

## Medicines in Development – Blood Disorders

### Anemias

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Accrufer®</b> ferric maltol	Shield Therapeutics Austin, TX	iron deficiency anemia (age 1 month to 17 years)	Phase III <a href="http://www.shieldtherapeutics.com">www.shieldtherapeutics.com</a>
AND017 (prolyl hydroxylase inhibitor)	Kind Pharmaceuticals Redwood City, CA	anemia in chronic kidney disease (CKD)	Phase II <a href="http://www.kindpharmaceuticals.com">www.kindpharmaceuticals.com</a>
ANX005 (complement C1 inhibitor)	Annexon Biosciences Brisbane, CA	warm autoimmune hemolytic anemia	Phase I <a href="http://www.annexonbio.com">www.annexonbio.com</a>
BGE-117 (HIF-PH inhibitor)	BioAgeLabs Richmond, CA	unexplained anemia of aging	Phase II <a href="http://www.bioagelabs.com">www.bioagelabs.com</a>
daprodustat (prolyl hydroxylase inhibitor)	GlaxoSmithKline Research Triangle Park, NC	anemia in CKD	application submitted <a href="http://www.gsk.com">www.gsk.com</a>
DISC-0974 (HFE2 inhibitor)	Disc Medicine Watertown, MA	anemia of inflammation	Phase I <a href="http://www.discmedicine.com">www.discmedicine.com</a>
<b>Empaveli®</b> pegcetacoplan ORPHAN DRUG	Apellis Pharmaceuticals Waltham, MA	cold agglutinin disease	Phase III <a href="http://www.apellis.com">www.apellis.com</a>

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INCB00928 (ALK2 inhibitor)	Incyte Wilmington, DE	anemia due to hematological disorders	Phase II <a href="http://www.incyte.com">www.incyte.com</a>
iptacopan (LNP023) (CFB inhibitor)	Novartis East Hanover, NJ	cold agglutinin disease	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
KER-047 (ACVR1 protein inhibitor)	Keros Therapeutics Lexington, MA	iron deficiency anemia, anemia from high hepcidin	Phase II <a href="http://www.kerostx.com">www.kerostx.com</a>
nipocalimab (Fc receptor antagonist) ORPHAN DRUG	Janssen Research & Development Raritan, NJ	warm autoimmune hemolytic anemia (Fast Track)	Phase III <a href="http://www.janssen.com">www.janssen.com</a>
		----- hemolytic disease of the fetus and new born	Phase II <a href="http://www.janssen.com">www.janssen.com</a>
parsaclisib (PI3K inhibitor)	Incyte Wilmington, DE	warm autoimmune hemolytic anemia	Phase III <a href="http://www.incyte.com">www.incyte.com</a>
<b>Pyrukynd</b> <sup>®</sup> mitapivat	Agios Pharmaceuticals Cambridge, MA	non-transfusion PK deficiency (pediatric), transfusion-dependent PK deficiency	Phase III <a href="http://www.agios.com">www.agios.com</a>
REGN7257 (IL-2Rg antibody)	Regeneron Pharmaceuticals Tarrytown, NY	aplastic anemia	Phase I <a href="http://www.regeneron.com">www.regeneron.com</a>

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rilzabrutinib (BTK inhibitor)	Sanofi Bridgewater, NJ	warm autoimmune hemolytic anemia	Phase II <a href="http://www.sanofi.com">www.sanofi.com</a>
roxadustat (HIF-PH inhibitor)	FibroGen San Francisco, CA	anemia in CKD, anemia in myelodysplastic syndromes (MDS)	Phase III <a href="http://www.fibrogen.com">www.fibrogen.com</a>
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		chemotherapy-induced anemia	Phase II <a href="http://www.fibrogen.com">www.fibrogen.com</a>
RP-L102 (mozafancogene autotemcel) (lentiviral vector-based gene therapy) <b>ORPHAN DRUG</b>	Rocket Pharmaceuticals Cranbury, NJ	Fanconi's anemia (Fast Track)	Phase II <a href="http://www.rocketpharma.com">www.rocketpharma.com</a>
SAR445088 (complement C1s inhibitor)	Sanofi Bridgewater, NJ	cold agglutinin disease	Phase II <a href="http://www.sanofi.com">www.sanofi.com</a>
<b>Sarclisa</b> <sup>®</sup> isatuximab	Sanofi Bridgewater, NJ	warm autoimmune hemolytic anemia	Phase II <a href="http://www.sanofi.com">www.sanofi.com</a>
<b>Tavalisse</b> <sup>®</sup> fostamatinib <b>ORPHAN DRUG</b>	Rigel Pharmaceuticals South San Francisco, CA	autoimmune hemolytic anemia (Fast Track)	Phase III <a href="http://www.rigel.com">www.rigel.com</a>

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<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
TP-0184 (itacnosertib) (ALK2/ALK5 inhibitor)	Sumitomo Pharma Oncology Cambridge, MA	anemia in MDS	Phase I/II <a href="http://www.oncology.sumitomo-pharma.com">www.oncology.sumitomo-pharma.com</a>
<b>Triferic®</b> ferric pyrophosphate citrate	Rockwell Medical Technologies Wixom, MI	iron deficiency anemia in end-stage renal disease (6 months - 17 years)	Phase III <a href="http://www.rockwellmd.com">www.rockwellmd.com</a>
		iron deficiency anemia in home infusion setting, iron deficiency in acute heart failure	Phase I <a href="http://www.rockwellmd.com">www.rockwellmd.com</a>
vadadustat (HIF-PH inhibitor)	Akebia Therapeutics Cambridge, MA	anemia in CKD	Phase III <a href="http://www.akebia.com">www.akebia.com</a>

## Blood Clots

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AB002 (E-WE thrombin) (protein C stimulant)	Aronora Portland, OR	thrombosis with end stage renal disease (ESRD) on chronic hemodialysis (Fast Track)	Phase II <a href="http://www.aronorabio.com">www.aronorabio.com</a>
AB023 (xisomab 3G3) (factor XI/XII inhibitor)	Aronora Portland, OR	thrombosis	Phase II <a href="http://www.aronorabio.com">www.aronorabio.com</a>

## Blood Clots

### Drug Name

### Sponsor

### Indication

### Development Phase

abelacimab  
(factor XI/Xia inhibitor)

Anthos Therapeutics  
Cambridge, MA

arterial thromboembolism,  
venous thromboembolism

Phase II  
[www.anthostherapeutics.com](http://www.anthostherapeutics.com)

apadamtase alfa (TAK-755)  
(ADAM protein replacement)  
ORPHAN DRUG

Takeda  
Deerfield, IL

congenital thrombotic  
thrombocytopenic purpura  
(Fast Track)

Phase III  
[www.takeda.com](http://www.takeda.com)

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idiopathic thrombotic  
thrombocytopenic purpura

Phase II  
[www.takeda.com](http://www.takeda.com)

asundexian  
(FXIa inhibitor)

Bayer Pharmaceuticals  
Whippany, NJ

prevention of thromboembolism

Phase II  
[www.bayer.com](http://www.bayer.com)

BMS-986209  
(factor XIa inhibitor)

Bristol-Myers Squibb  
Princeton, NJ

thrombosis

Phase I  
[www.bms.com](http://www.bms.com)

danaparoid  
(factor Xa inhibitor)

Aspen Global  
Grand Bay, Mauritius

heparin-induced thrombocytopenia

Phase III  
[www.aspengl.com](http://www.aspengl.com)

**Eliquis®**  
apixaban

Bristol-Myers Squibb  
Princeton, NJ  
Pfizer  
New York, NY

venous thromboembolism prevention  
in pediatric acute lymphocytic  
leukemia

Phase III  
[www.bms.com](http://www.bms.com)

## Blood Clots

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fesomersen (FXI-LICA antisense)	Bayer Pharmaceuticals Whippany, NJ	thrombosis prevention in ESRD	Phase II <a href="http://www.bayer.com">www.bayer.com</a>
ION547 (factor XII inhibitor)	Ionis Pharmaceuticals Carlsbad, CA	thrombosis	Phase I <a href="http://www.ionispharma.com">www.ionispharma.com</a>
milvexian (factor XIa inhibitor)	Bristol-Myers Squibb Princeton, NJ Janssen Research & Development Raritan, NJ	prevention and treatment of thrombosis	Phase II <a href="http://www.bms.com">www.bms.com</a>
osocimab (factor XIa inhibitor)	Aronora Portland, OR Bayer Pharmaceuticals Whippany, NJ	thrombosis prevention in ESRD	Phase II <a href="http://www.bayer.com">www.bayer.com</a>
REGN9933 (factor XI inhibitor)	Regeneron Pharmaceuticals Tarrytown, NY	thrombosis	Phase I <a href="http://www.regeneron.com">www.regeneron.com</a>
VLX-1005 (12-lipoxygenase inhibitor) ORPHAN DRUG	Veralox Therapeutics Frederick, MD	heparin-induced thrombocytopenia, heparin-induced thrombocytopenia with thrombosis	Phase I <a href="http://www.veralox.com">www.veralox.com</a>

## Hematologic Malignancies

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ABBV-155 (antibody drug conjugate)	AbbVie North Chicago, IL	hematological malignancies	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a>
ABBV-621 (eftozanermin alfa) (TRAIL receptor agonist)	AbbVie North Chicago, IL	hematologic malignancies	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a>
ABBV-CX-2029 (anti-CD71 probody drug conjugate)	AbbVie North Chicago, IL CytomX Therapeutics South San Francisco, CA	hematological malignancies	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a>
AMG 176 (tapotoclax) (MCL-1 inhibitor)	Amgen Thousand Oaks, CA	hematological malignancies	Phase I <a href="http://www.amgen.com">www.amgen.com</a>
AZD0466 (BCL2/xL inhibitor)	AstraZeneca Wilmington, DE	hematological malignancies	Phase I <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
AZD4573 (CDK9 inhibitor)	AstraZeneca Wilmington, DE	hematological malignancies (monotherapy and +acalabrutinib)	Phase II <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
AZD5991 (MCL1 inhibitor)	AstraZeneca Wilmington, DE	hematological malignancies	Phase I <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
AZD7789 (PD-1 x TIM3 bispecific antibody)	AstraZeneca Wilmington, DE	hematological malignancies	Phase I <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>

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<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
BGB-11417 (Bcl-2 inhibitor)	BeiGene Cambridge, MA	B-cell malignancies	Phase I <a href="http://www.beigene.com">www.beigene.com</a>
BGB-16673 (BTK degrader)	BeiGene Cambridge, MA	B-cell malignancies	Phase I <a href="http://www.beigene.com">www.beigene.com</a>
BMS-986158 (BET inhibitor)	Bristol-Myers Squibb Princeton, NJ	hematologic malignancies	Phase II <a href="http://www.bms.com">www.bms.com</a>
CC-90010 (trotabresib) (BET inhibitor)	Bristol-Myers Squibb Princeton, NJ	hematologic malignancies	Phase I <a href="http://www.bms.com">www.bms.com</a>
CK1-alpha degrader (casein kinase 1 alpha degrader)	Bristol-Myers Squibb Princeton, NJ	hematologic malignancies	Phase I <a href="http://www.bms.com">www.bms.com</a>
CLBR001 + SWI019 (CAR-T cell therapy)	AbbVie North Chicago, IL Calibr La Jolla, CA	B-cell malignancies (Fast Track)	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a>
CTX110 (CRISPR/Cas9 gene-edited CAR-T cell therapy) <b>ORPHAN DRUG</b>	CRISPR Therapeutics Cambridge, MA	B-cell malignancies	Phase I <a href="http://www.crisprtx.com">www.crisprtx.com</a>



## Hematologic Malignancies

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CYC140 (PLK1 inhibitor)	Cyclacel Pharmaceuticals Berkeley Heights, NJ	hematologic malignancies	Phase I <a href="http://www.cyclacel.com">www.cyclacel.com</a>
ECT-001-CB (stem cell therapy)	ExCellThera Montreal, Canada	hematologic malignancies, myeloid malignancies	Phase I/II <a href="http://www.excellthera.com">www.excellthera.com</a>
favezelimab (MK-4280) (CD223 antigen inhibitor)	Merck Kenilworth, NJ	hematologic malignancies	Phase II <a href="http://www.merck.com">www.merck.com</a>
fianlimab (REGN3767) (LAG3 antibody)	Regeneron Pharmaceuticals Tarrytown, NY	hematologic malignancies	Phase I <a href="http://www.regeneron.com">www.regeneron.com</a>
futibatinib (FGFR 1-4 inhibitor)	Taiho Oncology Princeton, NJ	lymphoid or myeloid neoplasms	Phase II <a href="http://www.taihooncology.com">www.taihooncology.com</a>
GEN-3009 (IgG1 bispecific antibody)	AbbVie North Chicago, IL Genmab US Plainsboro, NJ	B-cell malignancies	Phase I/II <a href="http://www.abbvie.com">www.abbvie.com</a>
GEN-3014 (CD38 hexabody)	Genmab US Plainsboro, NJ	hematologic malignancies	Phase I <a href="http://www.genmab.com">www.genmab.com</a>
glofitamab (RG6026) (CD20 x CD3 bispecific antibody)	Roche/Genentech South San Francisco, CA	hematologic malignancies	Phase I <a href="http://www.roche.com">www.roche.com</a>

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<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
HDM201 (siremadlin) (MDM2 inhibitor)	Novartis East Hanover, NJ	hematologic malignancies	Phase I <a href="http://www.novartis.com">www.novartis.com</a>
ianalumab (VAY736) (BAFF-R inhibitor)	Novartis East Hanover, NJ	hematologic malignancies (+ibrutinib)	Phase I <a href="http://www.novartis.com">www.novartis.com</a>
IGM-8444 (TRAIL receptor 2 agonist)	IGM Biosciences Mountain View, CA	hematologic malignancies	Phase I <a href="http://www.igmbio.com">www.igmbio.com</a>
JBH492	Novartis East Hanover, NJ	hematologic malignancies	Phase I <a href="http://www.novartis.com">www.novartis.com</a>
JNJ-4681 (BTK inhibitor)	Janssen Research & Development Raritan, NJ	hematologic malignancies	Phase I <a href="http://www.janssen.com">www.janssen.com</a>
JNJ-6617 (MLL1 inhibitor)	Janssen Research & Development Raritan, NJ	hematologic malignancies	Phase I <a href="http://www.janssen.com">www.janssen.com</a>
JNJ-6633 (MALT1 inhibitor)	Janssen Research & Development Raritan, NJ	hematologic malignancies	Phase I <a href="http://www.janssen.com">www.janssen.com</a>
JNJ-6665 (DHODH inhibitor)	Janssen Research & Development Raritan, NJ	hematologic malignancies	Phase I <a href="http://www.janssen.com">www.janssen.com</a>

## Hematologic Malignancies

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
JNJ-8780	Janssen Research & Development Raritan, NJ	hematologic malignancies	Phase I <a href="http://www.janssen.com">www.janssen.com</a>
LOXO-338 (Bcl-2 inhibitor)	Lilly Indianapolis, IN	hematologic malignancies	Phase I <a href="http://www.lilly.com">www.lilly.com</a>
LP-108 (Bcl-2 inhibitor)	Newave Pharmaceutical Pleasanton, CA	hematologic malignancies	Phase I
LP-118 (Bcl-XL inhibitor)	Newave Pharmaceutical Pleasanton, CA	hematologic malignancies	Phase I
LP-168	Newave Pharmaceutical Pleasanton, CA	hematologic malignancies	Phase I
LY3410738 (IDH1 inhibitor)	Lilly Indianapolis, IN	hematologic malignancies	Phase I <a href="http://www.lilly.com">www.lilly.com</a>
MB-102 (CAR-T cell therapy) ORPHAN DRUG	Mustang Bio Worcester, MA	blastic plasmacytoid dendritic cell neoplasm	Phase I/II <a href="http://www.mustangbio.com">www.mustangbio.com</a>
<b>Monjuvi</b> <sup>®</sup> tafasitamab-cxix	Incyte Wilmington, DE MorphoSys Boston, MA	B-cell malignancies	Phase I <a href="http://www.incyte.com">www.incyte.com</a> <a href="http://www.morphosys.com">www.morphosys.com</a>

## Hematologic Malignancies

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
mosunetuzumab (RG7828) (CD20/CD3 bispecific antibody)	Roche Genentech South San Francisco, CA	hematologic malignancies	Phase I <a href="http://www.roche.com">www.roche.com</a>
navtemadlin (KRT-232) (MDM2-p53 inhibitor)	Kartos Therapeutics Redwood City, CA	B-cell malignancies	Phase I <a href="http://www.kartisthera.com">www.kartisthera.com</a>
nemtabrutinib (MK-1026) (BTK inhibitor)	Merck Kenilworth, NJ	hematological malignancies	Phase II <a href="http://www.merck.com">www.merck.com</a>
<b>Nerofe™</b>	Immune System Key Jerusalem, Israel	hematological malignancies	Phase I <a href="http://www.immunesk.com">www.immunesk.com</a>
NKX019 (CAR-NK cell therapy)	Nkarta Therapeutics South San Francisco, CA	B-cell malignancies	Phase I <a href="http://www.nkartatx.com">www.nkartatx.com</a>
NX-2127 (BTK degrader)	Nurix Therapeutics San Francisco, CA	B-cell malignancies	Phase I <a href="http://www.nurixtx.com">www.nurixtx.com</a>
NX-5948 (BTK degrader)	Nurix Therapeutics San Francisco, CA	B-cell malignancies	Phase I <a href="http://www.nurixtx.com">www.nurixtx.com</a>
odronextamab (CD20 x CD3 bispecific antibody)	Regeneron Tarrytown, NY	B-cell malignancies	Phase I <a href="http://www.regeneron.com">www.regeneron.com</a>

## Hematologic Malignancies

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
omidubice (stem cell therapy) <b>ORPHAN DRUG</b>	Gamida Cell Boston, MA	hematologic malignancies (Breakthrough Therapy)	application submitted <a href="http://www.gamida-cell.com">www.gamida-cell.com</a>
<b>Opdivo®</b> nivolumab	Bristol-Myers Squibb Princeton, NJ	hematologic malignancies	Phase I <a href="http://www.bms.com">www.bms.com</a>
<b>Opdualag™</b> nivolumab and relatlimab	Bristol-Myers Squibb Princeton, NJ	hematologic malignancies	Phase I <a href="http://www.bms.com">www.bms.com</a>
orelabrutinib (ICP-022) (BTK inhibitor)	InnoCare Pharma Beijing, China	B-cell malignancies	Phase I/II <a href="http://www.innocarepharma.com">www.innocarepharma.com</a>
PF-07901801 (TTI-622) (CD47-SIPR-alpha fusion protein)	Pfizer New York, NY	hematologic malignancies	Phase II <a href="http://www.pfizer.com">www.pfizer.com</a>
pirtobrutinib (BTK inhibitor)	Lilly Indianapolis, IN	B-cell malignancies	Phase II <a href="http://www.lilly.com">www.lilly.com</a>
pivekimab sunirine (anti-CD123 ADC) <b>ORPHAN DRUG</b>	ImmunoGen Waltham, MA	blastic plasmacytoid dendritic cell neoplasm (Breakthrough Therapy)	Phase II <a href="http://www.immunogen.com">www.immunogen.com</a>

