PIPELINE REPORT
First Quarter 2015

Information on recently approved, soon-to-be-approved and Phase 3 trial specialty medications

The Walgreens Pipeline Report provides a summary of the specialty medications that may be approved by the FDA within the next few years. While not all-inclusive, this report focuses on medications in Phase 3 studies that may impact treatment for certain specialty disease states or conditions.

Drug information for approved products should be reviewed using the prescribing information (PI). For full PI, please refer to the DailyMed website. The medications with an asterisk indicate they have a boxed warning.

Note: This report is not intended for use by patients.

Walgreens

Medications recently approved

Manufacturer/ Drug name	Indication	Mechanism of action/ Drug class	Route of administration	Approval date	Comments
Bleeding disorders					
Baxter/Obizur™ (recombinant antihemophilic factor, porcine sequence)	For the treatment of bleeding episodes in adults with acquired hemophilia A (AHA)	Promotes blood clotting/Factor replacement therapy	IV infusion	10/23/14	• First recombinant porcine factor VIII treatment approved for AHA that allows physicians to manage the treatment's efficacy and safety by measuring factor VIII activity levels in addition to clinical assessments
Cancer					
Amgen/Blincyto™ (blinatumomab*)	For the treatment of Philadelphia chromosome-negative relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL)	Activates immune system to target cancer cells/Bispecific CD19-directed CD3 T-cell engager (BiTE®) antibody	IV infusion	12/3/14	• First FDA-approved BiTE® antibody
AstraZeneca/Lynparza™ (olaparib)	As monotherapy in patients with deleterious or suspected deleterious germline *BRCA* mutated (as detected by an FDA-approved test) advanced ovarian cancer who have been treated with three or more prior lines of chemotherapy	Inhibits cell growth and survival/Poly ADP-ribose polymerase (PARP) inhibitor	Oral	12/19/14	• First FDA-approved PARP inhibitor
Bristol-Myers Squibb/Opdivo™ (nivolumab)	For the treatment of patients with unresectable or metastatic melanoma and disease progression following Yervoy® (ipilimumab) and, if *BRAF* V600 mutation positive, a *BRAF* inhibitor	Activates immune system to target cancer cells/Programmed death receptor-1 (PD-1) inhibitor	IV infusion	12/22/14	• Second PD-1 inhibitor approved for the treatment of melanoma
Eli Lilly/Cyramza® (ramucirumab*)	In combination with paclitaxel* for the treatment of patients with advanced or metastatic, gastric or gastro-esophageal junction (GEJ) adenocarcinoma with disease progression on or after prior fluoropyrimidine- or platinum-containing chemotherapy In combination with docetaxel* for the treatment of patients with metastatic non-small cell lung cancer (NSCLC) with disease progression on or after platinum-based chemotherapy	Reduces tumor cell growth and blood supply/ Vascular endothelial growth factor receptor-2 (VEGFR2) antagonist	IV infusion	11/5/14 12/12/14	• Previously approved as a single agent for the treatment of gastric or GEJ cancer
Genentech/Avastin® (bevacizumab*)	In combination with paclitaxel*, pegylated liposomal doxorubicin* or topotecan* for the treatment of patients with platinum-resistant recurrent epithelial ovarian, fallopian tube or primary peritoneal cancer who received no more than two prior chemotherapy regimens	Reduces tumor cell growth and blood supply/Vascular endothelial growth factor (VEGF) inhibitor	IV infusion	11/14/14	• Previously approved for the treatment of metastatic colorectal cancer, non-squamous NSCLC, glioblastoma, metastatic renal cell carcinoma (RCC), and metastatic carcinoma of the cervix

*These medications have a boxed warning.

Medications recently approved (continued)

