

## 2020 Medicines in Development for Children

### Allergy

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AR101 (oral immunotherapy)	Aimmune Therapeutics Brisbane, CA	peanut allergy (Fast Track) <i>4 years to 17 years</i>	application submitted <a href="http://www.aimmune.com">www.aimmune.com</a>
		peanut allergy <i>1 year to 3 years</i>	Phase III <a href="http://www.aimmune.com">www.aimmune.com</a>
AR201 (oral immunotherapy)	Aimmune Therapeutics Brisbane, CA	hen egg allergy <i>4 years to 26 years</i>	Phase II <a href="http://www.aimmune.com">www.aimmune.com</a>
ASP0892 (plasmid-based DNA vaccine)	Astellas Pharma Northbrook, IL	peanut allergy (Fast Track) <i>12 years to 17 years</i>	Phase I <a href="http://www.astellas.com">www.astellas.com</a>
Dupixent® dupilumab	Regeneron Pharmaceuticals Tarrytown, NY Sanofi Bridgewater, NJ	peanut allergy (monotherapy) <i>6 years to 17 years</i>	Phase II <a href="http://www.regeneron.com">www.regeneron.com</a> <a href="http://www.sanofi.com">www.sanofi.com</a>
		peanut allergy (in combination with AR101) <i>6 years to 17 years</i>	Phase II <a href="http://www.aimmune.com">www.aimmune.com</a> <a href="http://www.regeneron.com">www.regeneron.com</a> <a href="http://www.sanofi.com">www.sanofi.com</a>

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HAL-MPE1 immunotherapy	HAL Allergy Leiden, Netherlands	peanut allergy <i>5 years and older</i>	Phase I <a href="http://www.hal-allergy.com">www.hal-allergy.com</a>
<b>Ragwitek®</b> short ragweed pollen allergen extract	ALK-Abello Round Rock, TX	seasonal allergic rhinitis <i>4 years to 17 years</i>	Phase III completed <a href="http://www.alk.net">www.alk.net</a>
<b>Ryaltris™</b> mometasone furoate and olopatadine	Glenmark Pharmaceuticals Mumbai, India	seasonal allergic rhinitis <i>12 years and older</i>	application submitted <a href="http://www.glenmark.com">www.glenmark.com</a>
		seasonal allergic rhinitis <i>6 years to 11 years</i> perennial allergic rhinitis <i>12 years and older</i>	Phase III completed <a href="http://www.glenmark.com">www.glenmark.com</a>
SAR439794 (toll-like receptor 4 agonist)	Sanofi Bridgewater, NJ	peanut hypersensitivity <i>12 years and older</i>	Phase I <a href="http://www.sanofi.com">www.sanofi.com</a>
STAGR320 (sublingual allergy immunotherapy)	Stallergenes Greer Boston, MA	allergic rhinitis <i>12 years and older</i>	Phase III completed <a href="http://www.stallergenesgreer.com">www.stallergenesgreer.com</a>
STMC-103H (live biotherapeutic product)	Siolta Therapeutics San Francisco, CA	atopic immunoglobulin E-mediated allergic disorder <i>2 years and older</i>	Phase I <a href="http://www.sioltatherapeutics.com">www.sioltatherapeutics.com</a>

## Allergy

### Drug Name

### Sponsor

### Indication

### Development Phase

VE416  
(live biotherapeutic product)

Vedanta Biosciences  
Cambridge, MA

peanut hypersensitivity  
*12 years and older*

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

**Viaskin Milk**  
milk allergy immunotherapy

DBV Technologies  
Summit, NJ

milk-induced eosinophilic esophagitis  
*4 years to 17 years*

Phase II completed  
[www.dbv-technologies.com](http://www.dbv-technologies.com)

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milk hypersensitivity (Fast Track)  
*2 years to 17 years*

Phase I/II  
[www.dbv-technologies.com](http://www.dbv-technologies.com)

**Viaskin Peanut**  
peanut allergy immunotherapy

DBV Technologies  
Summit, NJ

peanut hypersensitivity (Fast Track)  
(Breakthrough Therapy)  
*4 years to 11 years*

application submitted  
[www.dbv-technologies.com](http://www.dbv-technologies.com)

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peanut hypersensitivity  
*1 year to 3 years*

Phase III  
[www.dbv-technologies.com](http://www.dbv-technologies.com)

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peanut hypersensitivity  
*adolescents*

Phase I  
[www.dbv-technologies.com](http://www.dbv-technologies.com)

## Arthritis/Musculoskeletal

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Cimzia®</b> certolizumab pegol	UCB Smyrna, GA	polyarticular-course juvenile idiopathic arthritis <i>2 years to 17 years</i>	Phase III <a href="http://www.ucb.com">www.ucb.com</a>
<b>Cosentyx®</b> secukinumab	Novartis East Hanover, NJ	juvenile psoriatic arthritis, enthesitis-related arthritis <i>2 years to 17 years</i>	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
<b>Kevzara®</b> sarilumab	Regeneron Pharmaceuticals Tarrytown, NY Sanofi Bridgewater, NJ	polyarticular juvenile idiopathic arthritis, systemic juvenile idiopathic arthritis <i>2 years to 17 years</i>	Phase II <a href="http://www.regeneron.com">www.regeneron.com</a> <a href="http://www.sanofi.com">www.sanofi.com</a>
<b>MACI®</b> autologous cultured chondrocytes	Vericel Cambridge, MA	chondral or osteochondral defects of the knee <i>10 years to 17 years</i>	Phase III <a href="http://www.vcel.com">www.vcel.com</a>
MSC110 (lacnotuzumab)	Novartis East Hanover, NJ	pigmented villonodular synovitis <i>12 years and older</i>	Phase II completed <a href="http://www.novartis.com">www.novartis.com</a>
upadacitinib (ABT-494) (JAK1 inhibitor)	AbbVie North Chicago, IL	polyarticular juvenile idiopathic arthritis <i>2 years to 17 years</i>	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a>
<b>Xeljanz®</b> tofacitinib	Pfizer New York, NY	juvenile idiopathic arthritis <i>2 years to 17 years</i>	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>

## Blood Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Adakveo®</b> crizanlizumab (SEG101) ORPHAN DRUG	Novartis East Hanover, NJ	sickle cell disease <i>6 months to 12 years</i>	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
ALXN1210 (ravulizumab) ORPHAN DRUG	Alexion Pharmaceuticals Boston, MA	paroxysmal nocturnal hemoglobinuria <i>up to 18 years</i>	Phase III <a href="http://www.alexion.com">www.alexion.com</a>
BCL11a shRNA (miR) (gene therapy)	bluebird bio Cambridge, MA Boston Children's Hospital Boston, MA	sickle cell disease <i>3 years and older</i>	Phase I <a href="http://www.bluebirdbio.com">www.bluebirdbio.com</a>
<b>Brilinta®</b> ticagrelor	AstraZeneca Wilmington, DE	vaso-occlusive crises in sickle cell disease <i>2 years to 17 years</i>	Phase III <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
CNSA-001 (mixed function oxygenase modulator)	Censa Pharmaceuticals Wellesley, MA	hyperphenylalaninemia due to primary BH4 deficiency <i>12 years and older</i>	Phase I/II <a href="http://www.censapharma.com">www.censapharma.com</a>
eptacog beta (recombinant coagulation Factor VIIa)	Hema Biologics Framingham, MA	hemophilia A, hemophilia B <i>6 months and older</i>	Phase III <a href="http://www.lfb-usa.com">www.lfb-usa.com</a>
<b>Feraheme®</b> ferumoxytol injection	AMAG Pharmaceuticals Waltham, MA	iron deficiency anemia <i>2 years to 17 years</i>	Phase III <a href="http://www.amagpharma.com">www.amagpharma.com</a>

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fitusiran (RNAi targeting anti-thrombin) ORPHAN DRUG	Sanofi Genzyme Cambridge, MA	hemophilia A or B <i>12 years and older</i>	Phase III <a href="http://www.sanofi.com">www.sanofi.com</a>
<b>Injectafer®</b> ferric carboxymaltose injection	American Regent (Daiichi) Shirley, NY	iron deficiency anemia <i>1 year to 17 years</i>	Phase III <a href="http://www.dsi.com">www.dsi.com</a>
IW-1701 (soluble guanylate cyclase stimulator) ORPHAN DRUG	Cyclerion Therapeutics Cambridge, MA	sickle cell disease <i>16 years and older</i>	Phase II <a href="http://www.cyclerion.com">www.cyclerion.com</a>
<b>Jivi®</b> antihemophilic factor (recombinant) PEGylated-aucl	Bayer Pharmaceutical Whippany, NJ	hemophilia A <i>up to 12 years</i>	Phase III <a href="http://www.pharma.bayer.com">www.pharma.bayer.com</a>
<b>LentiGlobin™</b> beta-globin gene therapy ORPHAN DRUG	bluebird bio Cambridge, MA	transfusion-dependent $\beta$ -thalassemia (non- $\beta$ 0- $\beta$ 0 and $\beta$ 0- $\beta$ 0 genotypes) (Fast Track) <i>up to 50 years</i>	Phase III <a href="http://www.bluebirdbio.com">www.bluebirdbio.com</a>
		sickle cell disease (Fast Track) <i>12 years and older</i>	Phase I/II <a href="http://www.bluebirdbio.com">www.bluebirdbio.com</a>
<b>Lokelma™</b> sodium zirconium cyclosilicate	AstraZeneca Wilmington, DE	hyperkalemia <i>up to 18 years</i>	Phase III <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>

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mavoxifafor (CXCR4 inhibitor) ORPHAN DRUG	X4 Pharmaceuticals Cambridge, MA	neutropenia <i>12 years and older</i>	Phase I <a href="http://www.x4pharma.com">www.x4pharma.com</a>
NiCord® omidubicel ORPHAN DRUG	Gamida Cell Boston, MA	sickle cell disease, thalassemia <i>2 years and older</i>	Phase I/II <a href="http://www.gamida-cell.com">www.gamida-cell.com</a>
PF-06741086 (tissue factor pathway inhibitor) ORPHAN DRUG	Pfizer New York, NY	hemophilia (Fast Track) <i>12 years and older</i>	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
rivipansel (GMI-1070) (pan selectin inhibitor) ORPHAN DRUG	Pfizer New York, NY	vaso-occlusive crises associated with sickle cell disease (Fast Track) <i>7 years and older</i>	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
RP-L102 (lentiviral-based gene therapy)	Rocket Pharmaceuticals New York, NY	Fanconi's anemia (Fast Track) <i>1 year to 17 years</i>	Phase II <a href="http://www.rocketpharma.com">www.rocketpharma.com</a>
SC411 (docosahexaenoic acid) ORPHAN DRUG	Micelle BioPharma Riveria Beach, FL	sickle cell disease <i>5 years to 17 years</i>	Phase II <a href="http://www.micellebiopharma.com">www.micellebiopharma.com</a>
Soliris® eculizumab	Alexion Pharmaceuticals Boston, MA	paroxysmal nocturnal hemoglobinuria <i>2 years to 17 years</i>	Phase III completed <a href="http://www.alexion.com">www.alexion.com</a>

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<b>Triferic®</b> ferric pyrophosphate citrate	Rockwell Medical Wixom, MI	iron deficiency anemia <i>up to 17 years</i>	Phase I/II completed <a href="http://www.rockwellmed.com">www.rockwellmed.com</a>
<b>Veltassa®</b> patiromer	Relypsa Redwood City, CA	hyperkalemia <i>2 years to 17 years</i>	Phase II <a href="http://www.relypsa.com">www.relypsa.com</a>
voxelotor (sickle hemoglobin modulator) <b>ORPHAN DRUG</b>	Global Blood Therapeutics South San Francisco, CA	sickle cell disease (Fast Track) <i>12 years and older</i>	application submitted <a href="http://www.gbt.com">www.gbt.com</a>

## Cancer

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131I-MIBG (radiotherapy)	Jubilant DraxImage Kirkland, Canada	neuroblastoma <i>1 year and older</i>	Phase II <a href="http://www.draximage.com">www.draximage.com</a>
131 I-omburtamab (thernostic mAb) <b>ORPHAN DRUG</b>	Y-mAbs Therapeutics New York, NY	neuroblastoma, central nervous system (CNS) metastases, leptomeningeal metastases <i>up to 18 years</i>	Phase II/III <a href="http://www.ymabs.com">www.ymabs.com</a>
ABI-009 (mTOR inhibitor)	AADi Pacific Palisades, CA Children's Oncology Group Monrovia, CA	solid tumors (combination therapy) <i>12 months to 21 years</i>	Phase I <a href="http://www.aadibio.com">www.aadibio.com</a>



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<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Abraxane®</b> paclitaxel protein-bound particles for injectable suspension, albumin-bound	Celgene Summit, NJ	recurrent/refractory solid tumors <i>6 months to 24 years</i>	Phase I/II completed <a href="http://www.celgene.com">www.celgene.com</a>
Ad-RTS-hIL-12 plus veledimex (DNA-based in vivo therapy) ORPHAN DRUG	Ziopharm Oncology Boston, MA	diffuse intrinsic pontine glioma, pediatric brain tumor <i>up to 21 years</i>	Phase I <a href="http://www.ziopharm.com">www.ziopharm.com</a>
<b>Adcetris®</b> brentuximab vedotin	Takeda Oncology Deerfield, IL Seattle Genetics Bothell, WA	Hodgkin lymphoma (combination therapy) <i>5 years-17 years</i>	Phase I/II <a href="http://www.takeda.com">www.takeda.com</a> <a href="http://www.seattlegenetics.com">www.seattlegenetics.com</a>
AG-881 (vorasidenib)	Agios Pharmaceuticals Cambridge, MA	glioma <i>12 years and older</i>	Phase III <a href="http://www.agios.com">www.agios.com</a>
aglatimagene besadenovec (Adv-Tk gene therapy) ORPHAN DRUG	Advantagene Auburndale, MA	pediatric brain tumors <i>3 years to 21 years</i>	Phase I <a href="http://www.advantagene.com">www.advantagene.com</a>
<b>Aliqopa®</b> copanlisib	Bayer Pharmaceuticals Whippany, NJ	lymphoma, solid tumors <i>6 months to 21 years</i>	Phase I/II <a href="http://www.pharma.bayer.com">www.pharma.bayer.com</a>

## Cancer

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ALRN-6924 (MDM2/MDMX antagonist) ORPHAN DRUG	Aileron Therapeutics Watertown, MA Dana-Farber Cancer Institute Boston, MA	cancer <i>1 year to 21 years</i>	Phase I <a href="http://www.aileronrx.com">www.aileronrx.com</a>
AMG 337 (c-Met inhibitor)	NantPharma Culver City, CA	clear cell sarcoma <i>16 years and older</i>	Phase II <a href="http://www.nantpharma.com">www.nantpharma.com</a>
<b>Bavencio</b> <sup>®</sup> avelumab	EMD Serono Rockland, MA	lymphoma, solid tumors <i>up to 18 years</i>	Phase I/II <a href="http://www.emdserono.com">www.emdserono.com</a>
BGB-290 (PARP inhibitor)	BeiGene USA Cambridge, MA	glioma <i>13 years to 25 years</i>	Phase I <a href="http://www.beigene.com">www.beigene.com</a>
BMS-986016 (relatlimab) (anti-LAG3 mAb)	Bristol-Myers Squibb Princeton, NJ	solid tumors <i>12 years and older</i>	Phase I/II <a href="http://www.bms.com">www.bms.com</a>
BMS-986158 (BET inhibitor)	Bristol-Myers Squibb Princeton, NJ	advanced tumors <i>12 years and older</i>	Phase I/II <a href="http://www.bms.com">www.bms.com</a>
BXQ-350 (lysosomal activator protein Saposin C and phosphatidylserine)	Bexion Pharmaceuticals Covington, KY	solid tumors <i>1 year to 30 years</i>	Phase I <a href="http://www.bexionpharma.com">www.bexionpharma.com</a>

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camidanlumab tesirine (ADCT-301)	ADC Therapeutics Epalinges, Switzerland	Hodgkin lymphoma <i>16 years and older</i>	Phase II <a href="http://www.adctherapeutics.com">www.adctherapeutics.com</a>
CAR-T CD19 cell therapy (2nd generation shortened manufacture)	ZIOPHARM Oncology Boston, MA	leukemia, lymphoma <i>1 year and older</i>	Phase I <a href="http://www.ziopharm.com">www.ziopharm.com</a>
CLR 131 (radiopharmaceutical) <b>ORPHAN DRUG</b>	Cellectar Biosciences Florham Park, NJ	malignant tumors <i>2 years to 21 years</i>	Phase I <a href="http://www.cellectar.com">www.cellectar.com</a>
CMD-501 (natural killer T cells)	Cell Medica London, United Kingdom	neuroblastoma <i>1 year to 21 years</i>	Phase I <a href="http://www.cellmedica.com">www.cellmedica.com</a>
<b>Cometriq®</b> cabozantinib	Exelixis Alameda, CA	differentiated thyroid cancer <i>16 years and older</i>	Phase III <a href="http://www.exelixis.com">www.exelixis.com</a>
<b>Cotellic®</b> cobimetinib	Genentech South San Francisco, CA	solid tumors <i>6 months to 30 years</i>	Phase I/II <a href="http://www.gene.com">www.gene.com</a>
crenigacestat (LY3039478) (Notch signaling pathway inhibitor)	Eli Lilly Indianapolis, IN	acute lymphoblastic leukemia (ALL), T-cell lymphoblastic lymphoma <i>2 years and older</i>	Phase I/II completed <a href="http://www.lilly.com">www.lilly.com</a>

## Cancer

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Cyramza®</b> ramucirumab	Eli Lilly Indianapolis, IN	refractory solid tumors <i>12 months to 21 years</i>	Phase I <a href="http://www.lilly.com">www.lilly.com</a>
<b>Darzalex®</b> daratumumab	Janssen Research & Development Raritan, NJ	precursor B-cell lymphoblastic leukemia-lymphoma <i>1 year to 30 years</i>	Phase II <a href="http://www.janssen.com">www.janssen.com</a>
dilanubicef (ex vivo expanded hematopoietic stem and progenitor cell therapy) <b>ORPHAN DRUG</b>	Nohla Therapeutics Seattle, WA	acute myeloid leukemia (AML), ALL, chronic myeloid leukemia (CML), myelodysplastic syndromes (Fast Track) <i>6 months and older</i>	Phase II <a href="http://www.nohlatherapeutics.com">www.nohlatherapeutics.com</a>
eflornithine plus high-dose powder sachet	Cancer Prevention Pharmaceuticals Tucson, AZ	neuroblastoma <i>up to 17 years</i>	Phase II <a href="http://www.canprevent.com">www.canprevent.com</a>
enoblituzumab (CD276 protein inhibitor)	MacroGenics Rockville, MD	B7-H3-expressing solid tumors <i>1 year and older</i>	Phase I completed <a href="http://www.macrogenics.com">www.macrogenics.com</a>
fenretinide intravenous	CerRx Lubbock, TX	solid tumors <i>pediatric</i>	Phase I <a href="http://www.cerxx.com">www.cerxx.com</a>
fimepinostat (HDAC/pan-PI3K inhibitor)	Curis Lexington, MA	solid tumors <i>16 years and older</i>	Phase I completed <a href="http://www.curis.com">www.curis.com</a>

## Cancer

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
GD2-GD3 vaccine (immunostimulant vaccine) <b>ORPHAN DRUG</b>	Y-mAbs Therapeutics New York, NY	neuroblastoma <i>up to 17 years</i>	Phase II <a href="http://www.ymabs.com">www.ymabs.com</a>
GDC-0084 (PI3K inhibitor)	Kazia Therapeutics Sydney, Australia	diffuse intrinsic pontine glioma <i>2 years to 21 years</i>	Phase I <a href="http://www.kaziatherapeutics.com">www.kaziatherapeutics.com</a>
<b>Gilotrif®</b> afatinib	Boehringer Ingelheim Ridgefield, CT	neuroectodermal tumors, rhabdomyosarcoma <i>1 year to 18 years</i>	Phase I/II <a href="http://www.boehringer-ingelheim.com">www.boehringer-ingelheim.com</a>
<b>Halaven®</b> eribulin mesylate	Eisai Woodcliff Lake, NJ	relapsed/refractory rhabdomyo- sarcoma, non-rhabdomyosarcoma soft tissue sarcoma, Ewing sarcoma <i>12 months to 18 years</i>	Phase II <a href="http://www.eisai.com">www.eisai.com</a>
huGD2-BsAb (GD2xCD3) (Hu3F8 bispecific antibody)	Memorial Sloan Kettering New York, NY Y-mAbs Therapeutics New York, NY	refractory GD2-positive solid tumors <i>2 years and older</i>	Phase I <a href="http://www.ymabs.com">www.ymabs.com</a>
<b>Ibrance®</b> palbociclib	Pfizer New York, NY	recurrent/refractory solid tumors <i>2 years to 20 years</i>	Phase I <a href="http://www.pfizer.com">www.pfizer.com</a>