## Hematologic Malignancies

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
plamotamab (XmAb13676) (CD20 x CD3 bispecific antibody)	Janssen Research & Development Raritan, NJ Xencor Monrovia, CA	B-cell malignancies	Phase I <a href="http://www.xencor.com">www.xencor.com</a>
PRGN-3007 (UltraCar-T cell therapy)	Precigen Germantown, MD	ROR1+ hematologic malignancies	Phase I <a href="http://www.precigen.com">www.precigen.com</a>
PRT543 (PRMT5 inhibitor)	Prelude Therapeutics Wilmington, DE	hematologic malignancies	Phase I <a href="http://www.preludetx.com">www.preludetx.com</a>
RG6076 (RO7227166) (CD19-4-1 BBL)	Roche/Genentech South San Francisco, CA	hematologic malignancies	Phase I <a href="http://www.roche.com">www.roche.com</a>
Rivo-Cel™ (BPX-501) (allogenic polyclonal T-cell therapy)	Bellicum Pharmaceuticals Houston, TX	hematologic malignancies	Phase I/II <a href="http://www.bellicum.com">www.bellicum.com</a>
ROR1 CAR-T (CAR-T cell therapy)	Bristol-Myers Squibb Princeton, NJ	hematologic malignancies	Phase I <a href="http://www.bms.com">www.bms.com</a>
sargramostim (rhu GM-CSF)	Partner Therapeutics Lexington, MA	transplant-related hematologic malignancies	Phase II <a href="http://www.partnertx.com">www.partnertx.com</a>
TAK-940 (CAR-T cell therapy)	Takeda Deerfield, IL	CD19+ hematologic malignancies	Phase II <a href="http://www.takeda.com">www.takeda.com</a>

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Tecartus® brexucabtagene autoleucel	Gilead Sciences Foster City, CA	rare B-cell malignancies	Phase II <a href="http://www.gilead.com">www.gilead.com</a>
TG-1501 (cosibelimab) (anti-PD-L1 mAb)	TG Therapeutics Morrisville, NC	B-cell malignancies	Phase I <a href="http://www.tgtherapeutics.com">www.tgtherapeutics.com</a>
TG-1701 (BTK inhibitor)	TG Therapeutics Morrisville, NC	B-cell malignancies	Phase I <a href="http://www.tgtherapeutics.com">www.tgtherapeutics.com</a>
TG-1801 (CD47 x CD19 bispecific antibody)	TG Therapeutics Morrisville, NC	B-cell malignancies	Phase I <a href="http://www.tgtherapeutics.com">www.tgtherapeutics.com</a>
tinostamustine (HDAC inhibitor)	Mundipharma EDO Cambridge, United Kingdom	hematologic malignancies	Phase I <a href="http://www.mundipharma.com">www.mundipharma.com</a>
tiragolumab (RG6058) (TIGIT inhibitor)	Roche/Genentech South San Francisco, CA	hematologic malignancies	Phase I <a href="http://www.roche.com">www.roche.com</a>
vibostolimab + pembrolizumab coformulation (MK-7684A)	Merck Kenilworth, NJ	hematologic malignancies	Phase II <a href="http://www.merck.com">www.merck.com</a>
voruciclib (CDK9 inhibitor)	MEI Pharma San Diego, CA	hematologic malignancies	Phase I <a href="http://www.meipharma.com">www.meipharma.com</a>

## Hematologic Malignancies

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zandelisib (PI3K delta inhibitor)	MEI Pharma San Diego, CA	B-cell malignancies	Phase I <a href="http://www.meipharma.com">www.meipharma.com</a>
zilovertamab vedotin (MK-2140) (ROR1 antagonist) ORPHAN DRUG	Merck Kenilworth, NJ VelosBio San Diego, CA	hematologic malignancies	Phase II <a href="http://www.merck.com">www.merck.com</a>
ZX-101A (PI3K inhibitor)	Zenshine Pharmaceuticals Hangzhou, China	hematologic malignancies	Phase I/II <a href="http://www.zenshine-pharma.com">www.zenshine-pharma.com</a>

## Hemophilia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
concizumab (anti-TFPI mAb) ORPHAN DRUG	Novo Nordisk Plainsboro, NJ	hemophilia A, hemophilia B (Breakthrough Therapy)	Phase III <a href="http://www.novonordisk.com">www.novonordisk.com</a>
efanesoctocog alfa (blood coagulation factor replacement) ORPHAN DRUG	Sanofi Bridgewater, NJ	hemophilia A (Fast Track)	Phase III <a href="http://www.sanofi.com">www.sanofi.com</a>



## Hemophilia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
etranacogene dezaparvovec (AAV5-based gene therapy)	CSL Behring King of Prussia, PA uniQure Lexington, MA	hemophilia B (Breakthrough Therapy)	Phase III <a href="http://www.cslbehring.com">www.cslbehring.com</a> <a href="http://www.uniqure.com">www.uniqure.com</a>
fidanacogene elaparvovec (factor IX gene therapy) ORPHAN DRUG	Pfizer New York, NY Sparks Therapeutics Philadelphia, PA	hemophilia (Breakthrough Therapy)	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
fitusiran (RNAi targeting anti-thrombin) ORPHAN DRUG	Sanofi Bridgewater, NJ	hemophilia A (Fast Track), hemophilia B (Fast Track) (adult and pediatric)	Phase III <a href="http://www.sanofi.com">www.sanofi.com</a>
FLT180a (vergrinacogene setparvovec) (AAV gene therapy)	Freeline Therapeutics New York, NY	hemophilia B	Phase I/II <a href="http://www.freeline.life">www.freeline.life</a>
giroctocogene fitelparvovec (factor VIII gene therapy) ORPHAN DRUG	Pfizer New York, NY	hemophilia (Fast Track)	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
<b>Hemlibra®</b> emicizumab-kxwh	Roche/Genentech South San Francisco, CA	mild to moderate hemophilia A	Phase III <a href="http://www.roche.com">www.roche.com</a>

## Hemophilia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
marstacimab (PF-06741086) (tissue factor pathway inhibitor) <b>ORPHAN DRUG</b>	Pfizer New York, NY	hemophilia (Fast Track)	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
Mim8 (FVIII mimetic bispecific antibody)	Novo Nordisk Plainsboro, NJ	hemophilia A	Phase III <a href="http://www.novonordisk.com">www.novonordisk.com</a>
NXT007 (factor IXa x X bispecific antibody)	Chugai Pharma USA Berkeley Heights, NJ	hemophilia A	Phase I <a href="http://www.chugai-pharm-co.jp">www.chugai-pharm-co.jp</a>
OPK-88005 (factor VIIa-CTP) <b>ORPHAN DRUG</b>	OPKO Health Miami, FL	hemophilia	Phase II <a href="http://www.opko.com">www.opko.com</a>
peboctocogene camaparvovec (FVIII gene therapy)	Bayer Pharmaceuticals Whippany, NJ	hemophilia A	Phase I <a href="http://www.bayer.com">www.bayer.com</a>
RG6357 (SPK-8011) (factor VIII gene therapy) <b>ORPHAN DRUG</b>	Roche/Genentech South San Francisco, CA Sparks Therapeutics Philadelphia, PA	hemophilia A (Breakthrough Therapy)	Phase II <a href="http://www.roche.com">www.roche.com</a>
RG6358 (SPK-8016) (AAV-based gene therapy)	Roche/Genentech South San Francisco, CA Sparks Therapeutics Philadelphia, PA	hemophilia A with inhibitors to factor VIII	Phase II <a href="http://www.roche.com">www.roche.com</a>

## Hemophilia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
SerpinPC (APC inhibitor)	Centessa Boston, MA	hemophilia A, hemophilia B	Phase II <a href="http://www.centessa.com">www.centessa.com</a>
SIG-001 (BDD-FVIII cell therapy) ORPHAN DRUG	Sigilon Therapeutics Cambridge, MA	hemophilia A	Phase I/II <a href="http://www.sigilon.com">www.sigilon.com</a>
valoctocogene roxaparvovec (AAV5 gene therapy) ORPHAN DRUG	BioMarin Pharmaceutical San Rafael, CA	hemophilia A (Breakthrough Therapy)	application submitted <a href="http://www.biomarin.com">www.biomarin.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ABBV-IMAB-TJC4 (anti-CD47 mAb)	AbbVie North Chicago, IL	acute myeloid leukemia (AML)	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a>
Actimab-A (radioimmunoconjugate and hM195 mAb) ORPHAN DRUG	Actinium Pharmaceuticals New York, NY	AML	Phase I <a href="http://www.actiniumpharma.com">www.actiniumpharma.com</a>
AMG 427 (FLT3 x CD3 bispecific T-cell engager)	Amgen Thousand Oaks, CA	AML	Phase I <a href="http://www.amgen.com">www.amgen.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AMV565 (CD33 x CD3 bispecific T-cell engager) <b>ORPHAN DRUG</b>	Amphivena Therapeutics South San Francisco, CA	AML	Phase I <a href="http://www.amphivena.com">www.amphivena.com</a>
APG-115 (alrizomadlin) (MDM2-p53)	Ascentage Pharma Rockville, MD	AML	Phase I <a href="http://www.ascentagepharma.com">www.ascentagepharma.com</a>
APG-2575 (lisaftoclax) (Bcl-2 inhibitor) <b>ORPHAN DRUG</b>	Ascentage Pharma Rockville, MD	chronic lymphocytic leukemia (CLL), T-cell prolymphocytic leukemia	Phase II <a href="http://www.ascentagepharma.com">www.ascentagepharma.com</a>
APVO436 (CD123xCD3 bispecific antibody) <b>ORPHAN DRUG</b>	Aptevo Therapeutics Seattle, WA	AML	Phase I <a href="http://www.aptevotherapeutics.com">www.aptevotherapeutics.com</a>
ASP7517 (cell therapy)	Astellas Pharma US Northbrook, IL	AML	Phase II <a href="http://www.astellas.com">www.astellas.com</a>
aspacytarabine (BST-236) (anti-metabolite) <b>ORPHAN DRUG</b>	BioSight Lod, Israel	AML (Fast Track)	Phase II <a href="http://www.biosight-pharma.com">www.biosight-pharma.com</a>
azacitidine/cedazuridine (ASTX030) (oral DNMT inhibitor)	Astex Pharmaceuticals Pleasanton, CA	AML, chronic myelomonocytic leukemia (CMML)	Phase II/III <a href="http://www.astx.com">www.astx.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
bemcentinib (AXL inhibitor) <b>ORPHAN DRUG</b>	BerGenBio Bergen, Norway	AML	Phase I/II <a href="http://www.bergenbio.com">www.bergenbio.com</a>
BMS-986397 (CC-91633) (CRBN modulator CELMoD)	Bristol-Myers Squibb Princeton, NJ	AML	Phase I <a href="http://www.bms.com">www.bms.com</a>
<b>Blincyto®</b> blinatumomab	Amgen Thousand Oaks, CA	newly-diagnosed adults with Ph-negative B-cell precursor ALL	Phase III <a href="http://www.amgen.com">www.amgen.com</a>
BP1002 (Bcl-2 inhibitor)	Bio-Path Holdings Bellaire, TX	AML, CLL	Phase I <a href="http://www.biopathholdings.com">www.biopathholdings.com</a>
<b>Breyanzi®</b> lisocabtagene maraleucel	Bristol-Myers Squibb Princeton, NJ	3L+ CLL, 2L+ B-cell ALL (pediatric)	Phase II <a href="http://www.bms.com">www.bms.com</a>
<b>Brukinsa®</b> zanubrutinib <b>ORPHAN DRUG</b>	BeiGene Cambridge, MA	CLL (Breakthrough Therapy)	Phase III <a href="http://www.beigene.com">www.beigene.com</a>
BTX-1188 (CRBN protein modulator)	BioTheryX San Diego, CA	AML	Phase I <a href="http://www.biotheryx.com">www.biotheryx.com</a>
BTX-A51 (kinase inhibitor)	BioTheryX San Diego, CA	AML	Phase I <a href="http://www.biotheryx.com">www.biotheryx.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Calquence®</b> acalabrutinib	AstraZeneca Wilmington, DE	1L CLL (combination therapy)	Phase III <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
CAP-100 (gG1 monoclonal antibody)	Catapult Therapeutics Lelystad, The Netherlands	CLL	Phase I <a href="http://www.catapult-therapeutics.com">www.catapult-therapeutics.com</a>
CB-5339 (CDC48/P97 ATPase inhibitor)	Cleave Therapeutics San Francisco, CA	AML	Phase I <a href="http://www.cleavetherapeutics.com">www.cleavetherapeutics.com</a>
CC-90009 (GSPT1 CELMoD) (CRBN protein modulator) <b>ORPHAN DRUG</b>	Bristol-Myers Squibb Princeton, NJ	AML	Phase I <a href="http://www.bms.com">www.bms.com</a>
CC-95251 (SIRPA protein modulator)	Bristol-Myers Squibb Princeton, NJ	AML	Phase I <a href="http://www.bms.com">www.bms.com</a>
CC-96191 (NK cell engager)	Bristol-Myers Squibb Princeton, NJ	AML	Phase I <a href="http://www.bms.com">www.bms.com</a>
CFI-400945 (PLK4 protein inhibitor)	Treadwell Therapeutics New York, NY	AML (Fast Track), CMML	Phase I/II <a href="http://www.treadwelltx.com">www.treadwelltx.com</a>
CG-806 (luxetpinib) (BTK/FLT3 inhibitor) <b>ORPHAN DRUG</b>	Aptose Biosciences San Diego, CA	AML, CLL	Phase I <a href="http://www.aptose.com">www.aptose.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CLN 049 (FLT3xCD3 bispecific antibody)	Cullinan Oncology Cambridge, MA	AML	Phase I <a href="http://www.cullinanoncology.com">www.cullinanoncology.com</a>
<b>Copiktra®</b> duvelisib ORPHAN DRUG	Secura Bio Las Vegas, NV	CLL	Phase II <a href="http://www.securabio.com">www.securabio.com</a>
crenolanib (PDGFR inhibitor) ORPHAN DRUG	AROG Pharmaceuticals Plano, TX	AML (Fast Track)	Phase III <a href="http://www.arogpharma.com">www.arogpharma.com</a>
CYC140 (PLK1 inhibitor)	Cyclacel Berkeley Heights, NJ	AML	Phase I <a href="http://www.cyclacel.com">www.cyclacel.com</a>
CYNK-001 (taniraleucel) (natural killer cell therapy) ORPHAN DRUG	Celularity Florham Park, NJ	AML (Fast Track)	Phase I <a href="http://www.celularity.com">www.celularity.com</a>
DARIC33 (CAR-T cell therapy)	2seventy bio Cambridge, MA	AML (pediatric)	Phase I <a href="http://www.2seventybio.com">www.2seventybio.com</a>
DFP-10917 (DNA-directed polymerase inhibitor)	Delta-Fly Pharma Tokushima, Japan	AML	Phase III <a href="http://www.delta-flypharma.co.jp">www.delta-flypharma.co.jp</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
DS-1594 (menin-MLL binding inhibitor)	Daiichi Sankyo Basking Ridge, NJ	ALL, AML	Phase I <a href="http://www.daiichisankyo.com">www.daiichisankyo.com</a>
DSP107 (CD47 x 41BB bi-functional fusion protein)	KAHR Medical Jerusalem, Israel	AML, CMML	Phase I <a href="http://www.kahrbio.com">www.kahrbio.com</a>
DSP-5336 (menin-MIL inhibitor)	Sumitomo Pharma Oncology Cambridge, MA	ALL, AML	Phase I/II <a href="http://www.oncology.sumitomo-pharma.com">www.oncology.sumitomo-pharma.com</a>
DSTAT (dociparstat) (multi chemokine inhibitor) <b>ORPHAN DRUG</b>	Chimerix Durham, NC	AML (Fast Track)	Phase III <a href="http://www.chimerix.com">www.chimerix.com</a>
DTRM-555 (BTK inhibitor, everolimus, pomalidomide)	DTRM Biopharma Zhejiang, China	CLL	Phase II
DVX201 (NK cell therapy)	Deverra Therapeutics Seattle, WA	AML	Phase I <a href="http://www.deverratx.com">www.deverratx.com</a>
eltanexor (KPT-8602) (SINE inhibitor)	Karyopharm Therapeutics Newton, MA	AML	Phase I/II <a href="http://www.karyopharm.com">www.karyopharm.com</a>



## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Elzonris®</b> tagraxofusp-erzs	Stemline Therapeutics New York, NY	CMML	Phase II <a href="http://www.stemline.com">www.stemline.com</a>
emavusertib (IRAK4 inhibitor) <b>ORPHAN DRUG</b>	Curis Lexington, MA	IRAK4-driven AML	Phase II <a href="http://www.curis.com">www.curis.com</a>
entospletinib (SYK inhibitor)	Kronos Bio San Mateo, CA	1L fit NPM1-mutated AML	Phase III <a href="http://www.kronosbio.com">www.kronosbio.com</a>
epcoritamab (GEN-3013) (CD3 x CD20 bispecific antibody)	AbbVie North Chicago, IL Genmab US Plainsboro, NJ	CLL	Phase I/II <a href="http://www.abbvie.com">www.abbvie.com</a>
epratuzumab-cys-tesirine (ADCT-602) (DNA cross linking agent)	ADC Therapeutics New Providence, NJ	ALL	Phase I/II <a href="http://www.adctherapeutics.com">www.adctherapeutics.com</a>
eprenetapopt (APR-246) (therapeutic targeting mutant p53) <b>ORPHAN DRUG</b>	Apria Therapeutics Boston, MA	TP53-mutant AML (Fast Track), TP53-mutant CLL	Phase I <a href="http://www.apria.com">www.apria.com</a>
ERAS-007 (ERK1/2 inhibitor)	Erasca San Diego, CA	AML	Phase I <a href="http://www.erasca.com">www.erasca.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ERAS-601 (SHP2 inhibitor)	Erasca San Diego, CA	AML	Phase I <a href="http://www.erasca.com">www.erasca.com</a>
evorpacept (ALX148) (CD47 antigen inhibitor)	ALX Oncology South San Francisco, CA	AML (combination therapy)	Phase I/II <a href="http://www.alxoncology.com">www.alxoncology.com</a>
EX-103 (DNA cross linking agent) <b>ORPHAN DRUG</b>	Exinda Therapeutics El Dorado Hills, CA	CLL	Phase III <a href="http://www.exindarx.com">www.exindarx.com</a>
EX-104 (DNA cross linking agent) <b>ORPHAN DRUG</b>	Exinda Therapeutics El Dorado Hills, CA	CLL	Phase III <a href="http://www.exindarx.com">www.exindarx.com</a>
fadraciclib (CDK2/9 inhibitor)	Cyclacel Pharmaceuticals Berkeley Heights, NJ	AML, CLL (+venetoclax)	Phase I/II <a href="http://www.cyclacel.com">www.cyclacel.com</a>
FF-10101-01 (FLT3 inhibitor)	Fujifilm Pharmaceuticals U.S.A. Cambridge, MA	AML (adults)	Phase I/II completed <a href="http://www.fujifilmpharma.com">www.fujifilmpharma.com</a>
FHD286 (SMARCA2/4 protein inhibitor)	Foghorn Therapeutics Cambridge, MA	AML	Phase I <a href="http://www.foghornrx.com">www.foghornrx.com</a>
ficlatuzumab (anti-HGF/c-MET IgG1 mAb)	AVEO Oncology Boston, MA	AML	Phase II <a href="http://www.aveooncology.com">www.aveooncology.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
fosciclipirox (mitosis inhibitor)	CicloMed Kansas City, MO	AML	Phase I/II <a href="http://www.ciclomed.com">www.ciclomed.com</a>
FT516 (NK cell cancer immunotherapy)	Fate Therapeutics San Diego, CA	AML	Phase I <a href="http://www.fatetherapeutics.com">www.fatetherapeutics.com</a>
FT538 (NK cell cancer immunotherapy)	Fate Therapeutics San Diego, CA	AML	Phase I <a href="http://www.fatetherapeutics.com">www.fatetherapeutics.com</a>
FT596 (NK cell cancer immunotherapy)	Fate Therapeutics San Diego, CA	CLL	Phase I <a href="http://www.fatetherapeutics.com">www.fatetherapeutics.com</a>
FT819 (NK cell cancer immunotherapy)	Fate Therapeutics San Diego, CA	ALL, CLL	Phase I <a href="http://www.fatetherapeutics.com">www.fatetherapeutics.com</a>
galinpepimut-S (cancer peptide vaccine) ORPHAN DRUG	Sellas Life Sciences New York, NY	AML	Phase III <a href="http://www.sellaslifesciences.com">www.sellaslifesciences.com</a>
GDX012 (T lymphocyte cell therapy) ORPHAN DRUG	GammaDelta Therapeutics London, United Kingdom	AML	Phase I <a href="http://www.gammadeltatx.com">www.gammadeltatx.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Graspa®</b> eryaspase ORPHAN DRUG	Erytech Pharma Cambridge, MA	ALL (in patients hypersensitive to E-coli-derived pegylated asparaginase) (Fast Track)	Phase II <a href="http://www.erytech.com">www.erytech.com</a>
H3B-8800 (RVT-2001) (SF3b complex modulator) ORPHAN DRUG	Hemavant Sciences (Roviant) New York, NY	AML, CMML	Phase I <a href="http://www.roviant.com">www.roviant.com</a>
HM43239 (FLT-3/SKY inhibitor) ORPHAN DRUG	Aptose Biosciences San Diego, CA	AML	Phase I/II <a href="http://www.aptose.com">www.aptose.com</a>
HMPL-760 (BTK) inhibitor	Hutchison Medipharma Shanghai, China	CLL	Phase I <a href="http://www.hutch-med.com">www.hutch-med.com</a>
HQP1351 (olverembatinib) (BCR-ABL/KIT) ORPHAN DRUG	Ascentage Pharma Rockville, MD	resistant CML (Fast Track), Ph+ ALL	Phase I <a href="http://www.ascentagepharma.com">www.ascentagepharma.com</a>
<b>Iclusig®</b> ponatinib	Takeda Deerfield, IL	1L Ph+ ALL	Phase III <a href="http://www.takeda.com">www.takeda.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Idhifa</b> <sup>®</sup> enasidenib	Bristol-Myers Squibb Princeton, NJ	AML with IDH2 mutation	Phase III <a href="http://www.bms.com">www.bms.com</a>
		1L AML with IDH2 mutation	Phase II <a href="http://www.bms.com">www.bms.com</a>
IGM-2323 (CD20 x CD3 bispecific antibody)	IGM Biosciences Mountain View, CA	CLL	Phase I <a href="http://www.igmbio.com">www.igmbio.com</a>
<b>Imbruvica</b> <sup>®</sup> ibrutinib	Janssen Raritan, NJ Pharmacyclics Sunnyvale, CA	1L CLL (+venetoclax)	Phase III <a href="http://www.janssen.com">www.janssen.com</a> <a href="http://www.pharmacyclics.com">www.pharmacyclics.com</a>
<b>Inqovi</b> <sup>®</sup> decitabine and cedazuridine	Astex Pharmaceuticals Pleasanton, CA	AML (+venetoclax)	Phase I/II <a href="http://www.astx.com">www.astx.com</a>
IO-202 (LILRB4 protein inhibitor) <b>ORPHAN DRUG</b>	Immune-Onc Therapeutics Palo Alto, CA	AML (Fast Track), CMML	Phase I <a href="http://www.immune-onc.com">www.immune-onc.com</a>
Iomab-B (apamistamab-I-131) <b>ORPHAN DRUG</b>	Actinium Pharmaceuticals New York, NY	BMT conditioning in AML	Phase III <a href="http://www.actiniumpharma.com">www.actiniumpharma.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
IOV-2001 (adoptive cell therapy)	Iovance Biotherapeutics San Carlos, CA	CLL	Phase I <a href="http://www.iovance.com">www.iovance.com</a>
JBH492	Novartis East Hanover, NJ	CLL	Phase I <a href="http://www.novartis.com">www.novartis.com</a>
JEZ567 (CD123 CAR-T cell therapy)	Novartis East Hanover, NJ	AML	Phase I <a href="http://www.novartis.com">www.novartis.com</a>
JSP191 (anti-CD117 mAb)	Jasper Therapeutics Redwood City, CA	AML	Phase I <a href="http://www.jaspertherapeutics.com">www.jaspertherapeutics.com</a>
KITE-222 (anti-CLL-1 CAR-T cell therapy)	Gilead Sciences Foster City, CA	AML	Phase I <a href="http://www.gilead.com">www.gilead.com</a>
KO-539 (menin inhibitor) ORPHAN DRUG	Kura Oncology San Diego, CA	AML	Phase I/II <a href="http://www.kuraoncology.com">www.kuraoncology.com</a>
KT-333 (STAT3 modulator)	Kymera Therapeutics Watertown, MA	large granular lymphocytic leukemia	Phase I <a href="http://www.kymeratx.com">www.kymeratx.com</a>
KUR-502 (NKT cell therapy)	Athenex Buffalo, NY	leukemia	Phase I <a href="http://www.athenex.com">www.athenex.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Kymriah</b> <sup>®</sup> tisagenlecleucel	Novartis East Hanover, NJ	1L high-risk ALL (pediatric and young adults)	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
<b>Kyprolis</b> <sup>®</sup> carfilzomib	Amgen Thousand Oaks, CA	ALL (pediatric)	Phase II <a href="http://www.amgen.com">www.amgen.com</a>
Ianraplenib (SYK inhibitor)	Kronos Bio San Mateo, CA	AML	Phase I <a href="http://www.kronosbio.com">www.kronosbio.com</a>
liposomal annamycin (type II DNA topoisomerase inhibitor) <b>ORPHAN DRUG</b>	Moleculin Biotech Houston, TX	AML (Fast Track)	Phase I/II <a href="http://www.moleculin.com">www.moleculin.com</a>
LNK01002 (triple kinase inhibitor)	Lynk Pharmaceuticals Zhejiang, China	AML	Phase I <a href="http://www.en.lynkpharma.com">www.en.lynkpharma.com</a>
magrolimab (anti-CD47 mAb) <b>ORPHAN DRUG</b>	Gilead Sciences Foster City, CA	1L AML (Fast Track), 1L unfit AML	Phase III <a href="http://www.gilead.com">www.gilead.com</a>
MANA-312 (tumor-associated antigen T-cells)	Mana Therapeutics Waltham, MA	AML after HSCT	Phase I <a href="http://www.manatherapeutics.com">www.manatherapeutics.com</a>
MB-106 (CAR-T cell therapy)	Mustang Bio Worcester, MA	CLL	Phase I/II <a href="http://www.mustangbio.com">www.mustangbio.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
MGTA-117 (RNA polymerase II inhibitor)	Magenta Therapeutics Cambridge, MA	AML	Phase I <a href="http://www.magentatx.com">www.magentatx.com</a>
MGTA-145 (interleukin-8B receptor agonist) ORPHAN DRUG	Magenta Therapeutics Cambridge, MA	leukemia (stem cell mobilization)	Phase II <a href="http://www.magentatx.com">www.magentatx.com</a>
MIK665 (S64315) (MCL1 inhibitor)	Novartis East Hanover, NJ	AML	Phase I <a href="http://www.novartis.com">www.novartis.com</a>
MK-0482	Merck Whitehouse Station, NJ	AML	Phase I <a href="http://www.merck.com">www.merck.com</a>
MRX-2843 (FLT3/MerTk inhibitor)	Meryx Chapel Hill, NC	ALL, AML, mixed phenotype acute leukemia	Phase I <a href="http://www.meryxpharma.com">www.meryxpharma.com</a>
MS-553 (PKC inhibitor)	MingSight Pharmaceuticals San Diego, CA	CLL	Phase I/II <a href="http://www.mingsight.com">www.mingsight.com</a>
MT-401 (zelenoleucel) (T-cell therapy) ORPHAN DRUG	Marker Therapeutics Houston, TX	AML following stem cell transplantation	Phase II <a href="http://www.markertherapeutics.com">www.markertherapeutics.com</a>



## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
navtemadlin (KRT-232) (MDM2-p53 inhibitor)	Kartos Therapeutics Redwood City, CA	AML	Phase II <a href="http://www.kartisthera.com">www.kartisthera.com</a>
		AML (combination therapy), CML (combination therapy)	Phase I <a href="http://www.kartosthera.com">www.kartosthera.com</a>
	Kartos Therapeutics Redwood City, CA Telios Pharma Redwood City, CA	AML (+TL-895)	Phase I <a href="http://www.kartosthera.com">www.kartosthera.com</a>
NEXI-001 (adoptive cell therapy)	NexImmune Gaithersburg, MD	AML	Phase I <a href="http://www.neximmune.com">www.neximmune.com</a>
NKX101 (CAR-NK cell therapy) <b>ORPHAN DRUG</b>	Nkarta Therapeutics South San Francisco, CA	AML	Phase I <a href="http://www.nkartatx.com">www.nkartatx.com</a>
NTLA-5001 (TCR T-cell therapy) <b>ORPHAN DRUG</b>	Intellia Therapeutics Cambridge, MA	AML	Phase I/II <a href="http://www.intellia.com">www.intellia.com</a>
obe-cel (obecabtagene autoleucel) (CD19 CAR-T cell therapy) <b>ORPHAN DRUG</b>	Autolus London, United Kingdom	B-cell ALL (adults)	Phase I/II <a href="http://www.autolus.com">www.autolus.com</a>

## Leukemia

### Drug Name

### Sponsor

### Indication

### Development Phase

olutasidenib  
(IDH1M inhibitor)  
**ORPHAN DRUG**

FORMA Therapeutics  
Watertown, MA

AML

Phase II  
[www.formatherapeutics.com](http://www.formatherapeutics.com)

ONO-7475  
(Axl/Mer inhibitor)

Ono Pharmaceutical  
Osaka, Japan

acute leukemia

Phase I/II  
[www.ono-pharma.com](http://www.ono-pharma.com)

Orca-Q  
(stem cell therapy)

Orca Bio  
Menlo Park, CA

acute leukemia

Phase I  
[www.orcabio.com](http://www.orcabio.com)

Orca-T  
(stem cell therapy)

Orca Bio  
Menlo Park, CA

ALL, AML

Phase III  
[www.orcabio.com](http://www.orcabio.com)

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mixed phenotype acute leukemia

Phase I/II  
[www.orcabio.com](http://www.orcabio.com)

Oxi4503  
(combretastatin A1 phosphate)  
**ORPHAN DRUG**

Oncotelic Therapeutics  
Agoura Hills, CA

AML (Fast Track)  
(pediatric)

Phase I/II  
[www.oncotelic.com](http://www.oncotelic.com)

PBCAR0191  
(CAR-T cell therapy)  
**ORPHAN DRUG**

Precision Biosciences  
Durham, NC

B-cell ALL (Fast Track)

Phase I  
[www.precisionbiosciences.com](http://www.precisionbiosciences.com)

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
pegargiminas (ADI-PEG-20) (arginine deiminase replacement)	Polaris San Diego, CA	AML	Phase I <a href="http://www.polarispharma.com">www.polarispharma.com</a>
pirtobrutinib (BTK inhibitor)	Lilly Indianapolis, IN	CLL (monotherapy and combination therapy)	Phase III <a href="http://www.lilly.com">www.lilly.com</a>
pivekimab sunirine (anti-CD123 ADC) <b>ORPHAN DRUG</b>	ImmunoGen Waltham, MA	AML	Phase I <a href="http://www.immunogen.com">www.immunogen.com</a>
PLX2853 (BET inhibitor)	Daiichi Sankyo Basking Ridge, NJ Plexxikon South San Francisco, CA	AML	Phase I <a href="http://www.daiichisankyo.com">www.daiichisankyo.com</a>
PMB-101 (anti-BAFF CAR-T cell therapy)	PeproMene Bio Irvine, CA	B-cell ALL	Phase I <a href="http://www.pepromenebio.com">www.pepromenebio.com</a>
prexigebersen (GRB2 adaptor protein inhibitor) <b>ORPHAN DRUG</b>	Bio-Path Holdings Bellaire, TX	AML	Phase II <a href="http://www.biopathholdings.com">www.biopathholdings.com</a>
PRGN-3006 (UltraCar-T cell therapy) <b>ORPHAN DRUG</b>	Precigen Germantown, MD	AML (Fast Track)	Phase I <a href="http://www.precigen.com">www.precigen.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
PTX-200 (tricitabine) (AKT inhibitor)	Prescient Therapeutics Melbourne, Australia	acute leukemia	Phase I/II <a href="http://www.ptxtherapeutics.com">www.ptxtherapeutics.com</a>
quizartinib (FKT3 inhibitor) ORPHAN DRUG	Daiichi Sankyo Basking Ridge, NJ	1L AML (Fast Track) (Breakthrough Therapy)	Phase III <a href="http://www.daiichisankyo.com">www.daiichisankyo.com</a>
RG6007 (HLA-A2-WT1 x CD3 bispecific antibody)	Roche/Genentech South San Francisco, CA	AML	Phase I <a href="http://www.roche.com">www.roche.com</a>
RO7283420 (bispecific antibody)	Roche/Genentech South San Francisco, CA	AML	Phase I <a href="http://www.roche.com">www.roche.com</a>
RP7214 (DHODH inhibitor)	Rhizen Pharmaceuticals Bridgewater, NJ	AML	Phase I <a href="http://www.rhizen.com">www.rhizen.com</a>
RTX-240 (allogeneic cell therapy)	Rubius Therapeutics Cambridge, MA	AML (adults)	Phase I/II <a href="http://www.rubiustx.com">www.rubiustx.com</a>
RVU120 (CDK8/19 inhibitor) ORPHAN DRUG	Ryvu Therapeutics Kraków, Poland	AML	Phase I <a href="http://www.rvyu.com">www.rvyu.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Rydapt®</b> midostaurin	Novartis East Hanover, NJ	AML (pediatric)	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
sabatolimab (MBG453) (TIM3 antagonist)	Novartis East Hanover, NJ	AML (maintenance), unfit AML	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
sapacitabine (DNA polymerase inhibitor) <b>ORPHAN DRUG</b>	Cyclacel Pharmaceuticals Berkeley Heights, NJ	AML (+venetoclax)	Phase I/II <a href="http://www.cyclacel.com">www.cyclacel.com</a>
SAR443579 (IPH-6101) (anti-NKp46 x CD123 bispecific mAb)	Innate Pharma Rockville, MD Sanofi Bridgewater, NJ	ALL, AML	Phase I <a href="http://www.sanofi.com">www.sanofi.com</a>
SAR445419 (NK-cell-based immunotherapy)	Sanofi Bridgewater, NJ	AML	Phase I <a href="http://www.sanofi.com">www.sanofi.com</a>
<b>Sarclisa®</b> isatuximab	Sanofi Bridgewater, NJ	1-2L AML, ALL (pediatric)	Phase II <a href="http://www.sanofi.com">www.sanofi.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Scemblix®</b> asciminib	Novartis East Hanover, NJ	1L CML	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
		2L CML (pediatric)	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
SEA-CD70 (anti-CD70 antibody)	Seagen Bothell, WA	AML	Phase I <a href="http://www.seagen.com">www.seagen.com</a>
SEL24/MEN1703 (PIM/FLT3 inhibitor) ORPHAN DRUG	Menarini Florence, Italy	AML	Phase I/II <a href="http://www.menarini.com">www.menarini.com</a>
SKI-G-801 (FLT3 inhibitor)	Genosco Billerica, MA Oscotec Gyeonggi-do, South Korea	AML	Phase I <a href="http://www.genosco.com">www.genosco.com</a>
SL-172154	Shattuck Labs Austin, TX	AML	Phase I <a href="http://www.shattucklabs.com">www.shattucklabs.com</a>
SMART-101 (human T lymphoid progenitor) ORPHAN DRUG	Smart Immune Paris, France	acute leukemia (Fast Track)	Phase I/II <a href="http://www.smart-immune.com">www.smart-immune.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
SNDX-5613 (menin inhibitor) <b>ORPHAN DRUG</b>	Syndax Pharmaceuticals Waltham, MA	acute leukemia (Fast Track)	phase I/II <a href="http://www.syndax.com">www.syndax.com</a>
STAR-LLD (continuous delivery of low-dose lenalidomide)	Starton Therapeutics Paramus, NJ	CLL	Phase I <a href="http://www.startontx.com">www.startontx.com</a>
SUN-706 (vodobatinib) (Bcr-Abl1 kinase inhibitor)	Sun Pharma Advanced Research Mumbai, India	CML	Phase I/II <a href="http://www.sparc.life">www.sparc.life</a>
SY-2101 (arsenic trioxide oral) <b>ORPHAN DRUG</b>	Syros Pharmaceuticals Cambridge, MA	acute promyelocytic leukemia	Phase II <a href="http://www.syros.com">www.syros.com</a>
tamibarotene (oral RAR-alpha agonist) <b>ORPHAN DRUG</b>	Syros Pharmaceuticals Cambridge, MA	newly-diagnosed unfit AML	Phase II <a href="http://www.syros.com">www.syros.com</a>
TAS1440 (oral LSD1 inhibitor)	Astex Pharmaceuticals Pleasanton, CA	AML	Phase I <a href="http://www.astx.com">www.astx.com</a>
TAS1553 (oral RNR inhibitor)	Astex Pharmaceuticals Pleasanton, CA	AML	Phase I <a href="http://www.astx.com">www.astx.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
TBX-2400 (stem cell therapy) <b>ORPHAN DRUG</b>	Taiga Biotechnologies Aurora, CO	AML	Phase I <a href="http://www.taigabiotech.com">www.taigabiotech.com</a>
Tecartus® brexucabtagene autoleucel	Gilead Sciences Foster City, CA	ALL (pediatric)	Phase II <a href="http://www.gilead.com">www.gilead.com</a>
Tibsovo® ivosidenib	Servier Pharmaceuticals Boston, MA	1L IDH1-mutant AML (IC-ineligible) (combination therapy)	application submitted <a href="http://www.servier.us">www.servier.us</a>
treosulfan (bifunctional sulfonate alkylating agent) <b>ORPHAN DRUG</b>	Medexus Pharma USA Chicago, IL	AML (conditioning in allogeneic hematopoietic stem cell transplantation)	application submitted <a href="http://www.medexus.com">www.medexus.com</a>
UCART 22 (CAR-T cell therapy)	Collectis New York, NY	B-cell ALL	Phase I <a href="http://www.collectis.com">www.collectis.com</a>
UCART123 (CAR-T cell therapy)	Collectis New York, NY	AML	Phase I <a href="http://www.collectis.com">www.collectis.com</a>
uproleselan (GMI-1271) (E-selectin inhibitor) <b>ORPHAN DRUG</b>	GlycoMimetics Rockville, MD	AML (Fast Track) (Breakthrough Therapy)	Phase III <a href="http://www.glycomimetics.com">www.glycomimetics.com</a>



## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Venclexta®</b> venetoclax	AbbVie North Chicago, IL Genentech South San Francisco, CA	ALL	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a> <a href="http://www.gene.com">www.gene.com</a>
VIP152 (CDK9 inhibitor)	Vincerx Pharma Palo Alto, CA	leukemia	Phase I <a href="http://www.vincerx.com">www.vincerx.com</a>
volasertib (NBL-001) (PLK-1 inhibitor) <b>ORPHAN DRUG</b>	Notable Labs Foster City, CA	AML, leukemia/lymphoma (pediatric)	Phase I <a href="http://www.notablelabs.com">www.notablelabs.com</a>
VOR33 (CAR-T cell therapy) <b>ORPHAN DRUG</b>	Vor Biopharma Cambridge, MA	AML (Fast Track)	Phase I/II <a href="http://www.vorbio.com">www.vorbio.com</a>
<b>Vyxeos®</b> daunorubicin amd cytarabine	Jazz Pharmaceuticals Dublin, Ireland	newly-diagnosed older adults with AML	Phase II <a href="http://www.jazzpharma.com">www.jazzpharma.com</a>
WU-CART-007 (CAR-T cell therapy) <b>ORPHAN DRUG</b>	Wugen St. Louis, MO	T-cell ALL	Phase I/II <a href="http://www.wugen.com">www.wugen.com</a>
XmAb968 (CD38 x CD3 bispecific antibody)	Xencor Monrovia, CA	T-cell ALL, AML	Phase I <a href="http://www.xencor.com">www.xencor.com</a>

## Leukemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Xospata® gilteritinib	Astellas Pharma US Northbrook, IL	post-chemotherapy AML (maintenance), AML post stem cell transplant (maintenance), newly-diagnosed AML with high intensity induction of chemotherapy, AML (pediatric)	Phase III <a href="http://www.astellas.com">www.astellas.com</a>
		newly-diagnosed AML with low intensity induction of chemotherapy	Phase I <a href="http://www.astellas.com">www.astellas.com</a>
zandelisib (PI3K delta inhibitor)	MEI Pharma San Diego, CA	CLL	Phase II <a href="http://www.meipharma.com">www.meipharma.com</a>
zilovertamab (ROR1 mAb)	Octernal Therapeutics San Diego, CA	CLL	Phase II <a href="http://www.octernal.com">www.octernal.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AB-101 (natural killer cell therapy)	Artiva Biotherapeutics San Diego, CA	B-cell non-Hodgkin lymphoma (NHL)	Phase I <a href="http://www.artivabio.com">www.artivabio.com</a>
AB-205 (cell therapy)	Angiocrine Bioscience San Diego, CA	lymphoma (in patient undergoing autologous hematopoietic cell transplantation)	Phase III <a href="http://www.angiocrinebioscience.com">www.angiocrinebioscience.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
abequolixron (RGX-104) (LXR agonist)	Inspirna New York, NY	lymphoma	Phase I <a href="http://www.inspirna.com">www.inspirna.com</a>
abexinostat (HDAC inhibitor)	Xynomic Pharmaceuticals Poway, CA	follicular lymphoma	Phase II <a href="http://www.xynomicpharma.com">www.xynomicpharma.com</a>
abivertinib (EGFR tyrosine kinase inhibitor)	Sorrento Therapeutics San Diego, CA	B-cell lymphoma	Phase II <a href="http://www.sorrentotherapeutics.com">www.sorrentotherapeutics.com</a>
<b>Adcetris®</b> brentuximab vedotin	Seagen Bothell, WA	diffuse large B-cell lymphoma (DLBCL) (combination therapy)	Phase III <a href="http://www.seagen.com">www.seagen.com</a>
		Hodgkin lymphoma (combination therapy), Hodgkin lymphoma or peripheral T-cell lymphoma (PTCL) (unfit for combination chemotherapy), Hodgkin lymphoma and PTCL (retreatment), Hodgkin lymphoma (pediatric), PTCL (<10% CD30 expression)	Phase II <a href="http://www.seagen.com">www.seagen.com</a>
ADG106 (anti-CD137 mAb)	Adagene San Diego, CA	NHL	Phase I <a href="http://www.adagene.com">www.adagene.com</a>
ADI-001 (CAR-T cell therapy)	Adicet Bio Menlo Park, CA	NHL (Fast Track)	Phase I <a href="http://www.adicetbio.com">www.adicetbio.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ADX-2191 (intravitreal methotrexate) <b>ORPHAN DRUG</b>	Aldeyra Lexington, MA	primary vitreoretinal lymphoma	Phase II <a href="http://www.laderya.com">www.laderya.com</a>
AFM13 (natural killer cell antibody) <b>ORPHAN DRUG</b>	Affimed Therapeutics Heidelberg, Germany	CD30-positive T-cell lymphoma	Phase II <a href="http://www.affimed.com">www.affimed.com</a>
AG-270 (MAT2A inhibitor)	Servier Pharmaceuticals Boston, MA	lymphoma	Phase I <a href="http://www.servier.us">www.servier.us</a>
<b>Aliqopa®</b> copanlisib	Bayer Pharmaceuticals Whippany, NJ	NHL	application submitted <a href="http://www.bayer.com">www.bayer.com</a>
ALLO-501 (CAR-T cell therapy)	Allogene Therapeutics South San Francisco, CA	NHL	Phase I <a href="http://www.allogene.com">www.allogene.com</a>
ALLO-501A (CAR-T cell therapy)	Allogene Therapeutics South San Francisco, CA	NHL (Fast Track)	Phase I <a href="http://www.allogene.com">www.allogene.com</a>
ALLO-647 (anti-CD52 mAb)	Allogene Therapeutics South San Francisco, CA	enable persistence of allogeneic CAR-T therapies in NHL	Phase I <a href="http://www.allogene.com">www.allogene.com</a>
AN4005 (PD-L1 inhibitor)	Adlai Nortye Zhejiang, China	advanced lymphoma	Phase I <a href="http://www.adlainortye.com">www.adlainortye.com</a>

## Lymphoma

### Drug Name

### Sponsor

### Indication

### Development Phase

APG-2575 (Iisافتoclax)  
(Bcl-2 inhibitor)  
**ORPHAN DRUG**

Ascentage Pharma  
Rockville, MD

small lymphocytic lymphoma (SLL),  
Waldenstrom macroglobulinemia

Phase I  
[www.ascentagepharma.com](http://www.ascentagepharma.com)

AVM0703  
(AVM-NKT cells activator)

AVM Biotechnology  
Seattle, WA

lymphoid malignancies

Phase I/II  
[www.avmbiotech.com](http://www.avmbiotech.com)

AZD7789  
(PD-1 x TIM3 bispecific antibody)

AstraZeneca  
Wilmington, DE

Hodgkin disease

Phase I  
[www.astrazeneca.com](http://www.astrazeneca.com)

bbT369  
(CAR-T cell therapy)

2seventy bio  
Cambridge, MA

B-cell NHL

Phase I/II  
[www.2seventybio.com](http://www.2seventybio.com)

**Betalutin®**  
177-Lu-lilotomab satetraxetan  
**ORPHAN DRUG**

Nordic Nanovector  
Oslo, Norway

follicular lymphoma (Fast Track),  
NHL

Phase I/II  
[www.nordicnanovector.com](http://www.nordicnanovector.com)

BI-1206  
(CD32b mAb)  
**ORPHAN DRUG**

BioInvent International  
Lund, Sweden

B-cell NHL

Phase I/II  
[www.bioinvent.com](http://www.bioinvent.com)

BJ-005  
(anti-PD-L1 mAb)

BJ Bioscience  
Hangzhou, China

lymphoma

Phase I  
[www.bjbioscience.com](http://www.bjbioscience.com)

**Lymphoma**  
**Drug Name**

**Sponsor**

**Indication**

**Development Phase**

BNZ-1 (IL-2/9/15 inhibitor) <b>ORPHAN DRUG</b>	Bioniz Therapeutics Irvine, CA	cutaneous T-cell lymphoma (CTCL)	Phase III <a href="http://www.bioniz.com">www.bioniz.com</a>
BP1002 (Bcl-2 inhibitor)	Bio-Path Holdings Bellaire, TX	lymphoid malignancies	Phase I <a href="http://www.biopathholdings.com">www.biopathholdings.com</a>
<b>Breyanzi®</b> lisocabtagene maraleucel	Bristol-Myers Squibb Princeton, NJ	2L large B-cell lymphoma (transplant eligible)	Phase III <a href="http://www.bms.com">www.bms.com</a>
		2L large B-cell lymphoma (transplant ineligible), 3L+ follicular lymphoma/ marginal zone lymphoma, 2L+ primary CNS lymphoma, 1L high-grade B-cell lymphoma, 3L+ mantle cell lymphoma	Phase II <a href="http://www.bms.com">www.bms.com</a>
<b>Brukinsa®</b> zanubrutinib <b>ORPHAN DRUG</b>	BeiGene Cambridge, MA	SLL (Breakthrough Therapy)	Phase III <a href="http://www.beigene.com">www.beigene.com</a>
BTX-1188 (CRBN protein modulator)	BioTheryX San Diego, CA	NHL	Phase I <a href="http://www.biotheryx.com">www.biotheryx.com</a>
BTX-A51 (kinase inhibitor)	BioTheryX San Diego, CA	NHL	Phase I <a href="http://www.biotheryx.com">www.biotheryx.com</a>

**Lymphoma**  
**Drug Name**

**Sponsor**

**Indication**

**Development Phase**

<b>Calquence®</b> acalabrutinib <b>ORPHAN DRUG</b>	AstraZeneca Wilmington, DE	1L mantle cell lymphoma, 1L DLBCL	Phase III <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
camidanlumab tesirine (CD25 antibody drug conjugate)	ADC Therapeutics New Providence, NJ	Hodgkin lymphoma	Phase II <a href="http://www.adctherapeutics.com">www.adctherapeutics.com</a>
CAP-100 (gG1 monoclonal antibody)	Catapult Therapeutics Lelystad, The Netherlands	small lymphocytic lymphoma (SLL)	Phase I <a href="http://www.catapult-therapeutics.com">www.catapult-therapeutics.com</a>
capivasterib (AKT inhibitor)	AstraZeneca Wilmington, DE	B-cell NHL	Phase II <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
CB-010 (CRISPR-edited allogeneic anti-CD19 CAR-T cell Therapy)	Caribou Biosciences Berkeley, CA	B-cell NHL	Phase I <a href="http://www.cariboubio.com">www.cariboubio.com</a>
CC-90011 (pulrodemstat) (LSD1 inhibitor)	Bristol-Myers Squibb Princeton, NJ	NHL	Phase I <a href="http://www.bms.com">www.bms.com</a>
CC-95251 (SIRPA protein modulator)	Bristol-Myers Squibb Princeton, NJ	NHL	Phase I <a href="http://www.bms.com">www.bms.com</a>
CC-96673	Bristol-Myers Squibb Princeton, NJ	NHL	Phase I <a href="http://www.bms.com">www.bms.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CC-97540 (CD19 NEX T) (CAR-T cell therapy)	Bristol-Myers Squibb Princeton, NJ	NHL	Phase I <a href="http://www.bms.com">www.bms.com</a>
CC-99282 (A/I CELMoD)	Bristol-Myers Squibb Princeton, NJ	NHL	Phase II <a href="http://www.bms.com">www.bms.com</a>
CD19 x CD20 bispecific antibody	ImmPACT Bio Camarillo, CA	NHL	Phase I <a href="http://www.impact-bio.com">www.impact-bio.com</a>
CD47 x CD20 bispecific therapeutic	Bristol-Myers Squibb Princeton, NJ	NHL	Phase I <a href="http://www.bms.com">www.bms.com</a>
CFT7455 (IKZF1/3 degrader)	C4 Therapeutics Watertown, MA	NHL	Phase I/II <a href="http://www.c4therapeutics.com">www.c4therapeutics.com</a>
CG-806 (luxepatinib) (BTK/FLT3 inhibitor)	Aptose Biosciences San Diego, CA	NHL, SLL	Phase I <a href="http://www.aptose.com">www.aptose.com</a>
<b>Copiktra®</b> duvelisib ORPHAN DRUG	Secura Bio Las Vegas, NV	indolent NHL, PTCL (Fast Track), SLL, Richter's transformation (Fast Track)	Phase II <a href="http://www.securabio.com">www.securabio.com</a>
CPI-0209 (EZH2 inhibitor)	MorphoSys Boston, MA	lymphoma	Phase I <a href="http://www.morphosys.com">www.morphosys.com</a>



## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CPI 818 (ITK inhibitor)	Corvus Pharmaceuticals Burlingame, CA	T-cell lymphoma	Phase I <a href="http://www.corvuspharma.com">www.corvuspharma.com</a>
CPO107 (CD20 x CD47 bispecific fusion protein)	Conjupro Biotherapeutics Princeton, NJ	NHL	Phase I/II <a href="http://www.conjuprobio.com">www.conjuprobio.com</a>
CS5001 (DNA cross linking agent)	CStone Pharmaceuticals Suzhou, China	advanced lymphoma	Phase I <a href="http://www.cstonepharma.com">www.cstonepharma.com</a>
CTX130 (CRISPR/Cas9 gene-edited CAR-T cell therapy) <b>ORPHAN DRUG</b>	CRISPR Therapeutics Cambridge, MA	T-cell lymphoma	Phase I <a href="http://www.crisprtx.com">www.crisprtx.com</a>
danvatirsen (STAT3 inhibitor)	Flamingo Therapeutics San Diego, CA	DLBCL	Phase II <a href="http://www.flamingotx.com">www.flamingotx.com</a>
davocetcept (LPPN-202) (PD-L1/CTLA-4 antagonist)	Alpine Immune Sciences Seattle, WA	lymphoma	Phase I <a href="http://www.alpineimmunesciences.com">www.alpineimmunesciences.com</a>
devimistat	Rafael Pharmaceuticals Cranbury, NJ	B-cell lymphoma, Burkitt's lymphoma	Phase II <a href="http://www.rafaelpharma.com">www.rafaelpharma.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
DTRM-555 (BTK inhibitor, everolimus, pomalidomide)	DTRM Biopharma Zhejiang, China	DLBCL, follicular lymphoma, Richter's transformation	Phase II
DUET-01 (CpG-STAT3siRNA gene therapy)	Duet Therapeutics Pasadena, CA	NHL	Phase I <a href="http://www.duettx.com">www.duettx.com</a>
DZ-002	Da Zen Theranostics San Jose, CA	lymphoma	Phase I <a href="http://www.dazenthera.com">www.dazenthera.com</a>
DZD4205 (golidocitinib) (JAK inhibitor)	Dizal Pharmaceutical Shanghai, China	PTCL (Fast Track)	Phase II <a href="http://www.dizalpharma.com">www.dizalpharma.com</a>
E7766 (STING agonist)	Eisai Woodcliff Lake, NJ	lymphoma	Phase I <a href="http://www.eisai.com">www.eisai.com</a>
emavusertib (IRAK4 inhibitor)	Curis Lexington, MA	IRAK4-driven lymphoma	Phase II <a href="http://www.curis.com">www.curis.com</a>
epcoritamab (GEN-3013) (CD3 x CD20 bispecific antibody) <b>ORPHAN DRUG</b>	AbbVie North Chicago, IL Genmab US Plainsboro, NJ	DLBCL	Phase III <a href="http://www.abbvie.com">www.abbvie.com</a>
		follicular lymphoma, B-cell NHL, B-cell lymphoma, Richter's transformation	Phase I/II <a href="http://www.abbvie.com">www.abbvie.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
evorpcept (ALX148) (CD47 antigen inhibitor)	ALX Oncology South San Francisco, CA	NHL (combination therapy)	Phase I/II <a href="http://www.alxoncology.com">www.alxoncology.com</a>
EX-103 (DNA cross linking agent) <b>ORPHAN DRUG</b>	Exinda Therapeutics El Dorado Hills, CA	indolent NHL	Phase III <a href="http://www.exindarx.com">www.exindarx.com</a>
EX-104 (DNA cross linking agent) <b>ORPHAN DRUG</b>	Exinda Therapeutics El Dorado Hills, CA	indolent NHL	Phase III <a href="http://www.exindarx.com">www.exindarx.com</a>
exoLL-12 (engineered exosome)	Codiak Biosciences Cambridge, MA	CTCL	Phase I <a href="http://www.codiakbio.com">www.codiakbio.com</a>
EZM0414 (SETD2 inhibitor)	Epizyme Cambridge, MA	DLBCL (Fast Track)	Phase I <a href="http://www.epizyme.com">www.epizyme.com</a>
FF-10502 (DNA polymerase inhibitor)	Fujifilm Pharmaceuticals U.S.A. Cambridge, MA	lymphoma	Phase I/II <a href="http://www.fujifilmpharma.com">www.fujifilmpharma.com</a>
fimepinostat (HDAC inhibitor) <b>ORPHAN DRUG</b>	Curis Lexington, MA	lymphoma, including DLBCL (Fast Track)	Phase I completed <a href="http://www.curis.com">www.curis.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
FT516 (NK cell cancer immunotherapy)	Fate Therapeutics San Diego, CA	B-cell lymphoma	Phase I <a href="http://www.fatetherapeutics.com">www.fatetherapeutics.com</a>
FT596 (NK cell cancer immunotherapy)	Fate Therapeutics San Diego, CA	B-cell lymphoma	Phase I <a href="http://www.fatetherapeutics.com">www.fatetherapeutics.com</a>
FT819 (NK cell cancer immunotherapy)	Fate Therapeutics San Diego, CA	B-cell lymphoma	Phase I <a href="http://www.fatetherapeutics.com">www.fatetherapeutics.com</a>
GB5121 (BTK inhibitor)	Gossamer Bio San Diego, CA	primary CNS lymphoma	Phase I <a href="http://www.gossamerbio.com">www.gossamerbio.com</a>
glofitamab (RG6026) (CD20 x CD3 bispecific antibody)	Roche/Genentech South San Francisco, CA	2L+ DLBCL (+chemotherapy), 3L+ DLBCL	Phase III <a href="http://www.roche.com">www.roche.com</a>
		1L ctDNA high-risk DLBCL	Phase II <a href="http://www.roche.com">www.roche.com</a>
GZ 17-6.02	Genzada Pharmaceuticals Hutchinson, KS	lymphoma	Phase I <a href="http://www.ganzada.com">www.ganzada.com</a>
HH2853 (multi-protein inhibitor)	HaiHe Biopharma Shanghai, China	NHL	Phase I <a href="http://www.haihepharma.com">www.haihepharma.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
HMPL-523 (soveplenib) (Syk kinase inhibitor)	Hutchison MediPharma Shanghai, China	NHL	Phase I <a href="http://www.hutch-med.com">www.hutch-med.com</a>
HMPL-689 (amdizalisib) (PI3K delta inhibitor)	Hutchison MediPharma Shanghai, China	lymphoma	Phase I <a href="http://www.hutch-med.com">www.hutch-med.com</a>
HMPL-760 (BTK) inhibitor	Hutchison Medipharma Shanghai, China	NHL, SLL	Phase I <a href="http://www.hutch-med.com">www.hutch-med.com</a>
<b>HyBryte™</b> hypericin <b>ORPHAN DRUG</b>	Soligenix Princeton, NJ	CTCL (Fast Track)	Phase III <a href="http://www.soligenix.com">www.soligenix.com</a>
iberdomide (ubiquitin protein ligase complex modulator)	Bristol-Myers Squibb Princeton, NJ	NHL	Phase I <a href="http://www.bms.com">www.bms.com</a>
IGM-2323 (CD20 x CD3 bispecific antibody)	IGM Biosciences Mountain View, CA	non-Hodgkin lymphoma	Phase I <a href="http://www.igmbio.com">www.igmbio.com</a>
<b>Imbruvica®</b> ibrutinib	Janssen Raritan, NJ Pharmacyclics Sunnyvale, CA	indolent NHL, mantle cell lymphoma (+venetoclax), treatment naïve mantle cell lymphoma	Phase III <a href="http://www.janssen.com">www.janssen.com</a> <a href="http://www.pharmacyclics.com">www.pharmacyclics.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
IMC-002 (anti-CD47 mAb)	ImmuneOncia Therapeutics Gyeonggi-do, South Korea	lymphoma	Phase I <a href="http://www.immuneoncia.com">www.immuneoncia.com</a>
IMM0306 (CD47 x CD20 bispecific antibody)	ImmuneOnco Biopharmaceuticals Shanghai, China	B-cell NHL	Phase I <a href="http://www.immuneonco.com">www.immuneonco.com</a>
iopofosine (radiopharmaceutical) <b>ORPHAN DRUG</b>	Cellectar Biosciences Florham Park, NJ	Waldenstrom's macroglobulinemia (Fast Track)	Phase III <a href="http://www.cellectar.com">www.cellectar.com</a>
IOV-2001 (adoptive cell therapy)	Iovance Biotherapeutics San Carlos, CA	SLL	Phase I <a href="http://www.iovance.com">www.iovance.com</a>
<b>Istodax</b> <sup>®</sup> romidepsin	Bristol-Myers Squibb Princeton, NJ	1L peripheral T-cell lymphoma	Phase III <a href="http://www.bms.com">www.bms.com</a>
KB-0742 (CDK9 inhibitor)	Kronos Bio San Mateo, CA	NHL	Phase I <a href="http://www.kronosbio.com">www.kronosbio.com</a>
<b>Keytruda</b> <sup>®</sup> pembrolizumab	Merck Kenilworth, NJ	Hodgkin lymphoma, primary mediastinal large B-cell lymphoma	Phase II <a href="http://www.merck.com">www.merck.com</a>
KITE-363 (CD19 x CD20 bicistronic cell therapy)	Gilead Sciences Foster City, CA	3L+ large B-cell lymphoma	Phase I <a href="http://www.gilead.com">www.gilead.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
KT-333 (STAT3 modulator)	Kymera Therapeutics Watertown, MA	lymphoma	Phase I <a href="http://www.kymeratx.com">www.kymeratx.com</a>
KT-413 (IRAIMiD degrader)	Kymera Therapeutics Watertown, MA	lymphoma	Phase I <a href="http://www.kymeratx.com">www.kymeratx.com</a>
KUR-502 (NKT cell therapy)	Athenex Buffalo, NY	lymphoma	Phase I <a href="http://www.athenex.com">www.athenex.com</a>
<b>Kymriah®</b> tisagenlecleucel <b>ORPHAN DRUG</b>	Novartis East Hanover, NJ	follicular lymphoma	application submitted <a href="http://www.novartis.com">www.novartis.com</a>
lacutamab (KIR3DL2 antagonist) <b>ORPHAN DRUG</b>	Innate Pharma Rockville, MD	Sezary syndrome (Fast Track), T-cell lymphoma	Phase II <a href="http://www.innate-pharma.com">www.innate-pharma.com</a>
LB1901 (chimeric T-cell therapy)	Legend Biotech USA Somerset, NJ	T-cell lymphoma	Phase I <a href="http://www.legendbiotech.com">www.legendbiotech.com</a>
lenzilumab (GM-CSF antagonist)	Humanigen Short Hills, NJ	NHL (+CAR-T cell therapy)	Phase III <a href="http://www.humanigen.com">www.humanigen.com</a>
LNS8801 (G protein-coupled receptor agonist)	Linnaeus Therapeutics Haddonfield, NJ	lymphoma	Phase I/II <a href="http://www.linnaeustx.com">www.linnaeustx.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
magrolimab (anti-CD47 mAb)	Gilead Sciences Foster City, CA	DLBCL (Fast Track)	Phase I/II <a href="http://www.gilead.com">www.gilead.com</a>
MAK683 (EED inhibitor)	Novartis East Hanover, NJ	DLBCL	Phase I <a href="http://www.novartis.com">www.novartis.com</a>
maveropepimut-S (peptide cancer vaccine)	IMV Cambridge, MA	DLBCL (+pembrolizumab)	Phase II <a href="http://www.imv-inc.com">www.imv-inc.com</a>
mavorixafor (CXCR4 inhibitor)	X4 Pharmaceuticals Boston, MA	Waldenstrom's macroglobulinemia	Phase I <a href="http://www.x4pharma.com">www.x4pharma.com</a>
MB-106 (CAR-T cell therapy)	Mustang Bio Worcester, MA	follicular lymphoma, DLBCL, SLL Waldenstrom's macroglobulinemia	Phase I/II <a href="http://www.mustangbio.com">www.mustangbio.com</a>
MK-2118	Merck Kenilworth, NJ	lymphoma	Phase I <a href="http://www.merck.com">www.merck.com</a>
<b>Monjuvi</b> <sup>®</sup> tafasitamab-cxix <b>ORPHAN DRUG</b>	Incyte Wilmington, DE MorphoSys Boston, MA	1L DLBCL, follicular lymphoma, marginal zone lymphoma	Phase III <a href="http://www.incyte.com">www.incyte.com</a> <a href="http://www.morphosys.com">www.morphosys.com</a>



## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
mosunetuzumab (RG7828) (CD20 x CD3 bispecific antibody)	Roche/Genentech South San Francisco, CA	3L+ follicular lymphoma (Breakthrough Therapy)	application submitted <a href="http://www.roche.com">www.roche.com</a>
		2L+ follicular lymphoma (+ lenalidomide)	Phase III <a href="http://www.roche.com">www.roche.com</a>
mRNA-2752 (OX40L/IL-23/IL-36γ mRNA)	Moderna Therapeutics Cambridge, MA	lymphoma	Phase I <a href="http://www.modernatx.com">www.modernatx.com</a>
MS-553 (PKCβ inhibitor)	MingSight Pharmaceuticals San Diego, CA	SLL	Phase I/II <a href="http://www.mingsight.com">www.mingsight.com</a>
MT-101 (CD5-ATAK cell therapy)	Myeloid Therapeutics Cambridge, MA	PCTL	Phase I/II <a href="http://www.myeloidtx.com">www.myeloidtx.com</a>
MT-0169 (proteasome inhibitor)	Molecular Templates Austin, TX	NHL	Phase I <a href="http://www.mtem.com">www.mtem.com</a>
nana-val (nanatinostat + valganciclovir) <b>ORPHAN DRUG</b>	Viracta Therapeutics Cardiff, CA	relapsed/refractory EBV+ lymphoma (Fast Track)	Phase II <a href="http://www.viracta.com">www.viracta.com</a>
naratuximab emtansine (DEBIO 1562) (anti-CD37 antibody-drug conjugate) <b>ORPHAN DRUG</b>	Debiopharm Lausanne, Switzerland	DLBCL	Phase II completed <a href="http://www.debiopharm.com">www.debiopharm.com</a>

## Lymphoma

### Drug Name

### Sponsor

### Indication

### Development Phase

NK-17 (efineptakin alfa)  
(IL-7 replacement)

NeoImmuneTech  
Rockville, MD

lymphoma

Phase I  
[www.neoimmunetech.com](http://www.neoimmunetech.com)

NKTR-255  
(IL-15 receptor agonist)

Nektar Therapeutics  
San Francisco, CA

NHL (combination therapy)

Phase I/II  
[www.nektar.com](http://www.nektar.com)

NX-1607  
(CBL-B inhibitor)

Nurix Therapeutics  
San Francisco, CA

Richter's transformation

Phase I  
[www.nurixtx.com](http://www.nurixtx.com)

odronextamab  
(CD20 x CD2 bispecific antibody)

Regeneron Pharmaceuticals  
Tarrytown, NY

B-cell NHL

Phase II  
[www.regeneron.com](http://www.regeneron.com)

ONO-4685  
(PD-1 x CD3 bispecific antibody)

Ono Pharmaceutical  
Osaka, Japan

T-cell lymphoma

Phase I  
[www.ono-pharma.com](http://www.ono-pharma.com)

**Onureg**<sup>®</sup>  
azacitidine tablets

Bristol-Myers Squibb  
Princeton, MA

angioimmunoblastic T-cell lymphoma

Phase III  
[www.bms.com](http://www.bms.com)

**Opdivo**<sup>®</sup>  
nivolumab

Bristol-Myers Squibb  
Princeton, NJ

refractory Hodgkin lymphoma

Phase III  
[www.bms.com](http://www.bms.com)

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pediatric Hodgkin lymphoma,  
DLBCL, follicular lymphoma,  
primary testicular lymphoma

Phase II  
[www.bms.com](http://www.bms.com)

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
OT-82 (NAMPT inhibitor)	OncoTartis Buffalo, NY	lymphoma	Phase I <a href="http://www.oncotartis.com">www.oncotartis.com</a>
PBCAR0191 (CAR-T cell therapy)	Precision Biosciences Durham, NC	NHL	Phase I <a href="http://www.precisionbiosciences.com">www.precisionbiosciences.com</a>
PBCAR19B (CAR-T cell therapy)	Precision Biosciences Durham, NC	NHL	Phase I <a href="http://www.precisionbiosciences.com">www.precisionbiosciences.com</a>
pirtobrutinib (BTK inhibitor)	Lilly Indianapolis, IN	mantle cell lymphoma	Phase III <a href="http://www.lilly.com">www.lilly.com</a>
plamotamab (XmAb13676) (CD20 x CD3 bispecific antibody)	Janssen Research & Development Raritan, NJ Xencor Monrovia, CA	DLBCL (combination therapy)	Phase II <a href="http://www.xencor.com">www.xencor.com</a>
PLX2853 (BET inhibitor)	Plexikon South San Francisco, CA	lymphoma	Phase I <a href="http://www.plexikon.com">www.plexikon.com</a>
<b>Polivy</b> <sup>®</sup> polatuzumab vedotin	Roche/Genentech South San Francisco, CA	1L DLBCL	Phase III <a href="http://www.roche.com">www.roche.com</a>
<b>Poteligeo</b> <sup>®</sup> mogamulizumab-kpkc	Kyowa Kirin Bedminster, NJ	CTCL (four week dosing)	Phase II <a href="http://www.kkna.kyowakirin.com">www.kkna.kyowakirin.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
PRT811 (PRMT5 inhibitor)	Prelude Therapeutics Wilmington, DE	lymphoma	Phase I <a href="http://www.preludetx.com">www.preludetx.com</a>
PSB202 (T lymphocyte stimulant)	Sound Biologics Bothell, WA	B-cell lymphoma	Phase I <a href="http://www.soundbiologics.com">www.soundbiologics.com</a>
repotrectinib (ROS1/TRK inhibitor)	Turning Point Therapeutics San Diego, CA	lymphoma	Phase I <a href="http://www.tptherapeutics.com">www.tptherapeutics.com</a>
RG6333 (CD19 x CD28 bispecific antibody)	Roche/Genentech South San Francisco, CA	NHL (+glofitamab)	Phase I <a href="http://www.roche.com">www.roche.com</a>
rituximab-ADNAB (antibody-drug conjugate)	Sorrento Therapeutics San Diego, CA	B-cell lymphoma	Phase I <a href="http://www.sorrentotherapeutics.com">www.sorrentotherapeutics.com</a>
SAR442257 (CD38 x CD28 x CD3 trispecific antibody)	Sanofi Bridgewater, NJ	NHL	Phase I <a href="http://www.sanofi.com">www.sanofi.com</a>
SAR444245 (IL-2 replacement)	Sanofi Bridgewater, NJ	lymphoma	Phase II <a href="http://www.sanofi.com">www.sanofi.com</a>
SEA-CD40 (anti-CD70 antibody)	Seagen Bothell, WA	lymphoma	Phase I <a href="http://www.seagen.com">www.seagen.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
SEA-TGT (anti-TIGIT antibody)	Seagen Bothell, WA	lymphoma	Phase I <a href="http://www.seagen.com">www.seagen.com</a>
SGN-CD30C (anti-CD30 antibody-drug conjugate)	Seagen Bothell, WA	lymphoma	Phase I <a href="http://www.seagen.com">www.seagen.com</a>
SL-279252 (PD-1/OX40L inhibitor)	Shattuck Labs Austin, TX	lymphoma	Phase I <a href="http://www.shattucklabs.com">www.shattucklabs.com</a>
SNX281 (STING agonist)	Stingthera Boston, MA	lymphoma (+pembrolizumab)	Phase I
Sofusa® anti-PD-1 (anti-PD-1 lymphatic delivery system)	Sorrento Therapeutics San Diego, CA	CTCL	Phase I <a href="http://www.sorrentotherapeutics.com">www.sorrentotherapeutics.com</a>
STRO-001 (anti-CD74 antibody-drug conjugate)	Sutro Biopharma South San Francisco, CA	lymphoma	Phase I <a href="http://www.sutrobio.com">www.sutrobio.com</a>
TAK-007 (NK CAR-T cell therapy)	Takeda Deerfield, IL	B-cell NHL	Phase II <a href="http://www.takeda.com">www.takeda.com</a>

## Lymphoma

### Drug Name

### Sponsor

### Indication

### Development Phase

**Tazverik®**  
tazemetostat  
ORPHAN DRUG

Epizyme  
Cambridge, MA

follicular lymphoma (combination therapy)

Phase III  
[www.epizyme.com](http://www.epizyme.com)

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DLBCL (Fast Track),  
follicular lymphoma (WR-CHOP),  
mantle cell lymphoma

Phase II  
[www.epizyme.com](http://www.epizyme.com)

tenalisib  
(PI3 inhibitor)  
ORPHAN DRUG

Rhizen Pharmaceuticals  
Bridgewater, NJ

PTCL (Fast Track)

Phase II  
[www.rhizen.com](http://www.rhizen.com)

tirabrutinib  
(BTK inhibitor)

Ono Pharmaceutical  
Osaka, Japan

primary CNS lymphoma

Phase II  
[www.ono-pharma.com](http://www.ono-pharma.com)

tislelizumab  
(PD-1 antagonist)

BeiGene  
Cambridge, MA

T-cell lymphoma

Phase II  
[www.beigene.com](http://www.beigene.com)

TNB-486  
(CD19 x CD3 bispecific antibody)

TeneoTwo  
Newark, CA

NHL

Phase I

tolinapant (ASTX660)  
(dual IAP antagonist)  
ORPHAN DRUG

Astex Therapeutics  
Pleasanton, CA

lymphoma

Phase II  
[www.astx.com](http://www.astx.com)

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
TRPH-222 (anti-CD22 antibody drug conjugate)	Triphase Accelerator La Jolla, CA	B-cell lymphoma	Phase I <a href="http://www.triphaseco.com">www.triphaseco.com</a>
TT11 (CD30 CAR-T cell therapy)	Tessa Therapeutics Singapore	Hodgkin lymphoma	Phase II/III <a href="http://www.tessacell.com">www.tessacell.com</a>
TTX-030 (anti-CD39 antibody)	Trishula Therapeutics South San Francisco, CA	lymphoma	Phase I <a href="http://www.trishulatx.com">www.trishulatx.com</a>
valemetostat (EZH1/2 inhibitor) ORPHAN DRUG	Daiichi Sankyo Basking Ridge, NJ	PTCL	Phase II <a href="http://www.daiichisankyo.com">www.daiichisankyo.com</a>
VIP152 (CDK9 inhibitor)	Vincerx Pharma Palo Alto, CA	lymphoma	Phase I <a href="http://www.vincerx.com">www.vincerx.com</a>
volasertib (NBL-001) (PLK-1 inhibitor)	Notable Labs Foster City, CA	pediatric lymphoma	Phase I <a href="http://www.notablelabs.com">www.notablelabs.com</a>
WP1220 (STAT3 inhibitor)	Moleculin Biotech Houston, TX	CTCL	Phase I <a href="http://www.moleculin.com">www.moleculin.com</a>
WU-CART-007 (CAR-T cell therapy) ORPHAN DRUG	Wugen St. Louis, MO	lymphoblastic lymphoma	Phase I/II <a href="http://www.wugen.com">www.wugen.com</a>

## Lymphoma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
XmAb968 (CD38/CD3 bispecific antibody)	Xencor Monrovia, CA	T-cell lymphoblastic lymphoma	Phase I <a href="http://www.xencor.com">www.xencor.com</a>
<b>Xpovio</b> <sup>®</sup> selinexor	Karyopharm Therapeutics Newton, MA	DLBCL (combination therapy)	Phase III <a href="http://www.karyopharm.com">www.karyopharm.com</a>
<b>Yescarta</b> <sup>®</sup> axicabtagene ciloleucel	Gilead Sciences Foster City, CA	2L+ high-risk follicular lymphoma, 1L high-risk large B-cell lymphoma	Phase III <a href="http://www.gilead.com">www.gilead.com</a>
		1L large B-cell lymphoma, 2L large B-cell lymphoma outpatient	Phase II <a href="http://www.gilead.com">www.gilead.com</a>
YTB323 (CD19 CAR-T cell therapy)	Novartis East Hanover, NJ	2L DLBCL	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
zamtocabtagene autoleucel (CAR-T cell therapy)	Miltenyi Biotec Gaithersburg, MD	DLBCL	Phase II <a href="http://www.miltenyibiotec.com">www.miltenyibiotec.com</a>



## Lymphoma

### Drug Name

### Sponsor

### Indication

### Development Phase

zandelisib  
(PI3K delta inhibitor)  
**ORPHAN DRUG**

MEI Pharma  
San Diego, CA

2L+ follicular lymphoma (Fast Track),  
2L+ marginal zone lymphoma  
(combination therapy)

Phase III  
[www.meipharma.com](http://www.meipharma.com)

3L+ follicular lymphoma,  
3L+ marginal zone lymphoma

Phase II  
[www.meipharmacom](http://www.meipharmacom)

DLBCL

Phase I  
[www.meipharmacom](http://www.meipharmacom)

zilovetamab  
(ROR1 mAb)

Octernal Therapeutics  
San Diego, CA

mantle cell lymphoma

Phase II  
[www.octernal.com](http://www.octernal.com)

**Zynlonta®**  
loncastuximab tesirine-lpyl

ADC Therapeutics  
New Providence, NJ

2L NTE DLBCL (+rituximab)

Phase III  
[www.adctherapeutics.com](http://www.adctherapeutics.com)

3L+ DLBCL

Phase II  
[www.adctherapeutics.com](http://www.adctherapeutics.com)

follicular lymphoma

Phase I  
[www.adctherapeutics.com](http://www.adctherapeutics.com)

