

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

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

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VE416 (live biotherapeutic product)	Vedanta Biosciences Cambridge, MA	peanut hypersensitivity <i>12 years and older</i>	Phase I/II www.vedantabio.com
Viaskin Milk milk allergy immunotherapy	DBV Technologies Summit, NJ	milk-induced eosinophilic esophagitis <i>4 years to 17 years</i>	Phase II completed www.dbv-technologies.com
		milk hypersensitivity (Fast Track) <i>2 years to 17 years</i>	Phase I/II www.dbv-technologies.com
Viaskin Peanut peanut allergy immunotherapy	DBV Technologies Summit, NJ	peanut hypersensitivity (Fast Track) (Breakthrough Therapy) <i>4 years to 11 years</i>	application submitted www.dbv-technologies.com
		peanut hypersensitivity <i>1 year to 3 years</i>	Phase III www.dbv-technologies.com
		peanut hypersensitivity <i>adolescents</i>	Phase I www.dbv-technologies.com

Arthritis/Musculoskeletal

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

Blood Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Adakveo® crizanlizumab (SEG101) ORPHAN DRUG	Novartis East Hanover, NJ	sickle cell disease <i>6 months to 12 years</i>	Phase II www.novartis.com
ALXN1210 (ravulizumab) ORPHAN DRUG	Alexion Pharmaceuticals Boston, MA	paroxysmal nocturnal hemoglobinuria <i>up to 18 years</i>	Phase III www.alexion.com
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 www.bluebirdbio.com
Brilinta® ticagrelor	AstraZeneca Wilmington, DE	vaso-occlusive crises in sickle cell disease <i>2 years to 17 years</i>	Phase III www.astrazeneca.com
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 www.censapharma.com
eptacog beta (recombinant coagulation Factor VIIa)	Hema Biologics Framingham, MA	hemophilia A, hemophilia B <i>6 months and older</i>	Phase III www.lfb-usa.com
Feraheme® ferumoxytol injection	AMAG Pharmaceuticals Waltham, MA	iron deficiency anemia <i>2 years to 17 years</i>	Phase III www.amagpharma.com

Blood Disorders

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

Blood Disorders

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mavorixafor (CXCR4 inhibitor) ORPHAN DRUG	X4 Pharmaceuticals Cambridge, MA	neutropenia <i>12 years and older</i>	Phase I www.x4pharma.com
NiCord® omidubicel ORPHAN DRUG	Gamida Cell Boston, MA	sickle cell disease, thalassemia <i>2 years and older</i>	Phase I/II www.gamida-cell.com
PF-06741086 (tissue factor pathway inhibitor) ORPHAN DRUG	Pfizer New York, NY	hemophilia (Fast Track) <i>12 years and older</i>	Phase III www.pfizer.com
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 www.pfizer.com
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 www.rocketpharma.com
SC411 (docosahexaenoic acid) ORPHAN DRUG	Micelle BioPharma Riveria Beach, FL	sickle cell disease <i>5 years to 17 years</i>	Phase II www.micellebiopharma.com
Soliris® eculizumab	Alexion Pharmaceuticals Boston, MA	paroxysmal nocturnal hemoglobinuria <i>2 years to 17 years</i>	Phase III completed www.alexion.com

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

Cancer

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131I-MIBG (radiotherapy)	Jubilant DraxImage Kirkland, Canada	neuroblastoma <i>1 year and older</i>	Phase II www.draximage.com
131 I-omburtamab (thermostic mAb) ORPHAN DRUG	Y-mAbs Therapeutics New York, NY	neuroblastoma, central nervous system (CNS) metastases, leptomeningeal metastases <i>up to 18 years</i>	Phase II/III www.ymabs.com
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 www.aadibio.com

Cancer

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Abraxane® 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 www.celgene.com
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 www.ziopharm.com
Adcetris® brentuximab vedotin	Takeda Oncology Deerfield, IL Seattle Genetics Bothell, WA	Hodgkin lymphoma (combination therapy) <i>5 years-17 years</i>	Phase I/II www.takeda.com www.seattlegenetics.com
AG-881 (vorasidenib)	Agios Pharmaceuticals Cambridge, MA	glioma <i>12 years and older</i>	Phase III www.agios.com
aglatimagene besadenovec (Adv-Tk gene therapy) ORPHAN DRUG	Advantagene Auburndale, MA	pediatric brain tumors <i>3 years to 21 years</i>	Phase I www.advantagene.com
Aliqopa® copanlisib	Bayer Pharmaceuticals Whippany, NJ	lymphoma, solid tumors <i>6 months to 21 years</i>	Phase I/II www.pharma.bayer.com

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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 www.aileronrx.com
AMG 337 (c-Met inhibitor)	NantPharma Culver City, CA	clear cell sarcoma <i>16 years and older</i>	Phase II www.nantpharma.com
Bavencio® avelumab	EMD Serono Rockland, MA	lymphoma, solid tumors <i>up to 18 years</i>	Phase I/II www.emdserono.com
BGB-290 (PARP inhibitor)	BeiGene USA Cambridge, MA	glioma <i>13 years to 25 years</i>	Phase I www.beigene.com
BMS-986016 (relatlimab) (anti-LAG3 mAb)	Bristol-Myers Squibb Princeton, NJ	solid tumors <i>12 years and older</i>	Phase I/II www.bms.com
BMS-986158 (BET inhibitor)	Bristol-Myers Squibb Princeton, NJ	advanced tumors <i>12 years and older</i>	Phase I/II www.bms.com
BXQ-350 (lysosomal activator protein Saposin C and phosphatidylserine)	Bexion Pharmaceuticals Covington, KY	solid tumors <i>1 year to 30 years</i>	Phase I www.bexionpharma.com

