PRIME THERAPEUTICS®

Specialty Pipeline **MONTHLY UPDATE**

Critical updates in an ever changing environment

December 2020

NEW DRUG INFORMATION

- **Zokinvy™ (lonafarnib):** The United States Food and Drug Administration (FDA) has approved Eiger BioPharmaceuticals' Zokinvy for the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) and processing-deficient Progeroid Laminopathies (PL). In the United States there are 20 children and young adults affected with Progeria; if untreated, patients have an average life span of 14.5 years. Zokinvy is a disease-modifying agent that has demonstrated a statistically significant survival benefit in children and young adults with Progeria when compared to historical controls. In patients with Progeria, Zokinvy reduced the incidence of mortality by 60% and increased average survival time by 2.5 years.[1] Zokinvy is expected to launch January 2021 with pricing to follow.

- **Oxlumo™ (lumasiran):** The FDA approved Alnylam's Oxlumo (lumasiran) injection for subcutaneous use for the treatment of primary hyperoxaluria type 1 (PH1) to lower urinary oxalate levels in pediatric and adult patients. PH1 is an ultra-rare genetic disease that can lead to the formation of painful and recurrent kidney stones, or systemic organ dysfunction. Oxlumo is the first FDA-approved medication for PH1 and is initially administered in three monthly injections, then once quarterly. Oxlumo's approval was based on results from ILLUMINATE-A and ILLUMINATE-B that demonstrated clinically significant reductions in urinary oxalate (65% reduction) when compared to placebo (12%).[2] Oxlumo has launched with an annual wholesale acquisition cost (WAC) of $493,000.

- **Imcivree™ (setmelanotide):** Rhythm Pharmaceuticals' Imcivree is a melanocortin 4 (MC4) receptor agonist for chronic weight management of obesity due to proopiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1) or leptin receptor (LEPR) deficiency in patients 6 years of age and older. Obesity due to POMC, PCSK1 or LEPR deficiency are ultra-rare diseases caused by variants in POMC, PCSK1 or LEPR genes that impair the MC4 receptor pathway, which is a pathway in the hypothalamus that is responsible for regulating hunger, energy expenditure and consequently body weight. Prior to this approval, there were no FDA-approved medications available for these patients. In Phase 3 clinical trials, 80% of patients with obesity due to POMC or PCSK1 deficiency achieved greater than 10% weight loss and 45.5% of patients with obesity due to LEPR deficiency achieved greater than ten percent weight loss after one year of treatment with Imcivree.[3] Rhythm Pharmaceuticals plans to launch Imcivree in the first quarter of 2021 with pricing to follow.

- **Danyelza™ (naxitamab-gqgk):** The FDA granted approval of Y-mAbs' Danyelza in combination with granulocyte-macrophage colony-stimulating factor (GMCSF), for the treatment of pediatric patients 1 year of age and older and adult patients with relapsed or refractory high-risk neuroblastoma in the bone or bone marrow who have demonstrated a partial response, minor response, or stable disease to prior therapy. Danyelza is a GD2-binding monoclonal antibody administered by intravenous infusion. Danyelza demonstrated overall response rate (ORR) of 68% with a 59% complete responses (CR). Refractory patients demonstrated a 71% ORR with 64% CR. Relapsed patients demonstrated a 63% ORR with a 50% CR.[4] Danyelza has launched with an AWP $24,441 per vial.
- **Orladeyo™ (berotralstat):** BioCryst's Orladeyo has been approved as a prophylaxis to prevent attacks of hereditary angioedema (HAE) in adults and pediatric patients 12 years and older. Orladeyo is a once daily oral capsule that will be available as a 110mg and 150mg capsule in a 28-count blister pack. Orladeyo's approval was based on the Phase 3 APeX-2 trial that demonstrated a mean of 2.9 attacks per month at baseline to a mean of 1.0 attacks per month after 48 weeks.[5] APeX-2 found Orladeyo 110mg had a 30% reduction and Orladeyo 150mg had a 44.2% reduction in HAE attack rates when compared with placebo. Orladeyo will be exclusively available through OptimeCare Inc, a specialty pharmacy, by the end of December 2020 with a WAC of $485,004 per patient per year or $37,308 per 28-day pack of either 150mg or 110mg capsules.[6]
- **Margenza™ (margetuximab-cmkb):** The FDA has approved MacroGenics' Margenza for use in combination with chemotherapy for treatment of metastatic HER2-positive breast cancer patients who have received two or more prior anti-HER2-targeted therapies, at least one of which was for metastatic disease. Margenza is an intravenous Fc-engineered anti-HER2 monoclonal antibody. Margenza's approval was based on Phase 3 SOPHIA trial that demonstrated a statistically significant 24% reduction in the risk of disease progression or death when compared to trastuzumab plus chemotherapy. The objective response rate (ORR) for Margenza plus chemotherapy was 22% compared to 16% with trastuzumab plus chemotherapy.[7] MacroGenics is planning to launch in March 2021 with pricing to follow.
- **Riabni™ (rituximab-arrx):** The FDA has approved Amgen's Riabni as a biosimilar to Genentech's Rituxan® (rituximab). Riabni was approved based on two clinical trials that confirmed no clinically meaningful differences between Riabni and the originator biological, Rituxan. There are already two FDA-approved biosimilars for Rituxan; Teva/Celltrion's Truxima® and Pfizer's Ruxience®. Launch and price are pending for Riabni.[8]