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<b>Imbruvica®</b> ibrutinib	Janssen Research & Development Raritan, NJ Pharmacyclics Sunnyvale, CA	non-Hodgkin lymphoma <i>1 year to 30 years</i>	Phase III <a href="http://www.janssen.com">www.janssen.com</a> <a href="http://www.pharmacyclics.com">www.pharmacyclics.com</a>
<b>Imfinzi®</b> durvalumab	AstraZeneca Wilmington, DE	pediatric malignancies (with tremelimumab) <i>up to 18 years</i>	Phase I/II <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
<b>Imlygic®</b> talimogene laherparepvec	Amgen Thousand Oaks, CA	advanced non-CNS tumors <i>2 years to 21 years</i>	Phase I <a href="http://www.amgen.com">www.amgen.com</a>
INCB-59872 (LSD-1 inhibitor)	Incyte Wilmington, DE	Ewing's sarcoma <i>12 years and older</i>	Phase I <a href="http://www.incyte.com">www.incyte.com</a>
inhaled lipid-complexed cisplatin	Eleison Pharmaceuticals Princeton, NJ	pulmonary recurrent osteosarcoma <i>13 years and older</i>	Phase II completed <a href="http://www.eleison-pharma.com">www.eleison-pharma.com</a>
isatuximab (anti-CD33 mAb)	Sanofi Bridgewater, NJ	1L and 2L AML, ALL <i>up to 17 years</i>	Phase II <a href="http://www.sanofi.com">www.sanofi.com</a>
<b>Jakafi®</b> ruxolitinib	Incyte Wilmington, DE	ALL (with chemotherapy) <i>1 year to 21 years</i>	Phase II <a href="http://www.incyte.com">www.incyte.com</a>

**Cancer**

**Drug Name**

**Sponsor**

**Indication**

**Development Phase**

**Keytruda®**  
pembrolizumab

Merck  
Kenilworth, NJ

melanoma, merkel cell carcinoma  
*12 years and older*

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

melanoma, solid tumors  
*6 months to 17 years*

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

**Kymriah®**  
tisagenlecleucel

Novartis  
East Hanover, NJ

non-Hodgkin lymphoma  
*up to 18 years*

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

**Kyprolis®**  
carfilzomib

Amgen  
Thousand Oaks, CA

relapsing/refractory ALL  
*all ages*

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

L19-TNF  
(recombinant fusion protein)  
ORPHAN DRUG

Philogen  
Sovicille, Italy

soft tissue sarcoma  
*16 years and older*

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

**Lenvima®**  
lenvatinib

Eisai  
Woodcliff Lake, NJ

osteosarcoma, solid tumors  
*2 years to 21 years*

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

**Libtayo®**  
cemiplimab

Regeneron Pharmaceuticals  
Tarrytown, NY  
Sanofi Genzyme  
Cambridge, MA

relapsed/refractory solid tumors  
or CNS tumors  
*up to 25 years*

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

newly-diagnosed or recurrent glioma  
*up to 25 years*

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

## Cancer

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
lisocabtagene maraleucel (JCAR017) (CAR-T cell therapy)	Celgene Summit, NJ	relapsed/refractory B-cell ALL, B-cell non Hodgkin lymphoma <i>up to 25 years</i>	Phase I/II <a href="http://www.celgene.com">www.celgene.com</a>
LOXO-292 (RET inhibitor)	Loxo Oncology Stamford, CT	solid tumors, primary CNS tumors <i>6 months to 21 years</i>	Phase I/II <a href="http://www.loxooncology.com">www.loxooncology.com</a>
		advanced solid tumors, RET fusion-positive solid tumors, medullary thyroid cancer <i>6 months to 21 years</i>	Phase I/II <a href="http://www.loxooncology.com">www.loxooncology.com</a>
MB-102 (CAR-T cell therapy) <b>ORPHAN DRUG</b>	Mustang Bio Worcester, MA City of Hope National Medical Center Duarte, CA	AML, blastic plasmacytoid dendritic cell neoplasm <i>12 years and older</i>	Phase I <a href="http://www.mustangbio.com">www.mustangbio.com</a>
<b>Mekinist®</b> trametinib	Novartis East Hanover, NJ	BRAF V600 mutation positive cancer (mono and combination therapy) <i>1 month to 17 years</i>	Phase I/II <a href="http://www.novartis.com">www.novartis.com</a>
MTX110 (nano-inclusion) <b>ORPHAN DRUG</b>	Midatech Pharma Cardiff, United Kingdom	diffuse intrinsic pontine glioma <i>2 years to 21 years</i>	Phase II <a href="http://www.midatechpharma.com">www.midatechpharma.com</a>



## Cancer

### Drug Name

### Sponsor

### Indication

### Development Phase

navitoclax  
(Bcl-2 inhibitor)

AbbVie  
North Chicago, IL

relapsed/refractory ALL,  
relapsed/refractory lymphoblastic  
leukemia *4 years and older*

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

naxitamab  
(anti-GD2 3F8 mAb)  
ORPHAN DRUG

Y-mAbs Therapeutics  
New York, NY

neuroblastoma  
*1 year and older*

application submitted  
[www.ymabs.com](http://www.ymabs.com)

**NiCord®**  
omidubicel  
ORPHAN DRUG

Gamida Cell  
Boston, MA

leukemia, lymphoma,  
myelodysplastic syndrome  
*12 years and older*

Phase III  
[www.gamida-cell.com](http://www.gamida-cell.com)

ONC201  
(DRD2/DRD3 antagonist)  
ORPHAN DRUG

Oncoceutics  
Philadelphia, PA

glioma, recurrent glioblastoma  
(Fast Track) *16 years and older*

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

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diffuse intrinsic pontine glioma,  
H3K27M glioma (Fast Track)  
*2 years to 18 years*

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

**Cancer**

**Drug Name**

**Sponsor**

**Indication**

**Development Phase**

**Opdivo®**  
nivolumab

Bristol-Myers Squibb  
Princeton, NJ

metastatic melanoma  
*12 years and older*  
glioblastoma  
*all ages*

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

high-grade primary CNS malignancies  
*6 months to 21 years*  
Hodgkin lymphoma (with Adcetris®)  
*5 years to 30 years*  
solid tumors of high tumor  
mutational burden  
*12 years and older*

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

non-Hodgkin lymphoma  
*15 years and older*

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

OXS-1550  
(bispecific fusion protein  
drug conjugate)

GT Biopharma  
Beverly Hills, CA

B-cell malignancies  
*12 years and older*

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

PEP-CMV  
(dendritic cell vaccine)

Annias Immunotherapeutics  
Chapel Hill, NC  
Duke University  
Chapel Hill, NC

malignant glioma,  
recurrent medulloblastoma  
*3 years to 35 years*

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

## Cancer

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Pomalyst®</b> pomalidomide	Celgene Summit, NJ	recurrent or progressive primary brain tumors <i>1 year to 21 years</i>	Phase II <a href="http://www.celgene.com">www.celgene.com</a>
PTC596 (BMI1 protein inhibitor)	PTC Therapeutics South Plainfield, NJ	diffuse intrinsic pontine glioma, high grade glioma <i>36 months to 21 years</i>	Phase I <a href="http://www.ptcbio.com">www.ptcbio.com</a>
PVSRIP0 (recombinant polio virus vaccine) <b>ORPHAN DRUG</b>	Istari Oncology Research Triangle Park, NC	recurrent malignant glioma <i>12 years to 21 years</i>	Phase I <a href="http://www.istarioncology.com">www.istarioncology.com</a>
reprotrectinib (ALK, ROS1, TRK inhibitor)	Turning Point Therapeutics San Diego, CA	lymphoma, solid tumors <i>4 years to 12 years</i>	Phase I/II <a href="http://www.tptherapeutics.com">www.tptherapeutics.com</a>
<b>Revlimid®</b> lenalidomide	Celgene Summit, NJ	AML <i>1 year to 18 years</i>	Phase II completed <a href="http://www.celgene.com">www.celgene.com</a>
Rivo-cel (BPX-501) (T-cell replacement therapy) <b>ORPHAN DRUG</b>	Bellicum Pharmaceuticals Houston, TX	AML, myelodysplastic syndromes <i>12 years and older</i>	Phase II/III <a href="http://www.bellicum.com">www.bellicum.com</a>
<b>Rozlytrek™</b> entrectinib	Genentech South San Francisco, CA	recurrent or refractory solid tumors, primary CNS tumors <i>up to 22 years</i>	Phase I <a href="http://www.gene.com">www.gene.com</a>

## Cancer

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Rydapt®</b> midostaurin	Novartis East Hanover, NJ	newly-diagnosed FLT-3 mutated AML <i>3 months to 17 years</i>	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
seclidemstat (SP-2577) (LSD 1 inhibitor) ORPHAN DRUG	Salarius Pharmaceuticals Houston, TX	Ewing's sarcoma (Fast Track), solid tumors <i>13 years and older</i>	Phase I <a href="http://www.salariuspharma.com">www.salariuspharma.com</a>
<b>Tab-cel®</b> tabelecleucel	Atara Biotherapeutics South San Francisco, CA	Epstein-Barr virus associated post- transplant lymphoproliferative disease <i>all ages</i>	Phase III <a href="http://www.atarabio.com">www.atarabio.com</a>
		Epstein-Barr virus-associated nasopharyngeal carcinoma <i>12 years and older</i>	Phase I/II <a href="http://www.atarabio.com">www.atarabio.com</a>
<b>Taflinar®</b> dabrafenib	Novartis East Hanover, NJ	brain neoplasms <i>12 months to 17 years</i>	Phase I <a href="http://www.novartis.com">www.novartis.com</a>
<b>Taflinar® + Mekinist®</b> dabrafenib and trametinib	Novartis East Hanover, NJ	BRAF V600 mutation positive low grade glioma or relapsed/refractory high grade glioma <i>12 months to 17 years</i>	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
tazemetostat (EZH2 Inhibitor) ORPHAN DRUG	Epizyme Cambridge, MA	INI1-negative tumors, synovial sarcoma <i>6 months to 18 years</i>	Phase I <a href="http://www.epizyme.com">www.epizyme.com</a>

**Cancer**

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
TB-403 (placenta growth factor inhibitor)	BioInvent Lund, Sweden Oncurious Leuven, Belgium	relapsed/refractory medulloblastoma <i>6 months to 18 years</i>	Phase I/II <a href="http://www.oncurious.com">www.oncurious.com</a>
TK216 (proto oncogene protein inhibitor) ORPHAN DRUG	Oncternal Therapeutics San Diego, CA	Ewing's sarcoma (Fast Track) <i>10 years and older</i>	Phase I <a href="http://www.oncternal.com">www.oncternal.com</a>
UCART19 (CAR-T cell therapy)	Servier Paris, France	relapsed/refractory B-cell acute ALL <i>6 months to 17 years</i>	Phase I <a href="http://www.servier.com">www.servier.com</a>
varlitinib (EGFR antagonist, ERBB 2/4 receptor antagonist) ORPHAN DRUG	ASLAN Pharmaceuticals Singapore	biliary tract cancer <i>all ages</i>	Phase II/III <a href="http://www.aslanpharma.com">www.aslanpharma.com</a>
<b>Venclexta®</b> venetoclax	AbbVie North Chicago, IL Genentech South San Francisco, CA	AML (combination therapy) <i>12 years and older</i>	Phase III <a href="http://www.abbvie.com">www.abbvie.com</a> <a href="http://www.gene.com">www.gene.com</a>
		relapsed/refractory malignancies <i>up to 25 years</i> ALL, lymphoblastic leukemia <i>4 years and older</i>	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a> <a href="http://www.gene.com">www.gene.com</a>

## Cancer

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Vigil™</b> autologous cancer vaccine	Gradalis Carrollton, TX	Ewing's sarcoma <i>2 years and older</i>	Phase III <a href="http://www.gradalisinc.com">www.gradalisinc.com</a>
<b>Votrient®</b> pazopanib	Novartis East Hanover, NJ	solid tumors <i>1 year to 18 years</i>	Phase II <a href="http://www.novartis.com">www.novartis.com</a>
<b>Xalkori®</b> crizotinib	Pfizer New York, NY	ALK-positive malignant neoplasms <i>15 years and older</i>	Phase I <a href="http://www.pfizer.com">www.pfizer.com</a>
XmAb-18087 (SSTR2 x CD3 bispecific antibody)	Xencor Monrovia, CA	gastrointestinal stromal tumors, neuroendocrine tumors <i>12 years and older</i>	Phase I <a href="http://www.xencor.com">www.xencor.com</a>
<b>Xpovio®</b> selinexor	Karyopharm Therapeutics Newton, MA	liposarcoma <i>12 years and older</i>	Phase II/III <a href="http://www.karyopharm.com">www.karyopharm.com</a>
<b>Yescarta®</b> axicabtagene ciloleucel	Kite Pharma (Gilead) Foster City, CA	acute lymphoblastic leukemia <i>2 years to 21 years</i>	Phase I/II <a href="http://www.kitapharma.com">www.kitapharma.com</a>
<b>Zykadia</b> ceritinib	Novartis East Hanover, NJ	ALK-activated tumors <i>12 months to 17 years</i>	Phase I completed <a href="http://www.novartis.com">www.novartis.com</a>

## Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Adcirca</b> <sup>®</sup> tadalafil	Eli Lilly Indianapolis, IN United Therapeutics Silver Spring, MD	pulmonary arterial hypertension <i>6 months to 18 years</i>	Phase III <a href="http://www.lilly.com">www.lilly.com</a> <a href="http://www.unither.com">www.unither.com</a>
<b>Adempas</b> <sup>®</sup> riociguat	Bayer Pharmaceuticals Whippany, NJ	pulmonary arterial hypertension <i>6 years to 17 years</i>	Phase III <a href="http://www.pharma.bayer.com">www.pharma.bayer.com</a>
<b>Bevyxxa</b> <sup>®</sup> betrixaban	Portola South San Francisco, CA	venous thromboembolism <i>up to 17 years</i>	Phase I <a href="http://www.portola.com">www.portola.com</a>
<b>Edarbi</b> <sup>®</sup> azilsartan medoxomil	Arbor Pharmaceuticals Atlanta, GA	hypertension <i>6 years to 18 years</i>	Phase III <a href="http://www.arborpharma.com">www.arborpharma.com</a>

## Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Eliquis®</b> apixaban	Bristol-Myers Squibb Princeton, NJ Pfizer New York, NY	venous thromboembolism <i>3 months to 17 years</i>	Phase III <a href="http://www.bms.com">www.bms.com</a> <a href="http://www.pfizer.com">www.pfizer.com</a>
		prevention of blood clots in leukemia or lymphoma <i>1 year to 17 years</i>	
		thrombosis (congenital or acquired heart disease) <i>34 weeks to 17 years</i>	Phase II <a href="http://www.bms.com">www.bms.com</a> <a href="http://www.pfizer.com">www.pfizer.com</a>
		thromboembolism <i>37 weeks to 18 years</i>	Phase I <a href="http://www.bms.com">www.bms.com</a> <a href="http://www.pfizer.com">www.pfizer.com</a>
<b>Entresto®</b> sacubitril/valsartan	Novartis East Hanover, NJ	heart failure <i>1 year to 18 years</i>	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
		heart failure <i>1 month to 17 years</i>	Phase II/III <a href="http://www.novartis.com">www.novartis.com</a>
evinacumab (ANGPTL-3 antibody)	Regeneron Pharmaceuticals Tarrytown, NY	homozygous familial hypercholesterolemia <i>12 years and older</i>	Phase III <a href="http://www.regeneron.com">www.regeneron.com</a>
<b>Giapreza™</b> angiotensin II	La Jolla Pharmaceutical San Diego, CA	hypotension <i>2 years to 17 years</i>	Phase II <a href="http://www.lajollapharmaceutical.com">www.lajollapharmaceutical.com</a>



## Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
inclisiran (PCSK9 protein inhibitor, RNA interference) <b>ORPHAN DRUG</b>	Alnylam Pharmaceuticals Cambridge, MA The Medicines Company Parsippany, NJ	homozygous familial hypercholesterolemia <i>12 years and older</i>	Phase III completed <a href="http://www.alnylam.com">www.alnylam.com</a> <a href="http://www.themedicinescompany.com">www.themedicinescompany.com</a>
INOpulse (nitric oxide inhalation) <b>ORPHAN DRUG</b>	Bellerophon Therapeutics Warren, NJ	pulmonary arterial hypertension <i>16 years and older</i>	Phase II completed <a href="http://www.bellerophon.com">www.bellerophon.com</a>
intravenous citrulline	Asklepion Pharmaceuticals Baltimore, MD	acute lung injury in pediatrics undergoing cardiopulmonary bypass for congenital defects <i>up to 18 years</i>	Phase III <a href="http://www.asklepionpharm.com">www.asklepionpharm.com</a>
<b>Letairis®</b> ambrisentan	Gilead Sciences Foster City, CA GlaxoSmithKline Research Triangle Park, NC	pulmonary arterial hypertension <i>8 years to 18 years</i>	Phase II <a href="http://www.gilead.com">www.gilead.com</a> <a href="http://www.gsk.com">www.gsk.com</a>
<b>Opsumit®</b> macitentan	Actelion Pharmaceuticals South San Francisco, CA	pulmonary arterial hypertension <i>2 years to 17 years</i> congenital heart disease (fontan-palliated) <i>12 years and older</i>	Phase III <a href="http://www.actelion.com">www.actelion.com</a>
<b>Pradaxa®</b> dabigatran etexilate	Boehringer Ingelheim Ridgefield, CT	venous thromboembolism <i>up to 17 years</i>	Phase III <a href="http://www.boehringer-ingelheim.com">www.boehringer-ingelheim.com</a>

## Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Praluent®</b> alirocumab	Regeneron Pharmaceuticals Tarrytown, NY Sanofi US Bridgewater, NJ	heterozygous familial hypercholesterolemia <i>8 years to 17 years</i>	Phase III <a href="http://www.regeneron.com">www.regeneron.com</a> <a href="http://www.sanofi.com">www.sanofi.com</a>
<b>Praxbind®</b> idarucizumab	Boehringer Ingelheim Ridgefield, CT	reversal of the anticoagulant effects of Pradaxa® <i>up to 18 years</i>	Phase III <a href="http://www.boehringer-ingelheim.com">www.boehringer-ingelheim.com</a>
<b>Remodulin®</b> treprostinil	United Therapeutics Rockville, MD	pulmonary arterial hypertension <i>7 years to 17 years (oral)</i>	Phase II completed <a href="http://www.unither.com">www.unither.com</a>
		persistent pulmonary hypertension <i>up to 14 days (IV)</i>	Phase II <a href="http://www.unither.com">www.unither.com</a>
<b>Repatha®</b> evolocumab	Amgen Thousand Oaks, CA	heterozygous familial hypercholesterolemia, homozygous familial hypercholesterolemia <i>10 years to 17 years</i>	Phase III <a href="http://www.amgen.com">www.amgen.com</a>
<b>Savaysa®</b> edoxaban	Daiichi Sankyo Basking Ridge, NJ	prevention of thromboembolic complications in cardiac disease, venous thromboembolism <i>up to 17 years</i>	Phase III <a href="http://www.dsi.com">www.dsi.com</a>

## Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
udenafil (MZ101) (type 5 cyclic nucleotide phosphodiesterase inhibitor) <b>ORPHAN DRUG</b>	Mezzion Pharma Seoul, South Korea	single ventricle heart disease <i>12 years to 18 years</i>	Phase III <a href="http://www.mezzion.co.kr">www.mezzion.co.kr</a>
<b>Uptravi®</b> selexipag	Actelion Pharmaceuticals US South San Francisco, CA	pulmonary arterial hypertension <i>2 years to 18 years</i>	Phase II <a href="http://www.actelion.com">www.actelion.com</a>
<b>Xarelto®</b> rivaroxaban	Bayer Pharmaceutical Whippany, NJ Janssen Research & Development Raritan, NJ	venous thromboembolism <i>up to 17 years</i>	Phase III completed <a href="http://www.pharma.bayer.com">www.pharma.bayer.com</a> <a href="http://www.janssen.com">www.janssen.com</a>
		thromboprophylaxis following Fontan procedure <i>2 years to 8 years</i>	Phase III <a href="http://www.pharma.bayer.com">www.pharma.bayer.com</a> <a href="http://www.janssen.com">www.janssen.com</a>

