PRIME THERAPEUTICS®

Specialty Pipeline MONTHLY UPDATE

Critical updates in an ever changing environment

June 2020

NEW DRUG INFORMATION

- **Ferriprox™ (deferiprone):** The U.S. Food and Drug Administration (FDA) approved Chiesi Global Rare Diseases' Ferriprox for the treatment of patients with transfusional iron overload due to thalassemia syndromes when current chelation therapy in inadequate. The new formulation of twice a day Ferriprox 1000mg oral tablets eliminate the mid-day dose. Ferriprox was approved by the FDA based on the 505(b)2 pathway using Ferriprox® (deferiprone) as a reference drug and bioequivalence studies.[1] There are no controlled trials that demonstrate Ferriprox has improvement in disease-related symptoms or increases survival. Ferriprox launch plans and pricing are pending.

- **Kynmobi™ (apomorphine HCl) sublingual film:** Sunovion's Kynmobi was approved by the FDA for the acute, intermittent treatment of OFF episodes in patients with Parkinson's disease (PD). OFF episodes are the re-emergence or worsening of PD symptoms otherwise controlled with oral levodopa/carbidopa. Kynmobi can be taken up to five times a day, and will be available in doses ranging from 10 to 30mg. Kynmobi's approval was based on Phase 3 clinical trial results that demonstrated that patients with PD receiving Kynmobi experienced significant improvements in motor symptoms at 30 minutes after dosing at week 12, with a mean reduction of 7.6 points, compared to placebo, on the Movement Disorder Society Unified Parkinson's Disease Rating Scale (MDS-UPDRS). Kynmobi had a reduction of 11.1 points vs placebo's 3.5-point reduction.[2] Clinical improvements were seen at 15 minutes post-administration and persisted for up to 90 minutes. Patients treated with Kynmobi also had a higher percentage of patient-rated full ON response within 30 minutes at week 12, compared to people receiving placebo. Mylan's Apokyn® (apomorphine HCl) injection is the current gold standard for PD patients experiencing OFF episodes however injections have administration limitations. Kynmobi sublingual film is expected to provide an easier route of administration. In January 2019, Kynmobi received a complete response letter (CRL) from the FDA requesting more information and analyses but Sunovion stated no further studies were required. Sunovion plans to launch Kynmobi by September 2020 with pricing to follow.

- **Uplizna™ (inebilizumab-cdon):** The FDA has approved Viela Bio's Uplizna for the treatment of neuromyelitis optica spectrum disorder (NMOSD) in adult patients who are anti-aquaporin-4 (AQP4) antibody positive. Approximately 80% of all patients with NMOSD test positive for anti-AQP4 antibodies. Uplizna, a CD19-directed cytolytic antibody, is an IV infusion given as a two-dose

introduction, then once every six months. FDA approval is based in part on results from N-MOMENTUM trial, which measured Uplizna against placebo. Uplizna met its primary endpoint by demonstrating statistically significant benefits in reduction of NMOSD attacks. Specifically, 89% of patients in the anti-AQP4 antibody positive group remained relapse-free during the six-month period post treatment compared to 58% placebo patients.[3] There was no evidence of a benefit in patients who were anti-AQP4 antibody negative. Uplizna will compete with Alexion's Soliris® (eculizumab) which was FDA approved last June; as well as Rituxan and its biosimilars which are used off-label for NMOSD. Unlike Soliris, Uplizna will not carry a boxed warning. Viela has not disclosed Uplizna's price but estimates from analysts put it in the range of $200,000-$280,000 per year.[4] Viela Bio anticipates launch of Uplizna by the end of June.

- **Zepzelca™ (lurbinectedin):** The FDA granted accelerated approval to Jazz Pharmaceuticals' Zepzelca for the treatment of relapsed small cell lung cancer (SCLC) with disease progression on or after platinum-based chemotherapy. Zepzelca is administered via intravenous infusion in an outpatient setting once every 21 days until disease progression. The FDA approved Zepzelca based on a Phase 2 trial that demonstrated patients treated with Zepzelca had an overall response rate (ORR) of 35% and a median duration of response of 5.3 months.[5] Confirmatory trials are being conducted for Zepzelca. Jazz Pharmaceuticals plans to launch Zepzelca in early July with pricing to follow.