continued

NEW INDICATIONS

- **Imfinzi® (durvalumab):** The FDA granted approval of AstraZeneca's Imfinzi as a new four-week, fixed-dose regimen for the PD-L1 inhibitor immuno-oncologic, consistent with approved dosing in extensive-stage small cell lung cancer (ES-SCLC), for Imfinzi's approved indications in urothelial cancer and non-small cell lung cancer (NSCLC).
- **Benlysta® (belimumab):** The FDA expanded the indication of GlaxoSmithKline's Benlysta to include the treatment of lupus nephritis.

DECEMBER NEWS

- "The U.S. Food and Drug Administration issued the first emergency use authorization (EUA) for a vaccine for the prevention of coronavirus disease 2019 (COVID-19) caused by severe acute respiratory syndrome coronavirus 2 (SARS-CoV-2) in individuals 16 years of age and older. The emergency use authorization allows the Pfizer-BioNTech COVID-19 Vaccine to be distributed in the U.S."[9]
- "The FDA is likely to authorize the Moderna vaccine as soon as Friday, according to a person with knowledge of the situation who spoke on the condition of anonymity because they were not authorized to discuss the issue. Anticipating that decision shortly, Gen. Gustave Perna, who is overseeing the federal effort to distribute vaccines, said Monday that the United States was preparing to ship almost 6 million doses of the Moderna vaccines to 3,285 locations in the first week."[10]
- "Sanofi's response to nonfatal thrombotic events in a Phase 3 hemophilia program has delayed its plans to seek approval for RNAi drug fitusiran by 18 months. The delay will "allow for the appropriate collection and assessment of safety and efficacy data under the amended protocols." Thrombotic events emerged as a significant concern for the fitusiran program in 2017 when dosing in a phase 2 trial was paused after a hemophilia A patient died from a blood clot. Alnylam, which ceded control of fitusiran to Sanofi early in 2018, responded to the original clinical hold by introducing new risk-mitigation measures."[11]

SPECIALTY NEW PRODUCT APPROVALS IN THE PAST TWELVE MONTHS

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	MONTH APPROVED
berotralstat	Orladeyo™	BioCryst	Prevention of hereditary angioedema (HAE) attacks	Oral	December 2020
setmelanotide	Imcivree™	Rhythm Pharmaceuticals	Pro-opiomelanocortin (POMC) deficiency obesity	SC	November 2020
lumasiran	Oxlumo™	Alnylam	Primary hyperoxaluria type 1 (PH1)	SC	December 2020
lonafarnib	Zokinvy™	Eiger BioPharmaceuticals	Progeria and progeroid laminopathies	Oral	November 2020
mannitol for inhalation	Bronchitol™	Chiesi Group and Pharmaxis	Cystic fibrosis	Inhaled	November 2020
remdesivir	Veklury™	Gilead	COVID-19	IV	October 2020
atoltivimab, maftivimab and odesivimab-ebgn	Inmazeb™	Regeneron	Ebola	IV	October 2020
tofacitinib	Xeljanz™	Pfizer	Active polyarticular course juvenile idiopathic arthritis (pcJIA)	Oral	September 2020
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	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

