

Specialty Pipeline MONTHLY UPDATE

Critical updates in an ever changing environment

December 2021

NEW DRUG INFORMATION

- **Voxzogo™ (vosoritide):** The U.S. Food and Drug Administration (FDA) has granted accelerated approval to Biomarin Pharmaceuticals' Voxzogo™ (vosoritide) for injection, to increase linear growth in pediatric patients with achondroplasia five years of age and older with open epiphyses (growth plates). Achondroplasia is the most common type of short-limbed dwarfism, with a global incidence of 1 in 15,000 to 40,000 newborns. Most cases are not inherited; about 80% are a result from new mutations in the FGFR3 gene causing abnormal formation of cartilage and bone and ultimately shorter bones. Voxzogo, a once daily subcutaneous injection, is a first-in-class C-type natriuretic peptide analog that works by regulating the signaling pathway downstream of the fibroblast growth factor receptor 3 (FGFR3) gene to assist bone growth. Voxzogo was approved based on a clinical trial that demonstrated patients treated with Voxzogo grew 1.6 centimeters more over the course of a year than those who received placebo.¹ BioMarin does not yet have data on whether Voxzogo can prevent the hearing loss, sleep apnea, and life-threatening skeletal problems that can result from achondroplasia, complications that take years to develop. Voxzogo has launched with an average wholesale acquisition cost (WAC) of approximately \$320,000 per year for the treatment.
- **Fyarro™ (sirolimus protein-bound particles for injectable suspension):** The FDA approved Aadi Bioscience's Fyarro for injectable suspension, in adults with locally advanced unresectable or metastatic malignant perivascular epithelioid cell tumor (PEComa). Approval is based on a Phase 2 trial that demonstrated an overall response rate (ORR) of 39% (12/31 patients), with two patients achieving a complete response (CR) after prolonged follow up. Among responders, 92% had a response lasting greater than or equal to six months; 67% had a response lasting greater than or equal to 12 months; and 58% had a response lasting greater than or equal to two years.² The recommended dosage is 100mg/m² administered as an IV infusion over 30 minutes on days one and eight of each 21-day cycle until disease progression or unacceptable toxicity. Fyarro is set to launch in the first quarter of 2022 with a WAC of approximately \$39,000 a month, or \$468,000 a year.

- **Tarpeyo™ (budesonide) delayed release capsules:** Calliditas Therapeutics' Tarpeyo has been approved by the FDA to reduce proteinuria in adults with primary immunoglobulin A nephropathy (IgAN) at risk of rapid disease progression, generally a urine protein-to-creatinine ratio (UPCR) ≥ 1.5 g/g. This indication is approved under accelerated approval. It has not been established whether Tarpeyo slows kidney function decline in patients with IgAN. Continued approval may be contingent upon verification and description of clinical benefit in a confirmatory clinical trial. IgAN is a rare, progressive autoimmune disease, which has a high unmet need with more than 50% of patients potentially progressing to end-stage renal disease (ESRD)³. Patients taking Tarpeyo demonstrated a statistically significant 34% reduction in proteinuria from baseline vs 5% with RAS inhibitors alone at nine months meeting its primary endpoint in the Phase 3 clinical trial NeflgArd.³ Tarpeyo is scheduled to launch first quarter of 2022 with pricing to follow.

NEW INDICATIONS

- **Keytruda® (pembrolizumab):** The FDA expanded the indication of Merck's Keytruda to include adjuvant treatment of patients with renal cell carcinoma (RCC) at intermediate-high or high risk of recurrence following nephrectomy or following nephrectomy and resection of metastatic lesions.