## Developmental Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AB-2004 (microbiome modulator)	Axial Biotherapeutics Waltham, MA	gastrointestinal dysfunction and associated behavioral symptoms of autism spectrum disorder <i>12 years to 17 years</i>	Phase I/II <a href="http://www.axialbiotherapeutics.com">www.axialbiotherapeutics.com</a>

## Developmental Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
acamprosate SR sprinkles (GABA-A receptor agonist, glutamate receptor antagonist) ORPHAN DRUG	Confluence Pharmaceuticals Carmel, IN	fragile X syndrome symptoms <i>5 years to 17 years</i>	Phase II completed <a href="http://www.confluencepharma.com">www.confluencepharma.com</a>
balovaptan (RO5285119) (vasopressin 1 receptor antagonist)	Genentech South San Francisco, CA	autism spectrum disorder <i>5 years to 17 years</i>	Phase II <a href="http://www.gene.com">www.gene.com</a>
		autism spectrum disorder <i>2 years to 4 years</i>	Phase I <a href="http://www.gene.com">www.gene.com</a>
<b>Circadin®</b> melatonin prolonged release ORPHAN DRUG	Neurim Pharmaceuticals Tel Aviv, Israel	sleep disturbances in children with neurodevelopment disabilities <i>2 years to 17 years</i>	Phase III completed <a href="http://www.neurim.com">www.neurim.com</a>
CM-AT (enzyme modulator)	Curemark Rye, NY	autism spectrum disorder (Fast Track) <i>3 years to 8 years</i>	application submitted <a href="http://www.curemark.com">www.curemark.com</a>
GWP42006 (cannabidiol)	Greenwich Biosciences Carlsbad, CA	autism spectrum disorder <i>4 years to 18 years</i>	Phase II <a href="http://www.greenwichbiosciences.com">www.greenwichbiosciences.com</a>
JNJ-42165279 (FAAH inhibitor)	Janssen Research and Development Raritan, NJ	autism spectrum disorder <i>13 years and older</i>	Phase II <a href="http://www.janssen.com">www.janssen.com</a>

## Developmental Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
L1-79 (tyrosine hydroxylase inhibitor)	Yamo Pharmaceuticals New York, NY	autism spectrum disorder (Fast Track) <i>13 years to 21 years</i>	Phase II completed <a href="http://www.yamopharma.com">www.yamopharma.com</a>
OV101 (GABA A receptor agonist) ORPHAN DRUG	Ovid Therapeutics New York, NY	Angelman syndrome (Fast Track) <i>13 years and older</i> Fragile X syndrome (Fast Track) <i>13 years to 22 years</i>	Phase II <a href="http://www.ovidrx.com">www.ovidrx.com</a>
Zygel™ transdermal cannabidiol gel ORPHAN DRUG	Zynerba Pharmaceuticals Drevon, PA	fragile X syndrome (Fast Track) <i>3 years to 17 years</i>	Phase II/III <a href="http://www.zynerba.com">www.zynerba.com</a>
		autism spectrum disorder <i>4 years to 17 years</i>	Phase II <a href="http://www.zynerba.com">www.zynerba.com</a>
		development and epileptic encephalopathies <i>3 years to 17 years</i>	Phase II <a href="http://www.zynerba.com">www.zynerba.com</a>

## Diabetes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Adlyxin® lixisenatide	Sanofi Bridgewater, NJ	type 2 diabetes <i>10 years to 17 years</i>	Phase I <a href="http://www.sanofi.com">www.sanofi.com</a>

## Diabetes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Afrezza®</b> insulin (human) inhalation	MannKind Westlake Village, CA	type 1 diabetes <i>4 years to 17 years</i>	Phase II <a href="http://www.mannkind.com">www.mannkind.com</a>
AG019 (gene transference)	Intrexon T1D Partners Germantown, MD ActoBio Therapeutics Ghent, Belgium	type 1 diabetes <i>12 years and older</i>	Phase I/II <a href="http://www.actbio.com">www.actbio.com</a>
<b>Bydureon® BCise®</b> exenatide extended release	AstraZeneca Wilmington, DE	type 2 diabetes <i>10 years to 17 years</i>	Phase III <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
CLBS03 (autologous ex vivo polyclonal T regulatory cell therapy) <b>ORPHAN DRUG</b>	Caladrius Biosciences Basking Ridge, NJ	type 1 diabetes (Fast Track) <i>8 years to 17 years</i>	Phase II <a href="http://www.caladrius.com">www.caladrius.com</a>
dasiglucagon rescue pen (glucagon receptor agonist)	Zealand Pharma US New York, NY	congenital hyperinsulin <i>3 months to 12 years,</i> hypoglycemia in type 1 diabetes <i>6 years to 17 years</i>	Phase III <a href="http://www.zealnadpharma.com">www.zealnadpharma.com</a>
		congenital hyperinsulin <i>up to 364 days</i>	Phase II/III <a href="http://www.zealandpharma.com">www.zealandpharma.com</a>

## Diabetes

### Drug Name

### Sponsor

### Indication

### Development Phase

**Diamyd®/GABA**  
autoimmune diabetes vaccine  
ORPHAN DRUG

Diamyd Medical  
Stockholm, Sweden  
University of Alabama  
Birmingham, AL

type 1 diabetes  
*4 years to 18 years*

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

**Farxiga®**  
dapagliflozin

AstraZeneca  
Wilmington, DE

type 2 diabetes  
*10 years to 18 years*

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

**Fiasp®**  
insulin aspart injection  
100 units/mL

Novo Nordisk  
Plainsboro, NJ

type 1 diabetes  
*children and adolescents*

application submitted  
[www.novonordisk.com](http://www.novonordisk.com)

**Gvoke™**  
glucagon rescue pen  
ORPHAN DRUG

Xeris Pharmaceuticals  
Chicago, IL

congenital hyperinsulinism  
*up to 12 months*

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

insulin lispro (LY900014)  
(ultra rapid formulation)

Eli Lilly  
Indianapolis, IN

type 1 diabetes  
*1 year to 17 years*

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

**Invokana®**  
canagliflozin

Janssen  
Raritan, NJ

type 2 diabetes  
*10 years to 17 years*

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

**Janumet®**  
sitagliptin and metformin

Merck  
Kenilworth, NJ

type 2 diabetes  
*10 years to 17 years*

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

## Diabetes

### Drug Name

### Sponsor

### Indication

### Development Phase

**Janumet® XR**  
sitagliptin and metformin  
extended release

Merck  
Kenilworth, NJ

type 2 diabetes  
*10 years to 17 years*

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

**Januvia®**  
sitagliptin

Merck  
Kenilworth, NJ

type 2 diabetes  
*10 years to 17 years*

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

**Jardiance®**  
empagliflozin

Boehringer Ingelheim  
Ridgefield, CT  
Eli Lilly  
Indianapolis, IN

type 2 diabetes  
*10 years to 17 years*

Phase III  
[www.boehringer-ingelheim.com](http://www.boehringer-ingelheim.com)  
[www.lilly.com](http://www.lilly.com)

**Nesina®**  
alogliptin

Takeda  
Deerfield, IL

type 2 diabetes  
*10 years to 17 years*

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

**Onglyza®**  
saxagliptin

AstraZeneca  
Wilmington, DE

type 2 diabetes  
*10 years to 18 years*

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

**Ryzodeg®**  
insulin degludec and insulin  
aspart injection

Novo Nordisk  
Plainsboro, NJ

type 1 diabetes  
*1 year to 17 years*

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



## Diabetes

### Drug Name

### Sponsor

### Indication

### Development Phase

**Simponi®**  
golimumab

Janssen Research & Development  
Raritan, NJ

type 1 diabetes  
*6 years to 21 years*

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

pre-symptomatic type 1 diabetes  
*6 years to 21 years*

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

**Steglatro™**  
ertugliflozin

Merck  
Kenilworth, NJ

type 2 diabetes  
*10 years to 17 years*

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

teplizumab  
(CD3 antigen inhibitor)  
**ORPHAN DRUG**

Provention Bio  
Oldwick, NJ

type 1 diabetes  
*8 years to 17 years*

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

**Toujeo®**  
insulin glargine injection  
(300 units/mL)

Sanofi  
Bridgewater, NJ

type 1 diabetes  
*6 years to 17 years*

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

**Tradjenta®**  
linagliptin

Boehringer Ingelheim  
Ridgefield, CT  
Eli Lilly  
Indianapolis, IN

type 2 diabetes  
*10 years to 17 years*

Phase III  
[www.boehringer-ingelheim.com](http://www.boehringer-ingelheim.com)  
[www.lilly.com](http://www.lilly.com)

**Trulicity®**  
dulaglutide

Eli Lilly  
Indianapolis, IN

type 2 diabetes  
*10 years to 17 years*

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

## Diabetes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Victoza®</b> liraglutide	Novo Nordisk Plainsboro, NJ	type 2 diabetes <i>10 years to 17 years</i>	Phase III completed <a href="http://www.novonordisk.com">www.novonordisk.com</a>

## Eye Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
atropine micro formulation (muscarinic receptor antagonist)	Eyenovia New York, NY	progressive myopia <i>3 years to 12 years</i>	Phase III <a href="http://www.eyenoviabio.com">www.eyenoviabio.com</a>
brimonidine ophthalmic suspension	Sun Pharma Advanced Research Mumbai, India	ocular hypertension, open angle glaucoma <i>2 years and older</i>	Phase III <a href="http://www.sparc.life">www.sparc.life</a>
DE-117 ophthalmic solution (omidenepeg isopropyl)	Santen Pharmaceutical Emeryville, CA	glaucoma, ocular hypertension <i>12 months and older</i>	Phase III <a href="http://www.santenus.com">www.santenus.com</a>
<b>Eylea®</b> aflibercept	Regeneron Pharmaceuticals Tarrytown, NY	retinopathy of prematurity <i>all ages</i>	Phase III <a href="http://www.regeneron.com">www.regeneron.com</a>
ISV-305 (dexamethasone ophthalmic)	InSite Vision Alameda, CA	active symptomatic blepharitis <i>1 year and older</i>	Phase III
iVIEW-1201 (povidine iodine ophthalmic solution)	iVIEW Therapeutics Doylestown, PA	acute adenoviral conjunctivitis <i>15 years and older</i>	Phase II <a href="http://www.iviewtherapeutics.com">www.iviewtherapeutics.com</a>

## Eye Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Lotemax®</b> loteprednol etabonate	Bausch & Lomb Bridgewater, NJ	postoperative inflammation following cataract surgery <i>up to 11 years</i>	Phase III completed <a href="http://www.bausch.com">www.bausch.com</a>
<b>Lucentis®</b> ranibizumab	Novartis East Hanover, NJ	retinopathy of prematurity <i>all ages</i>	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
<b>MicroStat</b> phenylephrine/tropicamide ophthalmic solution	Eyenovia New York, NY	mydriasis (eye dilation) <i>all ages</i>	Phase III completed <a href="http://www.eyenoviabio.com">www.eyenoviabio.com</a>
NVK-002 (atropine ophthalmic)	Nevakar Bridgewater, NJ	progressive myopia <i>3 years to 17 years</i>	Phase III <a href="http://www.nevakar.com">www.nevakar.com</a>
RVL-1201 (oxymetazoline ophthalmic solution)	Osmotica Pharmaceutical Bridgewater, NJ	blepharoptosis <i>9 years and older</i>	Phase III <a href="http://www.osmotica.com">www.osmotica.com</a>
SYD-101 (atropine ophthalmic)	Sydnexis Del Mar, CA	progressive myopia <i>3 years to 14 years</i>	Phase III <a href="http://www.sydnexis.com">www.sydnexis.com</a>

## Gastrointestinal Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Cimzia®</b> certolizumab pegol	UCB Smyrna, GA	Crohn's disease <i>6 years to 17 years</i>	Phase II completed <a href="http://www.ucb.com">www.ucb.com</a>

## Gastrointestinal Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Dexilant®</b> dexlansoprazole	Takeda Deerfield, IL	erosive esophagitis, gastroesophageal reflux disease <i>1 year to 11 years</i>	Phase II <a href="http://www.takeda.com">www.takeda.com</a>
<b>Dupixent®</b> dupilumab ORPHAN DRUG	Regeneron Pharmaceuticals Tarrytown, NY Sanofi Bridgewater, NJ	eosinophilic esophagitis <i>12 years and older</i>	Phase II <a href="http://www.takeda.com">www.takeda.com</a>
<b>Entyvio®</b> vedolizumab	Takeda Deerfield, IL	Crohn's disease, ulcerative colitis <i>2 years to 17 years</i>	Phase II <a href="http://www.takeda.com">www.takeda.com</a>
etrasimod (sphingosine 1 phosphate receptor modulator)	Arena Pharmaceuticals Boston, MA	ulcerative colitis <i>16 years and older</i>	Phase III <a href="http://www.arenapharm.com">www.arenapharm.com</a>
etrolizumab (IgG1 MAb targeting beta 7 integrin subunit)	Genentech South San Francisco, CA	Crohn's disease, ulcerative colitis <i>4 years to 17 years</i>	Phase I <a href="http://www.gene.com">www.gene.com</a>
<b>Gattex®</b> teduglutide	Takeda Deerfield, IL	short bowel syndrome <i>4 months to 12 months</i>	Phase III <a href="http://www.takeda.com">www.takeda.com</a>
<b>Humira®</b> adalimumab	AbbVie North Chicago, IL	ulcerative colitis <i>4 years to 17 years</i>	Phase III <a href="http://www.abbvie.com">www.abbvie.com</a>

## Gastrointestinal Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Lialda®</b> mesalazine delayed release	Takeda Deerfield, IL	ulcerative colitis <i>5 years to 17 years</i>	Phase III completed <a href="http://www.takeda.com">www.takeda.com</a>
<b>Linzess®</b> linaclotide	Allergan Madison, NJ Ironwood Pharmaceuticals Cambridge, MA	functional constipation <i>6 years to 17 years</i>	Phase III <a href="http://www.allergan.com">www.allergan.com</a> <a href="http://www.ironwoodpharma.com">www.ironwoodpharma.com</a>
		functional constipation <i>2 years to 5 years</i>	Phase II <a href="http://www.allergan.com">www.allergan.com</a> <a href="http://www.ironwoodpharma.com">www.ironwoodpharma.com</a>
<b>Mytesi®</b> crofelemer	Napo Pharmaceuticals San Francisco, CA	acute diarrhea <i>pediatric</i>	Phase I <a href="http://www.napopharma.com">www.napopharma.com</a>
NTRA-2112	Nutrinia Tel Aviv, Israel	intestinal malabsorption <i>in preterm infants up to 5 days</i>	Phase III
SHP647 (alpha4beta7 integrin antagonist) <b>ORPHAN DRUG</b>	Takeda Deerfield, IL	Crohn's disease, ulcerative colitis <i>16 years and older</i>	Phase III <a href="http://www.takeda.com">www.takeda.com</a>
<b>Simponi®</b> golimumab	Janssen Research & Development Raritan, NJ	ulcerative colitis <i>2 years to 17 years</i>	Phase III <a href="http://www.janssen.com">www.janssen.com</a>

## Gastrointestinal Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Skyrizi®</b> risankizumab ORPHAN DRUG	AbbVie North Chicago, IL	Crohn's disease, ulcerative colitis <i>16 years and older</i>	Phase III <a href="http://www.abbvie.com">www.abbvie.com</a>
<b>Stelara®</b> ustekinumab	Janssen Research & Development Raritan, NJ	Crohn's disease <i>2 years to 17 years</i>	Phase I <a href="http://www.janssen.com">www.janssen.com</a>
TAK721 (SHP621) (budesonide oral suspension) ORPHAN DRUG	Takeda Deerfield, IL	eosinophilic esophagitis <i>11 years and older</i>	Phase III <a href="http://www.takeda.com">www.takeda.com</a>
<b>Trulance®</b> plecanatide	Bausch Health Bridgewater, NJ	chronic idiopathic constipation <i>12 years to 17 years</i>	Phase II completed <a href="http://www.bauschhealth.com">www.bauschhealth.com</a>
		irritable bowel syndrome <i>6 years to 17 years</i>	Phase II <a href="http://www.bauschhealth.com">www.bauschhealth.com</a>
upadacitinib (ABT-494) (JAK1 inhibitor)	AbbVie North Chicago, IL	ulcerative colitis <i>16 years and older</i>	Phase III <a href="http://www.abbvie.com">www.abbvie.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AAV-CNGA3 (AAV gene therapy) ORPHAN DRUG	Janssen Research & Development Raritan, NJ MeiraGTx New York, NY	congenital achromatopsia <i>3 years to 15 years</i>	Phase I/II <a href="http://www.meiragtx.com">www.meiragtx.com</a>
AAV-CNGB3 (AAV gene therapy) ORPHAN DRUG	Janssen Research & Development Raritan, NJ MeiraGTx New York, NY	congenital achromatopsia <i>3 years and older</i>	Phase I/II <a href="http://www.meiragtx.com">www.meiragtx.com</a>
AAV-RPE65 (AAV gene therapy) ORPHAN DRUG	MeiraGTx New York, NY	Leber congenital amaurosis with retinal RPE65-deficiency <i>3 months and older</i>	Phase I/II completed <a href="http://www.meiragtx.com">www.meiragtx.com</a>
AAV-RPGR (AAV gene therapy) ORPHAN DRUG	Janssen Research & Development Raritan, NJ MeiraGTx New York, NY	x-linked retinitis pigmentosa <i>5 years and older</i>	Phase I/II <a href="http://www.meiragtx.com">www.meiragtx.com</a>
ABO-101 (gene transference) ORPHAN DRUG	Abeona Therapeutics New York, NY	mucopolysaccharidosis type IIIB (Fast Track) <i>7 months and older</i>	Phase I/II <a href="http://www.abeonatherapeutics.com">www.abeonatherapeutics.com</a>
ABO-102 (gene transference) ORPHAN DRUG	Abeona Therapeutics New York, NY	mucopolysaccharidosis type IIIA (Fast Track) <i>7 months and older</i>	Phase I/II <a href="http://www.abeonatherapeutics.com">www.abeonatherapeutics.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ACHM-CNGA3 (AAV gene therapy)	Applied Genetic Technologies Alachua, GA	achromatopsia <i>6 years and older</i>	Phase I/II <a href="http://www.agtc.com">www.agtc.com</a>
ACHM-CNGB3 (AAV gene therapy)	Applied Genetic Technologies Alachua, GA	achromatopsia <i>6 years and older</i>	Phase I/II <a href="http://www.agtc.com">www.agtc.com</a>
ACP-015 (TRansCon CNP)	Ascendis Pharma Palo Alto, CA	achondroplasia <i>2 years to 10 years</i>	Phase II <a href="http://www.ascendispharma.com">www.ascendispharma.com</a>
AEB1102 (pegzilarginase) <b>ORPHAN DRUG</b>	Aeglea Biotherapeutics Austin, TX	arginase 1 deficiency (Fast Track) <i>2 years and older</i>	Phase III <a href="http://www.aegleabio.com">www.aegleabio.com</a>
AGN-151587 (CRISPR-based medicine)	Allergan Madison, NJ Editas Medicine Cambridge, MA	Leber congenital amaurosis 10 <i>3 years and older</i>	Phase I/II <a href="http://www.allergan.com">www.allergan.com</a>
AGT-181 (enzyme replacement therapy) <b>ORPHAN DRUG</b>	ArmaGen San Diego, CA	mucopolysaccharidosis type I (Fast Track) <i>2 years and older</i>	Phase I/II completed <a href="http://www.armagen.com">www.armagen.com</a>
AGTC-501 (AAV-gene transference) <b>ORPHAN DRUG</b>	Applied Genetic Technologies Alachua, FL	X-linked retinitis pigmentosa caused by RPGR mutations <i>6 years and older</i>	Phase I/II <a href="http://www.agtc.com">www.agtc.com</a>



## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AMO-02 (GSK3-beta inhibitor)	AMO Pharma London, United Kingdom	congenital myotonic dystrophy <i>6 years to 16 years</i>	Phase II/III <a href="http://www.amo-pharma.com">www.amo-pharma.com</a>
arimoclomol ORPHAN DRUG	Orphazyme Newton, MA	Niemann-Pick disease type C (Fast Track) <i>2 years to 18 years</i>	Phase II/III <a href="http://www.orphazyme.com">www.orphazyme.com</a>
AT-GAA (novel ERT + chaperone) (enzyme replacement therapy) ORPHAN DRUG	Amicus Therapeutics Cranbury, NJ	Pompe disease <i>12 years to 17 years</i>	Phase III <a href="http://www.amicusrx.com">www.amicusrx.com</a>
avalglucosidase alfa (recombinant alpha glucosidase)	Sanofi Genzyme Cambridge, MA	Pompe disease <i>3 years and older</i>	Phase III <a href="http://www.sanofigenzyme.com">www.sanofigenzyme.com</a>
		Pompe disease <i>6 months to 17 years</i>	Phase II <a href="http://www.sanofigenzyme.com">www.sanofigenzyme.com</a>
AVR-RD-01 (lentiviral-based gene therapy) ORPHAN DRUG	AvroBio Cambridge, MA	Fabry disease <i>16 years and older</i>	Phase I/II <a href="http://www.avrobio.com">www.avrobio.com</a>
BCX7353 (serine protease inhibitor) ORPHAN DRUG	BioCryst Pharmaceuticals Durham, NC	hereditary angioedema (Fast Track) <i>12 years and older</i>	Phase III <a href="http://www.biocryst.com">www.biocryst.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
BPM31510 topical cream <b>ORPHAN DRUG</b>	Berg Framingham, MA	epidermolysis bullosa <i>12 years and older</i>	Phase I <a href="http://www.berghealth.com">www.berghealth.com</a>
BT595 (immune globulin)	Biotest Pharmaceuticals Boca Raton, FL	primary immunodeficiency diseases <i>2 years and older</i>	Phase III
cannabidiol oral solution	INSYS Therapeutics Chandler, AZ	Prader-Willi syndrome (Fast Track) <i>8 years to 17 years</i>	Phase II <a href="http://www.insysrx.com">www.insysrx.com</a>
CAP-1002 (allogeneic cardiosphere-derived stem cell therapeutic) <b>ORPHAN DRUG</b>	Capricor Beverly Hills, CA	Duchenne muscular dystrophy <i>10 years and older</i>	Phase II <a href="http://www.capricor.com">www.capricor.com</a>
casimersen (SRP-4045) (RNA interference)	Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy <i>7 years to 13 years</i>	Phase III <a href="http://www.sarepta.com">www.sarepta.com</a>
CCP-020 (diacerein topical ointment) <b>ORPHAN DRUG</b>	Castle Creek Pharmaceuticals Parsippany, NJ	epidermolysis bullosa (Fast Track) <i>6 months and older</i>	Phase II <a href="http://www.castlecreekpharma.com">www.castlecreekpharma.com</a>
CLN3 gene therapy	Amicus Therapeutics Cranbury, NJ	CLN3 Batten disease <i>3 years to 10 years</i>	Phase I/II <a href="http://www.amicusrx.com">www.amicusrx.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Cerdelga®</b> eliglustat	Sanofi Genzyme Cambridge, MA	Gaucher disease type 1 <i>2 years to 17 years</i>	Phase III <a href="http://www.sanofi.com">www.sanofi.com</a>
CLN6 gene therapy	Amicus Therapeutics Cranbury, NJ	CLN6 Batten disease <i>1 year and older</i>	Phase I/II <a href="http://www.amicusrx.com">www.amicusrx.com</a>
CUTX-101 (copper histidine) <b>ORPHAN DRUG</b>	Cyprium Therapeutics New York, NY	Menkes disease (Fast Track) <i>all ages</i>	Phase III <a href="http://www.cypriumtx.com">www.cypriumtx.com</a>
dasiglucagon (glucagon receptor agonist) <b>ORPHAN DRUG</b>	Zealand Pharma US New York, NY	congenital hyperinsulinemia <i>3 months to 12 years</i>	Phase III <a href="http://www.zealandpharma.com">www.zealandpharma.com</a>
DCCR (diazoxide choline controlled release) <b>ORPHAN DRUG</b>	Soleno Therapeutics Redwood City, CA	Prader-Willi syndrome (Fast Track) <i>4 years and older</i>	Phase III <a href="http://www.soleno.life">www.soleno.life</a>
EB-101 (autologous ex-vivo gene-corrected cell therapy) <b>ORPHAN DRUG</b>	Abeona Therapeutics New York, NY	recessive dystrophic epidermolysis bullosa (Breakthrough Therapy) <i>13 years and older</i>	Phase I/II <a href="http://www.abeonatherapeutics.com">www.abeonatherapeutics.com</a>
edasalonexent (NF-kappa B inhibitor) <b>ORPHAN DRUG</b>	Catabasis Pharmaceuticals Cambridge, MA	Duchenne muscular dystrophy (Fast Track) <i>4 years to 7 years</i>	Phase III <a href="http://www.catabasis.com">www.catabasis.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
elamipretide (mitochondria-targeting short peptide) ORPHAN DRUG	Stealth BioTherapeutics Newton, MA	primary mitochondrial myopathy (Fast Track) <i>16 years and older</i>	Phase III <a href="http://www.stealthbt.com">www.stealthbt.com</a>
		Barth syndrome (Fast Track) <i>12 years and older</i>	Phase II/III <a href="http://www.stealthbt.com">www.stealthbt.com</a>
EPI-743 (NQO1 modulator) ORPHAN DRUG	BioElectron Technology Mountain View, CA	Rett syndrome <i>up to 18 years</i>	Phase II completed <a href="http://www.bioelectron.com">www.bioelectron.com</a>
<b>Epidiolex®</b> cannabidiol ORPHAN DRUG	Greenwich Biosciences Carlsbad, CA	Rett syndrome <i>2 years to 18 years</i> tuberous sclerosis complex <i>1 year and older</i>	Phase III <a href="http://www.greenwichbiosciences.com">www.greenwichbiosciences.com</a>
<b>EryDex</b> dexamethasone erythrocyte- encapsulated ORPHAN DRUG	EryDel Bresso, Italy	ataxia telangiectasia <i>6 years and older</i>	Phase III <a href="http://www.erydel.com">www.erydel.com</a>
<b>Firazyr®</b> icatibant	Takeda Deerfield, IL	hereditary angioedema <i>2 years to 17 years</i>	Phase III completed <a href="http://www.takeda.com">www.takeda.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Firdapse®</b> amifampridine ORPHAN DRUG	Catalyst Pharmaceuticals Coral Gables, FL	congenital myasthenic syndromes <i>2 years and older</i>	Phase III <a href="http://www.catalystpharma.com">www.catalystpharma.com</a>
		spinal muscular atrophy type 3 <i>6 years and older</i>	Phase II <a href="http://www.catalystpharma.com">www.catalystpharma.com</a>
<b>Galafold®</b> migalastat	Amicus Therapeutics Cranbury, NJ	Fabry's disease <i>12 years to 17 years</i>	Phase III <a href="http://www.amicusrx.com">www.amicusrx.com</a>
GALGT2 (Galgt2 gene therapy)	Sarepta Therapeutics Cambridge, MA Nationwide Children's Hospital Columbus, OH	Duchenne muscular dystrophy <i>4 years and older</i>	Phase I/II <a href="http://www.sarepta.com">www.sarepta.com</a>
ganaxolone (GABA A receptor agonist) ORPHAN DRUG	Marinus Pharmaceuticals Radnor, PA	CDKL5 deficiency disorder <i>2 years to 21 years</i> PCDH19-related epilepsy <i>1 year to 17 years</i>	Phase III <a href="http://www.marinuspharma.com">www.marinuspharma.com</a>
givinostat (HDAC inhibitor)	Italfarmaco Milan, Italy	Duchenne muscular dystrophy <i>6 years to 17 years</i>	Phase III <a href="http://www.italfarmaco.com">www.italfarmaco.com</a>
GS010 (lenadogene nolparvovec) (AAV-based gene therapy) ORPHAN DRUG	GenSight Biologics New York, NY	Leber's hereditary optic atrophy <i>15 years and older</i>	Phase III <a href="http://www.gensight-biologics.com">www.gensight-biologics.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Hyqvia®</b> immune globulin Infusion 10% (human) with recombinant human hyaluronidase	Takeda Deerfield, IL	primary immunodeficiency diseases <i>2 years to 15 years</i>	Phase III <a href="http://www.takeda.com">www.takeda.com</a>
IB1001 (calcium channel modulator) <b>ORPHAN DRUG</b>	IntraBio Oxfordshire, United Kingdom	ataxia telangiectasia, Niemann-Pick disease type C, Tay-Sachs disease, Sandhoff disease <i>7 years and older</i>	Phase II <a href="http://www.intrabio.com">www.intrabio.com</a>
idebenone <b>ORPHAN DRUG</b>	Santhera Pharmaceuticals Burlington, MA	Duchenne muscular dystrophy (Fast Track) <i>10 years and older</i> Leber's hereditary optic neuropathy <i>12 years and older</i>	Phase III <a href="http://www.santhera.com">www.santhera.com</a>
immune globulin intravenous (human) 10%	Liminal BioSciences Laval, Canada	primary immunodeficiency diseases <i>2 years and older</i>	Phase III <a href="http://www.liminalbiosciences.com">www.liminalbiosciences.com</a>
KB103 (bercolagene telserpavec) <b>ORPHAN DRUG</b>	Krystal Biotech Pittsburgh, PA	dystrophic epidermolysis bullosa (Fast Track) <i>5 years and older</i>	Phase II <a href="http://www.krystalbio.com">www.krystalbio.com</a>
lenabasum (CB2 agonist) <b>ORPHAN DRUG</b>	Corbus Pharmaceuticals Norwood, MA	cystic fibrosis (Fast Track) <i>12 years and older</i>	Phase II <a href="http://www.corbuspharma.com">www.corbuspharma.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
leniolisib (CDZ173) (PI3K $\delta$ inhibitor)	Novartis East Hanover, NJ	APDS/PASLI (activated PI3Kdelta syndrome/p110delta-activating mutation causing senescent T Cells lymphadenopathy and immunodeficiency) <i>12 years and older</i>	Phase II/III <a href="http://www.novartis.com">www.novartis.com</a>
<b>Lenti-D™</b> elivaldogene tavalentivec <b>ORPHAN DRUG</b>	bluebird bio Cambridge, MA	cerebral adrenoleukodystrophy <i>up to 17 years</i>	Phase III <a href="http://www.bluebirdbio.com">www.bluebirdbio.com</a>
livoletide (ghrelin antagonist) <b>ORPHAN DRUG</b>	Millendo Therapeutics Ann Arbor, MI	Prader-Willi syndrome (Fast Track) <i>8 years and older</i>	Phase II/III <a href="http://www.millendo.com">www.millendo.com</a>
LMIO70 (branaplam) (SMN2 RNA splicing modulator) <b>ORPHAN DRUG</b>	Novartis East Hanover, NJ	spinal muscular atrophy <i>up to 182 days</i>	Phase I/II <a href="http://www.novartis.com">www.novartis.com</a>
lonafarnib (farnesyl transferase inhibitor) <b>ORPHAN DRUG</b>	Eiger BioPharmaceuticals Palo Alto, CA	Hutchinson-Gilford progeria syndrome (Breakthrough Therapy) <i>18 months to 17 years</i>	application submitted <a href="http://www.eigerbio.com">www.eigerbio.com</a>
LV-101 (intranasal carbetocin) <b>ORPHAN DRUG</b>	Levo Therapeutics Skokie, IL	Prader-Willi syndrome (Fast Track) <i>7 years to 18 years</i>	Phase III <a href="http://www.levotx.com">www.levotx.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<p>LYS-SAF302 (SRP-9002) (AAV gene therapy) <b>ORPHAN DRUG</b></p>	<p>Lysogene Cambridge, MA Sarepta Therapeutics Cambridge, MA</p>	<p>mucopolysaccharidosis type IIIA <i>6 months and older</i></p>	<p>Phase II/III <a href="http://www.lysogene.com">www.lysogene.com</a> <a href="http://www.sarepta.com">www.sarepta.com</a></p>
<p>maralixibat (sodium-bile acid cotransporter inhibitor) <b>ORPHAN DRUG</b></p>	<p>Mirum Pharmaceuticals Foster City, CA</p>	<p>Alagille syndrome (Breakthrough Therapy) <i>2 year to 18 years</i></p>	<p>Phase II <a href="http://www.mirumpharma.com">www.mirumpharma.com</a></p>
<p>mavorixafor (CXCR4 inhibitor) <b>ORPHAN DRUG</b></p>	<p>X4 Pharmaceuticals Cambridge, MA</p>	<p>WHIM syndrome (warts, hypogammaglobulinemia, immunodeficiency, myelokathexis) (Breakthrough Therapy) <i>13 years and older</i></p>	<p>Phase III <a href="http://www.x4pharma.com">www.x4pharma.com</a></p>
<p>MB-107 (ex vivo lentiviral gene therapy)</p>	<p>Mustang Bio Worcester, MA St. Jude Children's Research Hospital Memphis, TN</p>	<p>X-linked severe combined immunodeficiency <i>up to 24 months</i></p>	<p>Phase I/II <a href="http://www.mustangbio.com">www.mustangbio.com</a></p>
<p>miransertib (ARQ092) (pan-AKT inhibitor) <b>ORPHAN DRUG</b></p>	<p>ArQule Burlington, MA</p>	<p>PIK3CA-related overgrowth spectrum, Proteus syndrome (Fast Track) <i>2 years and older</i></p>	<p>Phase II <a href="http://www.arqule.com">www.arqule.com</a></p>



## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
MNK-1411 (melanocortin receptor agonist) <b>ORPHAN DRUG</b>	Mallinckrodt St. Louis, MO	Duchenne muscular dystrophy (Fast Track) <i>4 years to 8 years</i>	Phase II <a href="http://www.mallinckrodt.com">www.mallinckrodt.com</a>
mRNA-3704 <b>ORPHAN DRUG</b>	Moderna Therapeutics Cambridge, MA	isolated methylmalonic acidemia (Fast Track) <i>1 year and older</i>	Phase I/II <a href="http://www.modernatx.com">www.modernatx.com</a>
MT-1621 (deoxyribonucleoside replacement) <b>ORPHAN DRUG</b>	Modis Therapeutics Oakland, CA	thymidine kinase 2 deficiency <i>all ages</i>	Phase I/II <a href="http://www.modistx.com">www.modistx.com</a>
NADPH oxidase gene therapy <b>ORPHAN DRUG</b>	Genethon Cedex, France	chronic granulomatous disease <i>23 months and older</i>	Phase I/II <a href="http://www.genethon.com">www.genethon.com</a>
NBI-74788 (CRF receptor 1 antagonist)	Neurocrine Biosciences San Diego, CA	congenital adrenal hyperplasia <i>14 years to 17 years</i>	Phase II <a href="http://www.neurocrine.com">www.neurocrine.com</a>
NS-065/NCNP-0 (antisense oligonucleotide) <b>ORPHAN DRUG</b>	NS Pharma Paramus, NJ	Duchenne muscular dystrophy (Fast Track) <i>4 years to 10 years</i>	Phase II <a href="http://www.nspharma.com">www.nspharma.com</a>
olipudase alfa (rhASM enzyme replacement therapy) <b>ORPHAN DRUG</b>	Sanofi Genzyme Cambridge, MA	acid sphingomyelinase deficiency <i>up to 17 years</i>	Phase II <a href="http://www.sanofigenzyme.com">www.sanofigenzyme.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
omigapil (GAPDH inhibitor) ORPHAN DRUG	Santhera Pharmaceuticals Burlington, MA	congenital muscular dystrophy (Fast Track) <i>5 years to 16 years</i>	Phase I completed <a href="http://www.sathera.com">www.sathera.com</a>
ORGN001 (cPMP replacement therapy) ORPHAN DRUG	Origin Biosciences San Francisco, CA	molybdenum cofactor deficiency (Breakthrough Therapy) <i>up to 28 days</i>	Phase II/III <a href="http://www.origintx.com">www.origintx.com</a>
		molybdenum cofactor deficiency <i>all ages</i>	Phase II <a href="http://www.origintx.com">www.origintx.com</a>
OT-58 (enzyme replacement therapy)	Orphan Technologies Rapperswil, Switzerland	homocystinuria <i>12 years and older</i>	Phase I/II <a href="http://www.orphantechnologies.com">www.orphantechnologies.com</a>
P-188 NF (blood cell modulator) ORPHAN DRUG	Phrixus Pharmaceuticals Ann Arbor, MI	Duchenne muscular dystrophy <i>12 years to 25 years</i>	Phase II <a href="http://www.phrixuspharmaceuticals.com">www.phrixuspharmaceuticals.com</a>
palovarotene (retinoic acid receptor gamma agonist) ORPHAN DRUG	Clementia Pharmaceuticals Montreal, Canada	fibrodysplasia ossificans progressiva (Fast Track) <i>4 years and older</i>	Phase III <a href="http://www.clementiapharma.com">www.clementiapharma.com</a>
		multiple osteochondromas <i>2 years to 14 years</i>	Phase II <a href="http://www.clementiapharma.com">www.clementiapharma.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
pamrevlumab (FG-3019) (CTGF inhibitor) <b>ORPHAN DRUG</b>	FibroGen San Francisco, CA	Duchenne muscular dystrophy <i>12 years and older</i>	Phase II <a href="http://www.fibrogen.com">www.fibrogen.com</a>
pexidartinib (PLX3397)	Plexxikon Berkeley, CA National Cancer Institute Bethesda, MD	neurofibromatosis type 1 <i>3 years to 35 years</i>	Phase I/II <a href="http://www.plexxikon.com">www.plexxikon.com</a>
PF-06939926 (minidystrophin gene therapy) <b>ORPHAN DRUG</b>	Pfizer New York, NY	Duchenne muscular dystrophy <i>5 years to 12 years</i>	Phase I <a href="http://www.pfizer.com">www.pfizer.com</a>
<b>Procysbi®</b> cysteamine bitartrate oral granule packets	Horizon Therapeutics Lake Forest, IL	nephropathic cystinosis <i>1 year and older</i>	application submitted <a href="http://www.horizontherapeutics.com">www.horizontherapeutics.com</a>
PXT3003 (baclofen/naltrexone/sorbitol) <b>ORPHAN DRUG</b>	Pharnext Paris, France	Charcot-Marie-Tooth disease type 1A (Fast Track) <i>16 years and older</i>	Phase III <a href="http://www.pharnext.com">www.pharnext.com</a>
QR-313 (RNA interference) <b>ORPHAN DRUG</b>	Wings Therapeutics Berkeley, CA	recessive dystrophic epidermolysis bullosa <i>6 years and older</i>	Phase I/II <a href="http://www.wings-tx.com">www.wings-tx.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
recifercept (TA-46) (soluble recombinant human FGFR3 decoy) <b>ORPHAN DRUG</b>	Pfizer New York, NY	achondroplasia <i>up to 10 years</i>	Phase I <a href="http://www.pfizer.com">www.pfizer.com</a>
reldesemtiv (troponin stimulant) <b>ORPHAN DRUG</b>	Astellas Pharma Northbrook, IL Cytokinetics South San Francisco, CA	spinal muscular atrophy <i>12 years and older</i>	Phase II completed <a href="http://www.astellas.com">www.astellas.com</a> <a href="http://www.cytokinetics.com">www.cytokinetics.com</a>
resamirigene bilparvovec (AAV8 gene therapy) <b>ORPHAN DRUG</b>	Audentes Therapeutics San Francisco, CA	X-Linked myotubular myopathy (Fast Track) <i>up to 5 years</i>	Phase I/II <a href="http://www.audentextx.com">www.audentextx.com</a>
RG6206 (BMS-986089) (anti-myostatin adnectin)	Bristol-Myers Squibb Princeton, NJ Genentech South San Francisco, CA	Duchenne muscular dystrophy <i>6 years to 11 years</i>	Phase II/III <a href="http://www.gene.com">www.gene.com</a>
RGN-137 (timbetasin topical gel) <b>ORPHAN DRUG</b>	Lenus Therapeutics Princeton, NJ RegeneRx Biopharmaceuticals Rockville, MD	junctional and dystrophic epidermolysis bullosa <i>4 years and older</i>	Phase II <a href="http://www.gtreebnt.com">www.gtreebnt.com</a> <a href="http://www.regenerx.com">www.regenerx.com</a>
RGX-111 (personalized gene therapy) <b>ORPHAN DRUG</b>	REGENXBIO Rockville, MD	mucopolysaccharidosis type I (Fast Track) <i>6 years and older</i>	Phase I <a href="http://www.regenxbio.com">www.regenxbio.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
RGX-121 (personalized gene therapy) <b>ORHAN DRUG</b>	REGENXBIO Rockville, MD	mucopolysaccharidosis type II (Fast Track) <i>4 months to 5 years</i>	phase I/II <a href="http://www.regenxbio.com">www.regenxbio.com</a>
risdiplam (SMN2 splicing modifier) <b>ORHAN DRUG</b>	PTC Therapeutics South Plainfield, NJ Genentech South San Francisco, CA	spinal muscular atrophy (Fast Track) <i>1 month to 25 years</i>	application submitted <a href="http://www.ptcbio.com">www.ptcbio.com</a>
		spinal muscular atrophy <i>up to 6 weeks</i>	Phase II <a href="http://www.ptcbio.com">www.ptcbio.com</a>
RP-A501 (AAV9 gene therapy)	Rocket Pharmaceuticals New York, NY	Danon disease (Fast Track) <i>8 years and older</i>	Phase I <a href="http://www.rocketpharma.com">www.rocketpharma.com</a>
RP-L102 (lentiviral vector-based gene therapy) <b>ORPHAN DRUG</b>	Rocket Pharmaceuticals New York, NY	Franconi anemia (Fast Track) <i>1 year to 12 years</i>	Phase I <a href="http://www.rocketpharma.com">www.rocketpharma.com</a>
RP-L201 (lentiviral vector-based gene therapy) <b>ORPHAN DRUG</b>	Rocket Pharmaceuticals New York, NY	leukocyte adhesion deficiency-type 1 (Fast Track) <i>3 months and older</i>	Phase I/II <a href="http://www.rocketpharma.com">www.rocketpharma.com</a>
<b>Ruconest®</b> C1 esterase inhibitor (recombinant)	Pharming Leiden, Netherlands	hereditary angioedema <i>2 years to 13 years</i>	Phase II completed <a href="http://www.pharming.com">www.pharming.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Ryplazim™ IV</b> plasminogen intravenous <b>ORPHAN DRUG</b>	Liminal BioSciences Laval, Canada	congenital plasminogen deficiency (Fast Track) <i>2 years and older</i>	application submitted <a href="http://www.liminalbiosciences.com">www.liminalbiosciences.com</a>
SAR422459 (ABCA4 gene therapy) <b>ORPHAN DRUG</b>	Sanofi Bridgewater, NJ	Stargardt disease <i>6 years and older</i>	Phase I/II <a href="http://www.sanofi.com">www.sanofi.com</a>
sarizotan (dopamine D2 receptor partial agonist, serotonin 1A receptor agonist) <b>ORPHAN DRUG</b>	Newron Pharmaceuticals US Morristown, NJ	Rett syndrome <i>4 years and older</i>	Phase II/III <a href="http://www.newron.com">www.newron.com</a>
SB-318 (gene transference) <b>ORPHAN DRUG</b>	Sangamo Therapeutics Brisbane, CA	mucopolysaccharidosis type I <i>5 years and older</i>	Phase I/II <a href="http://www.sangamo.com">www.sangamo.com</a>
SB-913 (gene transference) <b>ORPHAN DRUG</b>	Sangamo Therapeutics Brisbane, CA	mucopolysaccharidosis type II <i>5 years and older</i>	Phase I/II <a href="http://www.sangamo.com">www.sangamo.com</a>
selumetinib (MEK inhibitor) <b>ORPHAN DRUG</b>	AstraZeneca Wilmington, DE	neurofibromatosis type 1 (Breakthrough Therapy) <i>2 years and older</i>	application submitted <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
seprofarsen (QR-110) (RNA interference) <b>ORPHAN DRUG</b>	ProQR Therapeutics Cambridge, MA	Leber congenital amaurosis 10 (Fast Track) <i>8 years and older</i>	Phase II/III <a href="http://www.proqr.com">www.proqr.com</a>
setmelanotide (MC4R agonist) <b>ORPHAN DRUG</b>	Rhythm Pharmaceuticals Boston, MA	Alström syndrome, Bardet-Biedl syndrome, POMC deficiency obesity <i>6 years and older</i> LEPR deficiency obesity <i>12 years and older</i>	Phase III <a href="http://www.rhythmtx.com">www.rhythmtx.com</a>
		POMC epigenetic or heterozygous deficiency obesity <i>12 years and older</i>	Phase II/III <a href="http://www.rhythmtx.com">www.rhythmtx.com</a>
SGT-001 (AAV-based gene therapy) <b>ORPHAN DRUG</b>	Solid Biosciences Cambridge, MA	Duchenne muscular dystrophy (Fast Track) <i>4 years to 17 years</i>	Phase I/II <a href="http://www.solidbio.com">www.solidbio.com</a>
sirolimus topical ointment	Aucta Pharmaceuticals Piscataway, NJ	cutaneous angiofibromas in tuberous sclerosis complex <i>2 years to 18 years</i>	Phase II <a href="http://www.auctapharma.com">www.auctapharma.com</a>
SOBI003 (enzyme replacement therapy) <b>ORPHAN DRUG</b>	Swedish Orphan Biovitrum Waltham, MA	mucopolysaccharidosis IIIA (Fast Track) <i>12 months to 78 months</i>	Phase I/II <a href="http://www.sobi.com">www.sobi.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
SRK-015 (anti-promyostatin mAb) <b>ORPHAN DRUG</b>	Scholar Rock Cambridge, MA	spinal muscular atrophy, type 2 or 3 <i>2 years to 21 years</i>	Phase II <a href="http://www.scholarrock.com">www.scholarrock.com</a>
SRP-5051 (RNA-targeted therapy PPMO)	Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy <i>7 years to 21 years</i>	Phase II <a href="http://www.sarepta.com">www.sarepta.com</a>
SRP-9001 (gene therapy)	Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy <i>4 years to 7 years</i>	Phase II <a href="http://www.sarepta.com">www.sarepta.com</a>
SRP-9003 (AAV-Rh74 vectored gene therapy) <b>ORPHAN DRUG</b>	Sarepta Therapeutics Cambridge, MA	limb girdle muscular dystrophy-2E <i>4 years to 15 years</i>	Phase I/II <a href="http://www.sarepta.com">www.sarepta.com</a>
SRP-9004 (AAV gene therapy)	Sarepta Therapeutics Cambridge, MA	limb girdle muscular dystrophy type 2D <i>7 years and older</i>	Phase I/II completed <a href="http://www.sarepta.com">www.sarepta.com</a>
tadekinig alfa (recombinant IL-18)	AB2 Bio Lausanne, Switzerland	NLRC4-related macrophage activation syndrome, XIAP deficiency syndrome <i>up to 17 years</i>	Phase III <a href="http://www.ab2bio.com">www.ab2bio.com</a>
TAK611 (SHP611) (enzyme replacement therapy) <b>ORPHAN DRUG</b>	Takeda Deerfield, IL	late infantile metachromatic leukodystrophy <i>6 months to 72 months</i>	Phase II <a href="http://www.takeda.com">www.takeda.com</a>



## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
tralesinidase alfa (GILT rHNAGLU-GF2 fusion protein) <b>ORPHAN DRUG</b>	BioMarin Pharmaceutical San Rafael, CA	mucopolysaccharidosis type IIIB <i>1 year to 10 years</i>	Phase I/II <a href="http://www.biomarin.com">www.biomarin.com</a>
<b>Translarna®</b> ataluren <b>ORPHAN DRUG</b>	PTC Therapeutics South Plainfield, NJ	Duchenne muscular dystrophy (Fast Track) <i>5 years and older</i>	Phase III <a href="http://www.ptcbio.com">www.ptcbio.com</a>
		Duchenne muscular dystrophy <i>2 years to 5 years</i>	Phase II completed <a href="http://www.ptcbio.com">www.ptcbio.com</a>
		nonsense mutation aniridia <i>2 years and older</i>	Phase II <a href="http://www.ptcbio.com">www.ptcbio.com</a>
	PTC Therapeutics South Plainfield, NJ NYU Langone Health New York, New York	Dravet syndrome, CDKL5 deficiency <i>2 years to 12 years</i>	Phase II <a href="http://www.ptcbio.com">www.ptcbio.com</a>
trofinetide (NNZ-2566) <b>ORPHAN DRUG</b>	ACADIA Pharmaceuticals San Diego, CA Neuren Pharmaceuticals Camberwell, Australia	Rett syndrome (Fast Track) <i>5 years to 15 years</i>	Phase II completed <a href="http://www.acadia-pharm.com">www.acadia-pharm.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
UX007 (triheptanoin) (fatty acid triglyceride) <b>ORPHAN DRUG</b>	Ultragenyx Pharmaceutical Novato, CA	long-chain fatty acid oxidation disorders (Fast Track) <i>6 months and older</i>	application submitted <a href="http://www.ultragenyx.com">www.ultragenyx.com</a>
		glucose transporter type 1 deficiency syndrome <i>6 years and older</i>	Phase III <a href="http://www.ultragenyx.com">www.ultragenyx.com</a>
vamorolone (glucocorticoid receptor agonist) <b>ORPHAN DRUG</b>	ReveraGen BioPharma Rockville, MD	Duchenne muscular dystrophy (Fast Track) <i>4 years to 7 years</i>	Phase II <a href="http://www.reveragen.com">www.reveragen.com</a>
<b>Vonvendi®</b> vonicog alfa <b>ORPHAN DRUG</b>	Takeda Deerfield, IL	Von Willebrand disease <i>all ages</i>	Phase III <a href="http://www.takeda.com">www.takeda.com</a>
vosoritide (BMN 111) (natriuretic peptide stimulant) <b>ORPHAN DRUG</b>	BioMarin Pharmaceutical San Rafael, CA	achondroplasia <i>5 years to 18 years</i>	Phase III <a href="http://www.biomin.com">www.biomin.com</a>
		achondroplasia <i>up to 59 months</i>	Phase II <a href="http://www.biomin.com">www.biomin.com</a>
VTS-270 (adrabetadex) <b>ORPHAN DRUG</b>	Vtesse (Mallinckrodt) Cambridge, MA	Niemann-Pick disease type C <i>4 years to 21 years</i>	Phase II/III <a href="http://www.mallinckrodt.com">www.mallinckrodt.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
VX-371 (ENaC inhibitor)	Vertex Pharmaceuticals Boston, MA	cystic fibrosis (with Orkambi®) <i>12 years and older</i>	Phase II completed <a href="http://www.vrtx.com">www.vrtx.com</a>
		primary ciliary dyskineia (with Kalydeco®) <i>12 years and older</i>	Phase II completed <a href="http://www.vrtx.com">www.vrtx.com</a>
VX-440/ivacaftor/tezacaftor	Vertex Pharmaceuticals Boston, MA	cystic fibrosis <i>12 years and older</i>	Phase II completed <a href="http://www.vrtx.com">www.vrtx.com</a>
VX-445/ivacaftor/tezacaftor	Vertex Pharmaceuticals Boston, MA	cystic fibrosis (Breakthrough Therapy) <i>12 years and older</i>	application submitted <a href="http://www.vrtx.com">www.vrtx.com</a>
VX-659/ivacaftor/tezacaftor	Vertex Pharmaceuticals Boston, MA	cystic fibrosis <i>12 years and older</i>	Phase III <a href="http://www.vrtx.com">www.vrtx.com</a>
<b>Vyondys53™</b> golodirsen (SRP-4053) ORPHAN DRUG	Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy <i>children and adolescents</i>	application submitted <a href="http://www.sarepta.com">www.sarepta.com</a>
WVE-210201 (suvodirsen) (dystrophin expression modulator) ORPHAN DRUG	WaVe Life Sciences Cambridge, MA	Duchenne muscular dystrophy (Fast Track) <i>5 years to 12 years</i>	Phase II/III <a href="http://www.wavelifesciences.com">www.wavelifesciences.com</a>

## Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Zolgensma</b> <sup>®</sup> onasemnogene abeparvovec-xioi (intrathecal administration)	AveXis Bannockburn, IL	spinal muscular atrophy with 3 copies of SMN2 and deletion of SMN1 <i>6 months to 60 months</i>	Phase I <a href="http://www.avexis.com">www.avexis.com</a>
ZX008 (fenfluramine low dose) ORPHAN DRUG	Zogenix Emeryville, CA	Dravet syndrome (Fast Track) <i>2 years and older</i>	application submitted <a href="http://www.zogenix.com">www.zogenix.com</a>

## Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Ad26 ZEBOV vaccine/ MVA-BN Filo (prime-boost regimen)	Janssen Vaccines & Prevention Leiden, Netherlands Bavarian Nordic Morrisville, NC	Ebola virus infections <i>all ages</i>	Phase III <a href="http://www.janssen.com">www.janssen.com</a> <a href="http://www.bavarian-nordic.com">www.bavarian-nordic.com</a>
<b>AeroVanc</b> vancomycin dry powder inhalation ORPHAN DRUG	Savara Pharmaceuticals Austin, TX	methicillin-resistant <i>Staphylococcus aureus</i> infections in cystic fibrosis (Fast Track) <i>6 years and older</i>	Phase III <a href="http://www.savarapharma.com">www.savarapharma.com</a>
<b>Alinia</b> <sup>®</sup> nitazoxanide	Romark Laboratories Tampa, FL	enterovirus infections, rhinovirus infections, uncomplicated influenza <i>12 years and older</i>	Phase III <a href="http://www.romark.com">www.romark.com</a>
		gastroenteritis, norovirus infections <i>12 years and older</i>	Phase II <a href="http://www.romark.com">www.romark.com</a>

## Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Arikayce®</b> amikacin liposomal inhalation suspension ORPHAN DRUG	Insmed Bridgewater, NJ	<i>Pseudomonas aeruginosa</i> infections in cystic fibrosis patients <i>6 years and older</i>	Phase I/II completed <a href="http://www.insmed.com">www.insmed.com</a>
ARV-1801 (fusidic acid oral) ORPHAN DRUG	Arrevus Raleigh, NC	acute bacterial skin and skin structure infections <i>12 years and older</i>	Phase III completed <a href="http://www.arrevus.com">www.arrevus.com</a>
		staphylococcal bone or joint infections <i>12 years and older</i>	Phase II/III completed <a href="http://www.arrevus.com">www.arrevus.com</a>
<b>Bexsero®</b> meningococcal group B vaccine	GlaxoSmithKline Research Triangle Park, NC	prevention of meningococcal disease B caused by <i>Neisseria meningitidis</i> serogroup B <i>infants</i>	Phase III <a href="http://www.gsk.com">www.gsk.com</a>
<b>Biktarvy®</b> bictegravir/emtricitabine/tenofovir alafenamide	Gilead Sciences Foster City, CA	HIV-1 infections <i>2 years and older, 14kg to 25 kg</i>	Phase II/III <a href="http://www.gilead.com">www.gilead.com</a>
C16G2 (synthetic specifically-targeted antimicrobial peptide)	Armata Pharmaceuticals Marina del Ray, CA	dental caries (tooth decay) caused by <i>Streptococcus mutans</i> <i>12 years and older</i>	Phase II completed <a href="http://www.armatapharma.com">www.armatapharma.com</a>

## Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Cayston®</b> aztreonam inhalation	Gilead Sciences Foster City, NJ	<i>Pseudomonas aeruginosa</i> infections in cystic fibrosis <i>3 months to 17 years</i>	Phase III <a href="http://www.gilead.com">www.gilead.com</a>
<b>Contepo™</b> fosfomycin intravenous	Nabriva Therapeutics King of Prussia, PA	bacterial infections <i>up to 11 years</i>	Phase I <a href="http://www.nabriva.com">www.nabriva.com</a>
<b>Cresemba®</b> isavuconazonium sulfate	Astellas Pharma Northbrook, IL	invasive aspergillosis, invasive mucormycosis <i>1 year to 7 years</i>	Phase II <a href="http://www.astellas.com">www.astellas.com</a>
<b>Dalvance®</b> dalbavancin	Allergan Madison, NJ	acute bacterial skin and skin structure infections caused by gram- positive organisms, including MRSA <i>3 months to 17 years</i>	Phase III <a href="http://www.allergan.com">www.allergan.com</a>
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		bacterial infections <i>up to 28 days</i>	Phase I <a href="http://www.allergan.com">www.allergan.com</a>
DAS181 (virus internalization inhibitor) ORPHAN DRUG	Ansun Biopharma San Diego, CA	parainfluenza virus infections (Fast Track) <i>12 years and older</i>	Phase III <a href="http://www.ansunbiopharma.com">www.ansunbiopharma.com</a>
<b>Descovy®</b> emtricitabine/tenofovir alafenamide	Gilead Sciences Foster City, CA	HIV-1 infections <i>17kg to 25kg</i>	Phase II/III <a href="http://www.gilead.com">www.gilead.com</a>

## Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Dificid®</b> fidaxomicin	Merck Kenilworth, NJ	clostridium difficile infections <i>up to 17 years</i>	application submitted <a href="http://www.merck.com">www.merck.com</a>
dolutegravir/lamivudine/tenofovir disoproxil fumarate	Mylan Canonsburg, PA	HIV-1 infections <i>adolescents</i>	application submitted <a href="http://www.mylan.com">www.mylan.com</a>
<b>Dovato®</b> dolutegravir/lamivudine	ViiV Healthcare Research Triangle Park, NC	HIV-1 infections <i>12 years to 17 years</i>	Phase III <a href="http://www.viivhealthcare.com">www.viivhealthcare.com</a>
<b>Edurant®</b> rilpivirine	Janssen Research & Development Raritan, NJ	HIV-1 infections <i>2 years to 11 years</i>	Phase II <a href="http://www.janssen.com">www.janssen.com</a>
<b>Epclusa®</b> sofosbuvir/velpatasvir	Gilead Sciences Foster City, CA	hepatitis C infections <i>3 years to 17 years</i>	Phase II <a href="http://www.gilead.com">www.gilead.com</a>
<b>Eraxis®</b> anidulafungin	Pfizer New York, NY	invasive candidiasis <i>1 month to 17 years</i>	Phase III completed <a href="http://www.pfizer.com">www.pfizer.com</a>
<b>Fetroja®</b> cefiderocol	Shionogi Florham Park, NJ	gram-negative bacterial infections, complicated urinary tract infections <i>3 months to 17 years</i>	Phase II <a href="http://www.shionogi.com">www.shionogi.com</a>
gepoticidin (type II DNA topoisomerase inhibitor)	GlaxoSmithKline Research Triangle Park, NC	uncomplicated urinary tract infections, uncomplicated urogenital gonorrhea <i>12 years and older</i>	phase III <a href="http://www.gsk.com">www.gsk.com</a>

## Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
GS-6207 (capsid inhibitor)	Gilead Sciences Foster City, CA	HIV infections <i>12 years and older</i>	Phase II/III <a href="http://www.gilead.com">www.gilead.com</a>
GSK3389245A (replication-defective recombinant viral vector vaccine)	GlaxoSmithKline Research Triangle Park, NC	respiratory syncytial virus infections <i>12 months to 23 months</i>	Phase II <a href="http://www.gsk.com">www.gsk.com</a>
		respiratory syncytial virus infections <i>6 months to 7 months</i>	Phase I <a href="http://www.gsk.com">www.gsk.com</a>
GSK3536819A (Men ABCWY) (recombinant conjugated vaccine)	GlaxoSmithKline Research Triangle Park, NC	prevention of meningococcal ABCWY <i>10 years to 17 years</i>	Phase II completed <a href="http://www.gsk.com">www.gsk.com</a>
<b>Harvoni</b> <sup>®</sup> ledipasvir/sofosbuvir	Gilead Sciences Foster City, CA	hepatitis C infections <i>3 years to 17 years</i>	Phase II completed <a href="http://www.gilead.com">www.gilead.com</a>
IBP-9414 (bacteria replacement) ORPHAN DRUG	Infant Bacterial Therapeutics Stockholm, Sweden	necrotizing enterocolitis <i>up to 48 hours</i>	Phase III <a href="http://www.ibtherapeutics.com">www.ibtherapeutics.com</a>
ibrexafungerp (glucan synthase inhibitor) ORPHAN DRUG	SCYNEXIS Jersey City, NJ	vulvovaginal candidiasis (Fast Track) <i>12 years and older</i>	Phase III <a href="http://www.scynexis.com">www.scynexis.com</a>



## Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Infanrix hexa</b> Hib-DTaP-hepatitis B-poliovirus vaccine	GlaxoSmithKline Research Triangle Park, NC	prevention of Hib-DTaP-hepatitis B and poliovirus <i>6 weeks to 12 weeks</i>	Phase III completed <a href="http://www.gsk.com">www.gsk.com</a>
JNJ-53718678 (fusion protein inhibitor)	Janssen Research & Development Raritan, NJ	respiratory syncytial virus infections <i>up to 3 years</i> respiratory syncytial virus infections <i>13 years and older</i>	Phase II <a href="http://www.janssen.com">www.janssen.com</a>
JNJ-64400141 (pre-fusion F protein vaccine)	Janssen Research & Development Raritan, NJ	respiratory syncytial virus infection in RSV-seropositive toddlers <i>12 months to 24 months</i>	Phase I/II <a href="http://www.janssen.com">www.janssen.com</a>
M2SR (influenza vaccine)	FluGen Madison, WI	seasonal influenza <i>9 years to 17 years</i>	Phase I <a href="http://www.flugen.com">www.flugen.com</a>
<b>Mavyret®</b> glecaprevir/pibrentasvir	AbbVie North Chicago, IL	hepatitis C infections <i>3 years to 17 years</i>	Phase III <a href="http://www.abbvie.com">www.abbvie.com</a>
MEDI-8897 (RSV mAb)	MedImmune Gaithersburg, MD	prevention of respiratory syncytial virus infections (Fast Track) (Breakthrough Therapy) <i>up to 365 days</i>	Phase II/III <a href="http://www.medimmune.com">www.medimmune.com</a>

## Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>MenQuadfi™</b> advanced generation meningococcal ACYW conjugate vaccine	Sanofi Bridgewater, NJ	prevention of meningococcal ACWY <i>2 years and older</i>	application submitted <a href="http://www.sanofi.com">www.sanofi.com</a>
<b>Menveo® Liquid</b> (conjugated vaccine)	GlaxoSmithKline Research Triangle Park, NC	prevention of meningococcal ACWY <i>10 years to 17 years</i>	Phase II <a href="http://www.gsk.com">www.gsk.com</a>
miconazole	Hill Dermaceuticals Sanford, FL	otomycosis <i>25 months and older</i>	Phase II <a href="http://www.hillderm.com">www.hillderm.com</a>
<b>Mino-Lok®</b> disodium edetate/ethyl alcohol/ minocycline	Citius Pharmaceuticals Cranford, NJ	catheter-related infections (Fast Track) <i>12 years and older</i>	Phase III <a href="http://www.citiuspharma.com">www.citiuspharma.com</a>
MK-1654 (RSV F mAb)	Merck Kenilworth, NJ	respiratory syncytial virus infections <i>up to 8 months</i>	Phase I/II <a href="http://www.merck.com">www.merck.com</a>
MOB015B (terbinafine topical)	Moberg Pharma North America Cedar Knolls, NJ	onychomycosis <i>12 years and older</i>	Phase III <a href="http://www.mobergpharma.com">www.mobergpharma.com</a>
mRNA-1653 (RNA vaccine)	Moderna Therapeutics Cambridge, MA	metapneumovirus infections, parainfluenza infections <i>12 months and older</i>	Phase I <a href="http://www.modernatx.com">www.modernatx.com</a>
<b>Natroba®</b> spinosad	ParaPRO Carmel, IN	scabies <i>4 years and older</i>	Phase III completed <a href="http://www.parapro.com">www.parapro.com</a>

## Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
OP0201 (cholesteryl-palmitate/dipalmitoyl-phosphatidylcholine)	Novus Therapeutics Irvine, CA	acute otitis media <i>6 months to 24 months</i>	Phase II <a href="http://www.novustherapeutics.com">www.novustherapeutics.com</a>
<b>Orbactiv™</b> oritavancin	Melinta Therapeutics Morristown, NJ	bacterial infections <i>up to 18 years</i>	Phase I <a href="http://www.melinta.com">www.melinta.com</a>
PF-06482077 (multivalent pneumococcal vaccine)	Pfizer New York, NY	invasive and non-invasive pneumococcal infections (Breakthrough Therapy) <i>42 days to 98 days</i>	Phase II <a href="http://www.pfizer.com">www.pfizer.com</a>
pimodivir (JNJ-63623872) (viral protein inhibitor)	Janssen Research & Development Raritan, NJ	influenza A virus infections (Fast Track) <i>13 years and older</i>	Phase III <a href="http://www.janssen.com">www.janssen.com</a>
<b>Prevymis®</b> letermovir	Merck Kenilworth, NJ	cytomegalovirus infections following stem cell transplantation <i>up to 17 years</i>	Phase II <a href="http://www.merck.com">www.merck.com</a>
<b>Prezcobix®</b> cobicistat/darunavir FDC tablet	Janssen Research & Development Raritan, NJ	HIV-1 infections <i>12 years to 17 years</i>	Phase I <a href="http://www.janssen.com">www.janssen.com</a>
quadrivalent influenza vaccine	Seqirus Summit, NJ	influenza <i>6 months to 59 months</i>	Phase III completed <a href="http://www.seqirus.com">www.seqirus.com</a>

## Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Recarbrio®</b> imipenem, cilastatin, relebactam	Merck Kenilworth, NJ	gram-negative bacterial infections <i>up to 17 years</i>	Phase II/III <a href="http://www.merck.com">www.merck.com</a>
<b>ResVax</b> RSV F vaccine	Novavax Gaithersburg, MD	respiratory syncytial virus infections <i>6 months to 5 years</i>	Phase I completed <a href="http://www.novavax.com">www.novavax.com</a>
RSV vaccine	Sanofi Bridgewater, NJ	respiratory syncytial virus infections <i>4 months and older</i>	Phase I <a href="http://www.sanofi.com">www.sanofi.com</a>
SB206 (nitric oxide releasing topical gel)	Novan Morrisville, NC	molluscum contagiosum <i>6 months and older</i>	Phase III <a href="http://www.novan.com">www.novan.com</a>
<b>Shan 6</b> DTP-HepB-polio-Hib hexavalent vaccine	Sanofi Bridgewater, NJ	DTP-HepB-polio-Hib prevention <i>pediatric</i>	Phase III <a href="http://www.sanofi.com">www.sanofi.com</a>
<b>Sivextro®</b> tedizolid	Merck Kenilworth, NJ	acute bacterial skin and skin structure infections <i>up to 11 years</i>	Phase III <a href="http://www.merck.com">www.merck.com</a>
		gram-positive infections <i>up to 24 months</i>	Phase I <a href="http://www.merck.com">www.merck.com</a>
<b>Sovaldi®</b> sofosbuvir	Gilead Sciences Foster City, CA	hepatitis C infections <i>3 years to 17 years</i>	Phase II <a href="http://www.gilead.com">www.gilead.com</a>

## Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
TAK620 (SHP620) (UL97 kinase inhibitor) ORPHAN DRUG	Takeda Deerfield, IL	cytomegalovirus infections (Fast Track) <i>12 years and older</i>	Phase III <a href="http://www.takeda.com">www.takeda.com</a>
<b>Technivie®</b> ombitasvir/paritaprevir/ritonavir	AbbVie North Chicago, IL	hepatitis C infections genotype 1 or 4 <i>3 years to 17 years</i>	Phase II <a href="http://www.abbvie.com">www.abbvie.com</a>
terconazole/metronidazole fixed-dose combination	Curatek Pharmaceuticals Elk Grove Village, IL	vaginitis <i>12 years and older</i>	Phase III competed
<b>Tivicay®</b> dolutegravir 5mg dispersible tablet	Viiv Healthcare Research Triangle Park, NC	HIV infections <i>pediatric</i>	application submitted <a href="http://www.viivhealthcare.com">www.viivhealthcare.com</a>
V114 (pneumoconjugate vaccine)	Merck Kenilworth, NJ	pneumococcal disease <i>42 days and older</i>	Phase III <a href="http://www.merck.com">www.merck.com</a>
<b>Vabomere™</b> meropenem and vaborbactam	Melinta Therapeutics Morristown, NJ	bacterial infections <i>up to 17 years</i>	Phase I <a href="http://www.melinta.com">www.melinta.com</a>
VBP-245 (povidone iodine topical)	Veloce BioPharma Fort Lauderdale, FL	molluscum contagiosum <i>2 years to 18 years</i>	Phase II completed <a href="http://www.velocebiopharma.com">www.velocebiopharma.com</a>
<b>Vemlidy®</b> tenofovir alafenamide	Gilead Sciences Foster City, CA	hepatitis B infections <i>2 years to 17 years</i>	Phase II <a href="http://www.gilead.com">www.gilead.com</a>

## Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Viekira Pak®</b> ombitasvir, paritaprevir, ritonavir and dasabuvir <b>ORPHAN DRUG</b>	AbbVie North Chicago, IL	hepatitis C infections <i>3 years to 17 years</i>	Phase II <a href="http://www.abbvie.com">www.abbvie.com</a>
<b>Viekira XR®</b> dasabuvir, ombitasvir, paritaprevir, and ritonavir	AbbVie North Chicago, IL	hepatitis C infections <i>3 years to 17 years</i>	Phase II <a href="http://www.abbvie.com">www.abbvie.com</a>
VP-102 (cantharidin)	Verrica Pharmaceuticals West Chester, PA	molluscum contagiosum <i>2 years and older</i>	application submitted <a href="http://www.verriva.com">www.verriva.com</a>
VRC01LS (CD4 antigen inhibitor)	Xencor Monrovia, CA National Institutes of Health Rockville, MD	HIV infections <i>all ages</i>	Phase I <a href="http://www.xencor.com">www.xencor.com</a>
VT-1161 (otesaconazole)	Mycovia Pharmaceuticals Durham, NC	recurrent vulvovaginal candidiasis (Fast Track) <i>12 years and older</i>	Phase III <a href="http://www.mycovia.com">www.mycovia.com</a>
<b>Xerava™</b> eravacycline	Tetraphase Pharmaceuticals Watertown, MA	bacterial infections <i>8 years to 17 years</i>	Phase I <a href="http://www.tphase.com">www.tphase.com</a>

## Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Xofluza®</b> baloxavir marboxil	Genentech South San Francisco, CA	influenza <i>1 year to 12 years</i>	Phase III completed <a href="http://www.gene.com">www.gene.com</a>
		influenza <i>up to 1 year</i>	Phase III <a href="http://www.gene.com">www.gene.com</a>
<b>Zepatier®</b> elbasvir/grazoprevir	Merck Kenilworth, NJ	hepatitis C infections <i>3 years to 17 years</i>	Phase II <a href="http://www.merck.com">www.merck.com</a>
<b>Zerbaxa®</b> ceftolozane/tazobactam	Merck Kenilworth, NJ	complicated intra-abdominal infections, complicated urinary tract infections <i>up to 17 years</i>	Phase II <a href="http://www.merck.com">www.merck.com</a>
<b>Zinplava®</b> bezlotoxumab	Merck Kenilworth, NJ	<i>Clostridium difficile</i> infections <i>1 year to 17 years</i>	Phase III <a href="http://www.merck.com">www.merck.com</a>
zoliflodacin (antibiotic/DNA gyrase inhibitor)	Entasis Therapeutics Waltham, MA	uncomplicated gonorrhea (Fast Track)	Phase III <a href="http://www.entasistx.com">www.entasistx.com</a>

## Kidney Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ADV7103 (potassium bicarbonate and potassium citrate)	Advicenne Pharma Nimes, France	distal renal tubular acidosis <i>4 months and older</i>	Phase III <a href="http://www.advicenne.com">www.advicenne.com</a>
ALXN1210 (ravulizumab-cwvz) ORPHAN DRUG	Alexion Pharmaceuticals Boston, MA	atypical hemolytic uremic syndrome <i>up to 17 years</i>	Phase III <a href="http://www.alexion.com">www.alexion.com</a>
avacopan (complement C5a receptor inhibitor) ORPHAN DRUG	ChemoCentryx Mountain View, CA	C3 glomerulopathy <i>12 years and older</i>	Phase II <a href="http://www.chemocentryx.com">www.chemocentryx.com</a>
DCR-PHXC (RNA interference) ORPHAN DRUG	Dicerna Pharmaceuticals Cambridge, MA	primary hyperoxaluria <i>6 years and older</i>	Phase II <a href="http://www.dicerna.com">www.dicerna.com</a>
<b>Hectorol®</b> doxercalciferol capsules	Sanofi Bridgewater, NJ	secondary hyperparathyroidism in chronic kidney disease <i>5 years to 18 years</i>	Phase III <a href="http://www.sanofi.com">www.sanofi.com</a>
lixivaptan (vasopressin V2 receptor antagonist) ORPHAN DRUG	Palladio Biosciences Newtown, NY	autosomal recessive polycystic kidney disease <i>15 years and older</i>	Phase II <a href="http://www.palladiobio.com">www.palladiobio.com</a>



## Kidney Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
lumasiran (RNA interference) <b>ORPHAN DRUG</b>	Alnylam Pharmaceuticals Cambridge, MA	primary hyperoxaluria <i>6 years and older</i> primary hyperoxaluria <i>up to 5 years</i>	Phase III <a href="http://www.alnylam.com">www.alnylam.com</a>
narsoplimab (OMS721) (ASP2 protein inhibitor)	Omeros Corporation Seattle, WA	atypical hemolytic uremic syndrome (Fast Track) <i>12 years and older</i>	Phase III <a href="http://www.omeros.com">www.omeros.com</a>
<b>Orencia®</b> abatacept	Bristol-Myers Squibb Princeton, NJ	nephrotic syndrome due to focal segmental glomerulosclerosis or minimal change disease <i>6 years and older</i>	Phase II <a href="http://www.bms.com">www.bms.com</a>
<b>Oxabact®</b> lyophilised oxalobacter formigenes <b>ORPHAN DRUG</b>	OxThera Knoxville, TN	primary hyperoxaluria <i>2 years and older</i>	Phase III <a href="http://www.oxthera.com">www.oxthera.com</a>
<b>Parsabiv®</b> etelcalcetide	Amgen Thousand Oaks, CA	secondary hyperparathyroidism and chronic kidney disease <i>up to 18 years</i>	Phase III <a href="http://www.amgen.com">www.amgen.com</a>
reloxaliase (ALLN-177) (recombinant oxalate decarboxylase enzyme) <b>ORPHAN DRUG</b>	Allena Pharmaceuticals Newton, MA	enteric or primary hyperoxaluria and hyperoxalemia <i>12 years and older</i>	Phase II <a href="http://www.allenapharm.com">www.allenapharm.com</a>

## Kidney Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
sparsentan (RE-021) (DARA receptor antagonist) <b>ORPHAN DRUG</b>	Retrophin San Diego, CA	focal segmental glomerulosclerosis <i>8 years and older</i>	Phase III <a href="http://www.retrophin.com">www.retrophin.com</a>
tesevatinib (tyrosine kinase inhibitor) <b>ORPHAN DRUG</b>	Kadmon Pharmaceuticals New York, NY	autosomal recessive polycystic kidney disease <i>5 years to 12 years</i>	Phase I <a href="http://www.kadmon.com">www.kadmon.com</a>

## Liver Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Defitelio</b> <sup>®</sup> defibrotide <b>ORPHAN DRUG</b>	Jazz Pharmaceuticals Dublin, Ireland	prevention of hepatic veno-occlusive disease <i>1 month and older</i>	Phase III <a href="http://www.jazzpharma.com">www.jazzpharma.com</a>
elafibranor (PPAR alpha/delta receptor agonist)	Genfit Loos, France	non-alcoholic steatohepatitis (Fast Track) <i>8 years to 18 years</i>	Phase II <a href="http://www.genfit.com">www.genfit.com</a>
givosiran (5 aminolevulinate synthetase inhibitor conjugated to small- interfering RNA) <b>ORPHAN DRUG</b>	Alnylam Pharmaceuticals Cambridge, MA	acute hepatic porphyria (Breakthrough Therapy) <i>12 years and older</i>	application submitted <a href="http://www.alnylam.com">www.alnylam.com</a>

## Liver Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
IMM-124E (immunomodulator)	Immuron Blackburn North, Australia National Institute of Health Bethesda, MD	non-alcoholic fatty liver disease <i>6 years to 17 years</i>	Phase II <a href="http://www.immuron.com.au">www.immuron.com.au</a>
maralixibat (sodium-bile acid cotransporter inhibitor) ORPHAN DRUG	Mirum Pharmaceuticals Foster City, CA	progressive familial intrahepatic cholestasis <i>1 year to 17 years</i>	Phase III <a href="http://www.mirumpharma.com">www.mirumpharma.com</a>
odevixibat (A4250) (sodium-bile acid cotransporter inhibitor) ORPHAN DRUG	Albireo Pharma Boston, MA	intrahepatic cholestasis (Fast Track) <i>6 months to 18 years</i>	Phase III <a href="http://www.albireopharma.com">www.albireopharma.com</a>

## Mental Illness

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AR20	Arbor Pharmaceuticals Atlanta, GA	mental disorders <i>pediatric</i>	Phase I <a href="http://www.arborpharma.com">www.arborpharma.com</a>
AR33	Arbor Pharmaceuticals Atlanta, GA	mental disorders <i>pediatric</i>	Phase II <a href="http://www.arborpharma.com">www.arborpharma.com</a>

## Mental Illness

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
BI 409306 (PDE9A inhibitor)	Boehringer Ingelheim Ridgefield, CT	psychotic disorders <i>16 years and older</i>	Phase II <a href="http://www.boehringer-ingelheim.com">www.boehringer-ingelheim.com</a>
dasotraline (serotonin, norepinephrine and dopamine reuptake inhibitor)	Sunovion Marlborough, MA	attention-deficit/hyperactivity disorder (ADHD) <i>children and adolescents</i>	application submitted <a href="http://www.sunovion.com">www.sunovion.com</a>
<b>Fetzima</b> <sup>®</sup> levomilnacipran ER	Allergan Madison, NJ	major depressive disorder <i>7 years to 17 years</i>	Phase III <a href="http://www.allergan.com">www.allergan.com</a>
<b>Geodon</b> <sup>®</sup> ziprasidone	Pfizer New York, NY	bipolar 1 disorder <i>10 years to 17 years</i>	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
HLD-100 (dextroamphetamine sulfate modified release)	Ironshore Pharmaceuticals Durham, NC	ADHD <i>6 years to 12 years</i>	Phase II completed <a href="http://www.ironshorepharma.com">www.ironshorepharma.com</a>
KP415 (serdexmethylphenidate)	KemPharm Celebration, FL	ADHD <i>6 years to 12 years</i>	Phase III <a href="http://www.kempharm.com">www.kempharm.com</a>
KP484 (serdexmethylphenidate)	KemPharm Celebration, FL	ADHD <i>6 years to 12 years</i>	Phase III <a href="http://www.kempharm.com">www.kempharm.com</a>
<b>Lexapro</b> <sup>®</sup> escitalopram	Allergan Madison, NJ	generalized anxiety disorder <i>7 years to 17 years</i>	Phase III <a href="http://www.allergan.com">www.allergan.com</a>

## Mental Illness

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>NaBen®</b> sodium benzoate ORPHAN DRUG	SyneuRx New Taipei City, Taiwan	schizophrenia <i>12 years to 17 years</i>	Phase II/III <a href="http://www.syneurx.com">www.syneurx.com</a>
<b>Rexulti®</b> brexpiprazole	Lundbeck Deerfield, IL Otsuka Pharmaceutical Rockville, MD	schizophrenia <i>13 years to 17 years</i>	Phase III <a href="http://www.lundbeck.com">www.lundbeck.com</a> <a href="http://www.otsuka.com">www.otsuka.com</a>
SEP-363856 (serotonin 1-A agonist/ TARR1 agonist)	Sunovion Marlborough, MA	schizophrenia <i>13 years and older</i>	Phase III <a href="http://www.sunovion.com">www.sunovion.com</a>
SPN-810 (molindone)	Supernus Pharmaceuticals Rockville, MD	impulse aggression in ADHD (Fast Track) <i>6 years to 17 years</i>	Phase III <a href="http://www.supernus.com">www.supernus.com</a>
SPN-812 (viloxazine)	Supernus Pharmaceuticals Rockville, MD	ADHD <i>6 years to 17 years</i>	application submitted <a href="http://www.supernus.com">www.supernus.com</a>
<b>Spravato®</b> esketamine	Janssen Research & Development Raritan, NJ	major depressive disorder <i>12 years to 17 years</i>	Phase II <a href="http://www.janssen.com">www.janssen.com</a>
<b>Trintellix®</b> vortioxetine	Lundbeck Deerfield, IL Takeda Deerfield, IL	major depressive disorder <i>7 years to 11 years</i>	Phase III <a href="http://www.lundbeck.com">www.lundbeck.com</a> <a href="http://www.takeda.com">www.takeda.com</a>

