PHARMNOTES

June 2023

Date: 7/7/2023

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Drug Safety Alert Notification

- No drug safety communication published in June.

New FDA-Approved Drug Products

DRUG NAME	MANUFACTURER	APPROVAL DATE
COLUMVI™ (GLOFITAMAB-GXBM) INJECTION	GENENTECH INC.	6/15/2023

THERAPEUTIC CLASS
Antineoplastics

FDA-APPROVED INDICATION(S)
Columvi™ is a bispecific CD20-directed CD3 T-cell engager indicated for the treatment of adult patients with relapsed or refractory diffuse large B-cell lymphoma, not otherwise specified (DLBCL, NOS) or large B-cell lymphoma (LBCL) arising from follicular lymphoma, after two or more lines of systemic therapy.

DOSAGE AND ADMINISTRATION
- Pretreat with a single 1000mg dose of obinutuzumab intravenously 7 days before initiation of Columvi™ (Cycle 1 Day 1).
- Administer premedications as recommended.
- Administer only as an intravenous infusion.

Treatment Cycle[a]	Day	Dose of COLUMVI	
Cycle 1	Day 1	Obinutuzumab 1,000 mg	
	Day 8	Step-up dose 1	2.5 mg
	Day 15	Step-up dose 2	10 mg
Cycle 2-12	Day 1	30 mg	

- Administer in a facility equipped to monitor and manage CRS.
- Patients should be hospitalized for the 2.5 mg step-up dose and for subsequent infusions as recommended.

DOSAGE FORMS AND STRENGTHS
Injection: 2.5 mg/2.5 mL (1 mg/mL) in a single-dose vial; 10 mg/10 mL (1 mg/mL) in a single-dose vial

SAFETY PROFILE

CONTRAINDICATIONS
- None.

WARNINGS AND PRECAUTIONS
- **BLACK BOX WARNING: CYTOKINE RELEASE SYNDROME**
  - Cytokine Release Syndrome (CRS), including serious or fatal reactions, can occur in patients receiving Columvi™. Premedicate before each dose, and initiate treatment with the Columvi™ step-up dosing schedule to reduce the risk of CRS. Withhold Columvi™ until CRS resolves or permanently discontinue based on severity.
- Neurologic Toxicity: Can cause serious neurologic toxicity, including Immune Effector Cell-Associated Neurotoxicity Syndrome (ICANS). Monitor for neurologic toxicity; withhold or permanently discontinue based on severity.
- Serious Infections: Can cause serious or fatal infections. Monitor patients for signs and symptoms of infection and treat appropriately.
- Tumor Flare: Can cause serious tumor flare reactions. Monitor patients at risk for complications of tumor flare.
- Embryo-Fetal Toxicity: May cause fetal harm. Advise females of reproductive potential of the potential risk to the fetus and to use effective contraception.

ADVERSE REACTIONS
- The most common (≥ 20%) adverse reactions, excluding laboratory abnormalities, are cytokine release syndrome, musculoskeletal pain, rash, and fatigue. The most common (≥ 20%) Grade 3 to 4 laboratory abnormalities are lymphocyte count decreased, phosphate decreased, neutrophil count decreased, uric acid increased, and fibrinogen decreased.

USE IN SPECIFIC POPULATIONS
- Pregnancy: Based on its mechanism of action Columvi™ may cause fetal harm when administered to a pregnant woman.
- Females and Males of Reproductive Potential: Columvi™ may cause fetal harm when administered to a pregnant woman. Verify pregnancy status in females of reproductive potential prior to initiating Columvi™. Advise female patients of reproductive potential to use effective contraception during treatment with Columvi™ and for 1 month after the last dose of Columvi™.

Orphan status: No

DRUG NAME	MANUFACTURER	APPROVAL DATE
LIFTULO™ (RITLECITINIB) CAPSULES	PFIZER INC.	6/23/2023

THERAPEUTIC CLASS
Dermatological agents

FDA-APPROVED INDICATION(S)
Liftulo™ is a kinase inhibitor indicated for the treatment of severe alopecia areata in adults and adolescents 12 years and older.