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

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Cyramza® ramucirumab	Eli Lilly Indianapolis, IN	refractory solid tumors <i>12 months to 21 years</i>	Phase I www.lilly.com
Darzalex® daratumumab	Janssen Research & Development Raritan, NJ	precursor B-cell lymphoblastic leukemia-lymphoma <i>1 year to 30 years</i>	Phase II www.janssen.com
dilanubicel (ex vivo expanded hematopoietic stem and progenitor cell therapy) ORPHAN DRUG	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 www.nohlatherapeutics.com
eflornithine plus high-dose powder sachet	Cancer Prevention Pharmaceuticals Tucson, AZ	neuroblastoma <i>up to 17 years</i>	Phase II www.canprevent.com
enoblituzumab (CD276 protein inhibitor)	MacroGenics Rockville, MD	B7-H3-expressing solid tumors <i>1 year and older</i>	Phase I completed www.macrogenics.com
fenretinide intravenous	CerRx Lubbock, TX	solid tumors <i>pediatric</i>	Phase I www.cerrx.com
fimepinostat (HDAC/pan-PI3K inhibitor)	Curis Lexington, MA	solid tumors <i>16 years and older</i>	Phase I completed www.curis.com

Cancer

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
GD2-GD3 vaccine (immunostimulant vaccine) ORPHAN DRUG	Y-mAbs Therapeutics New York, NY	neuroblastoma <i>up to 17 years</i>	Phase II www.ymabs.com
GDC-0084 (PI3K inhibitor)	Kazia Therapeutics Sydney, Australia	diffuse intrinsic pontine glioma <i>2 years to 21 years</i>	Phase I www.kaziatherapeutics.com
Gilotrif® afatinib	Boehringer Ingelheim Ridgefield, CT	neuroectodermal tumors, rhabdomyosarcoma <i>1 year to 18 years</i>	Phase I/II www.boehringer-ingelheim.com
Halaven® 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 www.eisai.com
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 www.ymabs.com
Ibrance® palbociclib	Pfizer New York, NY	recurrent/refractory solid tumors <i>2 years to 20 years</i>	Phase I www.pfizer.com

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

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

melanoma, solid tumors
6 months to 17 years

Phase I/II
www.merck.com

Kymriah®
tisagenlecleucel

Novartis
East Hanover, NJ

non-Hodgkin lymphoma
up to 18 years

Phase II
www.novartis.com

Kyprolis®
carfilzomib

Amgen
Thousand Oaks, CA

relapsing/refractory ALL
all ages

Phase I
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

Lenvima®
lenvatinib

Eisai
Woodcliff Lake, NJ

osteosarcoma, solid tumors
2 years to 21 years

Phase I/II
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

newly-diagnosed or recurrent glioma
up to 25 years

Phase I/II
www.regeneron.com

Cancer

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
isocabtagene 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 www.celgene.com
LOXO-292 (RET inhibitor)	Loxo Oncology Stamford, CT	solid tumors, primary CNS tumors <i>6 months to 21 years</i>	Phase I/II www.loxooncology.com
		advanced solid tumors, RET fusion-positive solid tumors, medullary thyroid cancer <i>6 months to 21 years</i>	Phase I/II www.loxooncology.com
MB-102 (CAR-T cell therapy) ORPHAN DRUG	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 www.mustangbio.com
Mekinist® trametinib	Novartis East Hanover, NJ	BRAF V600 mutation positive cancer (mono and combination therapy) <i>1 month to 17 years</i>	Phase I/II www.novartis.com
MTX110 (nano-inclusion) ORPHAN DRUG	Midatech Pharma Cardiff, United Kingdom	diffuse intrinsic pontine glioma <i>2 years to 21 years</i>	Phase II www.midatechpharma.com

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

naxitamab
(anti-GD2 3F8 mAb)
ORPHAN DRUG

Y-mAbs Therapeutics
New York, NY

neuroblastoma
1 year and older

application submitted
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

ONC201
(DRD2/DRD3 antagonist)
ORPHAN DRUG

Oncocyte
Philadelphia, PA

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

Phase II
www.oncoceutics.com

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

Phase I
www.oncoceutics.com

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Opdivo® nivolumab	Bristol-Myers Squibb Princeton, NJ	metastatic melanoma <i>12 years and older</i> glioblastoma <i>all ages</i>	Phase III www.bms.com
		high-grade primary CNS malignancies <i>6 months to 21 years</i> Hodgkin lymphoma (with Adcetris®) <i>5 years to 30 years</i> solid tumors of high tumor mutational burden <i>12 years and older</i>	Phase II www.bms.com
		non-Hodgkin lymphoma <i>15 years and older</i>	Phase I/II www.bms.com
OXS-1550 (bispecific fusion protein drug conjugate)	GT Biopharma Beverly Hills, CA	B-cell malignancies <i>12 years and older</i>	Phase I completed www.gtbiopharma.com
PEP-CMV (dendritic cell vaccine)	Annias Immunotherapeutics Chapel Hill, NC Duke University Chapel Hill, NC	malignant glioma, recurrent medulloblastoma <i>3 years to 35 years</i>	Phase I www.anniasimmuno.com

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Pomalyst® pomalidomide	Celgene Summit, NJ	recurrent or progressive primary brain tumors <i>1 year to 21 years</i>	Phase II www.celgene.com
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 www.ptcbio.com
PVSRIP0 (recombinant polio virus vaccine) ORPHAN DRUG	Istari Oncology Research Triangle Park, NC	recurrent malignant glioma <i>12 years to 21 years</i>	Phase I www.istarioncology.com
reprotrectinib (ALK, ROS1, TRK inhibitor)	Turning Point Therapeutics San Diego, CA	lymphoma, solid tumors <i>4 years to 12 years</i>	Phase I/II www.tptherapeutics.com
Revlimid® lenalidomide	Celgene Summit, NJ	AML <i>1 year to 18 years</i>	Phase II completed www.celgene.com
Rivo-cel (BPX-501) (T-cell replacement therapy) ORPHAN DRUG	Bellicum Pharmaceuticals Houston, TX	AML, myelodysplastic syndromes <i>12 years and older</i>	Phase II/III www.bellicum.com
Rozlytrek™ entrectinib	Genentech South San Francisco, CA	recurrent or refractory solid tumors, primary CNS tumors <i>up to 22 years</i>	Phase I www.gene.com