DECEMBER NEWS

- "If both its BioNTech-partnered COVID-19 vaccine and in-house antiviral live up to their full promises, Pfizer may be sitting on a \$100 billion goldmine in 2022. That's according to SVB Leerink analyst Geoffrey Porges, who released his latest forecast complete with a lengthy breakdown of all the intricate factors going into his calculation. Bottom line: universal boosting and pediatric recommendations will shore up global COVID vaccine sales to \$59 billion in 2021 and \$48 billion in 2022, or \$107 billion in cumulative sales. While "tremendous uncertainty" remains about Paxlovid such as capacity, price, shelf-life, market share and labeling, Porges noted that one thing is certain: Orders of Paxlovid will be large, given there's already a \$5.3 billion contract in the U.S., and the revenue will be profitable."⁴
- "Johnson & Johnson expects to become a \$60-billion-a-year drugmaker by 2025, execs said during a pharmaceutical strategy review. Specifically, J&J expects to achieve compound annual revenue growth of at least 5% per year through the middle of the decade, with growth expected every year, Jennifer Taubert, executive vice president, worldwide chairman of pharmaceuticals at J&J, said on the call. To get there, the company will rely on a combination of established medicines, investigational drugs, and new, innovative therapeutics such as CAR-T meds and gene therapies, Taubert said. The company has 14 novel medicines on the way, all of which have more than \$1 billion in sales potential. Even sweeter, five of those products have the potential to reap \$5 billion or more, Taubert said. J&J stalwarts Darzalex, Imbruvica, Erleada and Tremfya are a few examples of existing drugs poised for growth, Taubert said. Cancer med Darzalex could soon get a boost from a combination regimen approval in frontline multiple myeloma. Together with Revlimid and the steroid dexamethasone, the combo therapy recently posted a progression-free survival improvement of nearly 30 months versus Revlimid alone, Taubert said. On the immunology front, J&J's IL-23 inhibitor Tremfya, approved in psoriasis and psoriatic arthritis, could soon make the jump into Crohn's disease and ulcerative colitis."⁵

- “The U.S. health regulator has added its strictest warning to the labels of drugs from Pfizer, Eli Lilly and AbbVie belonging to a class of anti-inflammatory treatments called JAK inhibitors, citing risk of serious health issues and death in patients 50 and over, the drugmakers said. The addition of the [warning](#) on the labels follows the agency's review of Pfizer's Xeljanz after initial results from a February trial showed an increased risk of serious heart-related problems and cancer in some patients being treated with the drug.”⁶

SPECIALTY NEW PRODUCT APPROVALS IN THE PAST TWELVE MONTHS

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	MONTH APPROVED*
budesonide	Tarpeyo®	Calliditas Therapeutics	Primary IgA nephropathy (IgAN)	Oral	December 2021
vosoritide	Voxzogo®	BioMarin	Achondroplasia	SC	November 2021
ropeginterferon alfa-2b-nift	Besremi®	PharmaEssentia	Polycythemia vera (PV)	SC	November 2021
treprostinil inhalation powder	Yutrepia® Tentative approval	Liquidia	Pulmonary arterial hypertension	Inhaled dry powder	November 2021
ranibizumab via ocular implant	Susvimo®	Genentech	Age-related macular degeneration	Intravitreal injection	October 2021
triamcinolone acetonide	Xipere®	Clearside Biomedical and Bausch + Lomb (Bausch Health)	Macular edema associated with uveitis	Suprachoroidal injection	October 2021
avacopan	Tavneos®	Chemocentryx	ANCA-associated vasculitis	Oral	October 2021
allogeneic processed thymus tissue-agdc	Rethymic®	Enzyvant (Sumitomo Dainippon, Sumitovant)	Certain forms of congenital athymia	Surgical	October 2021
maralixibat	Livmarli®	Mirum Pharmaceuticals	Alagille syndrome (ALGS)	Oral	September 2021
ruxolitinib cream	Opzelura®	Incyte Corporation	Atopic dermatitis	Topical	September 2021
lonapegsomatropin	Skytrofa®	Acendis	Pediatric growth hormone deficiency (GHD)	SC	September 2021
avalglucosidase alfa	Nexviazyme®	Sanofi-Aventis	Pompe disease	IV	August 2021
anifrolumab-fnia	Saphnelo®	AstraZeneca	Systemic lupus erythematosus (SLE)	IV	August 2021
selexipag	Uptravi®	Johnson & Johnson	Pulmonary arterial hypertension	IV	July 2021
odevixibat	Bylvay®	Albireo	Progressive familial intrahepatic cholestasis	Oral	July 2021
belumosudil	Rezurock®	Kadmon	Chronic graft-versus-host disease (cGVHD)	Oral	July 2021
allogeneic cultured keratinocytes and dermal fibroblasts in murine collagen-dsat	StrataGraft™	Mallinckrodt	Deep partial-thickness thermal burns	Topical	June 2021
sofosbuvir/velpatasvir	Epclusa® pellets	Gilead	Hepatitis C	Oral	June 2021