DOSAGE AND ADMINISTRATION
- Perform the following evaluations prior to Liftulo™ initiation: Tuberculosis infection valuation, viral hepatitis screening, blood count, immunizations.
- Recommended dosage: 50mg orally once daily with or without food. Swallow capsules whole. Do not crush, split or chew capsules.

DOSAGE FORMS AND STRENGTHS
Capsules: 50 mg of ritlecitinib

Orphan status: No

SAFETY PROFILE

CONTRAINDICATIONS
- Patients with known hypersensitivity to ritlecitinib or any of its excipients.

WARNINGS AND PRECAUTIONS
- **BLACK BOX WARNING: SERIOUS INFECTIONS, MORTALITY, MALIGNANCY, MAJOR ADVERSE CARDIOVASCULAR EVENTS (MACE) and THROMBOSIS**
  - Increased risk of serious bacterial, fungal, viral, and opportunistic infections that may lead to hospitalization or death, including tuberculosis (TB). Interrupt treatment if serious infection occurs until the infection is controlled. Liftulo™ should not be given to patients with active tuberculosis. Test for latent TB before and during therapy; start treating latent TB prior to use. Monitor all patients for active TB during treatment, even patients with initial negative, latent TB test. Monitor all patients for signs and symptoms of infection during and after treatment with Liftulo™.
  - Higher rate of all-cause mortality, including sudden cardiovascular death with another Janus kinase inhibitor (JAK) vs. TNF blockers in rheumatoid arthritis (RA) patients. Liftulo™ is not approved for use in RA patients.
  - Malignancies were reported in patients treated with Liftulo™. Higher rate of lymphomas and lung cancers with another JAK inhibitor vs. TNF blockers in RA patients.

WARNINGS AND PRECAUTIONS
- **BLACK BOX WARNING: SERIOUS INFECTIONS, MORTALITY, MALIGNANCY, MAJOR ADVERSE CARDIOVASCULAR EVENTS (MACE) and THROMBOSIS (continuation)**
  - Higher rate of MACE (defined as cardiovascular death, myocardial infarction, and stroke) with another JAK inhibitor vs. TNF blockers in RA patients.
  - Thrombosis has occurred in patients treated with Liftulo™. Increased incidence of pulmonary embolism, venous and arterial thrombosis with another JAK inhibitor vs. TNF blockers.
- Hypersensitivity: Discontinue Liftulo™ if a clinically significant hypersensitivity reaction occurs.
- Laboratory Abnormalities: Perform ALC and platelet counts prior to Liftulo™ initiation. Treatment interruption or discontinuation are recommended based on ALC and platelet count abnormalities.
- Vaccinations: Avoid use of live vaccines during or shortly prior to Liftulo™ treatment.

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DRUG NAME	MANUFACTURER	APPROVAL DATE
LIFTULO™ (RITLECITINIB) CAPSULES	PFIZER INC.	6/23/2023

THERAPEUTIC CLASS
Dermatological agents

FDA-APPROVED INDICATION(S)
Liftulo™ is a kinase inhibitor indicated for the treatment of severe alopecia areata in adults and adolescents 12 years and older.

DOSAGE AND ADMINISTRATION
- Perform the following evaluations prior to Liftulo™ initiation: Tuberculosis infection valuation, viral hepatitis screening, blood count, immunizations.
- Recommended dosage: 50mg orally once daily with or without food. Swallow capsules whole. Do not crush, split or chew capsules.

DOSAGE FORMS AND STRENGTHS
Capsules: 50 mg of ritlecitinib

Orphan status: No

SAFETY PROFILE

ADVERSE REACTIONS
- Most common adverse reactions (incidence ≥1%) are headache, diarrhea, acne, rash, urticaria, folliculitis, pyrexia, atopic dermatitis, dizziness, blood creatine phosphokinase increased, herpes zoster, red blood cell count decreased, and stomatitis.

DRUG INTERACTIONS
- Certain CYP3A Substrates: Additional monitoring and dose adjustment of CYP3A substrate should be considered.
- Certain CYP1A2 Substrates: Additional monitoring and dose adjustment of CYP1A2 substrate should be considered.
- Certain CYP3A Inducers: Coadministration with strong inducers of CYP3A is not recommended.