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Rydapt® midostaurin	Novartis East Hanover, NJ	newly-diagnosed FLT-3 mutated AML <i>3 months to 17 years</i>	Phase II www.novartis.com
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 www.saliariuspharma.com
Tab-cel® tabelecleucel	Atara Biotherapeutics South San Francisco, CA	Epstein-Barr virus associated post-transplant lymphoproliferative disease <i>all ages</i>	Phase III www.atarabio.com
		Epstein-Barr virus-associated nasopharyngeal carcinoma <i>12 years and older</i>	Phase I/II www.atarabio.com
Taflinar® dabrafenib	Novartis East Hanover, NJ	brain neoplasms <i>12 months to 17 years</i>	Phase I www.novartis.com
Taflinar® + Mekinist® 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 www.novartis.com
tazemetostat (EZH2 Inhibitor) ORPHAN DRUG	Epizyme Cambridge, MA	INI1-negative tumors, synovial sarcoma <i>6 months to 18 years</i>	Phase I www.epizyme.com

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TB-403 (placenta growth factor inhibitor)	BioInvent Lund, Sweden Oncurios Leuven, Belgium	relapsed/refractory medulloblastoma <i>6 months to 18 years</i>	Phase I/II www.oncurious.com
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 www.oncternal.com
UCART19 (CAR-T cell therapy)	Servier Paris, France	relapsed/refractory B-cell acute ALL <i>6 months to 17 years</i>	Phase I www.servier.com
varlitinib (EGFR antagonist, ERBB 2/4 receptor antagonist) ORPHAN DRUG	ASLAN Pharmaceuticals Singapore	biliary tract cancer <i>all ages</i>	Phase II/III www.aslanpharma.com
Venclexta® venetoclax	AbbVie North Chicago, IL Genentech South San Francisco, CA	AML (combination therapy) <i>12 years and older</i>	Phase III www.abbvie.com www.gene.com
		relapsed/refractory malignancies <i>up to 25 years</i> ALL, lymphoblastic leukemia <i>4 years and older</i>	Phase I www.abbvie.com www.gene.com

Cancer

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

Cardiovascular Disease

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

Cardiovascular Disease

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

Cardiovascular Disease

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
inclisiran (PCSK9 protein inhibitor, RNA interference) ORPHAN DRUG	Alynham Pharmaceuticals Cambridge, MA The Medicines Company Parsippany, NJ	homozygous familial hypercholesterolemia <i>12 years and older</i>	Phase III completed www.alnylam.com www.themedicinescompany.com
INOpulse (nitric oxide inhalation) ORPHAN DRUG	Bellerophon Therapeutics Warren, NJ	pulmonary arterial hypertension <i>16 years and older</i>	Phase II completed www.bellerophon.com
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 www.asklepionpharm.com
Letairis® ambrisentan	Gilead Sciences Foster City, CA GlaxoSmithKline Research Triangle Park, NC	pulmonary arterial hypertension <i>8 years to 18 years</i>	Phase II www.gilead.com www.gsk.com
Opsumit® 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 www.actelion.com
Pradaxa® dabigatran etexilate	Boehringer Ingelheim Ridgefield, CT	venous thromboembolism <i>up to 17 years</i>	Phase III www.boehringer-ingelheim.com

Cardiovascular Disease

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

Cardiovascular Disease

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

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 www.axialbiotherapeutics.com

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 www.confluencepharma.com
balovaptan (RO5285119) (vasopressin 1 receptor antagonist)	Genentech South San Francisco, CA	autism spectrum disorder <i>5 years to 17 years</i>	Phase II www.gene.com
		autism spectrum disorder <i>2 years to 4 years</i>	Phase I www.gene.com
Circadin® 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 www.neurim.com
CM-AT (enzyme modulator)	Curemark Rye, NY	autism spectrum disorder (Fast Track) <i>3 years to 8 years</i>	application submitted www.curemark.com
GWP42006 (cannabidiol)	Greenwich Biosciences Carlsbad, CA	autism spectrum disorder <i>4 years to 18 years</i>	Phase II www.greenwichbiosciences.com
JNJ-42165279 (FAAH inhibitor)	Janssen Research and Development Raritan, NJ	autism spectrum disorder <i>13 years and older</i>	Phase II www.janssen.com

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 www.yamopharma.com
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 www.ovidrx.com
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 www.zynerba.com
		autism spectrum disorder <i>4 years to 17 years</i>	Phase II www.zynerba.com
		development and epileptic encephalopathies <i>3 years to 17 years</i>	Phase II www.zynerba.com

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 www.sanofi.com

Diabetes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Afrezza® insulin (human) inhalation	MannKind Westlake Village, CA	type 1 diabetes <i>4 years to 17 years</i>	Phase II www.mannkind.com
AG019 (gene transference)	Intrexon T1D Partners Germantown, MD ActoBio Therapeutics Ghent, Belgium	type 1 diabetes <i>12 years and older</i>	Phase I/II www.actbio.com
Bydureon® BCise® exenatide extended release	AstraZeneca Wilmington, DE	type 2 diabetes <i>10 years to 17 years</i>	Phase III www.astrazeneca.com
CLBS03 (autologous ex vivo polyclonal T regulatory cell therapy) ORPHAN DRUG	Caladrius Biosciences Basking Ridge, NJ	type 1 diabetes (Fast Track) <i>8 years to 17 years</i>	Phase II www.caladrius.com
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 www.zealnadpharma.com
		congenital hyperinsulin <i>up to 364 days</i>	Phase II/III www.zealandpharma.com

Diabetes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Diamyd®/GABA autoimmune diabetes vaccine ORPHAN DRUG	Diamyd Medical Stockholm, Sweden University of Alabama Birmingham, AL	type 1 diabetes <i>4 years to 18 years</i>	Phase I www.diamyd.com
Farxiga® dapagliflozin	AstraZeneca Wilmington, DE	type 2 diabetes <i>10 years to 18 years</i>	Phase III www.astrazeneca.com
Fiasp® insulin aspart injection 100 units/mL	Novo Nordisk Plainsboro, NJ	type 1 diabetes <i>children and adolescents</i>	application submitted www.novonordisk.com
Gvoke™ glucagon rescue pen ORPHAN DRUG	Xeris Pharmaceuticals Chicago, IL	congenital hyperinsulinism <i>up to 12 months</i>	Phase II completed www.xerispharma.com
insulin lispro (LY900014) (ultra rapid formulation)	Eli Lilly Indianapolis, IN	type 1 diabetes <i>1 year to 17 years</i>	Phase III www.lilly.com
Invokana® canagliflozin	Janssen Raritan, NJ	type 2 diabetes <i>10 years to 17 years</i>	Phase III www.janssen.com
Janumet® sitagliptin and metformin	Merck Kenilworth, NJ	type 2 diabetes <i>10 years to 17 years</i>	Phase III www.merck.com