aducanumab-avwa	Aduhelm®	Biogen	Alzheimer's disease	IV	June 2021
plasminogen (human-tvmh)	Ryplazim®	Prometic Life Sciences Inc	Plasminogen deficiency type 1	IV	June 2021
pegcetacoplan	Empaveli®	Apellis	Paroxysmal nocturnal hemoglobinuria	SC	May 2021
ponesimod	Ponvory®	Janssen	Multiple sclerosis	Oral	March 2021
fosdenopterin	Nulibry®	Origin Biosciences (BridgeBio)	Molybdenum cofactor deficiency (MoCD) Type A	IV	February 2021
casimersen	Amondys 45®	Sarepta Therapeutics	Duchenne muscular dystrophy (DMD)	IV	February 2021
evinacumab	Evkeeza™	Regeneron	Homozygous familial hypercholesterolemia (HoFH)	IV	February 2021
voclosporin	Lupkyis™	Aurinia	Lupus nephritis	Oral	January 2021

NEW INDICATIONS FOR APPROVED SPECIALTY PRODUCTS

GENERIC NAME	BRAND NAME	MANUFACTURER	NEW INDICATION(S)	DATE APPROVED
dupilumab	Dupixent®	Sanofi and Regeneron	Add-on treatment of pediatric patients aged 6 to 11 years of age with uncontrolled moderate-to-severe asthma	October 2021
bictegravir, emtricitabine and tenofovir alafenamide	Biktarvy®	Gilead	Expanded pediatric indication for the integrase strand transfer inhibitor (INSTI)-based single-tablet regimen and new low-dose tablet formulation for treatment of HIV-1 infected pediatric patients weighing at least 14 kg	October 2021
ruxolitinib	Jakafi®	Incyte	Steroid-refractory chronic graft versus host disease (SR chronic GVHD) in adults and pediatric patients 12 years and older	September 2021
calcium, magnesium, potassium and sodium oxybates	Xywav®	Jazz Pharmaceuticals	Idiopathic hypersomnia	August 2021
mepolizumab	Nucala®	Glaxo Smith Kline	Chronic rhinosinusitis with nasal polyps	July 2021
tacrolimus	Prograf®	Astellas Pharma	Prevent organ rejection in adult and pediatric patients receiving lung transplantation	July 2021
ravulizumab-cwvz	Ultomiris®	Alexion	Pediatric patients with paroxysmal nocturnal hemoglobinuria (PNH)	June 2021
elexacaftor, tezacaftor, and ivacaftor	Trikafta®	Vertex Pharmaceuticals	Ages 6 to 11 with at least one F508del mutation in the CFTR gene (the gene defective in CF) or a CFTR mutation that is responsive to the therapy	June 2021
ozanimod	Zeposia®	Bristol-Myers Squibb	Treatment of adults with moderately to severely active ulcerative colitis (UC)	May 2021
omalizumab	Xolair®	Genentech	New prefilled syringe for treatment of persistent asthma and chronic idiopathic urticaria	April 2021
risankizumab-rzaa	Skyrizi®	AbbVie	New autoinjector and a prefilled syringe with needle stock prevention for treatment of moderate-to-severe plaque psoriasis in adults who are candidates for systemic therapy or phototherapy	April 2021
treprostinil	Tyvaso®	United Therapeutics Corp	Pulmonary hypertension associated with interstitial lung disease to improve exercise ability	March 2021
tocilizumab	Actemra®	Genentech	Slowing the rate of decline in pulmonary function in adult patients with systemic sclerosis-associated interstitial lung disease	March 2021
adalimumab	Humira®	AbbVie	Moderately to severely active ulcerative colitis in adults and pediatric patients 5 years of age and older	February 2021