## Multiple Myeloma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ABBV-383/TN-383B (BCMA x CD3 bispecific antibody) <b>ORPHAN DRUG</b>	AbbVie North Chicago, IL	multiple myeloma	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a>
ABBV-IMAB-TJC4 (anti-CD47 mAb)	AbbVie North Chicago, IL	multiple myeloma	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a>
<b>Abecma</b> <sup>®</sup> idecabtagene vicleucel	Bristol-Myers Squibb Princeton, NJ	3L multiple myeloma (Breakthrough Therapy)	Phase III <a href="http://www.bms.com">www.bms.com</a>
		2L multiple myeloma, 4L+ multiple myeloma, high-risk newly-diagnosed multiple myeloma	Phase II <a href="http://www.bms.com">www.bms.com</a>
AGENT-797 (natural killer cell therapy)	MiNK Therapeutics (Agenus) New York, NY	multiple myeloma	Phase I <a href="http://www.minktherapeutics.com">www.minktherapeutics.com</a>
AL102 (amyloid precursor protein secretase inhibitor)	Ayala Pharmaceuticals Wilmington, DE	multiple myeloma (+WVT078)	Phase I <a href="http://www.ayalapharma.com">www.ayalapharma.com</a>
ALLO-605 (CAR-T cell therapy) <b>ORPHAN DRUG</b>	Allogene Therapeutics South San Francisco, CA	multiple myeloma (Fast Track)	Phase I <a href="http://www.allogene.com">www.allogene.com</a>

## Multiple Myeloma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ALLO-647 (anti-CD52 mAb)	Allogene Therapeutics South San Francisco, CA	multiple myeloma	Phase I <a href="http://www.allogene.com">www.allogene.com</a>
ALLO-715 (CAR-T cell therapy) <b>ORPHAN DRUG</b>	Allogene Therapeutics South San Francisco, CA	multiple myeloma	Phase I <a href="http://www.allogene.com">www.allogene.com</a>
alnuctamab (BCMA TCE) (CC-93269) (BCMA T-cell engager)	Bristol-Myers Squibb Princeton, NJ	multiple myeloma	Phase I <a href="http://www.bms.com">www.bms.com</a>
AO-176 (CD47 antigen inhibitor)	Arch Oncology Brisbane, CA	multiple myeloma	Phase I/II <a href="http://www.archoncology.com">www.archoncology.com</a>
APG-2575 (lisaftoclax) (Bcl-2 inhibitor) <b>ORPHAN DRUG</b>	Ascentage Pharma Rockville, MD	multiple myeloma	Phase I/II <a href="http://www.ascentagepharma.com">www.ascentagepharma.com</a>
ARC-T Plus anti-BCMA SparX (CAR-T cell therapy) <b>ORPHAN DRUG</b>	Arcellx Gaithersburg, MD	multiple myeloma (Fast Track)	Phase I <a href="http://www.arcellx.com">www.arcellx.com</a>
BCMA NKE (natural killer cell engager)	Bristol-Myers Squibb Princeton, NJ	multiple myeloma	Phase I <a href="http://www.bms.com">www.bms.com</a>

## Multiple Myeloma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
BHV-1100 (NK cell therapy) ORPHAN DRUG	Biohaven Pharmaceuticals New Haven, CT	multiple myeloma	Phase I/II <a href="http://www.biohavenpharma.com">www.biohavenpharma.com</a>
Blenrep® belantamab mafodotin-blmf	GlaxoSmithKline Research Triangle Park, NC	3L+ multiple myeloma, 2L+ multiple myeloma (combination therapy)	Phase III <a href="http://www.gsk.com">www.gsk.com</a>
		1L multiple myeloma (combination therapy)	Phase I <a href="http://www.gsk.com">www.gsk.com</a>
CART-ddBCMA (BCMA CAR-T cell therapy) ORPHAN DRUG	Arcellx Gaithersburg, MD	multiple myeloma (Fast Track)	Phase I <a href="http://www.arcellx.com">www.arcellx.com</a>
Carvykti® ciltacabtagene autoleucel	Janssen Research & Development Raritan, NJ	multiple myeloma 1-3PL, 1L multiple myeloma TNI	Phase III <a href="http://www.janssen.com">www.janssen.com</a>
CC-92328 (NK cell engager)	Bristol-Myers Squibb Princeton, NJ	multiple myeloma	Phase I <a href="http://www.bms.com">www.bms.com</a>
CC-92480 (CELMoD) (CRBN protein modulator)	Bristol-Myers Squibb Princeton, NJ	multiple myeloma	Phase II <a href="http://www.bms.com">www.bms.com</a>

## Multiple Myeloma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CC-95266	Bristol-Myers Squibb Princeton, NJ	multiple myeloma	Phase I <a href="http://www.bms.com">www.bms.com</a>
CC-98633 (BCMA NEX T) (BCMA CAR-T cell therapy)	Bristol-Myers Squibb Princeton, NJ	multiple myeloma	Phase I <a href="http://www.bms.com">www.bms.com</a>
CC-99712 (BCMA ADC) (BCMA antibody drug conjugate) <b>ORPHAN DRUG</b>	Bristol-Myers Squibb Princeton, NJ Sutro Biopharma South San Francisco, CA	multiple myeloma	Phase I <a href="http://www.bms.com">www.bms.com</a> <a href="http://www.sutrobio.com">www.sutrobio.com</a>
CD33 NKE (natural killer cell engager)	Bristol-Myers Squibb Princeton, NJ	multiple myeloma	Phase I <a href="http://www.bms.com">www.bms.com</a>
CD38 ADC (STI-6129) (antibody-drug conjugate)	Sorrento Therapeutics San Diego, CA	multiple myeloma	Phase I <a href="http://www.sorrentotherapeutics.com">www.sorrentotherapeutics.com</a>
CD38 DAR-T (STI-1492) (anti-CD38 DAR-T cell therapy)	Sorrento Therapeutics San Diego, CA	multiple myeloma	Phase I <a href="http://www.sorrentotherapeutics.com">www.sorrentotherapeutics.com</a>
cevistamab (RG6160) (FcRH5 x CD3 bispecific antibody) <b>ORPHAN DRUG</b>	Roche/Genentech South San Francisco, CA	multiple myeloma	Phase I <a href="http://www.roche.com">www.roche.com</a>

## Multiple Myeloma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CFT7455 (IKZF1/3 degrader) <b>ORPHAN DRUG</b>	C4 Therapeutics Watertown, MA	multiple myeloma	Phase I/II <a href="http://www.c4therapeutics.com">www.c4therapeutics.com</a>
CTX120 (CRISPR/Cas9 gene-edited CAR-T cell therapy) <b>ORPHAN DRUG</b>	CRISPR Therapeutics Cambridge, MA	multiple myeloma	Phase I <a href="http://www.crisprtx.com">www.crisprtx.com</a>
CYAD-211 (CAR-T cell therapy)	Celyad Oncology New York, NY	multiple myeloma	Phase I <a href="http://www.celyad.com">www.celyad.com</a>
CYNK-001 (taniraleucel) (natural killer cell therapy) <b>ORPHAN DRUG</b>	Celularity Florham Park, NJ	multiple myeloma	Phase I <a href="http://www.celularity.com">www.celularity.com</a>
<b>Darzalex</b> <sup>®</sup> daratumumab	Janssen Research & Development Raritan, NJ	smoldering multiple myeloma, 1L multiple myeloma in transplant eligible and ineligible	Phase III <a href="http://www.janssen.com">www.janssen.com</a>
Descartes-08 (CAR-T cell therapy)	Cartesian Therapeutics Gaithersburg, MD	multiple myeloma	Phase II <a href="http://www.cartesiantherapeutics.com">www.cartesiantherapeutics.com</a>
Descartes-25 (RNA cell therapy)	Cartesian Therapeutics Gaithersburg, MD	multiple myeloma	Phase I <a href="http://www.cartesiantherapeutics.com">www.cartesiantherapeutics.com</a>

## Multiple Myeloma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>DIOPP®</b> copper gluconate/disulfiram ORPHAN DRUG	Cantex Pharmaceuticals Weston, MA	multiple myeloma	Phase I/II <a href="http://www.cantex.com">www.cantex.com</a>
elranatamab (PF-06863135) (BCMA x CD3 bispecific antibody) ORPHAN DRUG	Pfizer New York, NY	multiple myeloma (double-class exposed) (Fast Track)	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
		multiple myeloma (triple-class refractory)	Phase II <a href="http://www.pfizer.com">www.pfizer.com</a>
eltanexor (KPT-8602) (SINE inhibitor)	Karyopharm Therapeutics Newton, MA	multiple myeloma	Phase I/II <a href="http://www.karyopharm.com">www.karyopharm.com</a>
<b>Empliciti® + Revlimid®</b> elotuzumab + lenalidomide	Bristol-Myers Squibb Princeton, NJ	1L multiple myeloma	Phase III <a href="http://www.bms.com">www.bms.com</a>
EZM0414 (SETD2 inhibitor) ORPHAN DRUG	Epizyme Cambridge, MA	multiple myeloma	Phase I <a href="http://www.epizyme.com">www.epizyme.com</a>
FOR46 (anti-CD46 antibody drug conjugate)	Fortis Therapeutics San Diego, CA	multiple myeloma	Phase I <a href="http://www.fortistx.com">www.fortistx.com</a>
frenlosirsen (IRF4rx) (RNA interference)	Flamingo Therapeutics San Diego, CA	multiple myeloma	Phase I <a href="http://www.flamingotx.com">www.flamingotx.com</a>

## Multiple Myeloma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
FT538 (NK cell cancer immunotherapy)	Fate Therapeutics San Diego, CA	multiple myeloma	Phase I <a href="http://www.fatetherapeutics.com">www.fatetherapeutics.com</a>
FT576 (NK cell cancer immunotherapy)	Fate Therapeutics San Diego, CA	multiple myeloma	Phase I <a href="http://www.fatetherapeutics.com">www.fatetherapeutics.com</a>
galinpepimut-S (cancer peptide vaccine) <b>ORPHAN DRUG</b>	Sellas Life Sciences New York, NY	multiple myeloma (Fast Track)	Phase II completed <a href="http://www.sellaslifesciences.com">www.sellaslifesciences.com</a>
GPRC5D CAR-T (MCAH 109) (CAR-T cell therapy)	Bristol-Myers Squibb Princeton, NJ	multiple myeloma	Phase I <a href="http://www.bms.com">www.bms.com</a>
HDP-101 (DNA-directed RNA polymerase inhibitor)	Heidelberg Pharma Munich, Germany	multiple myeloma	Phase I/II <a href="http://www.heidelberg-pharma.com">www.heidelberg-pharma.com</a>
HPN-217 (CD3-BCMA) <b>ORPHAN DRUG</b>	AbbVie North Chicago, IL Harpoon Therapeutics South San Francisco, CA	multiple myeloma (Fast Track)	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a> <a href="http://www.harpoontx.com">www.harpoontx.com</a>
iberdomide (ubiquitin protein ligase complex modulator)	Bristol-Myers Squibb Princeton, NJ	multiple myeloma	Phase II <a href="http://www.bms.com">www.bms.com</a>



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<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
iopofosine (radiopharmaceutical) <b>ORPHAN DRUG</b>	Cellectar Biosciences Florham Park, NJ	multiple myeloma (Fast Track)	Phase II <a href="http://www.cellectar.com">www.cellectar.com</a>
ISB 1342 (CD38/CD3 bispecific antibody) <b>ORPHAN DRUG</b>	Glenmark Pharmaceuticals Mumbai, India Ichnos Sciences New York, NY	multiple myeloma	Phase I <a href="http://www.glenmarkpharma.com">www.glenmarkpharma.com</a> <a href="http://www.ichnossciences.com">www.ichnossciences.com</a>
<b>Kyprolis®</b> carfilzomib	Amgen Thousand Oaks, CA	relapsed multiple myeloma (twice weekly dosing)	Phase III <a href="http://www.amgen.com">www.amgen.com</a>
magrolimab (anti-CD47 mAb)	Gilead Sciences Foster City, CA	multiple myeloma	Phase II <a href="http://www.gilead.com">www.gilead.com</a>
MGTA-145 (interleukin-8B receptor agonist) <b>ORPHAN DRUG</b>	Magenta Therapeutics Cambridge, MA	multiple myeloma (stem cell mobilization)	Phase II <a href="http://www.magentatx.com">www.magentatx.com</a>
modakafusp alfa (CD38 targeted antibody) <b>ORPHAN DRUG</b>	Takeda Deerfield, IL	multiple myeloma	Phase I <a href="http://www.takeda.com">www.takeda.com</a>
motixafortide (BL-8040) (CXCR4 antagonist) <b>ORPHAN DRUG</b>	BioLineRx Modi'in, Israel	multiple myeloma (stem cell mobilization)	Phase III <a href="http://www.biolinerx.com">www.biolinerx.com</a>

## Multiple Myeloma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
MT-0169 (proteasome inhibitor) <b>ORPHAN DRUG</b>	Molecular Templates Austin, TX	multiple myeloma	Phase I <a href="http://www.mtem.com">www.mtem.com</a>
NEXI-002 (adoptive cell therapy)	NexImmune Gaithersburg, MD	multiple myeloma	Phase I <a href="http://www.neximmune.com">www.neximmune.com</a>
<b>Ninlaro®</b> ixazomib	Takeda Deerfield, IL	newly-diagnosed multiple myeloma post-stem cell transplant and no stem cell transplant (maintenance)	Phase III <a href="http://www.takeda.com">www.takeda.com</a>
nirogacestat (gamma secretase inhibitor)	SpringWorks Therapeutic Stamford, CT	multiple myeloma (+belantamab mafodotin)	Phase II <a href="http://www.springworkstx.com">www.springworkstx.com</a>
NKTR-255 (IL-15 receptor agonist)	Nektar Therapeutics San Francisco, CA	multiple myeloma (combination therapy)	Phase I/II <a href="http://www.nektar.com">www.nektar.com</a>
<b>Opdivo® + Empliciti®</b> nivolumab + elotuzumab	Bristol-Myers Squibb Princeton, NJ	multiple myeloma	Phase II <a href="http://www.bms.com">www.bms.com</a>
ORIC-533 (CD73 inhibitor)	ORIC Pharmaceuticals South San Francisco, CA	multiple myeloma	Phase I <a href="http://www.oricpharma.com">www.oricpharma.com</a>
pavurutamab (AMG 701) (BCMAxCD3 bispecific T-cell engager)	Amgen Thousand Oaks, CA	multiple myeloma	Phase I <a href="http://www.amgen.com">www.amgen.com</a>

## Multiple Myeloma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
PBCAR269A (CAR-T cell therapy) <b>ORPHAN DRUG</b>	Precision Biosciences Durham, NC	multiple myeloma (Fast Track)	Phase I <a href="http://www.precisionbiosciences.com">www.precisionbiosciences.com</a>
P-BCMA-ALLO1 (CAR-T cell therapy) <b>ORPHAN DRUG</b>	Poseida Therapeutics San Diego, CA	multiple myeloma	Phase I <a href="http://www.poseida.com">www.poseida.com</a>
PHE885 (BCMA CAR-T cell therapy)	Novartis East Hanover, NJ	4L multiple myeloma	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
PT-112 (imifoplatin) (apoptosis stimulant) <b>ORPHAN DRUG</b>	Phosplatin Therapeutics New York, NY	multiple myeloma	Phase I <a href="http://www.phosplatin.com">www.phosplatin.com</a>
PVX-410 (cancer vaccine) <b>ORPHAN DRUG</b>	OncoPep Cambridge, MA	smoldering multiple myeloma (combination therapy)	Phase I completed <a href="http://www.oncopep.com">www.oncopep.com</a>
RAPA-201 (autologous rapamycin resistant T-cell therapy)	Rapa Therapeutics Rockville, MD	multiple myeloma (Fast Track)	Phase II
REGN5458 (BCMA x CD3 bispecific antibody)	Regeneron Pharmaceuticals Tarrytown, NY	multiple myeloma	Phase II <a href="http://www.regeneron.com">www.regeneron.com</a>

## Multiple Myeloma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
RG6234	Roche/Genentech South San Francisco, CA	multiple myeloma	Phase I <a href="http://www.roche.com">www.roche.com</a>
SAR442257 (CD38 x CD28 x CD3 trispecific antibody)	Sanofi Bridgewater, NJ	multiple myeloma (Fast Track)	Phase I <a href="http://www.sanofi.com">www.sanofi.com</a>
<b>Sarclisa</b> <sup>®</sup> isatuximab	Sanofi Bridgewater, NJ	1L newly-diagnosed multiple myeloma, (transplant eligible & ineligible), smoldering multiple myeloma	Phase III <a href="http://www.sanofi.com">www.sanofi.com</a>
		multiple myeloma (combination therapy)	Phase II <a href="http://www.sanofi.com">www.sanofi.com</a>
SEA-BCMA (anti-BCMA antibody)	Seagen Bothwell, WA	multiple myeloma	Phase I <a href="http://www.seagen.com">www.seagen.com</a>
STAR-LLD (continuous delivery of low-dose lenalidomide)	Starton Therapeutics Paramus, NJ	multiple myeloma	Phase I <a href="http://www.startontx.com">www.startontx.com</a>
STRO-001 (anti-CD74 antibody-drug conjugate) <b>ORPHAN DRUG</b>	Sutro Biopharma South San Francisco, CA	multiple myeloma	Phase I <a href="http://www.sutrobio.com">www.sutrobio.com</a>

## Multiple Myeloma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
talquetamab (GPRC5D x CD3 duobody)	Janssen Research & Development Raritan, NJ	multiple myeloma	Phase II <a href="http://www.janssen.com">www.janssen.com</a>
<b>Tazverik</b> <sup>®</sup> tazemetostat	Epizyme Cambridge, MA	multiple myeloma	Phase II <a href="http://www.epizyme.com">www.epizyme.com</a>
teclistamab (BCMA x CD3 duobody)	Janssen Research & Development Raritan, NJ	1-3PL multiple myeloma	Phase III <a href="http://www.janssen.com">www.janssen.com</a>
TEG002 (autologous T-cell therapy)	Gadeta Utrecht, The Netherlands	multiple myeloma	Phase I <a href="http://www.gadeta.nl">www.gadeta.nl</a>
UCARTCS1A (CAR-T cell therapy)	Collectis New York, NY	multiple myeloma	Phase I <a href="http://www.collectis.com">www.collectis.com</a>
<b>Venclexta</b> <sup>®</sup> venetoclax	AbbVie North Chicago, IL Genentech South San Francisco, CA	multiple myeloma	Phase III <a href="http://www.abbvie.com">www.abbvie.com</a> <a href="http://www.gene.com">www.gene.com</a>
WVT078	Novartis East Hanover, NJ	multiple myeloma	Phase I <a href="http://www.novartis.com">www.novartis.com</a>

## Multiple Myeloma

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
XmAb306 (RG6323) (IL15/IL15Ra-Fc recombinant fusion protein)	Roche/Genentech South San Francisco, CA Xencor Monrovia, CA	multiple myeloma (+daratumumab)	Phase I <a href="http://www.roche.com">www.roche.com</a> <a href="http://www.xencor.com">www.xencor.com</a>
zevorcabtagene autoleucel (zevor-cel) (CAR-T cell therapy) ORPHAN DRUG	CARsgen Shanghai, China	multiple myeloma	Phase I/II <a href="http://www.carsgen.com">www.carsgen.com</a>

## Myelodysplastic Syndromes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ABBV-IMAB-TJC4 (anti-CD47 mAb)	AbbVie North Chicago, IL	myelodysplastic syndromes (MDS)	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a>
AMV565 (CD33/CD3 bispecific T-cell engager)	Amphivena Therapeutics South San Francisco, CA	MDS	Phase I <a href="http://www.amphivena.com">www.amphivena.com</a>
APG-115 (alrizomadlin) (MDM2-p53)	Ascentage Pharma Rockville, MD	MDS	Phase I <a href="http://www.ascentagepharma.com">www.ascentagepharma.com</a>
APR-548 (p53 reactivator)	Apra Boston, MA	MDS	Phase I <a href="http://www.aprea.com">www.aprea.com</a>

