibitor) (Orphan Drug)	Cell Therapeutics <i>Seattle, WA</i>	myelofibrosis (see also lymphoma)	Phase III celltherapeutics.com
PKC412 (midostaurin)	Novartis Pharmaceuticals <i>East Hanover, NJ</i>	aggressive systemic mastocytosis (see also leukemia)	Phase II novartis.com
<b>Pomalyst®</b> pomalidomide	Celgene <i>Summit, NJ</i>	myelofibrosis	Phase III celgene.com
pracinostat (HDAC inhibitor)	MEI Pharma <i>San Diego, CA</i>	myelofibrosis (see also leukemia)	Phase II meipharma.com

## Myeloproliferative Neoplasms

Product Name	Sponsor	Indication	Development Phase
PRM-151 (rhPTX-2 for injection)	Promedior <i>Lexington, MA</i>	myelofibrosis	Phase I promedior.com
SAR302503 (JAK2 inhibitor)	Sanofi US <i>Bridgewater, NJ</i>	myelofibrosis	Phase III sanofi.com
		polycythemia vera, myelofibrosis (combination therapy)	Phase II sanofi.com
simtuzumab (mAb)	Gilead Sciences <i>Foster City, CA</i>	myelofibrosis	Phase II gilead.com

The content of this report has been obtained through public, government and industry sources, and the Adis "R&D Insight" database based on the latest information. **Report current as of April 17, 2013.** The medicines in this report 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. The information in this report may not be comprehensive. For more specific information about a particular product, contact the individual company directly or go to [www.clinicaltrials.gov](http://www.clinicaltrials.gov). The entire series of Medicines in Development is available on PhRMA's web site.

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**adjunctive treatment**—An auxiliary treatment that is secondary to the main treatment.

**adjuvant**—A substance or drug that aids another substance in its action.

**allogeneic**—Refers to having cell types that are distinct and cause reactions in the immune system.

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

**B-cell**—A class of white blood cells important to the body's immune system.

**Fast Track**—A process designed to facilitate the development and expedite the review of drugs to treat serious diseases and fill an unmet medical need. The status is assigned by the U.S. Food and Drug Administration. The purpose is to get important new drugs to the patient earlier. Fast Track addresses a broad range of serious diseases. Generally, determining factors include whether the drug will have an impact on such factors 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. Filling an unmet medical need is defined as providing a therapy where none exists or providing a therapy which may be potentially superior to existing therapy. Once a drug receives Fast Track designation, early and frequent communication between the FDA and a drug company is encouraged throughout the entire drug development and review process. The frequency of communication assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients.

**hematological malignancies**—Cancers of the blood or blood-forming tissues, such as **leukemia**, **Hodgkin** and **non-Hodgkin lymphomas**, AIDS-related malignancies, **myeloma**, myelodysplasia and myeloproliferative disorders.

**leukemia**—A form of cancer in which abnormally growing white blood cells are scattered throughout the body and bone marrow. They can take over the marrow and prevent it from making enough normal blood cells (white, red and platelets), leaving the patient highly susceptible to serious infections, anemia and bleeding episodes. The cells can also spill into the blood, infiltrating and interfering with the function of other organs. The four main types of leukemia are: **acute lymphoblastic leukemia (ALL)**, **acute myeloid leukemia (AML)**, **chronic lymphocytic leukemia (CLL)**, and **chronic myeloid leukemia (CML)**. The acute types have a rapid onset, and overwhelming infection or blood loss can cause death. The chronic forms progress much more slowly.

**lymphoma**—Cancers in which the cells of lymphoid tissue, found mainly in the lymph nodes and spleen, multiply unchecked. Lymphomas fall into two categories: Hodgkin disease, characterized by a particular kind of abnormal cell, and **non-Hodgkin lymphomas**, which vary in their malignancy according to the nature and activity of the abnormal cells. **Mantle cell lymphoma** is a type of non-Hodgkin lymphoma.

**mAb (monoclonal antibodies)**—Large protein molecules produced by white blood cells that seek out and destroy harmful foreign substances.

**macroglobulinemia**—A disorder in which plasma cells produce an excessive quantity of macroglobulins (large antibodies) that accumulate in the blood. It results from a group of abnormal, cancerous lymphocytes and plasma cells. Men are affected more often than women, and the average age at onset is 65.

**myeloablation**—A severe form of myelosuppression, activity is decreased, resulting in fewer red blood cells, white blood cells, and platelets. It is a side effect of some cancer treatments.