## Mental Illness

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
TRN-110	Tris Pharma Monmouth Junction, NJ	ADHD <i>pediatric</i>	in clinical trials <a href="http://www.trispharma.com">www.trispharma.com</a>
TV-46000 (extended release injectable suspension risperidone)	Teva Pharmaceuticals North Wales, PA	schizophrenia <i>13 years and older</i>	Phase III <a href="http://www.tevausea.com">www.tevausea.com</a>
<b>Viiibryd</b> <sup>®</sup> vilazodone	Allergan Madison, NJ	major depressive disorder <i>7 years to 17 years</i>	Phase III completed <a href="http://www.allergan.com">www.allergan.com</a>
<b>Vraylar</b> <sup>®</sup> cariprazine	Allergan Madison, NJ	schizophrenia <i>13 years to 17 years</i>	Phase III <a href="http://www.allergan.com">www.allergan.com</a>
<b>Vyvanse</b> <sup>®</sup> lisdexamfetamine	Takeda Deerfield, IL	ADHD <i>4 years to 5 years</i>	Phase III completed <a href="http://www.takeda.com">www.takeda.com</a>
<b>Zulresso</b> <sup>™</sup> brexanolone	Sage Therapeutics Cambridge, MA	postpartum depression <i>15 years to 17 years</i>	Phase III <a href="http://www.sagerx.com">www.sagerx.com</a>

## Neurologic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ABI-009 (mTOR inhibitor)	AADi Pacific Palisades, CA	Leigh syndrome <i>2 years to 17 years</i>	Phase II <a href="http://www.aadibio.com">www.aadibio.com</a>
		surgically refractory epilepsy <i>3 years to 26 years</i>	Phase I <a href="http://www.aadibio.com">www.aadibio.com</a>
<b>Aimovig</b> <sup>®</sup> erenumab	Amgen Thousand Oaks, CA Novartis East Hanover, NJ	chronic and episodic migraine <i>6 years to 17 years</i>	Phase III <a href="http://www.amgen.com">www.amgen.com</a> <a href="http://www.novartis.com">www.novartis.com</a>
<b>Aubagio</b> <sup>®</sup> teriflunomide	Sanofi Genzyme Cambridge, MA	relapsing multiple sclerosis <i>10 years to 18 years</i>	Phase III <a href="http://www.sanofigenzyme.com">www.sanofigenzyme.com</a>
AUC025 (lamotrigine oral liquid)	Aucta Pharmaceuticals Piscataway, NJ Eton Pharmaceuticals Deer Park, IL	partial onset seizures, generalized tonic-clonic seizures, Lennox-Gastaut syndrome <i>2 years and older</i>	application submitted <a href="http://www.auctapharma.com">www.auctapharma.com</a> <a href="http://www.etonpharma.com">www.etonpharma.com</a>
<b>Austedo</b> <sup>®</sup> deutetrabenazine ORPHAN DRUG	Teva Pharmaceuticals USA Parsippany, NJ	dyskinesia in cerebral palsy <i>6 years to 18 years</i> tics in Tourette syndrome <i>6 years to 17 years</i>	Phase III <a href="http://www.tevausa.com">www.tevausa.com</a>

## Neurologic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Botox®</b> onabotulinumtoxin A	Allergan Madison, NJ	urinary incontinence due to neurogenic detrusor overactivity <i>5 years to 17 years</i>	Phase III <a href="http://www.allergan.com">www.allergan.com</a>
<b>Briviact®</b> brivaracetam intravenous	UCB Pharma Smyrna, GA	epilepsy <i>1 month to 16 years</i>	Phase II <a href="http://www.ucb.com">www.ucb.com</a>
cannabidiol oral solution <b>ORPHAN DRUG</b>	INSYS Therapeutics Chandler, AZ	infantile spasm <i>1 month to 24 months</i>	Phase III <a href="http://www.insysrx.com">www.insysrx.com</a>
		childhood absence seizures <i>3 years to 17 years</i>	Phase II <a href="http://www.insysrx.com">www.insysrx.com</a>
CE-fosphenytoin (sodium channel antagonist)	Sedor Pharmaceuticals Paoli, PA	status epilepticus <i>pediatric</i>	application submitted <a href="http://www.sedorpharmaceuticals.com">www.sedorpharmaceuticals.com</a>
CEVA101 (cell replacement therapy)	Cellvation New York, NY Fortress Biotech New York, NY	traumatic brain injury <i>5 years to 17 years</i>	Phase I/II <a href="http://www.cellvation.com">www.cellvation.com</a>
CX-8998 (calcium channel antagonist)	Cavion Charlottesville, VA	epilepsy <i>16 years and older</i>	Phase II <a href="http://www.cavionpharma.com">www.cavionpharma.com</a>



## Neurologic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ecopipam (dopamine D1 receptor antagonist)	Emalex Biosciences Northbrook, IL	Tourette's syndrome (Fast Track) <i>6 years to 17 years</i>	Phase II <a href="http://www.emalexbiosciences.com">www.emalexbiosciences.com</a>
<b>Emgality</b> <sup>®</sup> galcanezumab-gnlm	Eli Lilly Indianapolis, IN	episodic migraine <i>6 years to 17 years</i>	Phase III <a href="http://www.lilly.com">www.lilly.com</a>
EPI-743 (NQO1 modulator) ORPHAN DRUG	BioElectron Technology Mountain View, CA	Leigh syndrome <i>1 year to 18 years</i>	Phase II <a href="http://www.bioelectron.com">www.bioelectron.com</a>
<b>Epidiolex</b> <sup>®</sup> cannabidiol ORPHAN DRUG	Greenwich Biosciences Carlsbad, CA	infantile spasms <i>1 month to 24 months</i>	Phase III <a href="http://www.greenwichbiosciences.com">www.greenwichbiosciences.com</a>
fosmetpantotenate (RE-024) (replacement therapy) ORPHAN DRUG	Retrophin San Diego, CA	pantothenate kinase-associated neurodegeneration (Fast Track) <i>6 years and older</i>	Phase III <a href="http://www.retrophin.com">www.retrophin.com</a>
<b>Fycompa</b> <sup>®</sup> perampanel	Eisai Woodcliff Lake, NJ	Lennox-Gastaut syndrome <i>2 years and older</i> tonic clonic seizures <i>4 years to 12 years</i>	Phase III <a href="http://www.eisai.com">www.eisai.com</a>
		epilepsy <i>1 month to 24 months</i> epileptic syndrome, partial onset seizures <i>1 month to 18 years</i>	Phase II <a href="http://www.eisai.com">www.eisai.com</a>

## Neurologic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ganaxolone (GABA A receptor agonist) ORPHAN DRUG	Marinus Pharmaceuticals Radnor, PA	refractory status epilepticus <i>12 years and older</i>	Phase II <a href="http://www.marinuspharma.com">www.marinuspharma.com</a>
lasmiditan (serotonin 1F receptor agonist)	Eli Lilly Indianapolis, IN	migraine <i>6 years to 17 years</i>	Phase I <a href="http://www.lilly.com">www.lilly.com</a>
<b>Lemtrada</b> <sup>®</sup> alemtuzumab	Sanofi Genzyme Cambridge, MA	relapsing-remitting multiple sclerosis <i>10 years to 17 years</i>	Phase III <a href="http://www.sanofigenzyme.com">www.sanofigenzyme.com</a>
<b>Libervant</b> <sup>™</sup> diazepam buccal soluble film ORPHAN DRUG	Aquestive Therapeutics Warren, NJ	acute repetitive seizures (Fast Track) <i>2 years and older</i>	application submitted <a href="http://www.aquestive.com">www.aquestive.com</a>
		epilepsy <i>2 years to 17 years</i>	Phase II <a href="http://www.aquestive.com">www.aquestive.com</a>
<b>Lyrica</b> <sup>®</sup> pregabalin	Pfizer New York, NY	generalized tonic-clonic seizures <i>5 years and older</i>	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
<b>Neupro</b> <sup>®</sup> rotigotine transdermal patch	UCB Smyrna, GA	restless legs syndrome <i>13 years to 17 years</i>	Phase III <a href="http://www.ucb.com">www.ucb.com</a>
<b>ONZETRA</b> <sup>®</sup> Xsail <sup>®</sup> sumatriptan nasal powder	Avanir Pharmaceuticals Aliso Viejo, CA	migraine <i>12 years to 17 years</i>	Phase III <a href="http://www.avanir.com">www.avanir.com</a>

## Neurologic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Plegridy®</b> peginterferon beta-1a	Biogen Cambridge, MA	relapsing-remitting multiple sclerosis <i>10 years to 17 years</i>	Phase III <a href="http://www.biogen.com">www.biogen.com</a>
RT001 (lipid peroxidation inhibitor) ORPHAN DRUG	Retrotope Los Altos, CA	infantile neuroaxonal dystrophy <i>18 months to 10 years</i>	Phase II/III <a href="http://www.retrotope.com">www.retrotope.com</a>
TAK935 (OV935) (CH24H inhibitor) ORPHAN DRUG	Ovid Therapeutics New York, NY Takeda Deerfield, IL	epilepsy, Lennox-Gastaut syndrome <i>2 years to 17 years</i>	Phase II <a href="http://www.ovidrx.com">www.ovidrx.com</a> <a href="http://www.takeda.com">www.takeda.com</a>
<b>Tecfidera®</b> dimethyl fumarate	Biogen Cambridge, MA	relapsing-remitting multiple sclerosis <i>10 years to 17 years</i>	Phase III <a href="http://www.biogen.com">www.biogen.com</a>
<b>Toviaz®</b> fesoterodine	Pfizer New York, NY	neurogenic overactive bladder <i>6 years to 17 years</i>	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
TRN-146	Tris Pharma Monmouth Junction, NJ	CNS disorders <i>pediatric</i>	in clinical trials <a href="http://www.trispharma.com">www.trispharma.com</a>
TRN-148	Tris Pharma Monmouth Junction, NJ	CNS disorders <i>pediatric</i>	in clinical trials <a href="http://www.trispharma.com">www.trispharma.com</a>

## Neurologic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Valtoco™</b> diazepam nasal spray ORPHAN DRUG	Neurelis San Diego, CA	acute repetitive seizures (Fast Track) <i>6 years and older</i>	application submitted <a href="http://www.neurelis.com">www.neurelis.com</a>
<b>VESicare®</b> solifenacin	Astellas Pharma US Northbrook, IL	neurogenic detrusor overactivity <i>pediatric</i>	application submitted <a href="http://www.astellas.com">www.astellas.com</a>
<b>Vimpat®</b> lacosamide	UCB Atlanta, GA	partial onset seizures <i>1 month to 47 months</i> idiopathic generalized epilepsy, tonic clonic seizures <i>4 years and older</i>	Phase III <a href="http://www.ucb.com">www.ucb.com</a>
		epilepsy (intravenous) <i>1 month to 16 years</i>	Phase II/III completed <a href="http://www.ucb.com">www.ucb.com</a>
<b>Xeomin®</b> incobotulinumtoxinA	Merz North America Raleigh, NC	spasticity in cerebral palsy <i>2 years to 17 years</i>	Phase III completed <a href="http://www.merzusa.com">www.merzusa.com</a>
<b>Zomig®</b> zolmitriptan nasal spray	Amneal Pharmaceuticals Bridgewater, NJ	migraine <i>6 years to 11 years</i>	Phase III <a href="http://www.impaklabs.com">www.impaklabs.com</a>
ZX008 (fenfluramine low dose) ORPHAN DRUG	Zogenix Emeryville, CA	Lennox Gastaut syndrome <i>2 years and older</i>	Phase III <a href="http://www.zogenix.com">www.zogenix.com</a>

## Respiratory Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Aerosurf®</b> lucinactant for inhalation-aerosolized KL4 surfactant ORPHAN DRUG	Wintree Therapeutics Warrington, PA	neonatal respiratory distress syndrome (Fast Track) <i>26 weeks to 36 weeks</i>	Phase II <a href="http://www.windtreetx.com">www.windtreetx.com</a>
<b>AirDuo® Digihaler™</b> salmeterol/fluticasone propionate	Teva North America North Wales, PA	asthma <i>4 years to 11 years</i>	Phase III completed <a href="http://www.tevapharm.com">www.tevapharm.com</a>
<b>AirDuo™ RespiClick®</b> fluticasone propionate and salmeterol inhalation powder	Teva Pharmaceutical North Wales, PA	asthma <i>4 years to 11 years</i>	Phase III completed <a href="http://www.tevapharm.com">www.tevapharm.com</a>
<b>ArmonAir™ RespiClick®</b> fluticasone propionate inhalation powder	Teva Pharmaceutical North Wales, PA	asthma <i>4 years to 11 years</i>	Phase III completed <a href="http://www.tevapharm.com">www.tevapharm.com</a>
AZD7594 (inhaled SGRM)	AstraZeneca Wilmington, DE	asthma <i>12 years to 17 years</i>	Phase I <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
<b>Breo Ellipta®</b> fluticasone furoate/vilanterol	GlaxoSmithKline Research Triangle Park, NC	asthma <i>5 years to 17 years</i>	Phase III <a href="http://www.gsk.com">www.gsk.com</a>
<b>Cinqair®</b> reslizumab	Teva Pharmaceuticals North Wales, PA	asthma <i>12 years and older</i>	Phase III completed <a href="http://www.tevausa.com">www.tevausa.com</a>

## Respiratory Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Dupixent®</b> dupilumab	Regeneron Pharmaceuticals Tarrytown, NY Sanofi Bridgewater, NJ	asthma <i>6 years to 11 years</i>	Phase III <a href="http://www.regeneron.com">www.regeneron.com</a> <a href="http://www.sanofi.com">www.sanofi.com</a>
fevipirant (QAW039) (prostaglandin D2 receptor antagonist)	Novartis East Hanover, NJ	asthma <i>12 years and older</i>	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
<b>Infasurf®</b> calfactant	ONY Biotech Amherst, NY	bronchiolitis <i>up to 4 months</i>	Phase I <a href="http://www.onybiotech.com">www.onybiotech.com</a>
<b>InfasurfAero®</b> calfactant aerosolized	ONY Biotech Amherst, NY	neonatal respiratory distress syndrome <i>up to 24 hours</i>	Phase III completed <a href="http://www.onybiotech.com">www.onybiotech.com</a>
<b>Nucala®</b> mepolizumab ORPHAN DRUG	GlaxoSmithKline Research Triangle Park, NC	asthma <i>6 years to 11 years</i>	application submitted <a href="http://www.gsk.com">www.gsk.com</a>
		hypereosinophilic syndrome <i>12 years and older</i>	Phase III <a href="http://www.gsk.com">www.gsk.com</a>
OPN-375 (fluticasone propionate intranasal)	OptiNose US Yardley, PA	bilateral nasal polyps <i>12 years to 17 years</i>	Phase III <a href="http://www.optinose.com">www.optinose.com</a>

## Respiratory Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Pneumostem®</b> mesenchymal stem cell therapy <b>ORPHAN DRUG</b>	Medipost Seoul, South Korea	bronchopulmonary dysplasia <i>up to 14 days</i>	Phase I/II completed <a href="http://www.medi-post.com">www.medi-post.com</a>
PT001 (inhaled glycopyrrolate)	Pearl Therapeutics (AstraZeneca) Redwood City, CA	asthma <i>12 years and older</i>	Phase II/III <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
PT027 (budesonide/salbutamol)	Avillion London, United Kingdom Pearl Therapeutics (AstraZeneca) Redwood City, CA	asthma <i>4 years and older</i>	Phase III <a href="http://www.avillionllp.com">www.avillionllp.com</a> <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
QMF149 (indacaterol/mometasone)	Novartis East Hanover, NJ	asthma <i>12 years and older</i>	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
TAK607 (SHP607) (IGF-1/IGFBP3)	Takeda Deerfield, IL	chronic lung disease in extremely premature infants (prevention) (Fast Track) <i>up to 1 day</i>	Phase II <a href="http://www.takeda.com">www.takeda.com</a>
tezepelumab (TSLP inhibitor)	Amgen Thousand Oaks, CA AstraZeneca Wilmington, DE	severe asthma (Breakthrough Therapy) <i>12 years and older</i>	Phase III <a href="http://www.amgen.com">www.amgen.com</a> <a href="http://www.astrazeneca.com">www.astrazeneca.com</a>
VR647 (budesonide inhalation)	Vectura Chippenham, United Kingdom	asthma <i>4 years to 8 years</i>	Phase I completed <a href="http://www.vectura.com">www.vectura.com</a>

## Skin Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ADPS topical	Taro Pharmaceuticals USA Hawthorne, NY	acne vulgaris <i>9 years and older</i>	Phase II completed <a href="http://www.taro.com/usa">www.taro.com/usa</a>
ALX-101 (liver X receptor agonist)	Ralexar Therapeutics Malvern, PA	atopic dermatitis <i>12 years and older</i>	Phase II <a href="http://www.ralexar.com">www.ralexar.com</a>
ARQ-151	Arcutis Biotherapeutics Westlake Village, CA	plaque psoriasis <i>12 years and older</i>	Phase III <a href="http://www.arcutis.com">www.arcutis.com</a>
		atopic dermatitis <i>12 years and older</i>	Phase II <a href="http://www.arcutis.com">www.arcutis.com</a>
ARQ-154	Arcutis Biotherapeutics Westlake Village, CA	plaque psoriasis <i>12 years and older</i>	Phase II <a href="http://www.arcutis.com">www.arcutis.com</a>
B244 (ammonia oxidizing bacteria-based therapeutic)	AOBiome Cambridge, MA	atopic dermatitis <i>2 years to 17 years</i>	Phase I/II <a href="http://www.aobiome.com">www.aobiome.com</a>
BI 730357	Boehringer Ingelheim Ridgefield, CT	plaque psoriasis <i>all ages</i>	Phase II <a href="http://www.boehringer-ingelheim.com">www.boehringer-ingelheim.com</a>
BMS-986165 (TYK2 kinase inhibitor)	Bristol-Myers Squibb Princeton, NJ	plaque psoriasis <i>all ages</i>	Phase III <a href="http://www.bms.com">www.bms.com</a>



## Skin Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
BPX-01 (minocycline topical)	BioPharmX San Jose, CA	acne vulgaris <i>9 years and older</i>	Phase II completed <a href="http://www.biopharmx.com">www.biopharmx.com</a>
<b>Bryhali</b> <sup>®</sup> halobetasol propionate	Bausch Health Bridgewater, NJ	plaque psoriasis <i>6 years to 17 years</i>	Phase III <a href="http://www.bauschhealth.com">www.bauschhealth.com</a>
BTX1204 (cannabidiol)	Botanix Pharmaceuticals Plymouth Meeting, PA	atopic dermatitis <i>12 years and older</i>	Phase II <a href="http://www.botanixpharma.com">www.botanixpharma.com</a>
BTX1503 (cannabidiol)	Botanix Pharmaceuticals Plymouth Meeting, PA	moderate to severe acne vulgaris <i>12 years and older</i>	Phase II <a href="http://www.botanixpharma.com">www.botanixpharma.com</a>
<b>Cimzia</b> <sup>®</sup> certolizumab pegol	UCB Smyrna, GA	plaque psoriasis <i>6 years to 17 years</i>	Phase III <a href="http://www.ucb.com">www.ucb.com</a>
CLS-006 (furosemide topical gel)	Cutanea Life Sciences Wayne, PA	common cutaneous warts <i>2 years and older</i>	Phase III completed <a href="http://www.cutanea.com">www.cutanea.com</a>
<b>Cosentyx</b> <sup>®</sup> secukinumab	Novartis East Hanover, NJ	plaque psoriasis <i>6 years to 17 years</i>	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
DFD-03 (tazarotene lotion)	Dr. Reddys Laboratories Princeton, NJ	acne vulgaris <i>9 years and older</i>	Phase III completed <a href="http://www.drreddys.com">www.drreddys.com</a>

## Skin Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
dHACM (dehydrated human amnion/ chorion membrane)	MiMedx Marietta, GA	partial thickness burns <i>12 months and older</i>	Phase II <a href="http://www.mimedx.com">www.mimedx.com</a>
<b>Duobrii</b> ® halobetasol propionate and tazarotene	Bausch Health Bridgewater, NJ	plaque psoriasis <i>4 years to 17 years</i>	Phase III <a href="http://www.bauschhealth.com">www.bauschhealth.com</a>
<b>Dupixent</b> ® dupilumab	Regeneron Pharmaceuticals Tarrytown, NY Sanofi Bridgewater, NJ	atopic dermatitis <i>6 years to 11 years</i> atopic dermatitis <i>6 months to 5 years</i>	Phase III <a href="http://www.regeneron.com">www.regeneron.com</a> <a href="http://www.sanofi.com">www.sanofi.com</a>
FCD105 (adapalene/minocycline foam)	Foamix Pharmaceuticals Bridgewater, NJ	acne vulgaris <i>12 years and older</i>	Phase II <a href="http://www.foamix.com">www.foamix.com</a>
FMX101 (minocycline foam)	Foamix Pharmaceuticals Bridgewater, NJ	moderate to severe acne vulgaris <i>all ages</i>	application submitted <a href="http://www.foamix.com">www.foamix.com</a>
IDP-120 (benzoyl peroxide/tretinoin)	Bausch Health Bridgewater, NJ	acne vulgaris <i>9 years and older</i>	Phase III <a href="http://www.bauschhealth.com">www.bauschhealth.com</a>
IDP-123 (tazarotene lotion)	Bausch Health Bridgewater, NJ	acne vulgaris <i>9 years and older</i>	application submitted <a href="http://www.bauschhealth.com">www.bauschhealth.com</a>