Manufacturer/ Drug name	Indication	Mechanism of action/ Drug class	Route of administration	Approval date	Comments
Cancer					
Incyte Corporation/Jakafi® (ruxolitinib)	For treatment of patients with polycythemia vera who have had an inadequate response to or are intolerant of hydroxyurea	Inhibits the formation and development of blood cells/Janus kinase (JAK) inhibitor	Oral	12/4/14	• Previously approved for treatment of patients with intermediate or high-risk myelofibrosis
Ipsen/Somatuline® Depot (lanreotide)	For the treatment of patients with unresectable, well or moderately differentiated, locally advanced or metastatic gastroenteropancreatic neuroendocrine tumors (GEP-NETs) to improve progression-free survival	Binds somatostatin receptors/Somatostatin analogue	Subcutaneous injection	12/16/14	• Previously approved for the treatment of acromegaly
Cystic fibrosis					
Vertex Pharmaceuticals/ Kalydeco® (ivacaftor)	For the treatment of cystic fibrosis (CF) in patients ages 6 years and older who have an *R117H* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene	Increases chloride ion transport across cell membranes/*CFTR* potentiator	Oral	12/29/14	• Previously approved or the treatment of CF in patients ages 6 years and older who have one of the following mutations in the *CFTR* gene: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N*, or *S549R*
Hepatitis					
AbbVie/Viekira Pak™ (ombitasvir, paritaprevir, and ritonavir* tablets; dasabuvir tablets)	With or without ribavirin* for the treatment of patients with genotype 1 chronic hepatitis C virus (HCV) infection, including those with compensated cirrhosis	Prevents virus replication/NS5A inhibitor (ombitasvir), NS3/4A protease inhibitor (paritaprevir) human immunodeficiency virus type 1 (HIV-1) protease inhibitor (ritonavir*) and non-nucleoside NS5B palm polymerase inhibitor (dasabuvir)	Oral	12/19/14	• Viekira Pak is ombitasvir, paritaprevir, ritonavir* fixed dose combination tablets copackaged with dasabuvir tablets
Gilead Sciences/Harvoni® (ledipasvir and sofosbuvir)	For the treatment of chronic HCV infection in genotype 1 patients	Prevents virus replication/NS5A inhibitor (ledipasvir) and NS5B polymerase inhibitor (sofosbuvir)	Oral	10/10/14	• First once-daily single tablet regimen approved for this indication
Human immunodeficiency virus					
Gilead Sciences/Tybost® (cobicistat)	To increase systemic exposure of atazanavir or darunavir (once daily dosing regimen) in combination with other antiretroviral agents in the treatment of HIV-1 infection	Inhibits cytochrome P4503A/ Pharmacoenhancer	Oral	9/24/14	• Cobicistat was first approved as a component of Stribild® (elvitegravir, cobicistat, emtricitabine*, tenofovir disoproxil fumarate*), a once-daily single-tablet complete regimen for the treatment of HIV-1 infection in adults who are antiretroviral treatment-naïve
Gilead Sciences/Vitekta® (elvitegravir)	In combination with a human immunodeficiency virus (HIV) protease inhibitor coadministered with ritonavir* and with other antiretroviral drug(s) for the treatment of HIV-1 infection in antiretroviral treatment-experienced adults	Prevents virus replication/Integrase strand transfer inhibitor	Oral	9/24/14	• Elvitegravir was also first approved as a component of Stribild®

*These medications have a boxed warning.

Medications recently approved (continued)

Manufacturer/ Drug name	Indication	Mechanism of action/ Drug class	Route of administration	Approval date	Comments
Inflammatory diseases					
Celgene/Otezla® (apremilast)	For the treatment of patients with moderate to severe plaque psoriasis who are candidates for phototherapy or systemic therapy	Modulates the inflammatory response/ Phosphodiesterase 4 inhibitor	Oral	9/23/14	• Previously approved for the treatment of psoriatic arthritis (PsA)
Multiple sclerosis					
Genzyme/Lemtrada™ (alemtuzumab*)	For the treatment of patients with relapsing forms of multiple sclerosis (MS)	Binds to the CD52 antigen on B-cells and T-cells/Therapeutic antibody	IV infusion	11/14/14	• Also marketed as Campath® for the treatment of B-cell chronic lymphocytic leukemia (CLL)
Neuroendocrine disorders					
Novartis/Signifor® LAR (pasireotide)	For the treatment of patients with acromegaly who have had an inadequate response to surgery and/or for whom surgery is not an option	Binds somatostatin receptors/Somatostatin analogue	IM injection	12/15/14	• Long-acting formulation of Signifor® (pasireotide diaspartate)
Ophthalmology					
Regeneron Pharmaceuticals/ Eylea® (aflibercept)	For the treatment of macular edema following branch retinal vein occlusion	Binds vascular endothelial growth factor and placental growth factor/Antiangiogenesis inhibitor	Intravitreal injection	10/6/14	• Previously approved for the treatment of neovascular age-related macular degeneration (AMD), macular edema following central retinal vein occlusion and diabetic macular edema (DME)
Pulmonary fibrosis					
Boehringer Ingelheim Pharmaceuticals/Ofev® (nintedanib)	For the treatment of idiopathic pulmonary fibrosis (IPF)	Targets growth factors/Kinase inhibitor	Oral	10/15/14	• One of first two FDA-approved products for the treatment of IPF
InterMune/Esbriet® (pirfenidone)	For the treatment of IPF	Unknown but thought to interfere with the production of transforming growth factor (TGF)-beta/Pyridone	Oral	10/15/14	• One of first two FDA-approved products for the treatment of IPF

*These medications have a boxed warning.