**myelodysplastic syndromes (MDS)**—Blood disorders that ultimately are fatal.

Patients usually succumb to infections or bleeding. The term "preleukemia" has been used to describe these disorders.

**myeloma**—A malignant condition of middle to old age, characterized by the uncontrolled proliferation and disordered function of plasma cells in the bone marrow. The condition, which makes the patient particularly prone to infection, is rare, with about three new cases annually per 100,000 population. **Relapsed myeloma**, also called recurrent myeloma, is multiple myeloma that returns after a successful course of treatment. **Refractory myeloma** is multiple myeloma that does not respond to treatment—whether initial therapy or therapy for recurrent disease. **Smoldering multiple myeloma** refers to early disease without any symptoms. For example, there is no bone damage.

**myeloproliferative neoplasms (MPN)**—Types of blood cancer that begin with an abnormal mutation (change) in a stem cell in the bone marrow. That change leads to an overproduction of any combination of white cells, red cells and platelets. Such blood cancers include myelofibrosis, polycythemia vera, and essential thrombocythemia.

**Orphan Drug**—A drug to treat a disease that has a patient population of 200,000 or less in the United States, or a disease that has a patient population of more than 200,000 and a development cost that will not be recovered from sales in the United States.

**Phase 0**—First-in-human trials conducted in accordance with FDA's 2006 guidance on exploratory Investigational New Drug (IND) studies designed to speed up development of promising drugs by establishing very early on whether the agent behaves in human subjects as was anticipated from preclinical studies.

**Phase I**—Researchers test the drug in a small group of people, usually between 20 and 80 healthy adult volunteers, to evaluate its initial safety and tolerability

profile, determine a safe dosage range, and identify potential side effects.

**Phase II**—The drug is given to volunteer patients, usually between 100 and 300, to see if it is effective, identify an optimal dose, and to further evaluate its short-term safety.

**Phase III**—The drug is given to a larger, more diverse patient population, often involving between 1,000 and 3,000 patients (but sometime many more thousands), to generate statistically significant evidence to confirm its safety

and effectiveness. They are the longest studies, and usually take place in multiple sites around the world.

**preleukemia**—A condition in which the bone marrow does not produce enough blood cells. This condition may progress to become acute leukemia. Preleukemia also is called **myelodysplastic syndromes** or smoldering leukemia.

**Relapsed/Refractory**—Relapsed is a condition when cancer cells return to a patient's bone marrow following treatment, while refractory is a condition

when patients still have some cancer cells in their bone marrow after treatment.

**Smoldering**—See preleukemia.

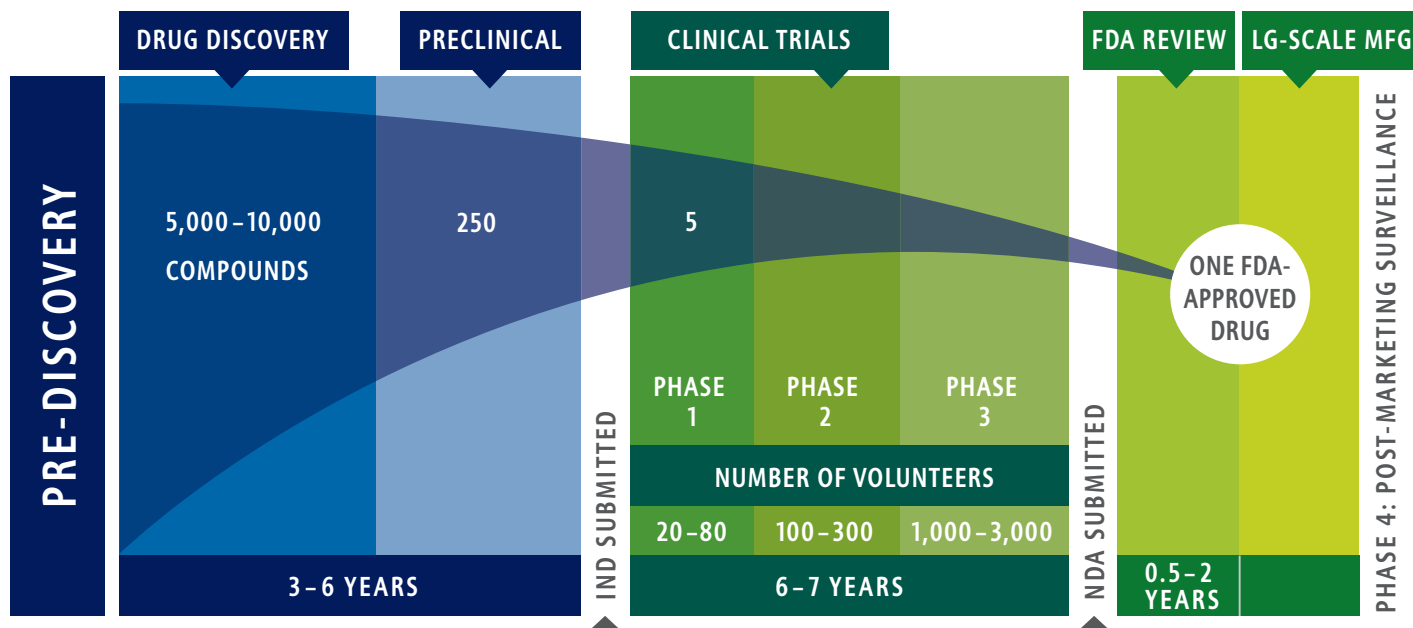
**T-cell**—One of two main classes of white blood cells called lymphocytes, which are important to the body's disease-fighting immune system.