- **Nyvepria™ (pegfilgrastim-apgf):** The FDA has approved Pfizer's Nyvepria, a biosimilar to Amgen's 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. FDA approval for Nyvepria was based on the review of a comprehensive data package using Neulasta® as its reference product.[6] Currently there are three pegfilgrastim biosimilars that have launched in the United States; Mylan's Fulphila® (pegfilgrastim-jmdb), Coherus' Udenyca® (pegfilgrastim-cbqv), and Sandoz's Ziextenzo® (pegfilgrastim-bmez). Due to ongoing legal battles with Amgen, Pfizer will delay Nyvepria's launch, hoping to make it available by the end of 2020. Pricing will follow.

- **Artesunate™ (artesunate):** The FDA has approved Amivas' Artesunate for the initial treatment of severe malaria in adult and pediatric patients. The safety and efficacy of IV Artesunate was studied in two trials that demonstrated a reduction in mortality by 34.7% and 22.5% respectively when compared with the injectable standard of care.[7] Prior to FDA approval access to investigational IV Artesunate had been managed by the Centers for Disease Control and Prevention (CDC) since 2007. Amivas will now manufacture, distribute, and commercialize Artesunate for injection. Artesunate launch and pricing are pending.

NEW INDICATIONS

- **Zejula® (niraparib):** The FDA granted approval of GlaxoSmithKline's Zejula for maintenance treatment in the first line setting for women with advanced ovarian cancer who responded to platinum chemotherapy regardless of biomarker status. Zejula is also indicated for treatment of adult patients with recurrent epithelial ovarian, fallopian tube or primary peritoneal cancer who are in a complete or partial response to platinum-based chemotherapy.

- **Lynparza® (olaparib):** The FDA granted approval of AstraZeneca's Lynparza for the treatment of metastatic castration-resistant prostate cancer (mCRPC) and deleterious or suspected deleterious germline or somatic homologous recombination repair (HRR) gene mutations in patients who have progressed following prior treatment with a new hormonal agent. Previously Lynparza was approved for ovarian and breast cancer.
- **Pomalyst® (pomalidomide):** Bristol-Myers Squibb's Pomalyst received FDA approval of a new indication for the thalidomide analog for treatment of AIDS-related Kaposi's sarcoma patients resistant to highly active antiretroviral therapy (HAART) and for treatment of Kaposi's sarcoma in patients who are HIV-negative. Previously Pomalyst was indicated, in combination with dexamethasone, for patients with multiple myeloma who have received at least two prior therapies including lenalidomide and a proteasome inhibitor and have demonstrated disease progression on or within 60 days of completion of the last therapy.
- **Alunbrig® (brigatinib):** The FDA granted approval of Takeda's Alunbrig for first-line treatment of anaplastic lymphoma kinase-positive (ALK+) metastatic non-small cell lung cancer (NSCLC) as detected by an FDA-approved test. Previously Alunbrig was approved for the treatment of patients with anaplastic lymphoma kinase (ALK)-positive metastatic non-small cell lung cancer (NSCLC) who have progressed on or are intolerant to crizotinib.
- **Dupixent® (dupilumab):** The FDA granted approval of Regeneron Pharmaceuticals' Dupixent as an add-on maintenance treatment for children aged 6 to 11 years with moderate-to-severe atopic dermatitis (AD) whose disease is not adequately controlled with topical prescription therapies or when those therapies are not advisable. Previously Dupixent was approved only for patients 12 years of age and older.
- **Opdivo® (nivolumab):** The FDA granted approval for Bristol-Myers Squibb's Opdivo for treatment of patients with unresectable advanced, recurrent or metastatic esophageal squamous cell carcinoma (ESCC) after prior fluoropyrimidine—and platinum-based chemotherapy.