Pipeline medications in Phase 3 trials

Manufacturer/ Drug name	Indication	Mechanism of action/ Drug class	Route of administration	Comments
Asthma				
GlaxoSmithKline/Mepolizumab	For the treatment of severe eosinophilic asthma	Interferes with the inflammatory response/IL-15 inhibitor	Subcutaneous injection	• Biologics license application (BLA) filed November 2014
Teva Pharmaceutical/Reslizumab	For the treatment of patients with inadequately controlled moderate to severe asthma with elevated levels of blood eosinophils	Interferes with the inflammatory response/IL-15 inhibitor	IV infusion	• Primary endpoint achieved in two Phase 3 trials September 2014 • BLA filing planned for first half 2015
Bleeding disorders				
Baxter/BAX 111 (recombinant von Willebrand factor)	For the treatment of bleeding in patients with von Willebrand disease	Promotes blood clotting/Factor replacement therapy	IV infusion	• Designated as an orphan drug • Primary endpoint achieved in Phase 3 trial April 2014 • BLA filed December 2014
Baxter/BAX 855 (recombinant factor VIII)	For the treatment and prevention of bleeding in patients with hemophilia A	Promotes blood clotting/Factor replacement therapy	IV infusion	• Primary endpoint achieved in Phase 3 trial August 2014 • BLA filed December 2014
Bayer HealthCare/BAY 81-8973 (recombinant factor VIII)	For the treatment of hemophilia A in adults and children	Promotes blood clotting/Factor replacement therapy	IV infusion	• BLA filed December 2014
CSL Behring/CSL654 (recombinant coagulation factor IX with recombinant albumin, rIX-FP)	For the treatment and prevention of bleeding in patients with hemophilia B	Promotes blood clotting/Factor replacement therapy	IV infusion	• Designated as an orphan drug • BLA filed December 2014
Bone disease				
Radius Health/Abaloparatide-SC	For the treatment of postmenopausal osteoporosis	Stimulates new bone formation/ Human parathyroid hormone-related protein (hPTHrP) analogue	SC injection	• Primary endpoint achieved in Phase 3 trial December 2014 • BLA filing planned for second half 2015
Cancer				
Amgen/Talimogene laherparepvec	For the treatment of metastatic melanoma	Immunotherapy/Initiates an immune response to target cancer cells that have metastasized	Intralesional	• Designated as an orphan drug • BLA filed in 2014 • A response to the BLA is expected July 2015
AstraZeneca/AZD9291	For the second-line treatment of patients with metastatic epidermal growth factor receptor (EGFR) T790M mutation-positive NSCLC	Inhibits the formation and development of blood cells/ Third-generation tyrosine kinase inhibitor (TKI)	Oral	• Designated as an orphan drug with fast-track status • FDA granted breakthrough therapy designation • New drug application (NDA) filing planned for second quarter 2015
AstraZeneca/Iressa (gefitinib)	For the first-line treatment of patients with advanced or metastatic EGFR mutation-positive NSCLC	Inhibits the formation and development of blood cells/TKI	Oral	• NDA filing accepted December 2014 • Previous FDA approval withdrawn in 2012

Pipeline medications in Phase 3 trials (continued)

