PRIME THERAPEUTICS®

Specialty Pipeline **MONTHLY UPDATE**

Critical updates in an ever changing environment

September 2020

NEW DRUG INFORMATION

- **Cystadrops™ (cysteamine ophthalmic solution 0.37%):** The United States Food and Drug Administration (FDA) has approved Recordati Rare Disease's Cystadrops for the treatment of corneal cystine crystal deposits in adults and children with cystinosis. Cystadrops is a new viscous eye drop solution that is a cystine-depleting agent. It is dosed four times a day. The FDA approval of Cystadrops was supported by two clinical trials that investigated the reduction in corneal cystine crystal density as assessed by in vivo confocal microscopy (IVCM). At day 90, patients treated with Cystadrops demonstrated a 40% reduction in IVCM total score across all corneal layer when compared with baseline.[1] Cystadrops demonstrated a significant reduction in cystine crystal deposits in the cornea of the eye. Cystadrops additionally demonstrated a 30% decrease in IVCM total score in a Phase 1/2a open-label trial that was maintained for five years. Cystadrops has launched at an average wholesale price (AWP) of $2,100 per bottle.

- **Kesimpta™ (ofatumumab):** Novartis and Genmab's Kesimpta has been approved by the FDA as an injectable disease-modifying therapy for adults with relapsing forms of multiple sclerosis (MS); including clinically isolated syndrome, relapsing-remitting MS and active secondary progressive MS. Kesimpta is a once monthly, subcutaneous self-administered B-cell therapy. The approval was based on two identical phase 3 studies, ACLEPIOS I and ACLEPIOS II, that compared Kesimpta to Sanofi Genzyme's oral once daily Aubagio® (teriflunomide) and had a primary outcome of annual rate of relapses. In the two trials, Kesimpta significantly reduced annualized relapse rate relative to Aubagio (relative reduction of 51 to 59%), active MRI-detected lesions (relative reduction of 94% or 98%), and new or enlarging lesions (relative reduction of 82 to 85%).[2] The disability progression was sustained over 3 months with a relative reduction of 34.4%. Kesimpta has launched with a wholesale acquisition cost (WAC) of $83,000 per year.[3]

- **Sogroya™ (somapacitan-beco):** The FDA approved Novo Nordisk's Sogroya for the replacement of endogenous growth hormone in adults with growth hormone deficiency (GHD). Sogroya is a once a week, self-administered subcutaneous injection that will be available as a 10mg/1.5mL (6.7mg/mL) solution. Until now, the only treatment option for patients with deficiency in growth hormone secretion has been a daily formulation of the hormone. In the clinical trial, REAL 1, Sogroya was compared to either a once weekly placebo or once daily somatropin. The effectiveness of Sogroya was determined by the percentage change of truncal fat. Sogroya decreased truncal fat by 1.06%, while it increased for placebo patients by 0.47%. The once daily somatropin group, truncal fat decreased by 2.23%.[4] Novo Nordisk has not announced launch date or pricing of Sogroya.

- **Onureg™ (azacitidine):** Bristol-Myers Squibb's oral Onureg has been approved by the FDA for the continued treatment of adult patients with acute myeloid leukemia (AML) who achieved first complete remission (CR) or CR with incomplete blood count recovery (CRi) following intensive induction chemotherapy and who are not able to complete intensive curative therapy. It is the first approved therapy oral ormulation of azacitadine which has been available as an IV formulation for years. Onureg's approval was based on the QUAZAR AML-001 trial, which demonstrated improvements that were statistically significant and clinically meaningful in overall survival (OS) when Onureg was compared to placebo. The median OS was greater than two years (24.7 months) with Onureg compared to 14.8 months with placebo patients.[5] A subgroup analysis showed consistency in the OS benefit for patients in either CR or CRi. Onureg is an 300mg oral version of azacitidine which is approved as Celgene's Vidaza® (azacitidine) IV solution. Onureg has a large difference in pharmacokinetic parameters and is not substitutable for intravenous or subcutaneous azacitidine as it may result in a fatal adverse reaction. Onureg caused new or worsening Grade 3 or 4 neutropenia in 49% of patients and thrombocytopenia in 22% of patients. Onureg has launched with an AWP of $25,389 per bottle (14 tablets).
- **Gavreto™ (pralsetinib):** The FDA granted accelerated approval of Blueprint's Gavreto for the treatment of adults with metastatic rearranged during transfection (RET) fusion-positive non-small cell lung cancer (NSCLC) as detected by an FDA-approved test. Gavreto is a once-daily, oral precision therapy that selectively inhibits RET-altered cancers. In NSCLC, RET fusions represent approximately 1-2% of patients. Gavreto's approval was based on results from the Phase I/II ARROW study which demonstrated an overall response rate (ORR) of 57% and complete response (CR) rate of 5.7% in the 87 people with NSCLC previously treated with platinum-based chemotherapy.[6] The median duration of response (DoR) was not reached. The ORR was 70% with an 11% CR rate in the patient's treatment-naïve NSCLC.[6] Gavreto has launched with a WAC of $231,000 annually.[7]