continued

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

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

NEW INDICATIONS FOR APPROVED SPECIALTY PRODUCTS

GENERIC NAME	BRAND NAME	MANUFACTURER	NEW INDICATION(S)	DATE APPROVED
belimumab	Benlysta®	GlaxoSmithKline	Lupus nephritis	December 2020
pitolisant	Wakix®	Harmony Biosciences	Cataplexy in adults with narcolepsy	October 2020
C1 Esterase Inhibitor Subcutaneous (Human)	Haegarda®	CSL Behring	Routine prophylaxis of hereditary angioedema attacks in patients six years of age and older	September 2020
golimumab	Simponi Aria®	Johnson & Johnson (Janssen)	Patients two years of age and older for the treatment of active psoriatic arthritis (PsA) or active polyarticular juvenile idiopathic arthritis (pJIA)	September 2020
ivacaftor	Kalydeco®	Vertex	Expanded population (ages four months to less than six months old) for the combination regimen of the cystic fibrosis	September 2020
tofacitinib	Xeljanz®	Pfizer	Pediatric patients two years and older with juvenile idiopathic arthritis (pcJIA)	September 2020
mepolizumab	Nucala®	GlaxoSmithKline	Pediatric patient 12 years and older with hypereosinophilic syndrome (HES)	September 2020
dolutegravir and lamivudine	Dovato®	ViiV Healthcare	Switch treatment for HIV-1 infection	August 2020
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

continued

NEW INDICATIONS FOR APPROVED SPECIALTY PRODUCTS *(continued)*

GENERIC NAME	BRAND NAME	MANUFACTURER	NEW INDICATION(S)	DATE APPROVED
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 six years and above	March 2020
nintedanib	Ofev®	Boehringer Ingelheim	Fibrosing interstitial lung diseases (ILD) with a progressive phenotype	March 2020

ONCOLOGY PRODUCT APPROVALS IN THE PAST TWELVE MONTHS

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	DATE APPROVED
margetuximab-cmkb	Margenza™	MacroGenics	Breast cancer	IV	December 2020
naxitamab-gqgk	Danyelza™	Y-mAbs	Relapsed or refractory high-risk neuroblastoma	IV	November 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
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

NEW INDICATIONS FOR APPROVED ONCOLOGY DRUGS

GENERIC NAME	BRAND NAME	MANUFACTURER	NEW INDICATION	DATE APPROVED
Durvalumab	Imfinzi®	AstraZeneca	New four-week, fixed-dose regimen for the PD-L1 inhibitor immuno-oncologic, consistent with approved dosing in extensive-stage small cell lung cancer (ES-SCLC), for Imfinzi's approved indications in urothelial cancer and non-small cell lung cancer (NSCLC)	November 2020
pembrolizumab	Keytruda®	Merck	In combination with chemotherapy for treatment of patients with locally recurrent unresectable or metastatic triple negative breast cancer (TNBC) whose tumors express PD-L1 (CPS ≥10)	November 2020
pembrolizumab	Keytruda®	Merck	Monotherapy for treatment of adults with relapsed or refractory classical Hodgkin lymphoma (cHL)	October 2020
nivolumab and ipilimumab	Opdivo® and Yervoy®	Bristol-Myers Squibb	First line treatment for malignant pleural mesothelioma	October 2020
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

continued

NEW INDICATIONS FOR APPROVED ONCOLOGY DRUGS *(continued)*

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 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
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
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

BIOSIMILAR PRODUCT APPROVALS IN THE PAST TWELVE MONTHS

GENERIC NAME	BRAND NAME	REFERENCE PRODUCT	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	MONTH APPROVED
rituximab-arrx	Riabni™	Rituxan®	Amgen	Oncology	IV	December 2020
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