**thrombocytopenia**—A reduction in the number of platelet cells in the blood, which causes a tendency to bleed, especially from the smaller blood vessels.

# The Drug Discovery, Development and Approval Process

Developing a new medicine takes an average of 10-15 years;  
For every 5,000-10,000 compounds in the pipeline, only 1 is approved.

## Drug Discovery and Development: A LONG, RISKY ROAD



## The Drug Development and Approval Process

**The U.S. system of new drug approvals is perhaps the most rigorous in the world.**

It takes 10-15 years, on average, for an experimental drug to travel from lab to U.S. patients, according to the Tufts Center for the Study of Drug Development. Only five in 5,000 compounds that enter preclinical testing make it to human testing. And only one of those five is approved for sale.

On average, it costs a company \$1.2 billion, including the cost of failures, to get one new medicine from the laboratory to U.S. patients, according to a recent study by the Tufts Center for the Study of Drug Development.

Once a new compound has been identified in the laboratory, medicines are usually developed as follows:

**Preclinical Testing.** A pharmaceutical company conducts laboratory and animal studies to show biological activity of the compound against the targeted disease, and the compound is evaluated for safety.

### Investigational New Drug Application (IND).

After completing preclinical testing, a company files an IND with the U.S. Food and Drug Administration (FDA) to begin to test the drug

in people. The IND shows results of previous experiments; how, where and by whom the new studies will be conducted; the chemical structure of the compound; how it is thought to work in the body; any toxic effects found in the animal studies; and how the compound is manufactured. All clinical trials must be reviewed and approved by the Institutional Review Board (IRB) where the trials will be conducted. Progress reports on clinical trials must be submitted at least annually to FDA and the IRB.

**Clinical Trials, Phase I**—Researchers test the drug in a small group of people, usually between 20 and 80 healthy adult volunteers, to evaluate its initial safety and tolerability profile, determine a safe dosage range, and identify potential side effects.

**Clinical Trials, Phase II**—The drug is given to volunteer patients, usually between 100 and 300, to see if it is effective, identify an optimal dose, and to further evaluate its short-term safety.

**Clinical Trials, Phase III**—The drug is given to a larger, more diverse patient population, often involving between 1,000 and 3,000 patients (but sometime many more thousands), to gener-

ate statistically significant evidence to confirm its safety and effectiveness. They are the longest studies, and usually take place in multiple sites around the world.

**New Drug Application (NDA)/Biologic License Application (BLA).** Following the completion of all three phases of clinical trials, a company analyzes all of the data and files an NDA or BLA with FDA if the data successfully demonstrate both safety and effectiveness. The applications contain all of the scientific information that the company has gathered. Applications typically run 100,000 pages or more.

**Approval.** Once FDA approves an NDA or BLA, the new medicine becomes available for physicians to prescribe. A company must continue to submit periodic reports to FDA, including any cases of adverse reactions and appropriate quality-control records. For some medicines, FDA requires additional trials (Phase IV) to evaluate long-term effects.

Discovering and developing safe and effective new medicines is a long, difficult, and expensive process. PhRMA member companies invested an estimated \$48.5 billion in research and development in 2012.