JUNE NEWS

- "We already knew from BioMarin's statement that their gene therapy for hemophilia A continued to protect patients against the bleeding episodes that can threaten their lives. Now, four years in, we're getting the hard data on durability for valrox—and here the biotech faces considerable potential trouble. At the end of the first, second and third years, post-infusion, mean Factor VIII activity level of the high dose 6e13 vg/kg cohort was 64.3, 36.4 and 32.7 IU/dL respectively as measured by the CS assay. Now, at the four-year mark, that figure has tumbled to 24.2 IU/dL. And that raises questions on whether or not the gene therapy's effect on Factor VIII—which hemophilia patients lack—will drop to the point it won't protect them anymore. That's not the picture they were looking for with a still likely FDA OK just a couple of months away."[8]

- "Pfizer and its hemophilia A gene therapy partners at Sangamo have posted the latest update on their Phase I/II trial for SB-525 — and it continues to look good as their rivals at BioMarin cruise to a likely approval with a not-so-perfect leader. Median Factor VIII expression levels for their gene therapy are running at 64.2% for the five patients now taking the 3e13 vg/kg dose. That includes patients up to 61 weeks after dosing, putting the crew on the trial at potentially better than par with BioMarin's valrox at a year in, which has attracted plenty of questions as FVIII levels declined over four years to a median of 16.4%, as we reported yesterday. A year in, the valrox median on the high dose was 60%."[9]
- "A Phase 1/2 clinical trial testing AXO-Lenti-PD (OXB102), Axovant's experimental gene therapy for Parkinson's disease, has completed dosing of all four adults in a second group in its dose-escalation part of the study. Six-month data on these patients is expected by year's end. AXO-Lenti-PD is a one-time gene therapy that works by delivering three genes involved in the production of dopamine directly to the brain. Dopamine is a neurotransmitter, or a chemical messenger, that is produced by specialized neurons that are gradually lost as a consequence of Parkinson's disease. One year after receiving the gene therapy, these patients continued to show gains, including a 37% improvement in off-period motor symptoms (periods when anti-parkinsonian medication wears off) and a better quality-of-life."[10]
- "In the high-stakes patent fight between Biogen and Mylan over Tecfidera's main remaining patent, Mylan has scored a major win in federal court. U.S. District Judge Irene Keeley said Mylan "demonstrated by clear and convincing evidence" that certain claims of Biogen's '514 patent are invalid for "lack of written description." The decision threatens Biogen's bestselling medicine with early generics; Tecfidera, a multiple sclerosis drug, generated $3.3 billion in the U.S. last year. The company's '514 patent is set to expire in 2028, meaning the decision, if upheld, could wipe out years of monopoly sales."[11]