romiplostim	Nplate®	Amgen	Increase survival in adults and in pediatric patients (including term neonates) acutely exposed to myelosuppressive doses of radiation (hematopoietic syndrome of acute radiation syndrome, or HSARS)	January 2021
carglumic acid	Carbaglu®	Recordati Rare Diseases	Adjunctive therapy to standard of care for treatment of acute hyperammonemia due to propionic acidemia (PA) or methylmalonic acidemia (MMA)	January 2021

ONCOLOGY PRODUCT APPROVALS IN THE PAST TWELVE MONTHS

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	DATE APPROVED
sirolimus albumin-bound nanoparticles for injectable suspension	Fyarro®	Aadi Bioscience	Metastatic or locally advanced malignant perivascular epithelioid cell tumor (PEComa)	IV	November 2021
asciminib	Scemblix®	Novartis	Certain forms of chronic myeloid leukemia (CML)	Oral	October 2021
tisotumab vedotin-tftv	Tivdak®	Seagen	Certain forms of cervical cancer	IV	September 2021
mobocertinib (TAK-788)	Exkivity®	Takeda Pharmaceuticals	Metastatic non-small cell lung cancer (mNSCLC)	Oral	September 2021
belzutifan	Welireg®	Merck	Von Hippel-Lindau (VHL) disease	Oral	August 2021
dostarlimab-gxly	Jemperli™	GlaxoSmithKline (Tesaro) and AnaptysBio	Certain forms of solid tumors	IV	August 2021
asparaginase erwinia chrysanthemi-rywn	Rylaze®	Jazz Pharmaceuticals	Acute lymphoblastic leukemia (ALL) or lymphoblastic lymphoma (LBL)	IV	June 2021
sotorasib	Lumakras®	Amaen	Certain forms of lung cancer	Oral	May 2021
infigratinib	Truseltiq®	QED Therapeutics (BridgeBio)	Certain forms of cholangiocarcinoma	Oral	May 2021
leuprolide mesylate 50 mg depot	Camcevi® 42mg	Foresee Pharmaceuticals	Palliative treatment of advanced prostate cancer	Injectable suspension	May 2021
amivantamab-vmjw	Rybrevant™	Johnson & Johnson	Certain forms of non-small cell lung cancer	IV	May 2021
dostarlimab-gxly	Jemperli™	GlaxoSmithKline (Tesaro) and AnaptysBio	Endometrial cancer	IV	April 2021
loncastuximab tesirine (lonca)	Zynlonta™	ADC Therapeutics	R/R diffuse large B-cell lymphoma (DLBCL)	IV	April 2021
tivozanib	Fotivda™	Aveo Oncology	R/R renal cell carcinoma (RCC)	Oral	March 2021
melphalan flufenamide	Pepaxto™	Oncopeptides	Certain forms of R/R multiple myeloma (RRMM)	IV	February 2021
trilaciclib	Cosela™	G1 Therapeutics	Myelopreservation	IV	February 2021
umbralisib	Ukoniq™	TG Therapeutics	Marginal zone lymphoma (MZL)	Oral	February 2021
tepotinib	Tepmetko®	EMD Serono	Certain forms of metastatic non-small cell lung cancer (NSCLC)	Oral	February 2021