Manufacturer/ Drug name	Indication	Mechanism of action/ Drug class	Route of administration	Comments
Cancer				
AstraZeneca, Array BioPharma/ Selumetinib	For the treatment of metastatic uveal melanoma	Inhibits cell growth and survival/ Mitogen-activated extracellular signal-regulated kinase (MEK) inhibitor	Oral	• Regulatory filing expected in 2015
Clovis Oncology/Rociletinib	For the treatment of EGFR T790M mutation-positive NSCLC	Targets the activating mutations to EGFR and inhibits T790M/EGFR inhibitor	Oral	• Designated as an orphan drug • FDA granted breakthrough therapy designation • NDA filing planned for mid-2015
CTI BioPharma/Opaxio (paclitaxel poliglumex)	For the treatment of ovarian cancer	Inhibits cell division/ Microtubule inhibitor	IV infusion	• Links paclitaxel to a biodegradable polyglutamate polymer that delivers more chemotherapy to tumor cells • Completed enrollment of Phase 3 trial January 2014
CTI BioPharma/Pacritinib	For the treatment of myelofibrosis (MF)	Inhibits the formation and development of blood cells/TKI	Oral	• FDA granted fast-track status • Top-line results from Phase 3 trial expected in first quarter 2015
Eisai/Lenvatinib	For the treatment of progressive radioiodine-refractory differentiated thyroid cancer	Inhibits cell growth and survival/TKI	Oral	• Designated as an orphan drug • NDA filed August 2014 • FDA granted priority review status • A response to the NDA expected April 2015
Eli Lilly/Necitumumab	For the treatment of metastatic squamous NSCLC	Reduces tumor cell growth and blood supply/EGFR inhibitor	IV infusion	• Primary endpoint achieved in Phase 3 trial • Filing anticipated end of 2014
Genentech/Cobimetinib	In combination with Zelboraf® (vemurafenib) for the treatment of *BRAF* V600 mutation-positive advanced melanoma	Inhibits cell growth and survival/ Mitogen-activated protein kinase (MAPK) inhibitor	Oral	• Designated as an orphan drug with fast-track status • Primary endpoint achieved in Phase 3 trial July 2014 • NDA filed December 2014
Janssen/Yondelis (trabectedin)	For the treatment of patients with advanced soft tissue sarcoma, including liposarcoma and leiomyosarcoma subtypes, who have received prior chemotherapy including an anthracycline	Prevents tumor cells from multiplying/Non-platinum antitumor agent	IV infusion	• Designated as an orphan drug • NDA filed November 2014
Novartis/Binimetinib	For the treatment of neuroblastoma RAS viral (v-ras) oncogene homolog (NRAS) mutant melanoma	Inhibits cell growth and survival/ MAPK inhibitor	Oral	• Designated as an orphan drug • Regulatory filings planned for 2016
Novartis/Midostaurin	For treatment of patients with FLT-3 mutated acute myeloid leukemia (AML)	Inhibits cell growth and survival/ Signal transduction inhibitor	Oral	• Designated as an orphan drug • Regulatory filings planned for 2015

Pipeline medications in Phase 3 trials (continued)

Manufacturer/ Drug name	Indication	Mechanism of action/ Drug class	Route of administration	Comments
Cancer				
Novartis/Farydak (panobinostat)	In combination with Velcade® (bortezomib) and dexamethasone for the treatment of relapsed or refractory multiple myeloma (MM)	Inhibits cell growth and survival/Histone deacetylase inhibitor	Oral	• Designated as an orphan drug • NDA accepted and granted priority review May 2014 • FDA extended the NDA review period by up to three months • A response to the NDA expected February 2015
Novartis/Sonidegib	For the treatment of advanced basal cell carcinoma	Inhibits hedgehog signaling pathway/Selective smoothened inhibitor	Oral	• NDA filed September 2014
Pfizer/Ibrance (palbociclib)	In combination with letrozole for the treatment ER+, HER2- advanced breast cancer who have not received previous systemic treatment	Prevents tumor cell progression/ Cyclin-dependent kinase inhibitor	Oral	• FDA granted breakthrough therapy designation • NDA filed August 2014 • FDA granted priority review status • A response to the NDA expected April 2015
Puma Biotechnology/Neratinib	For the extended adjuvant treatment of breast cancer	Inhibits cell growth and survival/TKI	Oral	• Primary endpoint achieved in Phase 3 trial July 2014 • Regulatory filings planned for first quarter 2016
Taiho Oncology/TAS-102 (trifluridine and tipiracil hydrochloride)	For the treatment of refractory metastatic colorectal cancer	Interferes with the function of DNA/ Antineoplastic nucleoside analogue (trifluridine) and enzyme inhibitor (tipiracil hydrochloride)	Oral	• FDA granted fast-track status • Primary endpoint achieved in Phase 3 trial May 2014 • Rolling NDA submission completed December 2014
Cystic fibrosis				
Vertex Pharmaceuticals/ Lumacaftor (VX-809)	In combination with Kalydeco® (ivacaftor) in patients CF who have two copies of the F508del mutation in the *CFTR* gene	Increases the movement of *CFTR* to the cell surface/*CFTR* corrector	Oral	• Designated as an orphan drug • FDA granted breakthrough therapy designation • NDA filed November 2014
Endocrine disorders				
NPS Pharmaceuticals/Natpara (recombinant human parathyroid hormone)	For the treatment of hypoparathyroidism	Replaces deficient hormone/ Hormone replacement therapy	Subcutaneous injection	• Designated as an orphan drug • BLA filed October 2013 • FDA extended the BLA review period by three months • A response to the BLA expected January 2015
Growth disorders				
Aeterna Zentaris/Macrilen (macimorelin acetate)	For the evaluation of adult growth hormone deficiency	Stimulates the secretion of growth hormone/Ghrelin receptor agonist	Oral	• Designated as an orphan drug • NDA filed November 2013 • Received a complete response letter November 2014 • FDA has requested a confirmatory clinical trial