NEW INDICATIONS

- **Tecentriq® (atezolizumab):** The FDA granted approval of Genentech's Tecentriq for use in combination with Roche's Cotellic® (cobimetinib) and Roche's Zelboraf® (vemurafenib) for treatment of patients with unresectable or metastatic BRAF V600 mutation-positive melanoma.
- **Kyprolis® (carfilzomib):** The FDA expanded the approval of Amgen's Kyprolis for use in combination with dexamethasone and Johnson & Johnson (Janssen)'s Darzalex® (daratumumab) for treatment of relapsed or refractory multiple myeloma patients who have received one to three lines of therapy.
- **Darzalex® (daratumumab):** The FDA expanded the approval of Johnson & Johnson and Genmab's Darzalex for use in combination with Kyprolis® (carfilzomib) and dexamethasone for treatment of relapsed or refractory multiple myeloma patients who have received one to three lines of therapy.

SEPTEMBER NEWS

- "While 8 targeted immune modulators (TIMs) on the market for ulcerative colitis have proved beneficial to patients, nonprofit cost-effectiveness watchdog ICER called the drugs unreasonably expensive, recommending some deep discounts to match the drugs with their actual value. In a report, ICER assessed the cost vs. clinical benefit of market leader Humira — for which AbbVie has faced price gouging accusations — as well as Janssen's Simponi, Stelara and Remicade, Merck's Renflexis, Pfizer's Inflectra and Xeljanz, and Takeda's Entyvio. The institute came up with a health benefit price benchmark (HBPB) — a price range indicating the highest price a manufacturer should charge for a treatment, based on the amount of improvement in overall health to patients — for each of the eight TIMs. Humira, which can cost more than $72,000 a year, would need to be discounted by 90% to 92% to meet ICER's suggested range ($6,000 to $7,000 per year)."[8]
- "The Oncologic Drugs Advisory Committee (ODAC) of the United States Food and Drug Administration (FDA) voted overwhelmingly in favor (9 to 1) that the available data support the efficacy of remestemcel-L (RYONCIL™) in pediatric patients with steroid-refractory acute graft versus host disease (SR-aGVHD). The ODAC is an independent panel of experts that evaluates efficacy and safety of data and makes appropriate recommendations to the FDA. Although the FDA will consider the recommendation of the panel, the final decision regarding the approval of the product is made solely by the FDA, and the recommendations by the panel are non-binding. RYONCIL has been accepted for Priority Review by the FDA with an action date of September 30, 2020, under the Prescription Drug User Fee Act (PDUFA). If approved by the PDUFA date, Mesoblast plans to launch RYONCIL in the United States in 2020."[9]
- "In the TRANSCEND NHL 001 study reported in The Lancet, doctors found that the autologous CD19-directed chimeric antigen receptor (CAR) T-cell agent lisocabtagene maraleucel (liso-cel) produced a high response rate in patients with relapsed or refractory large B-cell lymphomas. Median progression-free survival (after follow-up of 12.3 months) was 6.8 months; it was not reached among patients with complete response. Progression-free survival at 1 year was 44% for the total population and 65% among patients with complete response. Median overall survival was 21.1 months (after median follow-up of 17.5 months) and was not reached among patients with complete response; estimated 1-year rates were 58% for the total population and 86% among complete responders"[10]
- "The pharmaceutical company AstraZeneca said that it had resumed its coronavirus vaccine trial in Britain after suspending it over potential safety issues but that its trials in the United States and other countries were still on hold. The news came the same day that a competitor, Pfizer, said it was expanding the trial of its coronavirus vaccine to 44,000 people — a big increase from its previous goal of 30,000 — in an effort to recruit a more diverse group of participants and potentially cut down the time needed to get results from the trial. Both companies' announcements lacked crucial details, prompting criticism that they were not being open enough about the data they're collecting. AstraZeneca did not offer any information to support the decision to partially resume trials and would not give any details about the illness of a patient that had led to the suspension. Pfizer did not explain how it would determine the effectiveness of the vaccine in its expanded trials."[11]