USE IN SPECIFIC POPULATIONS
- Lactation: Because of the serious adverse effects in adults, including risks of serious infection and malignancy, advise women not to breastfeed during treatment with Liftulo™ and for approximately 14 hours after the last dose (approximately 6 elimination half-lives).
- Hepatic Impairment: Liftulo™ is not recommended in patients with severe (Child Pugh C) hepatic impairment.

DRUG NAME	MANUFACTURER	APPROVAL DATE
RYSTIGGO™ (ROZANOLIXIZUMAB-NOLI) INJECTION	UCB, INC.	6/26/2023

THERAPEUTIC CLASS
Antimyasthenic agents

FDA-APPROVED INDICATION(S)
Rystiggo™ is a neonatal Fc receptor blocker indicated for the treatment of generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AChR) or antimuscle-specific tyrosine kinase (MuSK) antibody positive.

DOSAGE AND ADMINISTRATION
- Evaluate the need to administer age-appropriate vaccines according to immunization guidelines before initiation of a new treatment cycle with Rystiggo™.
- For subcutaneous infusion only.
- The recommended dosage is administered as a subcutaneous infusion once weekly for 6 weeks.

Body Weight of Patient	Dose	Volume to be Infused
Less than 50 kg	420 mg	3 mL
50 kg to less than 100 kg	560 mg	4 mL
100 kg and above	840 mg	6 mL

- Administer subsequent treatment cycles based on clinical evaluation; the safety of initiating subsequent cycles sooner than 63 days from the start of the previous treatment cycle has not been established.

DOSAGE FORMS AND STRENGTHS
Injection: 280 mg/2 mL (140 mg/mL) in a single-dose vial

SAFETY PROFILE

WARNINGS AND PRECAUTIONS
- Infections: Delay administration of Rystiggo™ to patients with an active infection. Monitor for signs and symptoms of infection in patients treated with Rystiggo™. If serious infection occurs, administer appropriate treatment and consider withholding Rystiggo™ until the infection has resolved.
- Aseptic Meningitis: Serious events of aseptic meningitis have been reported. Monitor for symptoms; diagnostic workup and treatment should be initiated according to the standard of care.
- Hypersensitivity Reactions: Angioedema and rash have occurred. If a hypersensitivity reaction occurs, discontinue the infusion and institute appropriate therapy.

ADVERSE REACTIONS
- The most common adverse reactions (≥10%) in patients with gMG are headache, infections, diarrhea, pyrexia, hypersensitivity reactions, and nausea.

DRUG INTERACTIONS
- Closely monitor for reduced effectiveness of medications that bind to the human neonatal Fc receptor. When concomitant long-term use of such medications is essential for patient care, consider discontinuing Rystiggo™ and using alternate therapies.

USE IN SPECIFIC POPULATIONS
- Pregnancy: Based on animal data, may cause fetal harm.

Orphan status: Yes

DRUG NAME	MANUFACTURER	APPROVAL DATE
NGENLA™ (SOMATROGON-GHLA) INJECTION	PFIZER INC.	6/27/2023

THERAPEUTIC CLASS
Growth hormones

FDA-APPROVED INDICATION(S)
Ngenla™ is a a human growth hormone analog indicated for treatment of pediatric patients aged 3 years and older who have growth failure due to inadequate secretion of endogenous growth hormone.

DOSAGE AND ADMINISTRATION
- Administer Ngenla™ by subcutaneous injection once weekly, on the same day each week, at any time of the day in the abdomen, thighs, buttocks, or upper arms with weekly rotation of injection site.
- Recommended dosage is 0.66 mg/kg based on actual body weight administered once weekly.
- Individualize dosage for each patient based on the growth response.
- Patients switching from daily growth hormone may initiate treatment with once-weekly Ngenla™ on the day following their last daily injection.

DOSAGE FORMS AND STRENGTHS
Injection: 24 mg/1.2 mL (20 mg/mL) single-patient-use prefilled pen that delivers a dose in 0.2 mg increments; 60 mg/1.2 mL (50 mg/mL) single-patient-use prefilled pen that delivers a dose in 0.5 mg increments.