Pipeline medications in Phase 3 trials (continued)

Manufacturer/ Drug name	Indication	Mechanism of action/ Drug class	Route of administration	Comments
Hepatitis				
Bristol-Myers Squibb/Daclatasvir and asunaprevir	For the treatment of chronic HCV infection in genotype 1b patients	Prevents virus replication/NS5A inhibitor (daclatasvir), NS3 inhibitor (asunaprevir)	Oral	• Company has decided not to pursue approval of the dual regimen of daclatasvir and asunaprevir and has therefore withdrawn its NDA for asunaprevir
Bristol-Myers Squibb/Daclatasvir	In combination with other agents for the treatment of chronic HCV infection	Prevents virus replication/NS5A inhibitor	Oral	• NDA filed March 2014 • Received a complete response letter November 2014 • FDA has requested additional data for daclatasvir in combination with other antiviral agents
Merck/Grazoprevir and elbasvir	For the treatment of chronic HCV infection	Prevents virus replication/NS3/4A inhibitor (grazoprevir), NS5A inhibitor (elbasvir)	Oral	• FDA granted breakthrough therapy designation • Phase 3 results expected in first half 2015 • NDA filing planned for 2015
Human immunodeficiency virus				
Gilead Sciences/Elvitegravir, cobicistat, emtricitabine* and tenofovir alafenamide	For the treatment of HIV-1 infection in adults	Prevents virus replication/Integrase inhibitor (elvitegravir), pharmacoenhancer (cobicistat), nucleoside reverse transcriptase inhibitor (emtricitabine*), nucleotide reverse transcriptase inhibitor (tenofovir alafenamide)	Oral	• Once-daily single tablet regimen • NDA filed November 2014
Huntington s disease				
Auspex Pharmaceuticals/ Dutetrabenazine (SD-809)	For the treatment of chorea associated with Huntington's disease	Vesicular monoamine transporter 2 (VMAT-2) inhibitor/Depletes monoamines from nerve terminals	Oral	• Designated as an orphan drug • Primary endpoint achieved in Phase 3 trial December 2014 • NDA filing planned for first half 2015
Hypercholesterolemia				
Amgen/Evolocumab	For the treatment of hypercholesterolemia	Increases the removal of low-density lipoprotein cholesterol from the blood/Proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitor	Subcutaneous injection	• Designated as an orphan drug for the treatment of homozygous familial hypercholesterolemia • BLA filed August 2014 • A response to the BLA is expected August 2015
Novartis/Pradigastat	For the treatment of familial chylomicronemia syndrome	Interferes with triglyceride synthesis/ Diacylglycerol acyltransferase-1 (DGAT-1) inhibitor	Oral	• NDA filing planned for 2014
Sanofi and Regeneron Pharmaceuticals/Praluent (alirocumab)	For the treatment of hypercholesterolemia	Increases the removal of low-density lipoprotein cholesterol from the blood/PCSK9 inhibitor	Subcutaneous injection	• Primary endpoint achieved in nine Phase 3 trials July 2014 • BLA filing planned for 2014

*These medications have a boxed warning.

Pipeline medications in Phase 3 trials (continued)