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	2020
tanezumab	N/A	Pfizer And Lilly	Osteoarthritis	SC	December 2020
inclisiran	N/A	Novartis	Hyperlipidemia	SC	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
dostarlimab	N/A	GlaxoSmithKline (Tesaro) and AnaptysBio	Endometrial cancer	IV	January 2021
voclosporin	N/A	Aurinia	Lupus nephritis	Oral	January 2021
pegunigalsidase alfa (PRX-102)	N/A	Chiesi Global Rare Diseases and Protalix	Fabry disease	IV	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	Amondys 45®	Sarepta Therapeutics	Duchenne muscular dystrophy (DMD)	IV	February 2021
tepotinib	N/A	EMD Serono	Certain forms of metastatic non-small cell lung cancer (NSCLC)	Oral	February 2021
oral paclitaxel (paclitaxel and encequidar)	N/A	Athenex and Hanmi	Certain forms of breast cancer	Oral	February 2021
melflufen	N/A	Oncopeptides	Certain forms of relapsed refractory multiple myeloma (RRMM)	IV	February 2021
pralsetinib (Previously approved as Gavreto® under separate NDA in lung cancer)	N/A	Blueprint medicines corporation	RET mutant medullary thyroid cancer (MTC) and RET fusion-positive thyroid cancers	Oral	February 2021
fosdenopterin	N/A	Origin Biosciences (BridgeBio)	Molybdenum cofactor deficiency (MoCD) Type A	IV	March 2021
plasminogen (human plasma-derived purified)	Ryplazim®	Prometic Life Sciences Inc	Congenital plasminogen deficiency	IV	March 2021
ropeginterferon alfa-2b	N/A	PharmaEssentia	Polycythemia vera (PV)	SC	March 2021
aducanumab	N/A	Biogen	Alzheimer's disease	IV	March 2021
arimoclomol	N/A	Orphazyme	Niemann-Pick disease Type C (NPC)	Oral	March 2021
tivozanib	N/A	Aveo Oncology	Relapsed or refractory renal cell carcinoma (RCC)	Oral	March 2021
abrocitinib	N/A	Pfizer And Lilly	Atopic dermatitis	Oral	April 2021

SPECIALTY PIPELINE *(continued)*

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	ANTICIPATED APPROVAL DATE*
brincidofovir	N/A	Chimerix	Smallpox	Oral	April 2021
tralokinumab	N/A	Leo Pharma	Moderate to severe atopic dermatitis	IV/SC	May 2021
leuprolide mesylate 50 mg depot	Camcevi™ 42mg	Foresee Pharmaceuticals	Palliative treatment of advanced prostate cancer	Injectable suspension	May 2021
pegcetacoplan	N/A	Apellis	Paroxysmal nocturna hemoglobinuria	SC	May 2021
belumosudil	N/A	Kadmon	Chronic graft-versus-host disease (cGVHD)	Oral	May 2021
teplizumab	N/A	Provention Bio	Diabetes	SC	June 2021
infigratinib	N/A	QED Therapeutics (BridgeBio)	Certain forms of cholangiocarcinoma	Oral	June 2021
Lonapegsoma-tropin (TransCon hGH)	N/A	Acendis	Growth hormone deficiency (GHD)	Injection	June 2021
NexoBrid	N/A	MediWound	Burn tissue	Gel	June 2021
avacopan	N/A	Chemocentryx	ANCA-associated vasculitis	Oral	July 2021
amivantamab	N/A	Johnson & Johnson	Certain forms of non-small cell lung cancer	IV	July 2021
bimekizumab	N/A	UCB	Psoriasis	IV	July 2021
anifrolumab	N/A	AstraZeneca	Systemic lupus erythematosus (SLE)	IV	August 2021
vosoritide	N/A	BioMarin	Achondroplasia	SC	August 2021
loncastuximab tesirine (lonca)	N/A	ADC Therapeutics	R/R diffuse large B-cell lymphoma (DLBCL)	IV	September 2021
reltecimod	N/A	Atox bio	Organ failure	IV	September 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	2020
MYL-14020	N/A (Avastin® biosimilar)	Mylan NV and Biocon Ltd.	Oncology	IV	2020
BAT-1706	N/A (Avastin® biosimilar)	Bio-thera solutions	Oncology	IV	2021
Blood modifiers					
MSB11455	Stimufend® (Neulasta® biosimilar)	Fresenius Kabi	Neutropenia	SC	March 2021
Ophthalmology					
FYB201	N/A (Lucentis® biosimilar)	coherus biosciences/bioeq	Age-related macular degeneration	Injection into the eye	2020
SB11	N/A (Lucentis® biosimilar)	Samsung Bioepis	Age-related macular degeneration	Injection into the eye	September 2021
Autoimmune					
AVT02	N/A (Humira® biosimilar)	Alvotech	Autoimmune	SC	September 2021

FDA APPROVED GENE/CELL THERAPY PRODUCT APPROVALS

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	ANTICIPATED APPROVAL DATE*
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 FIRST QUARTER 2021 PIPELINE

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	ANTICIPATED APPROVAL DATE*
lisocabtagene maraleucel (liso-cel)	Breyanzi™	Bristol-Myers Squibb	R/R large B cell lymphoma	IV	2021
RVT-802	N/A	Enzyvant	Pediatric congenital athymia	IV	2021
idecabtagene vicleucel (bb2121, ide-cel)	N/A	Bristol-Myers Squibb and bluebird bio	Multiple myeloma	CAR-T	March 2021

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