SPECIALTY NEW PRODUCT APPROVALS IN THE PAST TWELVE MONTHS

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	MONTH APPROVED
inebilizumab-cdon	Uplizna™	Viela Bio	Neuromyelitis optica spectrum disorder (NMOSD)	IV	June 2020
apomorphine HCl sublingual film	Kynmobi™	Sunovion	Parkinson's disease OFF episodes	SL film	May 2020
artesunate	Artesunate™	Amivas	Malaria	IV	May 2020
deferiprone	Ferriprox™	ApoPharma	Thalassemia syndromes	Oral	May 2020
leuprolide acetate	Fensolvi™	Tolmar Pharmaceuticals	Central precocious puberty	SC	May 2020
coagulation factor VIIa (recombinant)-jncw	Sevenfact™	Laboratoire Francais du Fractionnement et des Biotechnologies	Hemophilia A or B	IV	April 2020
monomethyl fumarate delayed-release	Bafiertam™	Banner Life Sciences	Multiple sclerosis	Oral	April 2020
ozanimod	Zeposia™	BMS	Relapsing multiple sclerosis	Oral	March 2020
osilodrostat	Isturisa™	Recordati	Cushing's syndrome	Oral	March 2020
peanut (Arachis hypogaea) allergen powder-dnfp	Palforzia™	Aimmune Therapeutics	Peanut allergy	Oral	February 2020
octreotide acetate	Bynfezia pen™	Sun Pharmaceuticals	Acromegaly	SC	February 2020
teprotumumab – trbw	Tepezza™	Horizon	Thyroid eye disease	IV	January 2020
golodirsen	Vyondys 53™	Sarepta Therapeutics	Duchenne muscular dystrophy (DMD)	IV	December 2019
methotrexate	Reditrex™	Cumberland Pharmaceuticals	Rheumatoid arthritis/psoriasis	SC	December 2019
voxelotor	Oxbryta™	Global Blood Therapeutics	Sickle cell disease (SCD)	Oral	November 2019
riluzole	Exservan™	Aquestive	Adjunctive therapy for the treatment of ALS	Oral	November 2019
givosiran	Givlaari™	Alnylam	Acute hepatic porphyria (AHP)	SC	November 2019
crizanlizumab-tmca	Adakveo™	Novartis	Reduce the frequency of vaso-occlusive crises (VOCs) with sickle cell disease	IV	November 2019
luspatercept–aamt	Reblozyl™	Celgene Corp	Beta-thalassemia-associated anemia who require red blood cell transfusions	SC	November 2019
elexacaftor/ivacaftor/tezacaftor	Trikafta™	Vertex	Cystic fibrosis	Oral	October 2019
diroximel fumarate	Vumerity™	Alkermes and Biogen	Relapsing forms of MS	Oral	October 2019
afamelanotide	Scenesse™	Clinuvel Pharmaceuticals	Erythropoietic protoporphyria	SC	October 2019
brolucizumab-dbll	Beovu™	Novartis	Wet age-related macular degeneration	Intravitreal injection	October 2019
teriparatide	Bonsity™	Pfenex and Alvogen	Osteoporosis	SC	October 2019
ledipasvir and sofosbuvir pellets	Harvoni®	Gilead	Hepatitis C	Oral	September 2019

continued

SPECIALTY NEW PRODUCT APPROVALS IN THE PAST TWELVE MONTHS *(continued)*

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	MONTH APPROVED
sofosbuvir pellets	Sovaldi®	Gilead	Hepatitis C	Oral	September 2019
lefamulin	Xenleta™	Nabriva	Community acquired bacterial pneumonia	Oral and IV	August 2019
upadacitinb	Rinvoq™	Abbvie	Rheumatoid arthritis	Oral	August 2019
pretomanid	Pretomanid™	Global Alliance for TB Drug Development	Tuberculosis	Oral	August 2019
immune globulin subcutaneous, human-klhw	Xembify™	Grifols SA	Primary humoral immunodeficiency	SC	July 2019
bremelanotide	Vyleesi™	AMAG Pharmaceuticals and Palatin Technologies	Generalized hypoactive sexual desire disorder	SC	June 2019

NEW INDICATIONS FOR APPROVED SPECIALTY PRODUCTS

GENERIC NAME	BRAND NAME	MANUFACTURER	NEW INDICATION(S)	DATE APPROVED
dupilumab	Dupixent®	Regeneron Pharmaceuticals	Pediatric patients aged 6 to 11 years	May 2020
pomalidomide	Pomalyst®	Bristol-Myers Squibb	AIDS-related Kaposi's sarcoma patients resistant to highly active antiretroviral therapy (HAART) and for treatment of Kaposi's sarcoma in patients who are HIV-negative	May 2020
apremilast	Otezla®	Amgen	Moderate to severe plaque psoriasis of the scalp in adults	April 2020
nivolumab plus ipilimumab	Opdivo® plus Yervoy®	Bristol-Myers Squibb Co.	Advanced HCC previously treated with the kinase inhibitor Bayer's Nexavar® (sorafenib)	March 2020
sofosbuvir and velpatasvir	Epclusa®	Gilead Sciences Inc	Pediatric patients 6 years and above	March 2020
nintedanib	Ofev®	Boehringer Ingelheim	Fibrosing interstitial lung diseases (ILD) with a progressive phenotype	March 2020
benralizumab	Fasenra®	AstraZeneca	Prefilled auto-injector administration in a non-clinical setting for treatment of severe eosinophilic asthma	October 2019
ustekinumab	Stelara®	Johnson & Johnson	Moderately to severely active ulcerative colitis in adults	October 2019
ravulizumab-cwvz	Ultomiris®	Alexion Pharmaceuticals	Atypical hemolytic uremic syndrome (aHUS)	October 2019
von Willebrand factor/ coagulation factor VIII complex	Wilate®	Octapharma	Hemophilia A for routine prophylaxis	October 2019
emtricitabine/tenofovir alafenamide	Descovy®	Gilead	HIV pre-exposure prophylaxis (PrEP)	October 2019
glecaprevir and pibrentasvir	Mavyret®	AbbVie	Pediatric patients 12 years and above	September 2019
ledipasvir and sofosbuvir pellets	Harvoni®	Gilead	Pediatric patients 3 years and above	September 2019
sofosbuvir pellets	Sovaldi®	Gilead	Pediatric patients 3 years and above	September 2019
mepolizumab	Nucala®	GlaxoSmithKline	Pediatric patients 6-11 years	September 2019