Manufacturer/ Drug name	Indication	Mechanism of action/ Drug class	Route of administration	Comments
Inflammatory diseases				
Amgen/Brodalumab	For the treatment of plaque psoriasis	Interferes with the inflammatory response/IL-17 inhibitor	Subcutaneous injection	• Primary endpoint achieved in three Phase 3 trials • BLA filing planned for 2015
Eli Lilly/Ixekizumab	For the treatment of plaque psoriasis	Interferes with the inflammatory response/IL-17 inhibitor	Subcutaneous injection	• Primary endpoint achieved in three Phase 3 trials August 2014 • BLA filing planned for first half 2015
Novartis/Cosentyx (secukinumab, AIN457)	For the treatment of plaque psoriasis	Interferes with the inflammatory response/IL-17A inhibitor	Subcutaneous injection	• BLA filed October 2013 • A response to the BLA is expected January 2015
Sanofi and Regeneron Pharmaceuticals/Sarilumab	For the treatment of rheumatoid arthritis (RA)	Interferes with the inflammatory response/IL-16 inhibitor	Subcutaneous injection	• Co-primary endpoints achieved in Phase 3 trial November 2013 • Phase 3 trials ongoing
Lambert Eaton myasthenic syndrome				
Catalyst Pharmaceutical Partners/ Firdapse (amifampridine phosphate)	For the treatment of symptoms associated with Lambert-Eaton myasthenic syndrome	Improves impulse conduction in nerve fibers/Potassium channel blocker	Oral	• Designated as an orphan drug • FDA granted breakthrough therapy designation • Co-primary endpoints achieved in Phase 3 trial September 2014 • Rolling NDA submission planned for early 2015
Lysosomal storage diseases				
Amicus Therapeutics and GlaxoSmithKline/Amigal (migalastat HCl)	For the treatment of Fabry disease	Binds to and stabilizes alpha-galactosidase/Alpha-galactosidase A enhancer	Oral	• Designated as an orphan drug • Primary endpoint achieved in stage 2 of first Phase 3 trial April 2014 • Co-primary endpoints achieved in second Phase 3 trial August 2014
Synageva BioPharma/ Sebelipase alfa	For the treatment of early and late onset lysosomal acid lipase (LAL) deficiency	Replaces deficient LAL/Enzyme replacement therapy	IV infusion	• Designated as an orphan drug with fast-track status • FDA granted breakthrough therapy designation for early onset LAL deficiency • Rolling BLA submission completed December 2014
Metabolic disorders				
Alexion Pharmaceuticals/ Asfotase alfa	For the treatment of hypophosphatasia	Normalizes the genetically defective metabolic process/Targeted enzyme replacement therapy	Subcutaneous injection	• Designated as an orphan drug with fast-track status • FDA granted breakthrough therapy designation • Rolling BLA submission completed December 2014
Multiple sclerosis				
Teva Pharmaceuticals/Laquinimod	For the treatment of relapsing-remitting MS	Inhibits autoimmune and inflammatory disease activity/ Immunomodulatory agent	Oral	• Third Phase 3 trial is ongoing, results are expected 2016 • This trial is being conducted under a special protocol assessment

Pipeline medications in Phase 3 trials (continued)

Manufacturer/ Drug name	Indication	Mechanism of action/ Drug class	Route of administration	Comments
Muscular dystrophy				
Prosensa Holding N.V./Drisapersen	For the treatment of Duchenne muscular dystrophy (DMD)	Enables production of a functional dystrophin protein/Exon skipping therapy	Subcutaneous injection	• Designated as an orphan drug with fast-track status • FDA granted breakthrough therapy designation • Initiated rolling NDA submission October 2014; expecting to complete the application in fourth quarter 2014
PTC Therapeutics/Translarna (ataluren)	For the treatment for nonsense mutation Duchenne muscular dystrophy (nmDMD)	Enables formation of a functioning protein/Protein restoration therapy	Oral	• Designated as an orphan drug with fast-track status • Enrollment completed in Phase 3 trial September 2014 • Initiated rolling NDA submission December 2014; expecting to complete the application fourth quarter 2015
Sarepta Therapeutics/Eteplirsen	For the treatment of DMD	Enables production of a functional dystrophin protein/Exon skipping therapy	IV infusion	• Designated as an orphan drug with fast-track status • NDA filing planned by mid 2015
Primary immunodeficiency				
ADMA Biologics/RI-002	For the treatment of primary immune deficiency disorders	Replaces deficient immunoglobulin/ Immune globulin	IV infusion	• Primary endpoint achieved in Phase 3 trial December 2014 • BLA filing planned for first half 2015
Pulmonary hypertension				
Actelion/Uptravi (selexipag)	For the treatment of pulmonary arterial hypertension (PAH)	Reduces vascular smooth muscle constriction/Prostacyclin receptor agonist	Oral	• Designated as an orphan drug • NDA filed December 2014