## Myelodysplastic Syndromes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
APVO436 (CD123xCD3 bispecific antibody)	Aptevo Therapeutics Seattle, WA	MDS	Phase I <a href="http://www.aptevotherapeutics.com">www.aptevotherapeutics.com</a>
ASP7517 (cell therapy)	Astellas Pharma US Northbrook, IL	MDS	Phase II <a href="http://www.astellas.com">www.astellas.com</a>
aspyctarabine (BST-236) (anti-metabolite)	BioSight Lod, Israel	MDS	Phase II <a href="http://www.biosight-pharma.com">www.biosight-pharma.com</a>
azacitidine/cedazuridine (ASTX030) (oral DNMT inhibitor)	Astex Pharmaceuticals Pleasanton, CA	MDS	Phase II/III <a href="http://www.astx.com">www.astx.com</a>
bemcentinib (AXL inhibitor)	BerGenBio Bergen, Norway	MDS	Phase I/II <a href="http://www.bergenbio.com">www.bergenbio.com</a>
BMS-986397 (CC-91633) (CRBN modulator, CELMoD)	Bristol-Myers Squibb Princeton, NJ	MDS	Phase I <a href="http://www.bms.com">www.bms.com</a>
BTX-A51 (kinase inhibitor)	BioTheryX San Diego, CA	MDS	Phase I <a href="http://www.biotheryx.com">www.biotheryx.com</a>
CB-5339 (CDC48/P97 ATPase inhibitor)	Cleave Therapeutics San Francisco, CA	MDS	Phase I <a href="http://www.cleavetherapeutics.com">www.cleavetherapeutics.com</a>

## Myelodysplastic Syndromes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
CC-95251 (SIRPA protein modulator)	Bristol-Myers Squibb Princeton, NJ	MDS	Phase I <a href="http://www.bms.com">www.bms.com</a>
CFI-400945 (PLK4 protein inhibitor)	Treadwell Therapeutics New York, NY	MDS	Phase I/II <a href="http://www.treadwelltx.com">www.treadwelltx.com</a>
CG-806 (luxetpinib) (BTK/FLT3 inhibitor)	Aptose Biosciences San Diego, CA	MDS	Phase I <a href="http://www.aptose.com">www.aptose.com</a>
CYAD-02 (CAR-T cell therapy)	Celyad Oncology New York, NY	MDS	Phase I <a href="http://www.celyad.com">www.celyad.com</a>
CYC140 (PLK1 inhibitor)	Cyclacel Therapeutics Berkeley Heights, NJ	MDS	Phase I <a href="http://www.cyclacel.com">www.cyclacel.com</a>
DSP107 (CD47 x 41BB bi-functional fusion protein)	KAHR Medical Jerusalem, Israel	MDS	Phase I <a href="http://www.kahrbio.com">www.kahrbio.com</a>
DVX201 (NK cell therapy)	Deverra Therapeutics Seattle, WA	MDS	Phase I <a href="http://www.deverratx.com">www.deverratx.com</a>



## Myelodysplastic Syndromes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
eltanexor (KPT-8602) (SINE inhibitor) <b>ORPHAN DRUG</b>	Karyopharm Therapeutics Newton, MA	MDS	Phase I/II <a href="http://www.karyopharm.com">www.karyopharm.com</a>
emavusertib (IRAK4 inhibitor) <b>ORPHAN DRUG</b>	Curis Lexington, MA	IRAK4-driven MDS	Phase II <a href="http://www.curis.com">www.curis.com</a>
eprenetapopt (APR-246) (therapeutic targeting mutant p53) <b>ORPHAN DRUG</b>	Aprea Therapeutics Boston, MA	TP53-mutant MDS, (Fast Track) (Breakthrough Therapy)	Phase III <a href="http://www.aprea.com">www.aprea.com</a>
		IL TP53-mutant MDS	Phase II <a href="http://www.aprea.com">www.aprea.com</a>
evorpcept (ALX148) (CD47 antigen inhibitor)	ALX Oncology South San Francisco, CA	MDS (combination therapy)	Phase I/II <a href="http://www.alxoncology.com">www.alxoncology.com</a>
fadraciclib (CDK2/9 inhibitor)	Cyclacel Pharmaceuticals Berkeley Heights, NJ	MDS (+venetoclax)	Phase I/II <a href="http://www.cyclacel.com">www.cyclacel.com</a>
FHD286 (SMARCA2/4 protein inhibitor)	Foghorn Therapeutics Cambridge, MA	MDS	Phase I <a href="http://www.foghornrx.com">www.foghornrx.com</a>
H3B-8800 (RVT-2001) (SF3b complex modulator)	Hemavant Sciences (Roviant) New York, NY	MDS	Phase I <a href="http://www.roviant.com">www.roviant.com</a>

## Myelodysplastic Syndromes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
IGF-MTX (ifabotuzumab) (IGF-methotrexate conjugate)	IGF Oncology Birchwood, MN	MDS	Phase I/II <a href="http://www.igfoncology.com">www.igfoncology.com</a>
imetelstat (telomerase inhibitor) <b>ORPHAN DRUG</b>	Geron Foster City, CA	MDS (Fast Track)	Phase III <a href="http://www.geron.com">www.geron.com</a>
INKmune™ natural killer cell modulator	Inmune Bio Boca Raton, FL	MDS	Phase I <a href="http://www.inmunebio.com">www.inmunebio.com</a>
JSP191 (anti-CD117 mAb)	Jasper Therapeutics Redwood City, CA	MDS	Phase I <a href="http://www.jaspertherapeutics.com">www.jaspertherapeutics.com</a>
KER-050 (TGF-beta inhibitor)	Keros Therapeutics Lexington, MA	MDS	Phase II <a href="http://www.kerostx.com">www.kerostx.com</a>
LB-100 (PP2A inhibitor)	Lixte Biotechnology Pasadena, CA	MDS	Phase II <a href="http://www.lixe.com">www.lixe.com</a>
magrolimab (anti-CD47 mAb) <b>ORPHAN DRUG</b>	Gilead Sciences Foster City, CA	1L higher-risk MDS (Fast Track) (Breakthrough Therapy)	Phase III <a href="http://www.gilead.com">www.gilead.com</a>
MANA-312 (tumor-associated antigen T-cells)	Mana Therapeutics Waltham, MA	MDS after HSCT	Phase I <a href="http://www.manatherapeutics.com">www.manatherapeutics.com</a>

## Myelodysplastic Syndromes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
MGTA-117 (RNA polymerase II inhibitor)	Magenta Therapeutics Cambridge, MA	MDS	Phase I <a href="http://www.magentatx.com">www.magentatx.com</a>
NEXI-001 (adoptive cell therapy)	NexImmune Gaithersburg, MD	MDS	Phase I <a href="http://www.neximmune.com">www.neximmune.com</a>
NKX101 (CAR-NK cell therapy)	Nkarta Therapeutics South San Francisco, CA	MDS	Phase I <a href="http://www.nkartatx.com">www.nkartatx.com</a>
ONO-7475 (Axl/Mer inhibitor)	Ono Pharmaceutical Osaka, Japan	MDS	Phase I/II <a href="http://www.ono-pharma.com">www.ono-pharma.com</a>
<b>Onureg®</b> azacitidine	Bristol-Myers Squibb Princeton, MA	lower-risk MDS	Phase III <a href="http://www.bms.com">www.bms.com</a>
Orca-T (stem cell therapy)	Orca Bio Menlo Park, CA	MDS	Phase III <a href="http://www.orcabio.com">www.orcabio.com</a>
PLX2853 (BET inhibitor)	Plexikon South San Francisco, CA	MDS	Phase I <a href="http://www.plexikon.com">www.plexikon.com</a>
PRGN-3006 (UltraCar-T cell therapy)	Precigen Germantown, MD	MDS	Phase I <a href="http://www.precigen.com">www.precigen.com</a>

## Myelodysplastic Syndromes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
R289 (IRAK1/4 inhibitor)	Rigel Pharmaceuticals South San Francisco, CA	lower-risk MDS	Phase I <a href="http://www.rigel.com">www.rigel.com</a>
<b>Reblozyl®</b> luspatercept-aamt	Bristol-Myers Squibb Princeton, NJ	ESA naïve MDS	Phase III <a href="http://www.bms.com">www.bms.com</a>
RVU120 (CDK8/19 inhibitor)	Ryvu Therapeutics Kraków, Poland	high-risk MDS	Phase I <a href="http://www.rvu.com">www.rvu.com</a>
sabatolimab (MBG453) (TIM3 antagonist) <b>ORPHAN DRUG</b>	Novartis East Hanover, NJ	MDS (Fast Track)	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
		----- lower-risk MDS	Phase I <a href="http://www.novartis.com">www.novartis.com</a>
sapacitabine (DNA polymerase inhibitor) <b>ORPHAN DRUG</b>	Cyclacel Pharmaceuticals Berkeley Heights, NJ	MDS (+venetoclax)	Phase I/II <a href="http://www.cyclacel.com">www.cyclacel.com</a>
SEA-CD70 (anti-CD70 antibody)	Seagen Bothell, WA	MDS	Phase I <a href="http://www.seagen.com">www.seagen.com</a>
SL-172154 (recombinant fusion protein)	Shattuck Labs Austin, TX	MDS	Phase I <a href="http://www.shattucklabs.com">www.shattucklabs.com</a>

## Myelodysplastic Syndromes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
SX-682 (CXCR1/CXCR2 inhibitor)	Syntrix Biosystems Auburn, WA	MDS	Phase I/II <a href="http://www.syntrixbio.com">www.syntrixbio.com</a>
tamibarotene (oral RAR-alpha agonist) ORPHAN DRUG	Syros Pharmaceuticals Cambridge, MA	newly-diagnosed MDS	Phase III <a href="http://www.syros.com">www.syros.com</a>
TAS1553 (oral RNR inhibitor)	Astex Pharmaceuticals Pleasanton, CA	MDS	Phase I <a href="http://www.astx.com">www.astx.com</a>
<b>Tibsovo®</b> ivosidenib	Servier Pharmaceuticals Boston, MA	MDS w/IDH1 mutation (Breakthrough Therapy)	Phase I <a href="http://www.servier.us">www.servier.us</a>
treosulfan (bifunctional sulfonate alkylating agent) ORPHAN DRUG	Medexus Pharma USA Chicago, IL	MDS (conditioning in allogeneic hematopoietic stem cell transplantation)	application submitted <a href="http://www.medexus.com">www.medexus.com</a>
<b>Venclexta®</b> venetoclax	AbbVie North Chicago, IL Genentech South San Francisco, CA	1L MDS (+azacitidine) (Breakthrough Therapy)	Phase III <a href="http://www.abbvie.com">www.abbvie.com</a> <a href="http://www.gene.com">www.gene.com</a>
		MDS (+azacitidine)	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a> <a href="http://www.gene.com">www.gene.com</a>

## Myeloproliferative Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ABBV-744 (BDII-selective BET inhibitor)	AbbVie North Chicago, IL	myelofibrosis	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a>
APG-1252 (pelcitoclax) (Bcl-2/Bcl-xL)	Ascentage Pharma Rockville, MD	myelofibrosis	Phase I/II <a href="http://www.ascentagepharma.com">www.ascentagepharma.com</a>
<b>Besremi</b> <sup>®</sup> ropeginterferon alfa-2b-nfjt	PharmaEssentia Burlington, MA	essential thrombocythemia	Phase III <a href="http://www.pharmaessentia.com">www.pharmaessentia.com</a>
bomedemstat (LSD1 inhibitor) <b>ORPHAN DRUG</b>	Imago BioSciences South San Francisco, CA	essential thrombocythemia (Fast Track), myelofibrosis (Fast Track),	Phase II <a href="http://www.imagobio.com">www.imagobio.com</a>
elraglusib (GSK-3β inhibitor)	Actuate Therapeutics Fort Worth, TX	myelofibrosis	Phase II <a href="http://www.actuatetherapeutics.com">www.actuatetherapeutics.com</a>
<b>Elzonris</b> <sup>®</sup> tagraxofusp-erzs	Stemline Therapeutics New York, NY	myelofibrosis	Phase II <a href="http://www.stemline.com">www.stemline.com</a>
GB2064 (LOXL2 inhibitor)	Galecto Copenhagen, Denmark	myelofibrosis	Phase II <a href="http://www.galecto.com">www.galecto.com</a>
imetelstat (telomerase inhibitor) <b>ORPHAN DRUG</b>	Geron Foster City, CA	myelofibrosis (Fast Track)	Phase III <a href="http://www.geron.com">www.geron.com</a>

## Myeloproliferative Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
INCB57643 (BET inhibitor)	Incyte Wilmington, DE	myelofibrosis	Phase II <a href="http://www.incyte.com">www.incyte.com</a>
<b>Inrebic</b> <sup>®</sup> fedratinib	Bristol-Myers Squibb Princeton, NJ	myelofibrosis previously treated with ruxolitinib	Phase III <a href="http://www.bms.com">www.bms.com</a>
KER-050 (TGF beta inhibitor)	Keros Therapeutics Lexington, MA	myelofibrosis	Phase II <a href="http://www.kerostx.com">www.kerostx.com</a>
LNK01002 (triple kinase inhibitor)	Lynk Pharmaceuticals Zhejiang, China	myelofibrosis	Phase I <a href="http://www.en.lynkpharma.com">www.en.lynkpharma.com</a>
momelotinib (JAK 1/2 / ACVR1 inhibitor) <b>ORPHAN DRUG</b>	Sierra Oncology San Mateo, CA	myelofibrosis (Fast Track)	Phase III <a href="http://www.sierraoncology.com">www.sierraoncology.com</a>
navitoclax (BCL-XL x BCL-2 inhibitor)	AbbVie North Chicago, IL	myelofibrosis	Phase III <a href="http://www.abbvie.com">www.abbvie.com</a>
navtemadlin (KRT-232) (MDM2-p53 inhibitor)	Kartos Therapeutics Redwood City, CA	myelofibrosis	Phase III <a href="http://www.kartosthera.com">www.kartosthera.com</a>
		myelofibrosis (combination therapy)	Phase I/II <a href="http://www.kartosthera.com">www.kartosthera.com</a>

## Myeloproliferative Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
NS-018 (JAK2 inhibitor)	NS Pharma Paramus, NJ	myelofibrosis	Phase II <a href="http://www.nspharma.com">www.nspharma.com</a>
Orca-T (cell therapy)	Orca Bio Menlo Park, CA	myelofibrosis	Phase I/II <a href="http://www.orcabio.com">www.orcabio.com</a>
paraclisib and ruxolitinib (PI3K-delta/JAK1 inhibitor)	Incyte Wilmington, DE Novartis East Hanover, NJ	myelofibrosis	Phase II <a href="http://www.incyte.com">www.incyte.com</a> <a href="http://www.novartis.com">www.novartis.com</a>
pelabresib (BET inhibitor) ORPHAN DRUG	MorphoSys Boston, MA	myelofibrosis (Fast Track)	Phase III <a href="http://www.morphosys.com">www.morphosys.com</a>
<b>Pemazyre</b> <sup>®</sup> pemigatinib ORPHAN DRUG	Incyte Wilmington, DE	myeloproliferative neoplasms w/ FGFR1 rearrangement (Breakthrough Therapy)	Phase II <a href="http://www.incyte.com">www.incyte.com</a>
<b>Reblozyl</b> <sup>®</sup> luspatercept-aamt	Accelaron Pharma Cambridge, MA Bristol-Myers Squibb Princeton, NJ	myelofibrosis anemia	Phase III <a href="http://www.bms.com">www.bms.com</a>



## Myeloproliferative Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
RG6354 (PRM-151) (rhPTX-2) <b>ORPHAN DRUG</b>	Roche/Genentech South San Francisco, CA	myelofibrosis (Fast Track)	Phase II <a href="http://www.rocche.com">www.rocche.com</a>
rusfertide (PTG300) (hepcidin mimetic) <b>ORPHAN DRUG</b>	Protagonist Therapeutics Newark, CA	polycythemia vera (Fast Track) (Breakthrough Therapy)	Phase II <a href="http://www.protagonist-inc.com">www.protagonist-inc.com</a>
sapablursen (TMPRSS6 inhibitor)	Ionis Pharmaceuticals Carlsbad, CA	polycythemia vera	Phase II <a href="http://www.ionispharma.com">www.ionispharma.com</a>
TAS1553 (oral RNR inhibitor)	Astex Pharmaceuticals Pleasanton, CA	myeloproliferative disorders	Phase I <a href="http://www.astx.com">www.astx.com</a>
TBX-2400 (cell therapy)	Taiga Biotechnologies Aurora, CO	myelofibrosis	Phase I <a href="http://www.taigabiotech.com">www.taigabiotech.com</a>
TL-895 (BTK inhibitor)	Telios Pharma Redwood City, CA	myelofibrosis	Phase II
TP-3654 (PIM inhibitor)	Sumitomo Pharma Oncology Cambridge, MA	myelofibrosis	Phase I <a href="http://www.oncology.sumitomo-pharma.com">www.oncology.sumitomo-pharma.com</a>
<b>Xpovio</b> <sup>®</sup> selinexor	Karyopharm Therapeutics Newton, MA	myelofibrosis	Phase I <a href="http://www.karyopharm.com">www.karyopharm.com</a>

## Myeloproliferative Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
zelavespib (HSP90 heat-shock protein inhibitor) ORPHAN DRUG	Samus Therapeutics Topsfield, MA	myelofibrosis	Phase I <a href="http://www.samustherapeutics.com">www.samustherapeutics.com</a>

## Paroxysmal Nocturnal Hemoglobinuria

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ABP 959 (eculizumab biosimilar)	Amgen Thousand Oaks, CA	paroxysmal nocturnal hemoglobinuria	Phase III <a href="http://www.amgen.com">www.amgen.com</a>
ALXN2050 (factor D inhibitor) ORPHAN DRUG	Alexion AstraZeneca Rare Disease Boston, MA	paroxysmal nocturnal hemoglobinuria	Phase II <a href="http://www.alexion.com">www.alexion.com</a>
ARO-C3 (complement C3 inhibitor)	Arrowhead Pharmaceuticals Pasadena, CA	paroxysmal nocturnal hemoglobinuria	Phase I/II <a href="http://www.arrowheadpharma.com">www.arrowheadpharma.com</a>
BCX9930 (complement factor D inhibitor) ORPHAN DRUG	BioCryst Pharmaceuticals Durham, NC	paroxysmal nocturnal hemoglobinuria (Fast Track)	Phase II <a href="http://www.biocryst.com">www.biocryst.com</a>
crovalimab (RG6107) (complement C5 inhibitor)	Roche/Genentech South San Francisco, CA	paroxysmal nocturnal hemoglobinuria	Phase III <a href="http://www.roche.com">www.roche.com</a>