SPECIALTY NEW PRODUCT APPROVALS IN THE PAST TWELVE MONTHS

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	MONTH APPROVED
somapacitan-beco	Sogroya™	Novo Nordisk	Adult growth hormone deficiency	SC	August 2020
ofatumumab	Kesimpta™	Novartis and Genmab	Relapsing forms of multiple sclerosis (RMS)	SC	August 2020
cysteamine ophthalmic solution 0.37%	Cystadrops™	Recordati Rare Disease	Corneal cystine crystal deposits	Eye drop	August 2020
satralizumab-mwge	Enspryng™	Roche	Neuromyelitis optica spectrum disorder (NMOSD)	SC	August 2020
risdiplam	Evrysdi™	Genentech	Spinal muscular atrophy	Oral	August 2020
viltolarsen	Viltepso™	Nippon Shinyaku	Duchenne muscular dystrophy	IV	August 2020
triheptanoin	Dojolvi™	Ultragenyx	Long-chain fatty acid oxidation disorders	Oral	July 2020
calcium, magnesium, potassium, and sodium oxybates	Xywav™	Jazz Pharmaceuticals	Excessive daytime sleepiness	Oral	July 2020
Fenfluramine	Fintepla™	Zogenix	Seizures associated with Dravet syndrome	Oral	June 2020
triheptanoin	Dojolvi™	Ultragenyx	Long-chain fatty acid oxidation disorders	Oral	June 2020
octreotide	Mycapssa™	Chiasma	Acromegaly	Oral	June 2020
inebilizumab-cdon	Uplizna™	Viela Bio	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

continued

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

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	MONTH APPROVED
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

NEW INDICATIONS FOR APPROVED SPECIALTY PRODUCTS

GENERIC NAME	BRAND NAME	MANUFACTURER	NEW INDICATION(S)	DATE APPROVED
cannabidiol	Epidiolex®	GW Pharmaceuticals PLC	Seizures associated with tuberous sclerosis complex (TSC) in patients one year of age and older	July 2020
canakinumab	Ilaris®	Novartis	Active Still's disease, including AOSD and SJIA in patients aged two years and older	June 2020
secukinumab	Cosentyx®	Novartis	Non-radiographic axial spondyloarthritis (nr-axSpA)	June 2020
brolucizumab-dbll	Beovu®	Novartis	Retinal vasculitis and retinal vascular occlusion	June 2020
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	Hepatitis C in pediatric patients 6 years and above	March 2020

continued

NEW INDICATIONS FOR APPROVED SPECIALTY PRODUCTS *(continued)*

GENERIC NAME	BRAND NAME	MANUFACTURER	NEW INDICATION(S)	DATE APPROVED
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

ONCOLOGY PRODUCT APPROVALS IN THE PAST TWELVE MONTHS

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	DATE APPROVED
pralsetinib	Gavreto™	Blueprint	RET fusion-positive non-small cell lung cancer (NSCLC)	Oral	September 2020
azacitidine	Onureg™	Bristol-Myers Squibb	Acute myeloid leukemia (AML)	Oral	September 2020
cedazuridine and decitabine, C-DEC	Inqovi™	Astex Pharmaceuticals (Otsuka)	Myelodysplastic syndrome (MDS)	Oral	August 2020
tafasitamab-cxix	Monjuvi™	MorphoSys AG	Relapsed/refractory diffuse large B cell lymphoma	IV	August 2020
belantamab mafodotin-blmf	Blenrep™	GlaxoSmithKline	Multiple myeloma	IV	August 2020
lurbinectedin	Zepzelca™	PharmaMar and Jazz Pharmaceuticals	Small cell lung cancer (SCLC)	IV	August 2020
decitabine and cedazuridine	Inqovi™	Astex Pharmaceuticals	Myelodysplastic syndromes (MDS) and chronic myelomonocytic leukemia (CMML)	Oral	July 2020
pertuzumab, trastuzumab and hyaluronidase–zzxf	Phesgo™	Genentech	HER2-positive breast cancer	SC	June 2020
tazemtostat	Tazverik™	Epizyme	Relapsed or refractory (R/R) follicular lymphoma (FL)	Oral	June 2020
ripretinib	Qinlock™	Deciphera	Gastrointestinal stromal tumors (GIST)	Oral	May 2020

continued

ONCOLOGY PRODUCT APPROVALS IN THE PAST TWELVE MONTHS *(continued)*

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	DATE APPROVED
selpercatinib	Retevmo™	Lilly	RET-altered thyroid cancer and RET-altered NSCLC	Oral	May 2020
capmatinib	Tabrecta™	Novartis	Metastatic NSCLC	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