Orphan status: Yes

SAFETY PROFILE

CONTRAINDICATIONS
- Acute critical illness
- Hypersensitivity to somatrogon-ghla or excipients
- Closed epiphyses
- Active malignancy
- Active proliferative or severe non-proliferative diabetic retinopathy
- Prader-Willi syndrome who are severely obese or have severe respiratory impairment

WARNINGS AND PRECAUTIONS
- Severe Hypersensitivity: Severe hypersensitivity reactions may occur. In the event of an allergic reaction, seek prompt medical attention.
- Increased Risk of Neoplasms: Monitor patients with preexisting tumors for progression or recurrence. Increased risk of a second neoplasm in childhood cancer survivors treated with somatropin – in particular meningiomas in patients treated with radiation to the head for their first neoplasm.
- Glucose Intolerance and Diabetes Mellitus: Ngenla™ may decrease insulin sensitivity, particularly at higher doses. Monitor glucose levels periodically in all patients receiving Ngenla™, especially in patients with existing diabetes mellitus or at risk for its development.
- Intracranial Hypertension: Perform fundoscopic examinations prior to initiation of treatment with Ngenla™ and periodically thereafter. If preexisting papilledema is identified, evaluate the etiology and treat the underlying cause before initiating. If papilledema occurs with Ngenla™, stop treatment.

WARNINGS AND PRECAUTIONS
- Fluid Retention: May occur and may be dose dependent. Reduce dose as necessary.
- Hypoadrenalism: Monitor patients for reduced serum cortisol levels and/or need for glucocorticoid dose increases in those with known hypoadrenalism.
- Hypothyroidism: Monitor thyroid function periodically as hypothyroidism may become evident or worsen after initiation with Ngenla™.
- Slipped Capital Femoral Epiphysis: May develop. Evaluate patients with the onset of a limp or persistent hip or knee pain.
- Progression of Preexisting Scoliosis: Monitor for development or progression of scoliosis.
- Pancreatitis: Consider pancreatitis in patients with persistent severe abdominal pain.
- Lipoatrophy: May occur if Ngenla™ is administered in the same location over a long period of time. Rotate injection sites

ADVERSE RECTIONS
- Adverse reactions reported in ≥5% of patients treated with Ngenla™ are: injection site reactions, nasopharyngitis, headache, pyrexia, anemia, cough, vomiting, hypothyroidism, abdominal pain, rash, and oropharyngeal pain.

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DRUG NAME	MANUFACTURER	APPROVAL DATE
NGENLA™ (SOMATROGON-GHLA) INJECTION	PFIZER INC.	6/27/2023

THERAPEUTIC CLASS
Growth hormones

FDA-APPROVED INDICATION(S)
Ngenla™ is a a human growth hormone analog indicated for treatment of pediatric patients aged 3 years and older who have growth failure due to inadequate secretion of endogenous growth hormone.

DOSAGE AND ADMINISTRATION
- Administer Ngenla™ by subcutaneous injection once weekly, on the same day each week, at any time of the day in the abdomen, thighs, buttocks, or upper arms with weekly rotation of injection site.
- Recommended dosage is 0.66 mg/kg based on actual body weight administered once weekly.
- Individualize dosage for each patient based on the growth response.
- Patients switching from daily growth hormone may initiate treatment with once-weekly Ngenla™ on the day following their last daily injection.

DOSAGE FORMS AND STRENGTHS
Injection: 24 mg/1.2 mL (20 mg/mL) single-patient-use prefilled pen that delivers a dose in 0.2 mg increments; 60 mg/1.2 mL (50 mg/mL) single-patient-use prefilled pen that delivers a dose in 0.5 mg increments.

SAFETY PROFILE

DRUG INTERACTIONS
- Replacement Glucocorticoid Treatment: Patients treated with glucocorticoid for hypoadrenalism may require an increase in their maintenance or stress dose following initiation of Ngenla™.
- Pharmacologic Glucocorticoid Therapy and Supraphysiologic Glucocorticoid Treatment: Adjust glucocorticoid dosing in pediatric patients to avoid both hypoadrenalism and an inhibitory effect on growth.
- Cytochrome P450-Metabolized Drugs: Ngenla™ may alter the clearance. Monitor carefully if used with Ngenla™.
- Oral Estrogen: Larger doses of Ngenla™ may be required.
- Insulin and/or Other Antihyperglycemic Agents: Dose adjustment of insulin or antihyperglycemic agent may be required.