## Paroxysmal Nocturnal Hemoglobinuria

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
danicopan (ALXN2040) (factor D inhibitor) <b>ORPHAN DRUG</b>	Alexion AstraZeneca Rare Disease Boston, MA	paroxysmal nocturnal hemoglobinuria (Breakthrough Therapy)	Phase III <a href="http://www.alexion.com">www.alexion.com</a>
<b>Empaveli®</b> pegcetacoplan	Apellis Pharmaceuticals Waltham, MA	paroxysmal nocturnal hemoglobinuria (pediatric)	Phase II <a href="http://www.apellis.com">www.apellis.com</a>
iptacopan (LNP023) (CFB inhibitor) <b>ORPHAN DRUG</b>	Novartis East Hanover, NJ	paroxysmal nocturnal hemoglobinuria (Breakthrough Therapy)	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
NM 8074 (complement C3-C5 inhibitor)	NovelMed Therapeutics Cleveland, OH	paroxysmal nocturnal hemoglobinuria	Phase I <a href="http://www.novelmed.com">www.novelmed.com</a>
OMS906 (MASP-3 inhibitor)	Omeros Seattle, WA	paroxysmal nocturnal hemoglobinuria	Phase I <a href="http://www.omeros.com">www.omeros.com</a>
pozelimab + cemdisiran (C5 antibody + C5 siRNA) <b>ORPHAN DRUG</b>	Alnylam Pharmaceuticals Cambridge, MA Regeneron Pharmaceuticals Tarrytown, NY	paroxysmal nocturnal hemoglobinuria	Phase III <a href="http://www.alnylam.com">www.alnylam.com</a> <a href="http://www.regeneron.com">www.regeneron.com</a>
RLYB116 (complement C5 inhibitor)	Rallybio New Haven, CT	paroxysmal nocturnal hemoglobinuria	Phase I <a href="http://www.rallybio.com">www.rallybio.com</a>

## Paroxysmal Nocturnal Hemoglobinuria

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Ultomiris®</b> ravulizumab	Alexion AstraZeneca Rare Disease Boston, MA	paroxysmal nocturnal hemoglobinuria (subcutaneous)	Phase III <a href="http://www.alexion.com">www.alexion.com</a>

## Platelet Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
cevidoplenib (SKI-O-703) (SYK inhibitor)	Genosco Billerica, MA Ostotec Gyeonggi-do, South Korea	immune thrombocytopenia	Phase II <a href="http://www.genosco.com">www.genosco.com</a>
crovalimab (RG6107) (complement C5 inhibitor)	Roche/Genentech South San Francisco, CA	atypical hemolytic uremic syndrome	Phase III <a href="http://www.roche.com">www.roche.com</a>
<b>Doptelet®</b> avatrombopag	Swedish Orphan Biovitrum Waltham, MA	immune thrombocytopenia (pediatric)	Phase III <a href="http://www.sobi-northamerica.com">www.sobi-northamerica.com</a>
GC5101B (immunoglobulin)	GC Biopharma Gyeonggi-do, South Korea	immune thrombocytopenia	Phase III

## Platelet Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
iptacopan (LNP023) (CFB inhibitor)	Novartis East Hanover, NJ	atypical hemolytic uremic syndrome, immune thrombocytopenia	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
		autoimmune cytopenia	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
mezagitamab (TAK-079) (anti-CD38 mAb)	Takeda Deerfield, IL	immune thrombocytopenia	Phase II <a href="http://www.takeda.com">www.takeda.com</a>
narsoplimab (MASP-2 inhibitor) <b>ORPHAN DRUG</b>	Omeros Seattle, WA	atypical hemolytic uremic syndrome (Fast Track)	Phase III <a href="http://www.omeros.com">www.omeros.com</a>
<b>Nplate</b> <sup>®</sup> romiplostim	Amgen Thousand Oaks, CA	chemotherapy-induced thrombocytopenia	Phase III <a href="http://www.amgen.com">www.amgen.com</a>
OHB-101 (Fc gamma receptor modulator) <b>ORPHAN DRUG</b>	Oak Hill Bio Cambridge, MA	immune thrombocytopenia	Phase II <a href="http://www.oakhillbio.com">www.oakhillbio.com</a>
PF-06755347 (immunomodulation)	Pfizer New York, NY	primary immune thrombocytopenia	Phase I <a href="http://www.pfizer.com">www.pfizer.com</a>
PF-06835375 (CXCR5 antagonist)	Pfizer New York, NY	primary immune thrombocytopenia	Phase II <a href="http://www.pfizer.com">www.pfizer.com</a>

## Platelet Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
rilzabrutinib (BTK inhibitor) ORPHAN DRUG	Sanofi Bridgewater, NJ	immune thrombocytopenia (Fast Track)	Phase III <a href="http://www.sanofi.com">www.sanofi.com</a>
RLYB211 (anti-HPA-1a antibody) ORPHAN DRUG	Rallybio New Haven, CT	fetal/neonatal alloimmune thrombocytopenia (prevention)	Phase I <a href="http://www.rallybio.com">www.rallybio.com</a>
RLYB212 (anti-HPA-1a antibody) ORPHAN DRUG	Rallybio New Haven, CT	fetal/neonatal alloimmune thrombocytopenia (prevention)	Phase I <a href="http://www.rallybio.com">www.rallybio.com</a>
rozanolixizumab (neonatal Fc receptor antagonist) ORPHAN DRUG	UCB Smyrna, GA	primary immune thrombocytopenia	Phase III <a href="http://www.ucb.com">www.ucb.com</a>
<b>Ultomiris®</b> ravulizumab	Alexion AstraZeneca Rare Disease Boston, MA	atypical hemolytic uremic syndrome (subcutaneous)	Phase III <a href="http://www.alexion.com">www.alexion.com</a>
<b>Vonvendi®</b> von Willebrand factor (recombinant)	Takeda Deerfield, IL	von Willebrand disease (pediatric)	Phase III <a href="http://www.takeda.com">www.takeda.com</a>
<b>Vygart®</b> efgartigimod alfa ORPHAN DRUG	argenx Boston, MA	primary immune thrombocytopenia	Phase III <a href="http://www.argenx.com">www.argenx.com</a>

## Platelet Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Wilate®</b> von Willebrand factor/coagulation factor VIII complex (human)	Octapharma USA Paramus, NJ	von Willebrand disease (<6 years of age)	Phase III <a href="http://www.octapharmausa.com">www.octapharmausa.com</a>

## Sickle Cell Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Adakveo®</b> crizanlizumab-tmca	Novartis East Hanover, NJ	sickle cell disease in crisis (pediatric)	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
AG-946 (PK activator)	Agios Pharmaceuticals Cambridge, MA	sickle cell disease	Phase I <a href="http://www.agios.com">www.agios.com</a>
apadamtase alfa (TAK-755) (ADAM protein replacement) <b>ORPHAN DRUG</b>	Takeda Deerfield, IL	sickle cell disease	Phase II <a href="http://www.takeda.com">www.takeda.com</a>
ARU-1801 (lentiviral gene therapy) <b>ORPHAN DRUG</b>	Aruvant Sciences Millburn, NJ	sickle cell disease	Phase I/II <a href="http://www.aruvant.com">www.aruvant.com</a>
ASP8731/ML-0207 (BACH1 inhibitor)	Astellas Pharma US Northbrook, IL	sickle cell disease	Phase I <a href="http://www.astellas.com">www.astellas.com</a>

## Sickle Cell Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
BCL11a shRNA(miR) (miRNA-embedded shRNA)	bluebird bio Cambridge, MA	sickle cell disease	Phase I <a href="http://www.bluebirdbio.com">www.bluebirdbio.com</a>
benserazide (fetal globin gene promoter)	Phoenicia Biosciences Weston, MA	sickle cell disease	Phase I/II <a href="http://www.phbiosci.com">www.phbiosci.com</a>
crovalimab (RG6107) (complement C5 inhibitor)	Roche/Genentech South San Francisco, CA	sickle cell disease	Phase III <a href="http://www.roche.com">www.roche.com</a>
CSL889 (hemopexin replacement) <b>ORPHAN DRUG</b>	CSL Behring King of Prussia, PA	sickle cell disease	Phase I <a href="http://www.cslbehring.com">www.cslbehring.com</a>
Eclipse (decitabine/tetrahydrouridine) <b>ORPHAN DRUG</b>	EpiDestiny Solon, OH Novo Nordisk Plainsboro, NJ	sickle cell disease (Fast Track)	Phase I <a href="http://www.novonordisk.com">www.novonordisk.com</a>
ECT-001-CB (stem cell therapy)	ExCellThera Montreal, Canada	sickle cell disease	Phase I <a href="http://www.excellthera.com">www.excellthera.com</a>
EDIT-301 (autologous stem cell therapy)	Editas Medicine Cambridge, MA	sickle cell disease	Phase I/II <a href="http://www.editasmedicine.com">www.editasmedicine.com</a>



## Sickle Cell Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
etavopivat (PK activator) <b>ORPHAN DRUG</b>	FORMA Therapeutics Watertown, MA	sickle cell disease (Fast Track)	Phase II <a href="http://www.formatherapeutics.com">www.formatherapeutics.com</a>
exagamglogene autotemcel (CTX001) (gene-edited stem cell therapy) <b>ORPHAN DRUG</b>	CRISPR Therapeutics Cambridge, MA Vertex Pharmaceuticals Boston, MA	sickle cell disease (Fast Track)	Phase III <a href="http://www.crisprtx.com">www.crisprtx.com</a> <a href="http://www.vrtx.com">www.vrtx.com</a>
FTX-6058 (EED inhibitor) <b>ORPHAN DRUG</b>	Fulcrum Therapeutics Cambridge, MA	sickle cell disease	Phase I <a href="http://www.fulcrumtx.com">www.fulcrumtx.com</a>
GBT021601 (hemoglobin polymerization inhibitor)	Global Blood Therapeutics South San Francisco, CA	sickle cell disease	Phase I <a href="http://www.gbt.com">www.gbt.com</a>
GPH101 (gene edited stem cell therapy) <b>ORPHAN DRUG</b>	Graphite Bio South San Francisco, CA	sickle cell disease	Phase I/II <a href="http://www.graphitebio.com">www.graphitebio.com</a>
HIX763 (CRISPR/Cas9-based cell therapy)	Novartis East Hanover, NJ	sickle cell disease	Phase I <a href="http://www.novartis.com">www.novartis.com</a>
inclacumab (P selectin inhibitor)	Global Blood Therapeutics South San Francisco, CA	sickle cell disease	Phase III <a href="http://www.gbt.com">www.gbt.com</a>

## Sickle Cell Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
L-citrulline (amino acid replacement)	Asklepion Pharmaceuticals Baltimore, MD	sickle cell disease with vaso-occlusive crisis	Phase I/II <a href="http://www.asklepionpharma.com">www.asklepionpharma.com</a>
lovotibeglogene autotemcel (lovo-cel) (gene therapy) <b>ORPHAN DRUG</b>	bluebirdbio Cambridge, MA	sickle cell disease	Phase III <a href="http://www.bluebirdbio.com">www.bluebirdbio.com</a>
MGTA-145 (interleukin-8B receptor agonist)	Magenta Therapeutics Cambridge, MA	sickle cell disease	Phase I <a href="http://www.magentatx.com">www.magentatx.com</a>
olinciguat (sGC agonist) <b>ORPHAN DRUG</b>	Cyclerion Therapeutics Cambridge, MA	sickle cell disease	Phase II completed <a href="http://www.cyclerion.com">www.cyclerion.com</a>
OTQ923 (CRISPR/Cas9-based cell therapy)	Novartis East Hanover, NJ	sickle cell disease	Phase I <a href="http://www.novartis.com">www.novartis.com</a>
<b>Oxbryta</b> <sup>®</sup> voxelotor	Global Blood Therapeutics South San Francisco, CA	sickle cell disease (<4 years of age)	Phase I/II <a href="http://www.gbt.com">www.gbt.com</a>
PF-07209326 (E-selectin antagonist) <b>ORPHAN DRUG</b>	Pfizer New York, NY	sickle cell disease	Phase I <a href="http://www.pfizer.com">www.pfizer.com</a>

## Sickle Cell Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Pyrukynd®</b> mitapivat ORPHAN DRUG	Agios Pharmaceuticals Cambridge, MA	sickle cell disease (adults)	Phase II/III <a href="http://www.agios.com">www.agios.com</a>
rifaximin (rifamycin derivative antibiotic) ORPHAN DRUG	Salix Pharmaceuticals (Bausch Health) Bridgewater, NJ	sickle cell disease	Phase II <a href="http://www.salix.com">www.salix.com</a>
ST-400 (stem cell therapy)	Sangamo Therapeutics Brisbane, CA	sickle cell disease (Fast Track)	Phase I/II <a href="http://www.sangamo.com">www.sangamo.com</a>
vamifeport (ferroportin inhibitor) ORPHAN DRUG	Vifor Pharma Redwood City, CA	sickle cell disease	Phase II <a href="http://www.viforpharma.com">www.viforpharma.com</a>

## Thalassemia

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
benserazide (fetal globin gene promoter) ORPHAN DRUG	Phoenicia Biosciences Weston, MA	beta thalassemia intermedia	Phase I/II <a href="http://www.phbiosci.com">www.phbiosci.com</a>
betibeglogene autotemcel (beti-cel) (gene therapy) ORPHAN DRUG	bluebirdbio Cambridge, MA	beta-thalassemia (non- $\beta^0/\beta^0$ and $\beta^0/\beta^0$ genotypes) (Breakthrough Therapy) (Fast Track)	Phase III <a href="http://www.bluebirdbio.com">www.bluebirdbio.com</a>

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<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
EDIT-301 (autologous stem cell therapy)	Editas Medicine Cambridge, MA	transfusion-dependent thalassemia	Phase I/II <a href="http://www.editasmedicine.com">www.editasmedicine.com</a>
etavopivat (PKR activator)	FORMA Therapeutics Watertown, MA	thalassemia	Phase II <a href="http://www.formatherapeutics.com">www.formatherapeutics.com</a>
exagamglogene autotemcel (CTX001) (gene-edited stem cell therapy) <b>ORPHAN DRUG</b>	CRISPR Therapeutics Cambridge, MA Vertex Pharmaceuticals Boston, MA	beta thalassemia (Fast Track)	Phase III <a href="http://www.crisprtx.com">www.crisprtx.com</a> <a href="http://www.vrtx.com">www.vrtx.com</a>
<b>Pyrukynd®</b> mitapivat <b>ORPHAN DRUG</b>	Agios Pharmaceuticals Cambridge, MA	non-transfusion dependent thalassemia (adult), transfusion dependent thalassemia (adult)	Phase III <a href="http://www.agios.com">www.agios.com</a>
<b>Reblozyl®</b> luspatercept-aamt	Bristol-Myers Squibb Princeton, NJ	non-transfusion dependent beta thalassemia	Phase III <a href="http://www.bms.com">www.bms.com</a>
sapablursen (TMPRSS6 inhibitor)	Ionis Pharmaceuticals Carlsbad, CA	beta thalassemia	Phase II <a href="http://www.ionispharma.com">www.ionispharma.com</a>
vamifeport (ferroportin inhibitor) <b>ORPHAN DRUG</b>	Vifor Pharma Redwood City, CA	beta thalassemia	Phase II <a href="http://www.viforpharma.com">www.viforpharma.com</a>

## Other

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AB201 (recombinant nematode anticoagulant protein c2)	ARCA biopharma Westminster, CO	coagulation disorder associated with COVID-19 infections	Phase II/III <a href="http://www.arcabio.com">www.arcabio.com</a>
ALXN1820 (anti-properdin bi-specific antibody)	Alexion AstraZeneca Rare Disease Boston, MA	hematology	Phase I <a href="http://www.alexion.com">www.alexion.com</a>
bentracimab (PB2452) (recombinant mAb)	PhaseBio Pharmaceuticals Malvern, PA	reversal of the antiplatelet activity ticagrelor (Breakthrough Therapy)	Phase III <a href="http://www.phasebio.com">www.phasebio.com</a>
bitopertin (GlyT1 inhibitor)	Disc Medicine Watertown, MA	erythropoietic protoporphyria	Phase I <a href="http://www.discmedicine.com">www.discmedicine.com</a>
ciraparantag (factor Xa inhibitor) <b>ORPHAN DRUG</b>	AMAG Pharmaceuticals Waltham, MA	hemorrhage (anticoagulant reversal) (Fast Track)	Phase II <a href="http://www.amagpharma.com">www.amagpharma.com</a>
dersimelagon (MT-7117) (melanocortin type 1 agonist) <b>ORPHAN DRUG</b>	Mitsubishi Tanabe Pharma Jersey City, NJ	erythropoietic protoporphyria (Fast Track), x-linked protoporphyria	Phase III <a href="http://www.mt-pharma-america.com">www.mt-pharma-america.com</a>
<b>Empaveli</b> <sup>®</sup> pegcetacoplan	Apellis Pharmaceuticals Waltham, MA	transplant-associated thrombotic microangiopathy	Phase II <a href="http://www.apellis.com">www.apellis.com</a>

**Other**

<b><u>Drug Name</u></b>	<b><u>Sponsor</u></b>	<b><u>Indication</u></b>	<b><u>Development Phase</u></b>
fibrin sealant (human plasma-derived)	Grifols Barcelona, Spain	surgical blood loss (pediatric)	Phase III <a href="http://www.grifols.com">www.grifols.com</a>
KSI-301 (VEGF inhibitor)	Kodiak Sciences Palo Alto, CA	retinal vein occlusion	Phase III <a href="http://www.kodiak.com">www.kodiak.com</a>
<b>Lytenava™</b> bevacizumab ophthalmic	Outlook Therapeutics Iselin, NJ	branch retinal vein occlusion	Phase III <a href="http://www.outlooktherapeutics.com">www.outlooktherapeutics.com</a>
narsoplimab (MASP-2 inhibitor) <b>ORPHAN DRUG</b>	Omeros Seattle, WA	transplant-related thrombotic microangiopathies (Breakthrough Therapy)	Phase III <a href="http://www.omeros.com">www.omeros.com</a>
nomacopan (C5 complement inhibitor) <b>ORPHAN DRUG</b>	Akari Therapeutics New York, NY	stem cell transplantation-related thrombotic microangiopathy (Fast Track)	Phase III <a href="http://www.akaritx.com">www.akaritx.com</a>
<b>Octaplex®</b> human prothrombin complex	Octapharma Paramus, NJ	acute major bleeding on direct oral anticoagulant therapy with factor Xa inhibitor	Phase III <a href="http://www.octapharma.com">www.octapharma.com</a>
OTX-TKI (axitinib intravitreal implant)	Ocular Therapeutix Bedford, MA	retinal vein occlusion	Phase I <a href="http://www.ocutx.com">www.ocutx.com</a>

## Other

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Rivo-Cel™ (BPX-501) (allogenic polyclonal T-cell therapy)	Bellicum Pharmaceuticals Houston, TX	inherited blood disorders	Phase I/II <a href="http://www.bellicum.com">www.bellicum.com</a>
<b>Ultomiris®</b> ravulizumab ORPHAN DRUG	Alexion AstraZeneca Rare Disease Boston, MA	hematopoietic stem cell transplant-associated thrombotic microangiopathy, complement-mediated thrombotic microangiopathy	Phase III <a href="http://www.alexion.com">www.alexion.com</a>

The content of this report has been obtained through public, government and industry sources, and the Springer "Adis Insight" database based on the latest information. Report current as of **May 5, 2022**. The medicines in this listing include medicines being developed by U.S.-based companies conducting trials in the United States and abroad, PhRMA-member companies conducting trials in the United States and abroad, and foreign companies conducting clinical trials in the United States. Some products may not be in active clinical trials. The information may not be comprehensive. For more, specific information about a particular product, contact the individual company directly or go to [www.clinicaltrials.gov](http://www.clinicaltrials.gov). The entire series of *Medicines in Development* is available on PhRMA's website: [www.phrma.org](http://www.phrma.org).

## Definitions

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

**Breakthrough Therapy**—Upon request by a sponsor, the FDA can grant this designation to expedite the development and review of a drug or biologic intended, alone or in combination with one or more other drugs, to treat a serious or life threatening disease or condition and preliminary clinical evidence indicates that it 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.

## Definitions

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

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

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

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