NEW INDICATIONS FOR APPROVED ONCOLOGY DRUGS

GENERIC NAME	BRAND NAME	MANUFACTURER	NEW INDICATION	DATE APPROVED
daratumumab	Darzalex®	Johnson & Johnson (Janssen)	New combination for treatment of relapsed or refractory multiple myeloma	August 2020
carfilzomib	Kyprolis®	Amgen	New combination for treatment of relapsed or refractory multiple myeloma	August 2020
atezolizumab	Tecentriq®	Genentech (Roche)	New combination for treatment of patients with unresectable or metastatic BRAF V600 mutation-positive melanoma	July 2020
guselkumab	Tremfya®	Johnson & Johnson	Adults with active psoriatic arthritis (PsA)	July 2020
dupilumab	Dupixent®	Sanofi and Regeneron	New 300mg auto-injector formulation	June 2020
selinexor	Xpovio®	Karyopharm	Certain forms of relapsed or refractory diffuse large B-cell lymphoma (DLBCL)	June 2020
ramucirumab	Cyramza®	Lilly	New combination for certain forms of first-line treatment in metastatic non-small cell lung cancer (NSCLC)	June 2020
burosumab-twza	Crysvita®	Kyowa Kirin and Ultragenyx	FGF23-related hypophosphatemia associated with phosphaturic mesenchymal tumors	June 2020
tazemetostat	Tazverik®	Epizyme	Treatment of adults who are positive for an EZH2 mutation as detected by an FDA-approved test and a biomarker-independent use for adults with R/R FL who have no satisfactory alternative treatment options	June 2020
tazemetostat	Tazverik®	Epizyme	Relapsed or refractory follicular lymphoma (FL) patients who have received at least two prior lines of systemic therapy	June 2020
pembrolizumab	Keytruda®	Merck	Recurrent and/or metastatic cSCC that is not curable by surgery or radiation	June 2020
pembrolizumab	Keytruda®	Merck	New dosing schedule	June 2020
pembrolizumab	Keytruda®	Merck	Monotherapy in adult and pediatric patients with certain unresectable or metastatic solid tumors	June 2020
pembrolizumab	Keytruda®	Merck	First-line treatment of patients with unresectable or metastatic microsatellite instability-high (MSI-H) or mismatch repair deficient (dMMR) colorectal cancer	June 2020
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 NSCLC	June 2020
niraparib	Zejula®	GlaxoSmithKline	Certain forms of advanced ovarian cancer who responded to platinum chemotherapy regardless of biomarker status	June 2020
atezolizumab in combination with bevacizumab	Tecentriq® in combination with Avastin®	Genentech	Unresectable hepatocellular carcinoma (HCC) who have not received prior systemic therapy	May 2020
daratumumab	Darzalex®	Johnson & Johnson (Janssen)	Subcutaneous formulation for multiple myeloma	May 2020

continued

NEW INDICATIONS FOR APPROVED ONCOLOGY DRUGS *(continued)*

GENERIC NAME	BRAND NAME	MANUFACTURER	NEW INDICATION	DATE APPROVED
atezolizumab	Tecentriq®	Genentech	Advanced non-squamous and squamous 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 NSCLC	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
niraparib	Zejula®	GlaxoSmithKline (Tesaro)	First-line setting for women with advanced ovarian cancer who responded to platinum chemotherapy regardless of biomarker status	April 2020
luspatercept-aamt	Reblozyl®	Celgene	Certain forms of myelodysplastic syndromes (MDS)	April 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 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
pembrolizumab	Keytruda®	Merck	Certain forms of metastatic head and neck squamous cell carcinoma	October 2019

BIOSIMILAR PRODUCT APPROVALS IN THE PAST TWELVE MONTHS

GENERIC NAME	BRAND NAME	REFERENCE PRODUCT	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	MONTH APPROVED
adalimumab-fkjp	Hulio™	Humira®	Mylan	Autoimmune	SC	July 2020
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