## Skin Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
IDP-124 (pimecrolimus lotion)	Bausch Health Bridgewater, NJ	atopic dermatitis <i>2 years and older</i>	Phase III <a href="http://www.bauschhealth.com">www.bauschhealth.com</a>
IDP-126	Bausch Health Bridgewater, NJ	acne vulgaris <i>9 years and older</i>	Phase I <a href="http://www.bauschhealth.com">www.bauschhealth.com</a>
<b>Impozyz™</b> clobetasol propionate cream	Encore Dermatology Malvern, PA	plaque psoriasis <i>6 years to 17 years</i>	Phase II <a href="http://www.encorederm.com">www.encorederm.com</a>
lebrikizumab (injectable alpha-IL-13)	Dermira Menlo Park, CA	atopic dermatitis <i>12 years and older</i>	Phase III <a href="http://www.demira.com">www.demira.com</a>
LEO 124249 (delgocitinib)	LEO Pharma Madison, NJ	atopic dermatitis <i>2 years and older</i>	Phase I <a href="http://www.leo-pharma.us">www.leo-pharma.us</a>
<b>Lexette™</b> halobetasol foam	Mayne Pharma Greenville, NC	plaque psoriasis <i>12 years to 17 years</i>	Phase III <a href="http://www.maynepharma.com">www.maynepharma.com</a>
ligelizumab (QGE031) (anti-IgE mAb)	Novartis East Hanover, NJ	chronic spontaneous urticaria <i>12 years and older</i>	Phase III <a href="http://www.novartis.com">www.novartis.com</a>
lotamilast (RVT-501) (PDE4 inhibitor)	Dermavant Sciences Phoenix, AZ	atopic dermatitis <i>2 years to 17 years</i>	Phase II completed <a href="http://www.dermavant.com">www.dermavant.com</a>

## Skin Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
MM36 topical ointment (PDE4 inhibitor)	Medimetriks Pharmaceuticals Fairfield, NJ	atopic dermatitis <i>2 years to 17 years</i>	Phase II completed <a href="http://www.medimetriks.com">www.medimetriks.com</a>
nemolizumab (interleukin 31 receptor antagonist)	Galderma Fort Worth, TX	atopic dermatitis <i>12 years and older</i>	Phase III <a href="http://www.galderma.com">www.galderma.com</a>
<b>NexoBrid™</b> bromelain topical ORPHAN DRUG	MediWound Yavne, Israel	thermal wounds <i>up to 17 years</i>	Phase III <a href="http://www.mediwound.com">www.mediwound.com</a>
<b>Otezla®</b> apremilast	Celgene Summit, NJ	plaque psoriasis <i>6 years to 17 years</i>	Phase III <a href="http://www.celgene.com">www.celgene.com</a>
pegcantratinib (SNA-120) (TrkA receptor antagonist)	Sienna Biopharmaceuticals Westlake Village, CA	pruritus in psoriasis vulgaris <i>12 years and older</i>	Phase II completed <a href="http://www.siennabio.com">www.siennabio.com</a>
PF-04965842 (abrocitinib) (JAK1 inhibitor)	Pfizer New York, NY	atopic dermatitis (Breakthrough Therapy) <i>12 years to 17 years</i>	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
PF-06700841 (topical TYK2/JAK1 inhibitor)	Pfizer New York, NY	atopic dermatitis <i>12 years and older</i>	Phase II <a href="http://www.pfizer.com">www.pfizer.com</a>
Q301 cream (leukotriene inhibitor)	Qurient Seongnam-SI, South Korea	atopic dermatitis <i>12 years and older</i>	Phase II <a href="http://www.qurient.com">www.qurient.com</a>

## Skin Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Quzyttir™</b> cetirizine injection	JDP Therapeutics Blue Bell, PA	acute urticaria <i>pediatric</i>	application submitted <a href="http://www.jdptherapeutics.com">www.jdptherapeutics.com</a>
ruxolitinib cream (JAK1/JAK2 inhibitor)	Incyte Wilmington, DE	atopic dermatitis <i>12 years and older</i>	Phase III <a href="http://www.incyte.com">www.incyte.com</a>
		atopic dermatitis <i>2 years to 17 years</i>	Phase I <a href="http://www.incyte.com">www.incyte.com</a>
SB204 (nitric oxide releasing topical gel)	Novan Morrisville, NC	acne vulgaris <i>9 years and older</i>	Phase III <a href="http://www.novan.com">www.novan.com</a>
serlopitant (NK-1 receptor antagonist)	Menlo Therapeutics Redwood City, CA	pruritus in atopic dermatitis <i>13 years and older</i>	Phase II completed <a href="http://www.menlotherapeutics.com">www.menlotherapeutics.com</a>
<b>Sernivo®</b> betamethasone dipropionate	Encore Dermatology Malvern, PA Promius Pharma Princeton, NJ	plaque psoriasis <i>12 years to 16 years</i>	Phase III <a href="http://www.promiuspharma.com">www.promiuspharma.com</a>
<b>Skyrizi®</b> risankizumab	AbbVie North Chicago, IL	atopic dermatitis <i>12 years and older</i>	Phase II <a href="http://www.abbvie.com">www.abbvie.com</a>

## Skin Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Stelara®</b> ustekinumab	Janssen Research & Development Raritan, NJ	plaque psoriasis <i>6 years to 11 years</i> systemic lupus erythematosus <i>16 years and older</i>	Phase III <a href="http://www.janssen.com">www.janssen.com</a>
<b>Taltz®</b> ixekizumab	Eli Lilly Indianapolis, IN	plaque psoriasis <i>6 years to 17 years</i>	Phase III <a href="http://www.lilly.com">www.lilly.com</a>
TMB-001 (isotretinoin) ORPHAN DRUG	Timber Pharmaceuticals Woodcliff Lake, NJ	congenital ichthyosis <i>9 years and older</i>	Phase II <a href="http://www.timberpharma.com">www.timberpharma.com</a>
<b>Topicort®</b> desoximetasone topical spray	Taro Pharmaceuticals USA Hawthorne, NY	atopic dermatitis <i>6 months and older</i>	Phase III completed <a href="http://www.taro.com">www.taro.com</a>
		plaque psoriasis <i>2 years to 17 years</i>	Phase III <a href="http://www.taro.com">www.taro.com</a>
tralokinumab (MEK inhibitor)	LEO Pharma Madison, NJ	atopic dermatitis <i>12 years to 17 years</i>	Phase III <a href="http://www.leo-pharma.com">www.leo-pharma.com</a>
<b>Tremfya®</b> guselkumab	Janssen Research & Development Raritan, NJ	plaque psoriasis <i>6 years to 17 years</i>	Phase III <a href="http://www.janssen.com">www.janssen.com</a>

## Skin Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
trifarotene (retinoic acid receptor agonist)	Galderma Fort Worth, TX	moderate facial and truncal acne <i>9 years and older</i>	application submitted <a href="http://www.galderma.com">www.galderma.com</a>
TWIN (benzoyl peroxide/tretinoin)	Sol-Gel Technologies Ness Ziona, Israel	acne vulgaris <i>9 years and older</i>	Phase III <a href="http://www.sol-gel.com">www.sol-gel.com</a>
<b>Ultravate®</b> halobetasol propionate lotion	Sun Pharmaceutical Mumbai, India	plaque psoriasis <i>12 years to 16 years</i>	Phase III <a href="http://www.sunpharma.com">www.sunpharma.com</a>
upadacitinib (ABT-494) (JAK1 inhibitor)	AbbVie North Chicago, IL	atopic dermatitis <i>12 years and older</i>	Phase III <a href="http://www.abbvie.com">www.abbvie.com</a>
		atopic dermatitis <i>2 years to 11 years</i>	Phase I <a href="http://www.abbvie.com">www.abbvie.com</a>
<b>Winlevi™</b> clascoterone	Cassiopea Milan, Italy	acne vulgaris <i>9 years and older</i>	application submitted <a href="http://www.cassiopea.com">www.cassiopea.com</a>

## Transplantation

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Defitelio®</b> defibrotide ORPHAN DRUG	Jazz Pharmaceuticals Dublin, Ireland	acute graft-versus-host disease <i>1 year and older</i>	Phase II <a href="http://www.jazzpharma.com">www.jazzpharma.com</a>

## Transplantation

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
EQ001 (itolizumab) ORPHAN DRUG	Equillium La Jolla, CA	graft-versus-host disease (Fast Track) <i>12 years and older</i>	Phase I/II <a href="http://www.equillium.com">www.equillium.com</a>
<b>Imbruvica®</b> ibrutinib	Janssen Research & Development Raritan, NJ Pharmacyclics Sunnyvale, CA	chronic graft-versus-host disease <i>1 year to 21 years</i>	Phase I/II <a href="http://www.janssen.com">www.janssen.com</a> <a href="http://www.pharmacyclics.com">www.pharmacyclics.com</a>
itacitinib (JAK 1 inhibitor)	Incyte Wilmington, DE	graft-versus-host disease <i>up to 17 years</i>	Phase I/II <a href="http://www.incyte.com">www.incyte.com</a>
remestemcel-L (cell replacement therapy) ORPHAN DRUG	Mesoblast New York, NY	acute graft-versus-host disease (Fast Track) <i>pediatric</i>	application submitted <a href="http://www.mesoblast.com">www.mesoblast.com</a>
Rivo-cel (BPX-501) (polyclonal T cell replacement therapy) ORPHAN DRUG	Bellicum Pharmaceuticals Houston, TX	post-stem cell transplant (non-malignant diseases) <i>4 months and older</i>	Phase I <a href="http://www.bellicum.com">www.bellicum.com</a>
ruxolitinib	Incyte Wilmington, DE	chronic graft-versus-host disease <i>12 years and older</i>	Phase I <a href="http://www.incyte.com">www.incyte.com</a>
SNDX-6352 (IgG4 mAb)	Syndax Pharmaceuticals Waltham, MA	chronic graft-versus-host disease <i>12 years and older</i>	Phase I <a href="http://www.syndax.com">www.syndax.com</a>



## Transplantation

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
T-Guard (mAb CD30dgA/CD7-dgA immunotoxin)	Xenikos Nijmegen, The Netherlands	steroid-refractory graft-versus- host disease	Phase III <a href="http://www.xenikos.com">www.xenikos.com</a>
<b>Uvadex®</b> methoxsalen	Mallinckrodt St. Louis, MO	graft-versus-host disease with CELLEX® Photopheresis System <i>1 year to 21 years</i>	Phase III <a href="http://www.mallinckrodt.com">www.mallinckrodt.com</a>
<b>Zemaira®</b> alpha-1-antitrypsin	CSL Behring King of Prussia, PA	graft-versus-host disease in hematopoietic cell transplantation <i>12 years and older</i>	Phase II/III <a href="http://www.cslbehring.com">www.cslbehring.com</a>
<b>Zortress®</b> everolimus	Novartis East Hanover, NJ	prevention of kidney transplant rejection <i>1 year to 18 years</i>	Phase III completed <a href="http://www.novartis.com">www.novartis.com</a>

## Other Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
ACP-001 (TransCon hGH)	Ascendis Pharma Palo Alto, CA	growth hormone deficiency <i>3 years to 12 years</i>	Phase III <a href="http://www.ascendispharma.com">www.ascendispharma.com</a>
<b>Alkindi®</b> hydrocortisone	Diurnal Cardiff, United Kingdom	adrenal insufficiency <i>birth to 17 years</i>	application submitted <a href="http://www.diurnal.co.uk">www.diurnal.co.uk</a>

## Other Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
AR25	Arbor Pharmaceuticals Atlanta, GA	ear, nose, throat disorders <i>pediatric</i>	Phase III <a href="http://www.arborpharma.com">www.arborpharma.com</a>
BAY-2880376 (caffeine/naproxen)	Bayer Pharmaceutical Whippany, NJ	postoperative pain <i>16 years and older</i>	Phase II <a href="http://www.pharma.bayer.com">www.pharma.bayer.com</a>
BBI-4000 (sofipronium bromide)	Brickell Biotech Boulder, CO	primary axillary hyperhidrosis <i>12 years and older</i>	Phase III <a href="http://www.brickellbio.com">www.brickellbio.com</a>
		primary axillary hyperhidrosis <i>9 years to 16 years</i>	Phase II <a href="http://www.brickellbio.com">www.brickellbio.com</a>
<b>Botox</b> <sup>®</sup> onabotulinumtoxin A	Allergan Madison, NJ	urinary incontinence due to overactive bladder <i>12 years to 17 years</i>	Phase III <a href="http://www.allergan.com">www.allergan.com</a>
<b>Cymbalta</b> <sup>®</sup> duloxetine	Eli Lilly Indianapolis, IN	fibromyalgia <i>12 years to 17 years</i>	Phase III completed <a href="http://www.lilly.com">www.lilly.com</a>
<b>Exparel</b> <sup>®</sup> bupivacaine liposome injectable suspension	Pacira BioSciences Parsippany, NJ	postoperative pain <i>6 years to 17 years</i>	Phase III <a href="http://www.pacira.com">www.pacira.com</a>
<b>Hetlioz</b> <sup>®</sup> tasimelteon	Vanda Pharmaceuticals Washington, DC	non-24 sleep-wake disorder <i>pediatric</i>	Phase III <a href="http://www.vandapharma.com">www.vandapharma.com</a>

## Other Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
HTX-011 (bupivacaine and meloxicam)	Heron Therapeutics San Diego, CA	postoperative pain <i>3 years to 16 years</i>	Phase II <a href="http://www.herontx.com">www.herontx.com</a>
leuprolide acetate 45mg (6-month depot formulation)	AbbVie North Chicago, IL	central precocious puberty <i>2 years to 11 years</i>	Phase III <a href="http://www.tolmar.com">www.tolmar.com</a>
leuprolide acetate 45mg	Tolmar Pharmaceuticals Fort Collins, CO	central precocious puberty <i>2 years to 9 years</i>	Phase III completed <a href="http://www.tolmar.com">www.tolmar.com</a>
LF111 (drospirenone 4.0mg)	León Farma León, Spain	contraception <i>15 years and older</i>	Phase III completed <a href="http://www.chemopharmaceuticals.com">www.chemopharmaceuticals.com</a>
LUM-201 (ibutamoren) ORPHAN DRUG	Lumos Pharma Auston, TX	born small for gestational age, growth hormone deficiency, Turner syndrome <i>pediatric</i>	Phase I <a href="http://www.lumos-pharma.com">www.lumos-pharma.com</a>
netupitant (NK1 antagonist)	Helsinn Therapeutics Iselin, NJ	chemotherapy-induced nausea and vomiting (CINV) <i>up to 17 years</i>	Phase II <a href="http://www.helsinn.com">www.helsinn.com</a>
<b>Nucynta</b> <sup>®</sup> tapentadol oral solution	Collegium Pharmaceutical Stoughton, MA Grunenthal Aachen, Germany	acute pain <i>up to 17 years</i>	Phase III completed <a href="http://www.collegiumpharma.com">www.collegiumpharma.com</a>

## Other Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
NVK-009 (pregabalin)	Nevakar Bridgewater, NJ	dental pain <i>17 years and older</i>	Phase II <a href="http://www.nevakar.com">www.nevakar.com</a>
oxymorphone immediate release	Endo Pharmaceuticals Malvern, PA	postoperative pain (oral liquid) <i>up to 12 years</i> postoperative pain <i>12 years to 17 years</i>	Phase III completed <a href="http://www.endo.com">www.endo.com</a>
<b>Pedmark™</b> sodium thiosulfate <b>ORPHAN DRUG</b>	Fennec Pharmaceuticals Research Triangle Park, NC	chemotherapy-induced sensorineural hearing loss <i>1 month to 17 years</i>	application submitted <a href="http://www.fennecpharma.com">www.fennecpharma.com</a>
PF-06651600 (JAK3/TEC inhibitor)	Pfizer New York, NY	alopecia areata <i>12 years and older</i>	Phase III <a href="http://www.pfizer.com">www.pfizer.com</a>
<b>Qsymia®</b> phentermine/topiramate	VIVUS Campbell, CA	obesity <i>12 years to 16 years</i>	Phase III <a href="http://www.vivus.com">www.vivus.com</a>
<b>Rituxan®</b> rituximab <b>ORPHAN DRUG</b>	Biogen Cambridge, MA Genentech South San Francisco, CA	granulomatosis with polyangiitis or microscopic polyangiitis (combination therapy) <i>2 years and older</i>	application submitted <a href="http://www.biogen.com">www.biogen.com</a> <a href="http://www.gene.com">www.gene.com</a>
		granulomatosis with polyangiitis or microscopic polyangiitis (monotherapy) <i>2 years to 17 years</i>	Phase II completed <a href="http://www.biogen.com">www.biogen.com</a> <a href="http://www.gene.com">www.gene.com</a>

## Other Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
<b>Soliris®</b> eculizumab ORPHAN DRUG	Alexion Pharmaceuticals Boston, MA	generalized myasthenia gravis <i>6 years to 17 years</i>	Phase III <a href="http://www.alexion.com">www.alexion.com</a>
somapacitsan (long acting growth hormone)	Novo Nordisk Plainsboro, NJ	growth hormone deficiency <i>2 years to 11 years</i>	Phase III <a href="http://www.novonordisk.com">www.novonordisk.com</a>
		short stature in children born small for gestational age <i>2 years to 11 years</i>	Phase II <a href="http://www.novonordisk.com">www.novonordisk.com</a>
somatrogon (hGH-CTP) (HGH agonist) ORPHAN DRUG	OPKO Health Miami, FL Pfizer New York, NY	growth hormone deficiency <i>3 years to 17 years</i>	Phase III <a href="http://www.opko.com">www.opko.com</a> <a href="http://www.pfizer.com">www.pfizer.com</a>
<b>Victoza®</b> liraglutide	Novo Nordisk Plainsboro, NJ	weight management in obesity <i>12 years to 17 years</i>	Phase III completed <a href="http://www.novonordisk.com">www.novonordisk.com</a>
		weight management in Prader-Willi syndrome <i>6 years to 17 years</i>	Phase III <a href="http://www.novonordisk.com">www.novonordisk.com</a>
		obesity <i>7 years to 11 years</i>	Phase I completed <a href="http://www.novonordisk.com">www.novonordisk.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 **January 10, 2020**. The medicines in this listing include medicines being developed by U.S.-based companies conducting trials in the United States abroad, PhRMA-member companies conducting trials in the United States and abroad, and foreign companies conducting clinical trials in the United States. Some products may not be in active clinical trials. The information may not be comprehensive. For more, specific information about a particular product, contact the individual company directly or go to [www.clinicaltrials.gov](http://www.clinicaltrials.gov). The entire series of *Medicines in Development* is available on PhRMA's website: [www.phrma.org](http://www.phrma.org).

## Definitions

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

**Breakthrough Therapy**—Upon request by a sponsor, the FDA can grant this designation to expedite the development and review of a drug or biologic intended, alone or in combination with one or more other drugs, to treat a serious or life threatening disease or condition and preliminary clinical evidence indicates that it may demonstrate substantial improvement over existing therapies on one or more clinically-significant endpoints, such as substantial treatment effects observed early in clinical development. If a drug or biologic is designated as a Breakthrough Therapy, the FDA will expedite the development and review. With this designation, all Fast Track features convey to the medicine.

**Fast Track**—Upon request by a sponsor, the FDA can grant this designation to facilitate the development and expedite the review of a drug or biologic to treat a serious condition and fill an unmet medical need. When considering a biopharmaceutical company's request for Fast Track designation for an investigational drug or biologic, the FDA evaluates whether it will affect factors such as survival, day-to-day functioning, or the likelihood that the disease, if left untreated, will progress from a less severe condition to a more serious one, and whether a condition can be adequately addressed by available therapy. With Fast Track designation, early and frequent communication between the FDA and the biopharmaceutical company is encouraged throughout the entire drug development and review process to help to quickly resolve any questions or issues that arise, potentially leading to an earlier approval and access by patients.

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

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

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

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