continued

NEW INDICATIONS FOR APPROVED SPECIALTY PRODUCTS *(continued)*

GENERIC NAME	BRAND NAME	MANUFACTURER	NEW INDICATION(S)	DATE APPROVED
nintedanib	Ofev®	Boehringer Ingelheim	Systemic sclerosis-associated interstitial lung disease	September 2019
ixekizumab	Taltz®	Lilly	Active ankylosing spondylitis	August 2019
bedaquiline	Sirturo®	Johnson & Johnson	Pediatric patients 12 years and older	August 2019
apremilast	Otezla®	Celgene Corp	Oral ulcers associated with Behcet's disease	July 2019

ONCOLOGY PRODUCT APPROVALS IN THE PAST TWELVE MONTHS

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	DATE APPROVED
lurbinectedin	Zepzelca™	Jazz Pharmaceuticals	Relapsed small cell lung cancer (SCLC)	IV	June 2020
ripretinib	Qinlock™	Deciphera	Gastrointestinal stromal tumors (GIST)	Oral	May 2020
selpercatinib	Retevmo™	Lilly	RET-altered thyroid cancer and RET-altered non-small cell lung cancer (NSCLC)	Oral	May 2020
capmatinib	Tabrecta™	Novartis	Metastatic non-small cell lung cancer	Oral	May 2020
sacituzumab govitecan-hziy	Trodelvy™	Immunomedics	Breast cancer	IV	April 2020
pemigatinib	Pemazyre™	Incyte Corp.	Second-line treatment for cholangiocarcinoma	Oral	April 2020
tucatinib	Tukysa™	Seattle Genetics	Advanced HER2 breast cancer	Oral	April 2020
mitomycin gel	Jelmyto™	UroGen Pharma	Upper tract urothelial cancer	Gel	April 2020
selumetinib	Koselugo™	Merck and AstraZeneca	Pediatric neurofibromatosis type 1 (NF1)	Oral	April 2020
isatuximab	Sarclisa™	Sanofi	Multiple myeloma	IV	February 2020
tazemetostat	Tazverik™	Epizyme	Epithelioid sarcoma	Oral	January 2020
avapritinib	Ayvakit™	Blueprint Medicines	Unresectable or metastatic gastrointestinal stromal tumor (GIST)	Oral	January 2020
fam-trastuzumab deruxtecan-nxki	Enhertu™	Daiichi Sankyo and AstraZeneca	Breast cancer	IV	December 2019
enfortumab vedotin-ejfv	Padcev™	Astellas and Seattle Genetics	Advanced/metastatic/urothelial cancer	IV	December 2019
zanubrutinib	Brukinsa™	BeiGene	Mantle cell lymphoma	Oral	November 2019
pemetrexed	Pemfexy™	Eagle Pharmaceuticals	In combination with cisplatin or as a single agent for certain types of cancer	IV	October 2019
bortezomib	Bortezomib™	Dr. Reddy's	Multiple Myeloma; Non-Hodgkin's Lymphoma; Mantle cell lymphoma	IV or SC	October 2019
eedratinib	Inrebic™	Celgene	Myelofibrosis	Oral	August 2019
entrectinib	Rozlytrek™	Genentech	ROS1-positive non-small cell lung cancer (NSCLC) and certain NTRK gene fusion cancers	Oral	August 2019
pexidartinib	Turalio™	Daiichi Sankyo	Symptomatic tenosynovial giant cell tumor	Oral	August 2019
darolutamide	Nubeqa™	Bayer and Orion	Non-metastatic castration-resistant prostate cancer	Oral	July 2019
selinexor	Xpovio™	Karyopharm	Multiple myeloma	Oral	July 2019
polatuzumab vedotin-piiq	Polivy™	Genentech	Relapsed or refractory diffuse large B-cell lymphoma	IV	June 2019