Diabetes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Janumet® XR sitagliptin and metformin extended release	Merck Kenilworth, NJ	type 2 diabetes <i>10 years to 17 years</i>	Phase III www.merck.com
Januvia® sitagliptin	Merck Kenilworth, NJ	type 2 diabetes <i>10 years to 17 years</i>	Phase III www.merck.com
Jardiance® empagliflozin	Boehringer Ingelheim Ridgefield, CT Eli Lilly Indianapolis, IN	type 2 diabetes <i>10 years to 17 years</i>	Phase III www.boehringer-ingelheim.com www.lilly.com
Nesina® alogliptin	Takeda Deerfield, IL	type 2 diabetes <i>10 years to 17 years</i>	Phase III www.takeda.com
Onglyza® saxagliptin	AstraZeneca Wilmington, DE	type 2 diabetes <i>10 years to 18 years</i>	Phase III www.astrazeneca.com
Ryzodeg® insulin degludec and insulin aspart injection	Novo Nordisk Plainsboro, NJ	type 1 diabetes <i>1 year to 17 years</i>	Phase III completed www.novonordisk.com

Diabetes

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Simponi® golimumab	Janssen Research & Development Raritan, NJ	type 1 diabetes <i>6 years to 21 years</i>	Phase II www.janssen.com
		pre-symptomatic type 1 diabetes <i>6 years to 21 years</i>	Phase I www.janssen.com
Steglatro™ ertugliflozin	Merck Kenilworth, NJ	type 2 diabetes <i>10 years to 17 years</i>	Phase III www.merck.com
teplizumab (CD3 antigen inhibitor) ORPHAN DRUG	Provention Bio Oldwick, NJ	type 1 diabetes <i>8 years to 17 years</i>	Phase III www.proventionbio.com
Toujeo® insulin glargine injection (300 units/mL)	Sanofi Bridgewater, NJ	type 1 diabetes <i>6 years to 17 years</i>	Phase III completed www.sanofi.com
Tradjenta® linagliptin	Boehringer Ingelheim Ridgefield, CT Eli Lilly Indianapolis, IN	type 2 diabetes <i>10 years to 17 years</i>	Phase III www.boehringer-ingelheim.com www.lilly.com
Trulicity® dulaglutide	Eli Lilly Indianapolis, IN	type 2 diabetes <i>10 years to 17 years</i>	Phase III www.lilly.com

Diabetes

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

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 www.eyenoviabio.com
brimonidine ophthalmic suspension	Sun Pharma Advanced Research Mumbai, India	ocular hypertension, open angle glaucoma <i>2 years and older</i>	Phase III www.sparc.life
DE-117 ophthalmic solution (omidenepag isopropyl)	Santen Pharmaceutical Emeryville, CA	glaucoma, ocular hypertension <i>12 months and older</i>	Phase III www.santenusa.com
Eylea® aflibercept	Regeneron Pharmaceuticals Tarrytown, NY	retinopathy of prematurity <i>all ages</i>	Phase III www.regeneron.com
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 www.iviewtherapeutics.com

Eye Disorders

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

Gastrointestinal Disorders

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

Gastrointestinal Disorders

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

Gastrointestinal Disorders

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

Gastrointestinal Disorders

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

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 www.meiragtx.com
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 www.meiragtx.com
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 www.meiragtx.com
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 www.meiragtx.com
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 www.abeonatherapeutics.com
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 www.abeonatherapeutics.com

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 www.agtc.com
ACHM-CNGB3 (AAV gene therapy)	Applied Genetic Technologies Alachua, GA	achromatopsia <i>6 years and older</i>	Phase I/II www.agtc.com
ACP-015 (TRansCon CNP)	Ascendis Pharma Palo Alto, CA	achondroplasia <i>2 years to 10 years</i>	Phase II www.ascendispharma.com
AEB1102 (pegzilarginase) ORPHAN DRUG	Aeglea Biotherapeutics Austin, TX	arginase 1 deficiency (Fast Track) <i>2 years and older</i>	Phase III www.aegleabio.com
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 www.allergan.com
AGT-181 (enzyme replacement therapy) ORPHAN DRUG	ArmaGen San Diego, CA	mucopolysaccharidosis type I (Fast Track) <i>2 years and older</i>	Phase I/II completed www.armagen.com
AGTC-501 (AAV-gene transference) ORPHAN DRUG	Applied Genetic Technologies Alachua, FL	X-linked retinitis pigmentosa caused by RPGR mutations <i>6 years and older</i>	Phase I/II www.agtc.com

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 www.amo-pharma.com
arimoclomol ORPHAN DRUG	Orphazyme Newton, MA	Niemann-Pick disease type C (Fast Track) <i>2 years to 18 years</i>	Phase II/III www.orphazyme.com
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 www.amicusrx.com
avalglucosidase alfa (recombinant alpha glucosidase)	Sanofi Genzyme Cambridge, MA	Pompe disease <i>3 years and older</i>	Phase III www.sanofigenzyme.com
		Pompe disease <i>6 months to 17 years</i>	Phase II www.sanofigenzyme.com
AVR-RD-01 (lentiviral-based gene therapy) ORPHAN DRUG	AvroBio Cambridge, MA	Fabry disease <i>16 years and older</i>	Phase I/II www.avrobio.com
BCX7353 (serine protease inhibitor) ORPHAN DRUG	BioCryst Pharmaceuticals Durham, NC	hereditary angioedema (Fast Track) <i>12 years and older</i>	Phase III www.biocryst.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
BPM31510 topical cream ORPHAN DRUG	Berg Framingham, MA	epidermolysis bullosa <i>12 years and older</i>	Phase I www.berghealth.com
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 www.insysrx.com
CAP-1002 (allogeneic cardiosphere-derived stem cell therapeutic) ORPHAN DRUG	Capricor Beverly Hills, CA	Duchenne muscular dystrophy <i>10 years and older</i>	Phase II www.capricor.com
casimersen (SRP-4045) (RNA interference)	Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy <i>7 years to 13 years</i>	Phase III www.sarepta.com
CCP-020 (diacerein topical ointment) ORPHAN DRUG	Castle Creek Pharmaceuticals Parsippany, NJ	epidermolysis bullosa (Fast Track) <i>6 months and older</i>	Phase II www.castlecreekpharma.com
CLN3 gene therapy	Amicus Therapeutics Cranbury, NJ	CLN3 Batten disease <i>3 years to 10 years</i>	Phase I/II www.amicusrx.com