New dosage forms in the pipeline

Manufacturer/ Drug name	Indication	Mechanism of action/ Drug class	Current route of administration	Investigational route of administration†	Comments
Human immunodeficiency virus					
Janssen, Gilead Sciences/ Prezista (darunavir) and Tybost (cobicistat)	In combination with other antiretroviral agents for the treatment of HIV-1	Prevents virus replication/Protease inhibitor (darunavir) and pharmacoenhancer (cobicistat)	Oral	Oral	• New combination tablet • NDA filed March 2014 • A response to the NDA is expected March 2015
Merck/Isentress (raltegravir) and lamivudine*	In combination with other antiretroviral agents, for the treatment of HIV-1	Prevents virus replication/Integrase inhibitor (raltegravir) and nucleoside analogue reverse transcriptase inhibitor (lamivudine*)	Oral	Oral	• New combination tablet • NDA accepted for review June 2014
Multiple sclerosis					
Biogen Idec and AbbVie/ Zinbryta (daclizumab* high-yield process)	For the treatment of relapsing-remitting MS	Binds to the CD25 receptor on T-cells/ Therapeutic antibody	IV infusion	Subcutaneous injection	• Primary endpoint achieved in Phase 3 trial June 2014 • Filing planned for first half 2015 • Previously marketed as Zenapax® (daclizumab*) for the prevention of acute kidney rejection

*These medications have a boxed warning.
†Dosage form is not available. Only investigational route of administration is available at this time.

New indications in the pipeline

Manufacturer/ Drug name	Current indication	Investigational indication	Mechanism of action/ Drug class	Route of administration	Comments
Cancer					
Bristol-Myers Squibb/Opdivo™ (nivolumab)	For the treatment of patients with unresectable or metastatic melanoma and disease progression following Yervoy® (ipilimumab) and, if *BRAF* V600 mutation positive, a *BRAF* inhibitor	For the third-line treatment of squamous cell NSCLC	Activates immune system to target cancer cells/PD-1 inhibitor	IV infusion	• FDA granted fast-track status • Initiated rolling BLA submission April 2014; expecting to complete the application by the end of 2014
Celgene/Revlimid® (lenalidomide*)	For the treatment of previously treated MM, myelodysplastic syndromes and relapsed or refractory mantle cell lymphoma (MCL)	For the treatment of newly diagnosed MM	Possesses immunomodulatory, anti-inflammatory and antiangiogenic properties/Thalidomide analogue	Oral	• Primary endpoint achieved in Phase 3 trial July 2013 • Supplemental new drug application (sNDA) filed April 2014 • A response to the sNDA is expected February 2015
Pharmacyclics, Janssen/ Imbruvica® (ibrutinib)	For the treatment of patients with MCL or CLL who have received at least one prior therapy, and for CLL patients with a deletion of the short arm of chromosome 17	For the treatment of Waldenstrom's macroglobulinemia	Inhibits cell growth and survival/Bruton's tyrosine kinase (BTK) inhibitor	Oral	• FDA granted breakthrough therapy designation • Designated as an orphan drug • sNDA filed October 2014 • A response to the sNDA is expected April 2015
Cystic fibrosis					
Vertex Pharmaceuticals/ Kalydeco® (ivacaftor)	For the treatment of CF in patients ages 6 years and older who have one of the following mutations in the *CFTR* gene: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N*, *S549R*, or *R117H*	For treatment of CF patients ages 2 to 5 who have one of the following nine mutations in the *CFTR* gene: *G551D*, *G178R*, *S549N*, *S549R*, *G551S*, *G1244E*, *S1251N*, *S1255P* or *G1349D*	Increases chloride ion transport across cell membranes/*CFTR* potentiator	Oral	• sNDA filed October 2014
Inflammatory diseases					
AbbVie/Humira® (adalimumab*)	For the treatment of RA, polyarticular juvenile idiopathic arthritis, PsA, ankylosing spondylitis (AS), Crohn's disease (CD), ulcerative colitis (UC) and psoriasis	For the treatment of hidradenitis suppurativa	Targets tumor necrosis factor (TNF) alpha, which is involved in the inflammatory process/TNF inhibitor	Subcutaneous injection	• Primary endpoint achieved in Phase 3 trial September 2014 • Supplemental biologics license application (sBLA) filing expected in late 2014
Pfizer/Xeljanz® (tofacitinib*)	For the treatment of RA	For the treatment of moderate-to-severe chronic plaque psoriasis	Interferes with the inflammatory and immune responses/JAK inhibitor	Oral	• sNDA filing planned for 2015

*These medications have a boxed warning.