NEW INDICATIONS FOR APPROVED ONCOLOGY DRUGS

GENERIC NAME	BRAND NAME	MANUFACTURER	NEW INDICATION(S)	DATE APPROVED
pembrolizumab	Keytruda®	Merck	Adjuvant treatment of renal cell carcinoma (RCC)	November 2021
atezolizumab	Tecentriq®	Roche (Genentech)	Stage II-IIIa non-small cell lung cancer (NSCLC)	October 2021
pembrolizumab	Keytruda®	Merck	Combination with chemotherapy, with or without bevacizumab, treatment of patients with persistent, recurrent or metastatic cervical cancer whose tumors express PD-L1	October 2021
abemaciclib	Verzenio®	Lilly	In combination with endocrine therapy (tamoxifen or aromatase inhibitor) for adjuvant treatment of adults with hormone receptor-positive (HR+), human epidermal growth factor receptor 2-negative (HER2-)	October 2021
brexucabtagene autoleucel	Tecartus®	Gilead Sciences	Adults with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL)	October 2021
evolocumab	Repatha®	Amgen	Add-on treatment to diet alone or together with certain other therapies for patients aged 10 years and older with heterozygous familial hypercholesterolemia (HeFH) and homozygous familial hypercholesterolemia (HoFH)	September 2021
cabozantinib	Cabometyx®	Exelixis and Ipsen	Patients aged 12 and older with locally advanced or metastatic differentiated thyroid cancer (DTC) that has progressed following prior vascular endothelial growth factor receptor (VEGFR)-targeted therapy and who are radioactive iodine-refractory or ineligible	September 2021
cetuximab	Erbitux®	Lilly	Certain forms of metastatic colorectal cancer (CRC) with a BRAF V600E mutation	September 2021
Ivosidenib	Tibsovo®	Agios Pharmaceuticals	Locally advanced or metastatic cholangiocarcinoma with an IDH1 mutation, which is a rare type of cancer in the bile ducts in the liver	September 2021
zanubrutinib	Brukinsa®	Beigene USA	Marginal zone lymphoma (MZL) who have received at least one prior anti-CD20-based therapy	September 2021
zanubrutinib	Brukinsa®	Beigene USA	Rare indolent B-cell lymphoma Waldenstrom's Macroglobulinemia (WM)	August 2021
Nivolumab	Opdivo®	Bristol-Myers Squibb	Certain forms of urothelial carcinoma (UC)	August 2021
pembrolizumab	Keytruda®	Merck	First-line treatment of patients with RCC	August 2021
pembrolizumab	Keytruda®	Merck	Certain forms of triple negative breast cancer (TNBC) in combination with chemotherapy	July 2021
pembrolizumab and lenvatinib	Keytruda® and Lenvima®	Merck and Eisai	Certain forms of advanced endometrial carcinoma that is not microsatellite instability-high (MSI-H) or mismatch repair deficient (dMMR)	July 2021