USE IN SPECIFIC POPULATIONS
- Females and Males of Reproductive Potential: Interference with hCG blood and urine pregnancy testing in patients receiving somatrogon-ghla may be possible, leading to either false positive or false negative results. Alternative methods (i.e., not reliant on hCG) are recommended to determine pregnancy.

USE IN SPECIFIC POPULATIONS (continuation)
- Pediatric Use: Risks in pediatric patients associated with growth hormone use include:
  - Increased risk of second neoplasm in pediatric cancer survivors treated with radiation to the brain and/or head
  - Slipped capital femoral epiphysis
  - Progression of preexisting scoliosis
  - Pancreatitis
  - Sudden death in pediatric patients with Prader-Willi Syndrome. Ngenla™ is not indicated for the treatment of pediatric patients with growth failure secondary to genetically confirmed Prader-Willi syndrome.

Orphan status: Yes

New Biosimilar Products

- No biosimilar product approved by the FDA in June.

New Formulations, Combination Products & Line Extensions

Drug Name and Manufacturer	Date	Therapeutic Class	Indication(s)	Additional Information
Suflave™ (polyethylene glycol 3350, sodium sulfate, potassium chloride, magnesium sulfate, and sodium chloride for oral solution) / Sebela Pharmaceuticals	6/15/2023	Gastrointestinal agents	For cleansing of the colon in preparation for colonoscopy in adults	Suflave™ is a new low-volume, lemon-lime flavored liquid osmotic laxative for colonoscopy preparation meant to improve palatability for patients. Orphan: No
Lodoco™ (colchicine) tablets / AGEPHA Pharma USA, LLC	6/16/2023	Cardiovascular agents	To reduce the risk of myocardial infarction (MI), stroke, coronary revascularization, and cardiovascular death in adult patients with established atherosclerotic disease or with multiple risk factors for cardiovascular disease	Lodoco™ is the first drug approved by the FDA to target inflammation that is an underlying cause of atherosclerotic cardiovascular disease. Orphan: No
Vyvgart Hytrulo™ (efgartigimod alfa and hyaluronidase-human [recombinant]) injection / Argenx US	6/20/2023	Antimyasthenic agents	Treatment of generalized myasthenia gravis in adult patients who are anti-acetylcholine receptor antibody positive	Vyvgart Hytrulo™ is the first FDA-approved subcutaneous (SC) injectable for generalized myasthenia gravis (gMG). It contains a drug delivery technology to facilitate subcutaneous delivery of biologics. The product is to be administered subcutaneously by a healthcare professional as a single injection over 30-90 seconds in cycles of once weekly injections for four weeks. Orphan: Yes

New First-Time Generic Approvals

Product	Manufacturer	Approval Date	Generic For:	Therapeutic Class	Indication(s)	Projected Launch Date
Sugammadex sodium intravenous solution 200mg (base)/2mL and 500mg (base)/5mL	Aspiro Pharma Ltd.	6/9/2023	Bridion™	Antidotes	Reversal of neuromuscular blockade	01/2026
Tipiracil hydrochloride and trifluridine tablets 6.14mg (base)/15mg and 8.19mg (base)/20mg	Natcho Pharma Ltd.	6/13/2023	Lonsurf™	Antineoplastics	Colorectal cancer/gastric cancer	02/03/2037
Amlodipine benzoate oral suspension 1mg (base)/mL	Amneal Pharmaceuticals LLC.	6/13/2023	Katerzia™	Cardiovascular agents	Hypertension/coronary artery disease	2029-2030

New First-Time Generic Approvals

Product	Manufacturer	Approval Date	Generic For:	Therapeutic Class	Indication(s)	Projected Launch Date
Safinamide mesylate tablets 50mg (base) and 100mg (base)	Aurobindo Pharma Limited	6/14/2023	Xadago™	Antiparkinson agents	Parkinson's disease	12/10/2028
Mometasone furoate metered nasal spray 0.05mg/spray (OTC)	Amneal Pharmaceuticals LLC	6/14/2023	Nasonex™	Anti-inflammatories, inhaler corticosteroids	Allergic rhinitis	Unknown
Tiotropium bromide inhalation powder 0.018mg (base) per inhalation	Lupin Pharmaceuticals, Inc.	6/20/2023	Spiriva™	Bronchodilators, anticholinergic	Chronic obstructive pulmonary disease	09/2023