Genetic Disorders

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

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 www.stealthbt.com
		Barth syndrome (Fast Track) <i>12 years and older</i>	Phase II/III www.stealthbt.com
EPI-743 (NQO1 modulator) ORPHAN DRUG	BioElectron Technology Mountain View, CA	Rett syndrome <i>up to 18 years</i>	Phase II completed www.bioelectron.com
Epidiolex® 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 www.greenwichbiosciences.com
EryDex dexamethasone erythrocyte- encapsulated ORPHAN DRUG	EryDel Bresso, Italy	ataxia telangiectasia <i>6 years and older</i>	Phase III www.erydel.com
Firazyr® icatibant	Takeda Deerfield, IL	hereditary angioedema <i>2 years to 17 years</i>	Phase III completed www.takeda.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Firdapse® amifampridine ORPHAN DRUG	Catalyst Pharmaceuticals Coral Gables, FL	congenital myasthenic syndromes <i>2 years and older</i>	Phase III www.catalystpharma.com
		spinal muscular atrophy type 3 <i>6 years and older</i>	Phase II www.catalystpharma.com
Galafold® migalastat	Amicus Therapeutics Cranbury, NJ	Fabry's disease <i>12 years to 17 years</i>	Phase III www.amicusrx.com
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 www.sarepta.com
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 www.marinuspharma.com
givinostat (HDAC inhibitor)	Italfarmaco Milan, Italy	Duchenne muscular dystrophy <i>6 years to 17 years</i>	Phase III www.italfarmaco.com
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 www.gensight-biologics.com

Genetic Disorders

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

Genetic Disorders

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

Genetic Disorders

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

Genetic Disorders

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

Genetic Disorders

Drug Name

Sponsor

Indication

Development Phase

omigapil
(GAPDH inhibitor)
ORPHAN DRUG

Santhera Pharmaceuticals
Burlington, MA

congenital muscular dystrophy
(Fast Track) *5 years to 16 years*

Phase I completed
www.sathera.com

ORGN001
(cPMP replacement therapy)
ORPHAN DRUG

Origin Biosciences
San Francisco, CA

molybdenum cofactor deficiency
(Breakthrough Therapy)
up to 28 days

Phase II/III
www.origintx.com

molybdenum cofactor deficiency
all ages

Phase II
www.origintx.com

OT-58
(enzyme replacement therapy)

Orphan Technologies
Rapperswil, Switzerland

homocystinuria
12 years and older

Phase I/II
www.orphantechnologies.com

P-188 NF
(blood cell modulator)
ORPHAN DRUG

Phrixus Pharmaceuticals
Ann Arbor, MI

Duchenne muscular dystrophy
12 years to 25 years

Phase II
www.phrixuspharmaceuticals.com

palovarotene
(retinoic acid receptor
gamma agonist)
ORPHAN DRUG

Clementia Pharmaceuticals
Montreal, Canada

fibrodysplasia ossificans progressiva
(Fast Track) *4 years and older*

Phase III
www.clementiapharma.com

multiple osteochondromas
2 years to 14 years

Phase II
www.clementiapharma.com

Genetic Disorders

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

Genetic Disorders

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

Genetic Disorders

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

Genetic Disorders

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

Genetic Disorders

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

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
SRK-015 (anti-promyostatin mAb) ORPHAN DRUG	Scholar Rock Cambridge, MA	spinal muscular atrophy, type 2 or 3 <i>2 years to 21 years</i>	Phase II www.scholarrock.com
SRP-5051 (RNA-targeted therapy PPMO)	Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy <i>7 years to 21 years</i>	Phase II www.sarepta.com
SRP-9001 (gene therapy)	Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy <i>4 years to 7 years</i>	Phase II www.sarepta.com
SRP-9003 (AAV-Rh74 vectored gene therapy) ORPHAN DRUG	Sarepta Therapeutics Cambridge, MA	limb girdle muscular dystrophy-2E <i>4 years to 15 years</i>	Phase I/II www.sarepta.com
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 www.sarepta.com
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 www.ab2bio.com
TAK611 (SHP611) (enzyme replacement therapy) ORPHAN DRUG	Takeda Deerfield, IL	late infantile metachromatic leukodystrophy <i>6 months to 72 months</i>	Phase II www.takeda.com

Genetic Disorders

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

Genetic Disorders

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

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 www.vrtx.com
		primary ciliary dyskinea (with Kalydeco®) <i>12 years and older</i>	Phase II completed www.vrtx.com
VX-440/ivacaftor/tezacaftor	Vertex Pharmaceuticals Boston, MA	cystic fibrosis <i>12 years and older</i>	Phase II completed www.vrtx.com
VX-445/ivacaftor/tezacaftor	Vertex Pharmaceuticals Boston, MA	cystic fibrosis (Breakthrough Therapy) <i>12 years and older</i>	application submitted www.vrtx.com
VX-659/ivacaftor/tezacaftor	Vertex Pharmaceuticals Boston, MA	cystic fibrosis <i>12 years and older</i>	Phase III www.vrtx.com
Vyondys53™ golodirsen (SRP-4053) ORPHAN DRUG	Sarepta Therapeutics Cambridge, MA	Duchenne muscular dystrophy <i>children and adolescents</i>	application submitted www.sarepta.com
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 www.wavelifesciences.com

Genetic Disorders

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Zolgensma® 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 www.avexis.com
ZX008 (fenfluramine low dose) ORPHAN DRUG	Zogenix Emeryville, CA	Dravet syndrome (Fast Track) <i>2 years and older</i>	application submitted www.zogenix.com

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 www.janssen.com www.bavarian-nordic.com
AeroVanc 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 www.savarapharma.com
Alinia® nitazoxanide	Romark Laboratories Tampa, FL	enterovirus infections, rhinovirus infections, uncomplicated influenza <i>12 years and older</i>	Phase III www.romark.com
		gastroenteritis, norovirus infections <i>12 years and older</i>	Phase II www.romark.com

Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Arikayce® 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 www.insmed.com
ARV-1801 (fusidic acid oral) ORPHAN DRUG	Arrebus Raleigh, NC	acute bacterial skin and skin structure infections <i>12 years and older</i>	Phase III completed www.arrebus.com
		staphylococcal bone or joint infections <i>12 years and older</i>	Phase II/III completed www.arrebus.com
Bexsero® 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 www.gsk.com
Biktarvy® 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 www.gilead.com
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 www.armatapharma.com

Infectious Diseases

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

Infectious Diseases

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

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 www.gilead.com
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 www.gsk.com
		respiratory syncytial virus infections <i>6 months to 7 months</i>	Phase I www.gsk.com
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 www.gsk.com
Harvoni® ledipasvir/sofosbuvir	Gilead Sciences Foster City, CA	hepatitis C infections <i>3 years to 17 years</i>	Phase II completed www.gilead.com
IBP-9414 (bacteria replacement) ORPHAN DRUG	Infant Bacterial Therapeutics Stockholm, Sweden	necrotizing enterocolitis <i>up to 48 hours</i>	Phase III www.ibtherapeutics.com
ibrexafungerp (glucan synthase inhibitor) ORPHAN DRUG	SCYNEXIS Jersey City, NJ	vulvovaginal candidiasis (Fast Track) <i>12 years and older</i>	Phase III www.scynexis.com

Infectious Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Infanrix hexa 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 www.gsk.com
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 www.janssen.com
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 www.janssen.com
M2SR (influenza vaccine)	FluGen Madison, WI	seasonal influenza <i>9 years to 17 years</i>	Phase I www.flugen.com
Mavyret® glecaprevir/pibrentasvir	AbbVie North Chicago, IL	hepatitis C infections <i>3 years to 17 years</i>	Phase III www.abbvie.com
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 www.medimmune.com

Infectious Diseases

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

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 www.novustherapeutics.com
Orbactiv™ oritavancin	Melinta Therapeutics Morristown, NJ	bacterial infections <i>up to 18 years</i>	Phase I www.melinta.com
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 www.pfizer.com
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 www.janssen.com
Prevymis® letermovir	Merck Kenilworth, NJ	cytomegalovirus infections following stem cell transplantation <i>up to 17 years</i>	Phase II www.merck.com
Prezcobix® cobicistat/darunavir FDC tablet	Janssen Research & Development Raritan, NJ	HIV-1 infections <i>12 years to 17 years</i>	Phase I www.janssen.com
quadrivalent influenza vaccine	Seqirus Summit, NJ	influenza <i>6 months to 59 months</i>	Phase III completed www.seqirus.com

Infectious Diseases

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

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

Infectious Diseases

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

Infectious Diseases

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

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 www.advicenne.com
ALXN1210 (ravulizumab-cwvz) ORPHAN DRUG	Alexion Pharmaceuticals Boston, MA	atypical hemolytic uremic syndrome <i>up to 17 years</i>	Phase III www.alexion.com
avacopan (complement C5a receptor inhibitor) ORPHAN DRUG	ChemoCentryx Mountain View, CA	C3 glomerulopathy <i>12 years and older</i>	Phase II www.chemocentryx.com
DCR-PHXC (RNA interference) ORPHAN DRUG	Dicerna Pharmaceuticals Cambridge, MA	primary hyperoxaluria <i>6 years and older</i>	Phase II www.dicerna.com
Hectorol® doxercalciferol capsules	Sanofi Bridgewater, NJ	secondary hyperparathyroidism in chronic kidney disease <i>5 years to 18 years</i>	Phase III www.sanofi.com
lixivaptan (vasopressin V2 receptor antagonist) ORPHAN DRUG	Palladio Biosciences Newtown, NY	autosomal recessive polycystic kidney disease <i>15 years and older</i>	Phase II www.palladiobio.com

Kidney Diseases

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

Kidney Diseases

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

Liver Diseases

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

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 www.immuron.com.au
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 www.mirumpharma.com
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 www.albireopharma.com

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 www.arborpharma.com
AR33	Arbor Pharmaceuticals Atlanta, GA	mental disorders <i>pediatric</i>	Phase II www.arborpharma.com

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 www.boehringer-ingelheim.com
dasotraline (serotonin, norepinephrine and dopamine reuptake inhibitor)	Sunovion Marlborough, MA	attention-deficit/hyperactivity disorder (ADHD) <i>children and adolescents</i>	application submitted www.sunovion.com
Fetzima® levomilnacipran ER	Allergan Madison, NJ	major depressive disorder <i>7 years to 17 years</i>	Phase III www.allergan.com
Geodon® ziprasidone	Pfizer New York, NY	bipolar 1 disorder <i>10 years to 17 years</i>	Phase III www.pfizer.com
HLD-100 (dextroamphetamine sulfate modified release)	Ironshore Pharmaceuticals Durham, NC	ADHD <i>6 years to 12 years</i>	Phase II completed www.ironshorepharma.com
KP415 (serdexmethylphenidate)	KemPharm Celebration, FL	ADHD <i>6 years to 12 years</i>	Phase III www.kempharm.com
KP484 (serdexmethylphenidate)	KemPharm Celebration, FL	ADHD <i>6 years to 12 years</i>	Phase III www.kempharm.com
Lexapro® escitalopram	Allergan Madison, NJ	generalized anxiety disorder <i>7 years to 17 years</i>	Phase III www.allergan.com

Mental Illness

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

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 www.trispharma.com
TV-46000 (extended release injectable suspension risperidone)	Teva Pharmaceuticals North Wales, PA	schizophrenia <i>13 years and older</i>	Phase III www.tevausea.com
Vilbryd® vilazodone	Allergan Madison, NJ	major depressive disorder <i>7 years to 17 years</i>	Phase III completed www.allergan.com
Vraylar® cariprazine	Allergan Madison, NJ	schizophrenia <i>13 years to 17 years</i>	Phase III www.allergan.com
Vyvanse® lisdexamfetamine	Takeda Deerfield, IL	ADHD <i>4 years to 5 years</i>	Phase III completed www.takeda.com
Zulresso™ brexanolone	Sage Therapeutics Cambridge, MA	postpartum depression <i>15 years to 17 years</i>	Phase III www.sagerx.com