enfortumab vedotin-ejfy	Padcev®	Astellas and Seagen	Treatment of patients with locally advanced or metastatic urothelial cancer who have received a PD-1/L1 inhibitor and are ineligible for cisplatin	July 2021
daratumumab and hyaluronidase-fihj	Darzalex Faspro®	Janssen Biotech and Genmab	Combination regimen with pomalidomide and dexamethasone (d-Pd) for treatment of patients with relapsed or refractory multiple myeloma who have received at least one prior line of therapy	July 2021
avapritinib	Ayvakit®	Blueprint Medicines Corp	Adults with advanced systemic mastocytosis (SM)	June 2021
pembrolizumab	Keytruda®	Merck	Certain combination for first-line treatment of patients with locally advanced unresectable or metastatic HER2-positive gastric or gastroesophageal junction adenocarcinoma	May 2021
nivolumab	Opdivo®	Bristol-Myers Squibb	Adjuvant treatment of patients with resected esophageal or gastroesophageal junction (GEJ) cancer after neoadjuvant chemoradiation therapy (CRT)	May 2021
nivolumab	Opdivo®	Bristol-Myers Squibb	Certain forms of advanced or metastatic gastric cancer, gastroesophageal junction cancer (GEJC) or esophageal adenocarcinoma (EAC)	May 2021
lorlatinib	Lorbrena®	Pfizer	Treatment of previously untreated advanced ALK-positive non-small cell lung cancer (NSCLC)	April 2021
sacituzumab qovitecan-hziy	Trodelvy®	Gilead Sciences	Triple-negative breast cancer that has spread to other parts of the body	April 2021
nivolumab	Opdivo®	Bristol-Myers Squibb	Initial treatment of patients with advanced or metastatic gastric cancer, gastroesophageal junction cancer and esophageal adenocarcinoma	April 2021
pembrolizumab	Keytruda®	Merck	Certain forms of locally advanced unresectable or metastatic carcinoma of the esophagus and gastroesophageal junction (GEJ)	March 2021
axicabtagene ciloleucel	Yescarta®	Kite Pharm	Relapsed or refractory follicular lymphoma and marginal zone lymphoma after two or more prior lines of systemic therapy	March 2021
cytarabine and daunorubicin	Vyxeos®	Jazz Pharmaceuticals	Certain forms of acute myeloid leukemia (t-AML) or AML with myelodysplasia-related changes (AML-MRC)	March 2021
sacituzumab govitecan-hziy	Trodelvy®	Gilead Sciences	Adult patients with locally advanced or metastatic urothelial cancer (UC)	March 2021
isatuximab-irfc	Sarclisa®	Sanofi PS	Patients with multiple myeloma who have received at least one prior therapy	March 2021
cemiplimab-rwlc	Libtayo®	Regeneron	Monotherapy for treatment of first line locally advanced or metastatic non-small cell lung cancer (NSCLC) with PD-L1 expression	February 2021
cemiplimab-rwlc	Libtayo®	Regeneron	Treatment of locally advanced or metastatic basal cell carcinoma (BCC)	February 2021
pralsetinib	Gavreto®	Blueprint medicines	Treatment of patients with advanced or metastatic RET mutant medullary thyroid cancer (MTC) and RET fusion-positive thyroid cancers.	February 2021

darolutamide	Nubeqa®	Bayer Pharmaceutical	Non-metastatic castration-dependent prostate cancer	January 2021
daratumumab and hyaluronidase-fihj	Darzalex Faspro®	Janssen Biotech and Genmab	Light chain (AL) amyloidosis	January 2021
fam-trastuzumab deruxtecan-nxki	Enhertu®	Daiichi Pharmaceutical	Patients with HER2-positive metastatic gastric or gastroesophageal junction (GEJ) adenocarcinoma	January 2021
cabozantinib	Cabometyx®	Exelixis	In combination with the immuno-oncologic nivolumab (Bristol-Myers Squibb's Opdivo) for first-line treatment of advanced renal cell carcinoma (RCC)	January 2021
nivolumab	Opdivo®	Bristol-Myers Squibb	In combination with cabozantinib (Exelixis' Cabometyx) for first-line treatment of advanced renal cell carcinoma (RCC)	January 2021
crizotinib	Xalkori®	Pfizer	Pediatric patients with certain forms of relapsed or refractory systemic anaplastic large cell lymphoma	January 2021

BIOSIMILAR PRODUCT APPROVALS IN THE PAST TWELVE MONTHS

GENERIC NAME	BRAND NAME	REFERENCE PRODUCT	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	MONTH APPROVED
ranibizumab-nuna	Byooviz™	Lucentis® biosimilar	Samsung Bioepis	Age related macular degeneration	Intravitreal	September 2021
insulin glargine-yfgn <i>First interchangeable biosimilar for Lantus</i>	Semglee™	Lantus®	Mylan and Biocon	Diabetes	SC	July 2021

SPECIALTY PIPELINE

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	ANTICIPATED APPROVAL DATE*
CPP-1X/sul (eflornithine and sulindac)	N/A	Cancer Prevention Pharmaceuticals	Familial adenomatous polyposis (FAP)	Oral	Delayed
donislecel purified allogeneic deceased donor pancreas derived islets of Langerhans	Lantidra®	CellTrans	Brittle type 1 diabetes	IV	Delayed
abrocitinib	N/A	Pfizer And Lilly	Atopic dermatitis	Oral	Delayed
SH-111	N/A	Shorla Pharma	Pediatric patients with T-cell leukemia	IV	Delayed
FT218 (sodium oxybate, controlled release)	N/A	Avadel Pharmaceuticals	Narcolepsy	Oral	Delayed
bimekizumab	Bemzelx®	UCB Pharma	Psoriasis	SC	Delayed