New First-Time Generic Approvals

Product	Manufacturer	Approval Date	Generic For:	Therapeutic Class	Indication(s)	Projected Launch Date
Amphetamine extended-release orally disintegrating tablets 31.mg, 6.3mg, 9.4mg, 12.5mg, 15.7mg and 18.8mg	Actavis Pharma, Inc.	6/22/2023	Adzenys™ XR-ODT	Attention deficit hyperactivity disorder(ADHD) agents, amphetamines	ADHD	09/01/2025
Alcaftadine ophthalmic solution 0.25%	Eugia Pharma Specialties Limited	6/23/2023	Lastacaft™	Ophthalmic anti-allergy agents	Allergic conjunctivitis	2026-2027
Cyanocobalamin metered nasal spray 0.5mg/spray	Lupin Pharmaceuticals, Inc.	6/30/2023	Nascobal™	Cobalamins	Vitamin B12 deficiency	2023

New FDA-Approved Indications for Existing Drugs

New FDA-Approved Indications

Drug Name and Manufacturer	Therapeutic Class	Previous Indication(s)	New Indication(s)	Date
Prevymis™ (letermovir) tablets and injection / Merck Sharp & Dohme LLC	Anti-cytomegalovirus agents	For prophylaxis of cytomegalovirus (CMV) infection and disease in adult CMV-seropositive recipients [R+] of an allogeneic hematopoietic stem cell transplant	For prophylaxis of CMV disease in adult kidney transplant recipients at high risk (Donor CMV seropositive/recipient CMV seronegative [D+/R-])	6/5/2023
Linzess™ (linaclotide) capsules / Allergan, Inc.	Anti-constipation agents	Treatment of: [1] Irritable bowel syndrome with constipation in adults; [2] Chronic idiopathic constipation in adults	Treatment of functional constipation in pediatric patients 6 to 17 years of age	6/12/2023
Bylvay™ (odevixibat) capsules / Albireo Pharma, Inc.	Gastrointestinal agents	Treatment of pruritus in patients 3 months of age and older with progressive familial intrahepatic cholestasis	Treatment of cholestatic pruritus in patients 12 months of age and older with Alagille syndrome	6/13/2023
Synjardy™ (empagliflozin and metformin) tablets / Boehringer Ingelheim Pharmaceuticals, Inc.	Antidiabetic combinations	As an adjunct to diet and exercise to improve glycemic control in adults with type 2 diabetes mellitus	As an adjunct to diet and exercise to improve glycemic control in adults and pediatric patients aged 10 years and older with type 2 diabetes mellitus	6/20/2023

New FDA-Approved Indications

Drug Name and Manufacturer	Therapeutic Class	Previous Indication(s)	New Indication(s)	Date
Jardiance™ (empagliflozin) tablets / Boehringer Ingelheim Pharmaceuticals, Inc.	Antidiabetic agents	[1] To reduce the risk of cardiovascular death and hospitalization for heart failure in adults with heart failure; [2] To reduce the risk of cardiovascular death in adults with type 2 diabetes mellitus and established cardiovascular disease; [3] As an adjunct to diet and exercise to improve glycemic control in adults with type 2 diabetes mellitus	As an adjunct to diet and exercise to improve glycemic control in adults and pediatric patients aged 10 years and older with type 2 diabetes mellitus	6/20/2023
Talzenna™ (talazopraib) capsules / Pfizer	Antineoplastics – Molecular target inhibitors	Treatment of adult patients with deleterious or suspected deleterious germline breast cancer susceptibility gene (BRCA)-mutated (gBRCAm) human epidermal growth factor receptor 2 (HER2)-negative locally advanced or metastatic breast cancer	In combination with enzalutamide for the treatment of adult patients with homologous recombination repair (HRR) gene-mutated metastatic castration-resistant prostate cancer (mCRPC)	6/20/2023