NEW INDICATIONS FOR APPROVED ONCOLOGY DRUGS

GENERIC NAME	BRAND NAME	MANUFACTURER	NEW INDICATION	DATE APPROVED
nivolumab	Opdivo®	Bristol-Myers Squibb	Certain forms of esophageal squamous cell carcinoma (ESCC)	June 2020
brigatinib	Alunbrig®	Takeda	First-line treatment of anaplastic lymphoma kinase-positive (ALK+) metastatic non-small cell lung cancer (NSCLC)	June 2020
niraparib	Zejula®	GlaxoSmithKline	Certain forms of advanced ovarian cancer who responded to platinum chemotherapy regardless of biomarker status	June 2020
atezolizumab	Tecentriq®	Genentech	Advanced non-squamous and squamous non-small cell lung cancer (NSCLC) patients who do not have EGFR or ALK mutations and who have high PD-L1 expression (TC3/IC3 wild-type)	May 2020
olaparib	Lynparza®	AstraZeneca	Certain forms of metastatic castration-resistant prostate cancer (mCRPC)	May 2020
nivolumab plus ipilimumab	Opdivo® plus Yervoy®	Bristol-Myers Squibb Co.	First-line treatment for patients with certain forms of metastatic non-small cell lung cancer	May 2020
rucaparib	Rubraca®	Clovis Oncology	Monotherapy for treatment of adults with BRCA1/2-mutant recurrent, metastatic castrate-resistant prostate cancer (CRPC)	May 2020
olaparib	Lynparza®	AstraZeneca	Combination of certain forms of ovarian cancer	May 2020
ibrutinib	Imbruvica® in combination with rituximab	AbbVie and Janssen	First-line treatment of chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL)	May 2020
neratinib	Nerlynx®	Puma	Combination with Roche's Xeloda® (capecitabine) for certain forms of breast cancer	April 2020
encorafenib	Braftovi®	Pfizer	Combination regimen for treatment of advanced BRAF V600E-mutant metastatic colorectal cancer (mCRC) patients following one or two lines of therapy	April 2020
durvalumab	Imfinzi®	AstraZeneca	Certain forms of extensive-stage small cell lung cancer (ES-SCLC)	March 2020
pembrolizumab	Keytruda®	Merck	Bacillus Calmette-Guerin (BCG) – non-muscle invasive bladder cancer (NMIBC)	January 2020
enzalutamide	Xtandi®	Pfizer/Astellas Pharma	Metastatic hormone-sensitive prostate cancer (mHSPC)	December 2019
atezolizumab	Tecentriq®	Genentech (Roche)	Metastatic non-small cell lung cancer (NSCLC)	December 2019
olaparib	Lynparza®	AstraZeneca	Certain forms of advanced cancer	December 2019
acalabrutinib	Calquence®	AstraZeneca	Chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL)	November 2019
niraparib	Zejula®	GSK	Certain forms of ovarian, fallopian tube, or peritoneal cancer	October 2019
neratinib	Nerlynx®	Puma	Certain forms of breast cancer	October 2019
rituximab	Rituxan®	Genentech (Roche)	Granulomatosis with polyangiitis and microscopic polyangiitis in children 2 years of age and older	October 2019

continued

NEW INDICATIONS FOR APPROVED ONCOLOGY DRUGS *(continued)*

GENERIC NAME	BRAND NAME	MANUFACTURER	NEW INDICATION	DATE APPROVED
pembrolizumab	Keytruda®	Merck	Certain forms of metastatic head and neck squamous cell carcinoma	October 2019
daratumumab	Darzalex®	Johnson & Johnson	Certain forms of multiple myeloma	September 2019
apalutamide	Erleada®	Janssen	Metastatic castration-sensitive prostate cancer	September 2019
pembrolizumab and lenvatinib	Keytruda® and Lenvima®	Merck and Eisai	Certain forms of advanced endometrial carcinoma	September 2019
pembrolizumab	Keytruda®	Merck	Certain types of squamous cell carcinoma of the esophagus	August 2019