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 www.aadibio.com
		surgically refractory epilepsy <i>3 years to 26 years</i>	Phase I www.aadibio.com
Aimovig® erenumab	Amgen Thousand Oaks, CA Novartis East Hanover, NJ	chronic and episodic migraine <i>6 years to 17 years</i>	Phase III www.amgen.com www.novartis.com
Aubagio® teriflunomide	Sanofi Genzyme Cambridge, MA	relapsing multiple sclerosis <i>10 years to 18 years</i>	Phase III www.sanofigenzyme.com
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 www.auctapharma.com www.etonpharma.com
Austedo® 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 www.tevausa.com

Neurologic Disorders

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

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 www.emalexbiosciences.com
Emgality® galcanezumab-gnlm	Eli Lilly Indianapolis, IN	episodic migraine <i>6 years to 17 years</i>	Phase III www.lilly.com
EPI-743 (NQ01 modulator) ORPHAN DRUG	BioElectron Technology Mountain View, CA	Leigh syndrome <i>1 year to 18 years</i>	Phase II www.bioelectron.com
Epidiolex® cannabidiol ORPHAN DRUG	Greenwich Biosciences Carlsbad, CA	infantile spasms <i>1 month to 24 months</i>	Phase III www.greenwichbiosciences.com
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 www.retrophin.com
Fycompa® 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 www.eisai.com
		epilepsy <i>1 month to 24 months</i> epileptic syndrome, partial onset seizures <i>1 month to 18 years</i>	Phase II www.eisai.com

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

Neurologic Disorders

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

Neurologic Disorders

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

Respiratory Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Aerosurf® 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 www.windtreetx.com
AirDuo® Digihaler™ salmeterol/fluticasone propionate	Teva North America North Wales, PA	asthma <i>4 years to 11 years</i>	Phase III completed www.tevapharm.com
AirDuo™ RespiClick® fluticasone propionate and salmeterol inhalation powder	Teva Pharmaceutical North Wales, PA	asthma <i>4 years to 11 years</i>	Phase III completed www.tevapharm.com
ArmonAir™ RespiClick® fluticasone propionate inhalation powder	Teva Pharmaceutical North Wales, PA	asthma <i>4 years to 11 years</i>	Phase III completed www.tevapharm.com
AZD7594 (inhaled SGRM)	AstraZeneca Wilmington, DE	asthma <i>12 years to 17 years</i>	Phase I www.astrazeneca.com
Breo Ellipta® fluticasone furoate/vilanterol	GlaxoSmithKline Research Triangle Park, NC	asthma <i>5 years to 17 years</i>	Phase III www.gsk.com
Cinqair® reslizumab	Teva Pharmaceuticals North Wales, PA	asthma <i>12 years and older</i>	Phase III completed www.tevausa.com

Respiratory Diseases

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

Respiratory Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Pneumostem® mesenchymal stem cell therapy ORPHAN DRUG	Medipost Seoul, South Korea	bronchopulmonary dysplasia <i>up to 14 days</i>	Phase I/II completed www.medi-post.com
PT001 (inhaled glycopyrrolate)	Pearl Therapeutics (AstraZeneca) Redwood City, CA	asthma <i>12 years and older</i>	Phase II/III www.astrazeneca.com
PT027 (budesonide/salbutamol)	Avillion London, United Kingdom Pearl Therapeutics (AstraZeneca) Redwood City, CA	asthma <i>4 years and older</i>	Phase III www.avillionllp.com www.astrazeneca.com
QMF149 (indacaterol/mometasone)	Novartis East Hanover, NJ	asthma <i>12 years and older</i>	Phase III www.novartis.com
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 www.takeda.com
tezepelumab (TSLP inhibitor)	Amgen Thousand Oaks, CA AstraZeneca Wilmington, DE	severe asthma (Breakthrough Therapy) <i>12 years and older</i>	Phase III www.amgen.com www.astrazeneca.com
VR647 (budesonide inhalation)	Vectura Chippenham, United Kingdom	asthma <i>4 years to 8 years</i>	Phase I completed www.vectura.com

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 www.taro.com/usa
ALX-101 (liver X receptor agonist)	Ralexar Therapeutics Malvern, PA	atopic dermatitis <i>12 years and older</i>	Phase II www.ralexar.com
ARQ-151	Arcutis Biotherapeutics Westlake Village, CA	plaque psoriasis <i>12 years and older</i>	Phase III www.arcutis.com
		atopic dermatitis <i>12 years and older</i>	Phase II www.arcutis.com
ARQ-154	Arcutis Biotherapeutics Westlake Village, CA	plaque psoriasis <i>12 years and older</i>	Phase II www.arcutis.com
B244 (ammonia oxidizing bacteria-based therapeutic)	AOBiome Cambridge, MA	atopic dermatitis <i>2 years to 17 years</i>	Phase I/II www.aobiome.com
BI 730357	Boehringer Ingelheim Ridgefield, CT	plaque psoriasis <i>all ages</i>	Phase II www.boehringer-ingelheim.com
BMS-986165 (TYK2 kinase inhibitor)	Bristol-Myers Squibb Princeton, NJ	plaque psoriasis <i>all ages</i>	Phase III www.bms.com

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

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 www.mimedx.com
Duobrii ® halobetasol propionate and tazarotene	Bausch Health Bridgewater, NJ	plaque psoriasis <i>4 years to 17 years</i>	Phase III www.bauschhealth.com
Dupixent ® 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 www.regeneron.com www.sanofi.com
FCD105 (adapalene/minocycline foam)	Foamix Pharmaceuticals Bridgewater, NJ	acne vulgaris <i>12 years and older</i>	Phase II www.foamix.com
FMX101 (minocycline foam)	Foamix Pharmaceuticals Bridgewater, NJ	moderate to severe acne vulgaris <i>all ages</i>	application submitted www.foamix.com
IDP-120 (benzoyl peroxide/tretinoin)	Bausch Health Bridgewater, NJ	acne vulgaris <i>9 years and older</i>	Phase III www.bauschhealth.com
IDP-123 (tazarotene lotion)	Bausch Health Bridgewater, NJ	acne vulgaris <i>9 years and older</i>	application submitted www.bauschhealth.com