SPECIALTY PIPELINE

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	ANTICIPATED APPROVAL DATE*
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	Zokinvy™	Eiger BioPharmaceuticals	Progeria and progeroid laminopathies	Oral	November 2020
LIQ861	N/A	Liquidia	Pulmonary arterial hypertension (PAH)	Inhaled dry powder	November 2020
naxitamab	Danyelza™	Y-mAbs	Relapsed or refractory high-risk neuroblastoma	IV	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
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	Evrenzo™	FibroGen Inc. and AstraZeneca PLC	Anemia of chronic kidney disease (CKD)	Oral	December 2020
relugolix	Relumin™	Myovant	Prostate cancer	Oral	December 2020
furosemide subcutaneous	Furoscix™	ScPharmaceuticals	Heart failure	Wearable SC	December 2020
maralixibat	N/A	mirum pharmaceuticals	Alagille syndrome (ALGS)	Oral	First quarter 2021
voclosporin	N/A	Aurinia	Lupus nephritis	Oral	January 2021
StrataGraft™	N/A	Mallinckrodt	Deep partial-thickness thermal burns	Skin replacement	February 2021
evinacumab	N/A	Regeneron	Homozygous familial hypercholesterolemia (HoFH)	IV	February 2021
trilaciclib	N/A	G1 Therapeutics	Myelopresservation	IV	February 2021
umbralisib	N/A	TG Therapeutics	Marginal zone lymphoma (MZL)	Oral	February 2021
casimersen	N/A	Sarepta Therapeutics	Duchenne muscular dystrophy (DMD)	IV	February 2021
ropeginterferon alfa-2b	N/A	PharmaEssentia	Polycythemia vera (PV)	SC	March 2021
aducanumab	N/A	Biogen	Alzheimer's disease	IV	March 2021

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SPECIALTY PIPELINE *(continued)*

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	ANTICIPATED APPROVAL DATE*
tivozanib	N/A	Aveo Oncology	Relapsed or refractory renal cell carcinoma (RCC)	Oral	March 2021
remdesivir	Veklury™	Gilead	COVID-19	IV	April 2021
tralokinumab	N/A	Leo Pharma	Moderate to severe atopic dermatitis	IV/SC	May 2021

* 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
BAT-1706	N/A (Avastin® biosimilar)	Bio-thera solutions	Oncology	IV	2021
Blood modifiers					
MSB11455	N/A (Neulasta® biosimilar)	Fresenius Kabi	Neutropenia	SC	March 2021

FDA APPROVED GENE/CELL THERAPY NEW PRODUCT APPROVALS

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	MONTH APPROVED
brexucabtagene autoleucel	Tecartus™	Kite	Mantle cell lymphoma	CAR T-cell therapy	July 2020
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
remestemcel-L	Ryoncil™	Mesoblast	Children with acute graft-versus host disease (GVHD)	IV	September 2020
lisocabtagene maraleucel (liso-cel)	Breyanzi™	Bristol-Myers Squibb	R/R large B cell lymphoma	IV	November 2020
RVT-802	N/A	Enzyvant	Pediatric congenital athymia	IV	2020

REFERENCES

1. https://www.prnewswire.com/news-releases/us-fda-approves-cystadrops-cysteamine-ophthalmic-solution-0-37-a-new-practical-treatment-option-for-the-ocular-manifestations-of-cystinosis-301118026.html
2. https://www.globenewswire.com/news-release/2020/08/20/2081600/0/en/U-S-FDA-Approves-Kesimpta-ofatumumab-in-Relapsing-Multiple-Sclerosis.html#:~:text=Kesimpta%20(ofatumumab)%20is%20approved%20in,patients%20using%20a%20Sensoready%20pen.
3. https://www.nationalmssociety.org/About-the-Society/News/FDA-Approves-Kesimpta%C2%AE-(ofatumumab),-Similar-to-Oc#:~:text=B%20cells%20return.-,Q.,announced%20as%20%2483%2C000%20per%20year.
4. https://www.novonordisk-us.com/media/news-releases.html?122995
5. https://www.businesswire.com/news/home/20200901005914/en/U.S.-Food-Drug-Administration-Approves-Onureg%C2%AE-azacitidine
6. https://www.businesswire.com/news/home/20200904005515/en/Genentech-Announces-FDA-Approval-Gavreto-pralsetinib-Treatment
7. https://www.biopharmadive.com/news/blueprint-gavreto-cancer-drug-approval-lilly/584836/#:~:text=The%20companies%20said%20their%20drug,tumor%20types%20with%20RET%20fusions.
8. https://endpts.com/icer-criticizes-cost-of-8-targeted-immune-modulators-for-uc-recommending-some-deep-discounts/
9. https://www.globenewswire.com/news-release/2020/08/14/2078495/0/en/U-S-FDA-Advisory-Committee-Votes-Nine-to-One-in-Favor-of-Remestemcel-L-Ryoncil-for-Efficacy-in-Children-With-Steroid-Refractory-Acute-Graft-Versus-Host-Disease.html
10. https://ascopost.com/news/september-2020/lisocabtagene-maraleucel-for-relapsed-or-refractory-large-b-cell-lymphomas/
11. https://www.nytimes.com/2020/09/12/health/astrazeneca-coronavirus-vaccine-trial-resumes.html