Pipeline

Pipeline

Drug Name and Manufacturer	Date	Indication(s)	Additional Information	Impact
Tovorafenib / Day One Biopharmaceuticals	6/4/2023	Relapsed or progressive pediatric low-grade glioma (pLGG)	Tovorafenib is an investigational, oral, brain-penetrant, highly-selective type II pan-RAF kinase inhibitor designed to target a key enzyme in the MAPK signaling pathway, which may offer an alternative for individuals with primary brain tumors or brain metastases of solid tumors. NDA submitted.	High high
NVK002 (low dose atropine 0.01%) / Vyluma	6/6/2023	Pediatric myopia	NVK002 is a proprietary, investigational, preservative-free eye drop administered nightly and intended for the use in individuals ages 3 to 17. A Prescription Drug User Fee Act (PDUFA) has been set for January 31, 2024. NDA accepted.	Moderate
Vonoprazan / Phathom Pharmaceuticals, Inc.	6/12/2023	Erosive gastroesophageal reflux disease	Phathom Pharmaceuticals resubmitted the NDA as a response to the Complete Response Letter issued by the FDA in February 2023 relating to the specifications and controls for an impurity (N-nitroso-vonoprazan). The FDA has assigned a PDUFA goal date of November 17, 2023. NDA accepted.	Moderate

Pipeline

Drug Name and Manufacturer	Date	Indication(s)	Additional Information	Impact
Imetelstat / Geron Corporation	6/20/2023	Transfusion-dependent anemia in adult patients with low- to intermediate-1 risk myelodysplastic syndromes who have failed to respond or have lost response to or are ineligible for erythropoiesis-stimulating agents	Imetelstat is a novel, fist-in-class that targets telomerase to inhibit the uncontrolled proliferation of malignant stem and progenitor cells in myeloid hematologic malignancies resulting in malignant cell apoptosis and potential disease-modifying activity. Imetelstat has been granted Fast Track designation by the FDA. NDA submitted.	High
Fidanacogene elaparvovec / Pfizer Inc.	6/27/2023	Treatment of adults with hemophilia B	Fidanacogene elaparvovec is a novel, investigational gene therapy that contains a bio-engineered adeno-associated virus (AAV) capsid (protein shell) and a high-activity variant of human coagulation Factor IX (FIX) gene. The FDA has set a PDUFA goal date in the second quarter pf 2024. BLA accepted.	High high
Ensifentrine / Verona Pharma	6/27/2023	Maintenance treatment of patients with chronic obstructive pulmonary disease	Ensifentrine is a selective dual inhibitor of the enzymes phosphodiesterase 3 and 4 combining bronchodilator and non-steroidal anti-inflammatory activities in one molecule. NDA submitted.	Moderate

Pipeline

Drug Name and Manufacturer	Date	Indication(s)	Additional Information	Impact
Exblifep™ (cefepime/enmetazobactam) / Allecra Therapeutics	6/27/2023	Treatment of complicated urinary tract infections	Exblifep™ is an antibiotic combination of the fourth-generation cephalosporin cefepime with the proprietary beta lactamase inhibitor, enmetazolabctam. It has been designed to combat anti-microbial resistance in gram-negative bacteria. NDA submitted.	Moderate
Givinostat / Italfarmaco Group	6/29/2023	Treatment of Duchenne muscular dystrophy	Givinostat inhibits histone deacetylases (HDACs). HDACs are enzymes that prevent gene translation by changing the three-dimensional folding of DNA in the cell. Givinostat was observed to slow disease progression. It was granted Priority review by the FDA and assigned a PDUFA date of December 21, 2023. NDA submitted.	High high
Resmetirom / Madrigal Pharmaceuticals, Inc.	6/30/2023	Treatment of patients with nonalcoholic steatohepatitis (NASH) with liver fibrosis	Resmetirom is a once-daily, oral thyroid hormone receptor beta-selective agonist designed specifically to treat the underlying causes of NASH in the liver, while improving multiple atherogenic lipid profiles. It has been granted Breakthrough Therapy designation in April 2023. NDA submitted.	Moderate

References

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