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

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

Skin Disorders

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

Skin Disorders

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

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 www.galderma.com
TWIN (benzoyl peroxide/tretinoin)	Sol-Gel Technologies Ness Ziona, Israel	acne vulgaris <i>9 years and older</i>	Phase III www.sol-gel.com
Ultravate® halobetasol propionate lotion	Sun Pharmaceutical Mumbai, India	plaque psoriasis <i>12 years to 16 years</i>	Phase III www.sunpharma.com
upadacitinib (ABT-494) (JAK1 inhibitor)	AbbVie North Chicago, IL	atopic dermatitis <i>12 years and older</i>	Phase III www.abbvie.com
		atopic dermatitis <i>2 years to 11 years</i>	Phase I www.abbvie.com
Winlevi™ clascoterone	Cassiopea Milan, Italy	acne vulgaris <i>9 years and older</i>	application submitted www.cassiopea.com

Transplantation

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

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 www.equillium.com
Imbruvica® ibrutinib	Janssen Research & Development Raritan, NJ Pharmacyclics Sunnyvale, CA	chronic graft-versus-host disease <i>1 year to 21 years</i>	Phase I/II www.janssen.com www.pharmacyclics.com
itacitinib (JAK 1 inhibitor)	Incyte Wilmington, DE	graft-versus-host disease <i>up to 17 years</i>	Phase I/II www.incyte.com
remestemcel-L (cell replacement therapy) ORPHAN DRUG	Mesoblast New York, NY	acute graft-versus-host disease (Fast Track) <i>pediatric</i>	application submitted www.mesoblast.com
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 www.bellicum.com
ruxolitinib	Incyte Wilmington, DE	chronic graft-versus-host disease <i>12 years and older</i>	Phase I www.incyte.com
SNDX-6352 (IgG4 mAb)	Syndax Pharmaceuticals Waltham, MA	chronic graft-versus-host disease <i>12 years and older</i>	Phase I www.syndax.com

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 www.xenikos.com
Uvadex® methoxsalen	Mallinckrodt St. Louis, MO	graft-versus-host disease with CELLEX® Photopheresis System <i>1 year to 21 years</i>	Phase III www.mallinckrodt.com
Zemaira® 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 www.cslbehring.com
Zortress® everolimus	Novartis East Hanover, NJ	prevention of kidney transplant rejection <i>1 year to 18 years</i>	Phase III completed www.novartis.com

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 www.ascendispharma.com
Alkindi® hydrocortisone	Diurnal Cardiff, United Kingdom	adrenal insufficiency <i>birth to 17 years</i>	application submitted www.diurnal.co.uk

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 www.arborpharma.com
BAY-2880376 (caffeine/naproxen)	Bayer Pharmaceutical Whippany, NJ	postoperative pain <i>16 years and older</i>	Phase II www.pharma.bayer.com
BBI-4000 (sofipirionium bromide)	Brickell Biotech Boulder, CO	primary axillary hyperhidrosis <i>12 years and older</i>	Phase III www.brickellbio.com
		primary axillary hyperhidrosis <i>9 years to 16 years</i>	Phase II www.brickellbio.com
Botox® onabotulinumtoxin A	Allergan Madison, NJ	urinary incontinence due to overactive bladder <i>12 years to 17 years</i>	Phase III www.allergan.com
Cymbalta® duloxetine	Eli Lilly Indianapolis, IN	fibromyalgia <i>12 years to 17 years</i>	Phase III completed www.lilly.com
Exparel® bupivacaine liposome injectable suspension	Pacira BioSciences Parsippany, NJ	postoperative pain <i>6 years to 17 years</i>	Phase III www.pacira.com
Hetlioz® tasimelteon	Vanda Pharmaceuticals Washington, DC	non-24 sleep-wake disorder <i>pediatric</i>	Phase III www.vandapharma.com

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

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 www.nevakar.com
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 www.endo.com
Pedmark™ sodium thiosulfate ORPHAN DRUG	Fennec Pharmaceuticals Research Triangle Park, NC	chemotherapy-induced sensorineural hearing loss <i>1 month to 17 years</i>	application submitted www.fennecpharma.com
PF-06651600 (JAK3/TEC inhibitor)	Pfizer New York, NY	alopecia areata <i>12 years and older</i>	Phase III www.pfizer.com
Qsymia® phentermine/topiramate	VIVUS Campbell, CA	obesity <i>12 years to 16 years</i>	Phase III www.vivus.com
Rituxan® rituximab ORPHAN DRUG	Biogen Cambridge, MA Genentech South San Francisco, CA	granulomatosis with polyangiitis or microscopic polyangiitis (combination therapy) <i>2 years and older</i>	application submitted www.biogen.com www.gene.com
		granulomatosis with polyangiitis or microscopic polyangiitis (monotherapy) <i>2 years to 17 years</i>	Phase II completed www.biogen.com www.gene.com

Other Diseases

<u>Drug Name</u>	<u>Sponsor</u>	<u>Indication</u>	<u>Development Phase</u>
Soliris® eculizumab ORPHAN DRUG	Alexion Pharmaceuticals Boston, MA	generalized myasthenia gravis <i>6 years to 17 years</i>	Phase III www.alexion.com
somapacitsan (long acting growth hormone)	Novo Nordisk Plainsboro, NJ	growth hormone deficiency <i>2 years to 11 years</i>	Phase III www.novonordisk.com
		short stature in children born small for gestational age <i>2 years to 11 years</i>	Phase II www.novonordisk.com
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 www.opko.com www.pfizer.com
Victoza® liraglutide	Novo Nordisk Plainsboro, NJ	weight management in obesity <i>12 years to 17 years</i>	Phase III completed www.novonordisk.com
		weight management in Prader-Willi syndrome <i>6 years to 17 years</i>	Phase III www.novonordisk.com
		obesity <i>7 years to 11 years</i>	Phase I completed www.novonordisk.com

The content of this report has been obtained through public, government and industry sources, and the Springer "Adis Insight" database based on the latest information. Report current as of **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. The entire series of *Medicines in Development* is available on PhRMA's website: www.phrma.org.

Definitions

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

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

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

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