BIOSIMILAR PRODUCT APPROVALS IN THE PAST TWELVE MONTHS

GENERIC NAME	BRAND NAME	REFERENCE PRODUCT	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	MONTH APPROVED
pegfilgrastim-apgf	Nyvepria™	Neulasta®	Pfizer	Decrease the incidence of infection, as manifested by febrile neutropenia, in patients with nonmyeloid malignancies receiving myelosuppressive anti-cancer drug	SC	June 2020
infliximab-axxq	Avsola™	Remicade®	Janssen	Crohn's disease, pediatric Crohn's, ulcerative colitis, rheumatoid arthritis in combination with methotrexate, psoriatic arthritis	IV	December 2019
adalimumab-afzb	Abrilada™	Humira®	Pfizer	Autoimmune	SC	November 2019
pegfilgrastim-bmez	Ziextenzo™	Neulasta®	Sandoz	Decrease the incidence of infection, as manifested by febrile neutropenia, in patients with nonmyeloid malignancies receiving myelosuppressive anti-cancer drug	IV	November 2019
adalimumab-bwwd	Hadlima™	Humira®	Samsung Bioepis/ Merck	Certain forms of: rheumatoid arthritis, juvenile idiopathic arthritis, psoriatic arthritis, ankylosing spondylitis, adult Crohn's disease, ulcerative colitis, plaque psoriasis	SC	July 2019
rituximab-pvvr	Ruxience™	Rituxan®	Pfizer	Certain forms of non-Hodgkin's lymphoma, chronic lymphocytic leukemia, granulomatosis with polyangiitis	IV	July 2019
bevacuzynav-bvzr	Zirabev™	Avastin®	Pfizer	Certain forms of cancer	IV	July 2019

SPECIALTY PIPELINE

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	ANTICIPATED APPROVAL DATE*
padeliporfin di-potassium	Tookad™	Steba Oncology	Prostate cancer	IV	May 2020
filgotinib	N/A	Gilead	Rheumatoid arthritis	Oral	June 2020
viltolarsen	N/A	Nippon Shinyaku	Duchenne muscular dystrophy	IV	June 2020
HTX-011 (bupivacaine and meloxicam)	N/A	Heron Therapeutics	Pain	Injectable	June 2020
octreotide	Mycapssa™	Chiasma	Acromegaly	Oral	June 2020
abicipar pegol	N/A	Allergan	Macular degeneration	Eye injection	July 2020
JZP-258	N/A	Jazz Pharmaceuticals	Excessive daytime sleepiness	Oral	July 2020
UX007 (triheptanoin)	N/A	Ultragenyx	Long-chain fatty acid oxidation disorders	Oral	July 2020
belantamab mafodotin	N/A	GlaxoSmithKline	Multiple myeloma	IV	July 2020
risdiplam	N/A	Genentech	Spinal muscular atrophy	Oral	August 2020
satralizumab	N/A	Roche	Neuromyelitis optica spectrum disorder (NMOSD)	SC	August 2020
viaskin peanut	Viaskin Peanut™	DBV Technologies	Peanut allergy	Patch	August 2020
sodium thiosulfate, STS	Pedmark™	Fennec Pharmaceuticals	Prevention of ototoxicity	IV	August 2020
ASTX727 (cedazuridine and decitabine, C-DEC)	N/A	Astex Pharmaceuticals (Otsuka)	Myelodysplastic syndrome (MDS)	Oral	August 2020
lurbinectedin	Zepsyre™	PharmaMar and Jazz Pharmaceuticals	Small cell lung cancer (SCLC)	IV	August 2020
tafasitamab	N/A	MorphoSys AG	Relapsed/refractory diffuse large B cell lymphoma	IV	August 2020
CC-486 (azacitidine)	N/A	Bristol-Myers Squibb	Acute myeloid leukemia (AML)	Oral	September 2020
eflapegrastim	Rolontis™	Spectrum Pharmaceuticals and Hanmi Pharmaceutical	Chemotherapy-induced neutropenia	SC	October 2020
REGN-EB3 (REGN3470-3471-3479)	N/A	Regeneron	Ebola	IV	October 2020
mannitol for inhalation	Bronchitol	Chiesi Group and Pharmaxis	Cystic fibrosis	Inhaled	November 2020
sutimlimab	N/A	Sanofi	Cold agglutinin disease	IV	November 2020
lonafarnib	N/A	Eiger BioPharmaceuticals	Progeria and progeroid laminopathies	Oral	November 2020
setmelanotide	N/A	Rhythm Pharmaceuticals	Pro-opiomelanocortin (POMC) deficiency obesity	SC	November 2020
tanezumab	N/A	Pfizer And Lilly	Osteoarthritis	SC	December 2020
inclisiran	N/A	Novartis	Hyperlipidemia	SC	December 2020