inolimomab	Leukotac®	Farmaceutici Spa	Steroid-refractory acute graft-versus-host disease	IV	Delayed
efgartigimod	N/A	Argenx	Generalized myasthenia gravis	IV	December 2021
balstilimab	N/A	Agenus	Recurrent or metastatic cervical cancer	IV	December 2021
LV-101 (carbetocin)	N/A	Levo Therapeutics, Inc.	Hyperphagia and behavioral distress associated with Prader-Willi syndrome	Intranasal	December 2021
somatrogon	N/A	Pfizer	Growth hormone deficiency	SC	January 2022
levoketoconazole	Recorlev®	Strongbridge Biopharma	Cushing's syndrome	Oral	January 2022
inclisiran	Leqvio®	Novartis	Hyperlipidemia	SC	January 2022
faricimab	N/A	Genentech	Diabetic macular edema	Intravitreal	January 2022
tezepelumab	Tezspire®	AstraZeneca and Amgen	Asthma across phenotypes	SC	January 2022
penpulimab (AK105)	N/A	Akeso and Sino Biopharmaceutical	Metastatic nasopharyngeal carcinoma	IV	January 2022
oleogel-S10	Filsuvez®	Amryt Pharma	Dystrophic epidermolysis bullosa	Topical	February 2022
bardoxolone	N/A	Reata Pharmaceuticals	Autosomal dominant polycystic kidney disease	Oral	February 2022
pacritinib	Enpaxiq®	CTI BioPharma	Myelofibrosis patients with severe thrombocytopenia	Oral	February 2022
GC5107 (immune globulin intravenous [human], 10% liquid)	N/A	GC Pharma	Primary humoral immunodeficiency	IV	February 2022
mitapivat	N/A	Agios Pharmaceuticals	Pyruvate kinase (PK) deficiency, a chronic hemolytic anemia.	Oral	February 2022
tebentafusp	N/A	Immunocore	Metastatic uveal melanoma (mUM)	IV	February 2022
sintilimab	N/A	Innovent Biologics and Lilly	Nonsquamous non-small cell lung cancer (NSCLC)	IV	March 2022
vadadustat	Vafseo™	Akebia Therapeutics	Anemia due to chronic kidney disease (CKD)	Oral	March 2022
ublituximab	N/A	TG Therapeutics	Certain forms of chronic lymphocytic leukemia (CLL)	IV	March 2022
relatlimab and nivolumab	N/A	Bristol Myers Squibb	Certain forms of metastatic melanoma	IV	March 2022

ganaxolone	Ztalmy™	Marinus Pharmaceuticals, Inc.	Seizures associated with CDKL5 deficiency disorder (CDD)	Oral	March 2022
relatlimab and nivolumab	N/A	Bristol Myers Squibb	Certain forms of unresectable or metastatic melanoma	IV	March 2022
efbemalenograstim alfa	Ryzneuta™	Evive Biotech	Chemotherapy-induced neutropenia	SC	March 2022
mavacamten	N/A	Bristol-Myers Squibb	Symptomatic obstructive hypertrophic cardiomyopathy (oHCM)	Oral	April 2022
vutrisiran	N/A	Amylam Pharmaceuticals	Polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis	SC	April 2022
surufatinib	N/A	Hutchmed	Pancreatic and extra-pancreatic neuroendocrine tumor	Oral	April 2022
toripalimab	N/A	Junshi Biosciences and Coherus	Certain forms of recurrent or metastatic nasopharyngeal carcinoma	IV	April 2022
parsaclisib	N/A	Incyte	Certain forms of mantle cell lymphoma (MCL)	Oral	April 2022
trientine tetrahydrochloride (TETA 4HCl)	N/A	Orphalan	Wilson's disease	Oral	May 2022
tapinarof	N/A	Dermavant (Roivant)	Plaque psoriasis	Topical	May 2022
miglustat (for AT-GAA combination with cipaglucosidase alfa)	N/A	Amicus Therapeutics	Pompe disease	Oral	May 2022
sodium phenylbutyrate	N/A	Acer Therapeutics and Relief Therapeutics	Urea cycle disorders (UCDs)	Oral	June 2022
tislelizumab	N/A	BeiGene and Novartis	Certain forms of esophageal squamous cell carcinoma (ESCC)	Injection	July 2022
cipaglucosidase alfa* (in combination with miglustat)	N/A	Amicus Therapeutics	Pompe disease	IV	July 2022
parsaclisib	N/A	Incyte	Certain forms of follicular lymphoma (FL)	Oral	August 2022