New indications in the pipeline (continued)

Manufacturer/ Drug name	Current indication	Investigational indication	Mechanism of action/ Drug class	Route of administration	Comments
Ophthalmology					
Genentech/Lucentis® (ranibizumab)	For the treatment of neovascular AMD, macular edema following retinal vein occlusion (RVO) and DME	For the treatment of diabetic retinopathy	Binds vascular endothelial growth factor/Antiangiogenesis inhibitor	Intravitreal injection	• FDA granted breakthrough therapy designation • sBLA filed August 2014 • FDA granted priority review status • A response to the sNDA is expected February 2015
Regeneron Pharmaceuticals/ Eylea® (aflibercept)	For the treatment of neovascular AMD, macular edema following RVO and DME	For the treatment of diabetic retinopathy in patients with DME	Binds vascular endothelial growth factor and placental growth factor/ Antiangiogenesis inhibitor	Intravitreal injection	• FDA granted breakthrough therapy designation • sBLA accepted and granted priority review December 2014 • A response to the sBLA expected March 2015

New biosimilars in the pipeline

Manufacturer/ Drug name	Reference Manufacturer/Product	Investigational indication	Mechanism of action/ Drug class	Route of administration	Comments
Cancer					
Apotex/Pegfilgrastim	Amgen/Neulasta® (pegfilgrastim)	To decrease the incidence of infection, as manifested by febrile neutropenia, in patients with non-myeloid malignancies receiving myelosuppressive anti-cancer drugs associated with a clinically significant incidence of febrile neutropenia	Stimulates production of neutrophils/Granulocyte colony-stimulating factor	Subcutaneous injection	• BLA accepted December 2014 • BLA filed under the 351(k) abbreviated approval pathway
Sandoz/Zarzio (filgrastim)	Amgen/Neupogen® (filgrastim)	To decrease the incidence of infection, as manifested by febrile neutropenia, in patients with non-myeloid malignancies receiving myelosuppressive anti-cancer drugs associated with a significant incidence of severe neutropenia with fever	Stimulates production of neutrophils/Granulocyte colony-stimulating factor	Subcutaneous injection, IV infusion, or subcutaneous infusion	• BLA accepted July 2014 • BLA filed under the 351(k) abbreviated approval pathway
Inflammatory diseases					
Celltrion/Remsima (infliximab*)	Janssen/Remicade® (infliximab*)	For the treatment of CD, UC, RA, AS, PsA and psoriasis	Targets TNF alpha, which is involved in the inflammatory process/TNF inhibitor	IV infusion	• BLA filed August 2014 • BLA filed under the 351(k) abbreviated approval pathway

*These medications have a boxed warning.

Glossary of terms

351(k) abbreviated approval pathway
Abbreviated licensure pathway for biological products that are demonstrated to be "biosimilar" to or "interchangeable" with an FDA-licensed biological product

BLA
Stands for "biologics license application," similar to an NDA but used for investigational medications that are considered to be biologic agents.

Breakthrough therapy designation Intended to expedite the development and review of a potential new drug for serious or life-threatening diseases.

Complete-response letter

Issued to let the applicant know that the review period for an investigational agent is complete, and that the NDA or BLA is not yet ready for approval.

Fast track

Designation granted by the FDA to an investigational agent indicating an expedited review of the NDA or BLA; usually applies to medications that treat serious or life-threatening conditions and demonstrate the potential to address unmet medical needs.

NDA

Stands for "new drug application," the process by which a manufacturer submits information to the FDA to gain approval for the agent; conducted after Phase 3 development is completed.

Orphan drug

A medication that treats a rare disease that affects fewer than 200,000 Americans. A medication granted orphan drug status is entitled to seven years of marketing exclusivity.

Phase 2
Second phase of medication development; may involve a few dozen to a couple hundred patients to determine safety and preliminary data on efficacy.

Phase 3

Last phase of medication development; involves safety and efficacy trials of the new medication. This phase of development can take years to complete.

Glossary of terms (continued)

Priority review

Designation granted by the FDA to an investigational agent after it has been submitted to the FDA for approval. A priority designation means that the FDA will review and take action on the application (approve or not approve) within six months instead of the standard 10 months for all other medication filings.

Rolling submission

Usually applies to fast-track medications; indicates that the review process can be started even before the FDA receives all the information. However, the FDA requires all the information before a final decision about approval can be made.

sBLA

Stands for "supplemental biologics license application," similar to sNDA but used for already approved investigational medications that are considered to be biologic agents.

sNDA

Stands for "supplemental new drug application;" the process by which a pharmaceutical company submits information to the FDA to gain approval for a new indication for an agent that has already been approved by the FDA.

References

Websites:

ClinicalTrials—clinicaltrials.gov

DailyMed—dailymed.nlm.nih.gov

EvaluatePharma—evaluategroup.com

Manufacturers' websites

U.S. Food and Drug Administration—fda.gov

Information in the report is current as of December 2014 and was accessed on December 30, 2014.

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