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SPECIALTY PIPELINE *(continued)*

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	ANTICIPATED APPROVAL DATE*
berotralstat (BCX7353)	N/A	BioCryst	Hereditary angioedema (HAE) attacks	Oral	December 2020
lumasiran	N/A	Alnylam	Primary hyperoxaluria type 1 (PH1)	SC	December 2020
margetuximab	N/A	MacroGenics	Breast cancer	IV	December 2020
roxadustat	N/A	FibroGen Inc. and AstraZeneca PLC	Anemia of chronic kidney disease (CKD)	Oral	December 2020

* Anticipated approval dates are predictions made by Prime Therapeutics based on industry information.

BIOSIMILAR PIPELINE

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	ANTICIPATED APPROVAL DATE*
Oncology					
SB8	N/A (Avastin® biosimilar)	Samsung Bioepis and Merck	Non-small cell lung cancer	IV	September 2020
ABP 798	N/A (Rituxan® biosimilar)	Amgen	Oncology	IV	December 2020
MYL-1402O	N/A (Avastin® biosimilar)	Mylan NV and Biocon Ltd.	Oncology	IV	December 2020
Blood modifiers					
TX-01 (CRL)	N/A (Neupogen® biosimilar)	Tanvex BioPharma	Neutropenia	IV/SC	October 2020

FDA APPROVED GENE/CELL THERAPY NEW PRODUCT APPROVALS

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	MONTH APPROVED
onasemnogene abeparvovec-xioi	Zolgensma™	AveXis	Spinal muscular atrophy	IV	May 2019
voretigene neparvovec-rzyl	Luxturna®	Spark Therapeutics	Biallelic RPE65 mutation-associated retinal dystrophy	Subretinal injection	December 2017
axicabtagene ciloleucel	Yescarta®	Kite	Large B-cell lymphoma	IV	October 2017
tisagenlecleucel	Kymriah®	Novartis	Acute lymphoblastic leukemia	IV	August 2017

GENE/CELL THERAPY 2020 PIPELINE

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	ANTICIPATED APPROVAL DATE
nadofaragene firadenovec	Instiladrin™	FerGene	Non-muscle invasive urothelial bladder cancer	Intravesical	May 25, 2020
KTE-X19 (axicabtagene ciloleucel)	Yescarta™	Kite	Mantle cell lymphoma	CAR T-cell therapy	August 10, 2020
lisocabtagene maraleucel (liso-cel)	N/A	Bristol-Myers Squibb	R/R large B cell lymphoma	IV	August 17, 2020
valoctogene roxaparvovec (BMN 270)	Roctavian™	BioMarin	Hemophilia A	IV	August 21, 2020
RVT-802	N/A	Enzyvant	Pediatric congenital athymia	IV	2020
remestemcel-L	Ryoncil™	Mesoblast	Children with acute graft-versus host disease (GVHD)	IV	2020

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