* 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					
MYL-1402O	N/A (Avastin® biosimilar)	Mylan NV and Biocon Ltd.	Oncology	IV	2022
SB8	N/A (Avastin® biosimilar)	Samsung Bioepis and Merck	Oncology	IV	2022
FKB238	N/A (Avastin® biosimilar)	Centus Biotherapeutics	Oncology	IV	2022
BAT-1706	N/A (Avastin® biosimilar)	Bio-thera solutions	Oncology	IV	2022
BEVZ92	N/A (Avastin® biosimilar)	mAbxience, Insud Pharma; Amneal	Oncology	IV	2Q2022
CT-P16	N/A (Avastin® biosimilar)	Celltrion	Oncology	IV	4Q2022
Blood Modifiers					
MSB11455	Stimufend® (Neulasta® biosimilar)	Fresenius Kabi	Neutropenia	IV or SC	2022
TPI-120	N/A (Neulasta® biosimilar)	Amneal and Kashiv (Adello)	Neutropenia	IV or SC	2022
lupifil-p	N/A (Neulasta® biosimilar)	Lupin	Neutropenia	SC	April 2022
filgrastim kashiv	N/A (Neupogen® biosimilar)	Adello Biologics	Neutropenia	IV	2022
TX-01	N/A (Neupogen® biosimilar)	Tanvex BioPharma	Neutropenia	SC	2022
Ophthalmology					
FYB201	N/A (Lucentis® biosimilar)	Coheres biosciences/bioeq	Age-related macular degeneration	Injection into the eye	2022
MYL-1701P	N/A (Eylea® biosimilar)	Mylan	Age-related macular degeneration	Injection into the eye	2022
Autoimmune					
AVT02	N/A (Humira® biosimilar)	Alvotech	Autoimmune	SC	2022
CHS-1420	N/A (Humira® biosimilar)	Coherus	Autoimmune	SC	2022
CT-P17	N/A (Humira® biosimilar)	Celltrion	Autoimmune	SC	4Q2022

FDA APPROVED GENE/CELL THERAPY PRODUCT APPROVALS

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	MONTH APPROVED
allogeneic processed thymus tissue adgc	Rethymic™	Enzyvant	Congenital athymia	Surgical	October 2021
idecabtagene vicleucel (bb2121, ide-cel)	Abecma™	Bristol-Myers Squibb and bluebird bio	Multiple myeloma	CAR-T	March 2021
lisocabtagene maraleucel (liso-cel)	Breyanzi™	Bristol-Myers Squibb	Large B cell lymphoma (LBCL)	IV	February 2021
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	LBCL/follicular lymphoma (FL)	IV	October 2017
tisagenlecleucel	Kymriah®	Novartis	Acute lymphoblastic leukemia/LBCL	IV	August 2017

GENE/CELL THERAPY 1H2022 PIPELINE

GENERIC NAME	BRAND NAME	MANUFACTURER	INDICATION(S)	ROUTE OF ADMINISTRATION	ANTICIPATED APPROVAL DATE*
ciltacabtagene autoleucel (ciltacel)	N/A	Johnson & Johnson (Janssen)	Relapsed and refractory multiple myeloma	CAR-T	February 2022
betibeglogene autotemcel	N/A	Bluebird Bio	Transfusion-dependent beta-thalassemia (TDT)	IV	May 2022

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