PHARMNOTES

September 2023

Date: 10/9/2023

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Drug Safety Alert Notification

- No drug safety communication published in September.

New FDA-Approved Drug Products

New Molecular Entity

DRUG NAME	MANUFACTURER	APPROVAL DATE
APHEXDA™ (MOTIXAFORTIDE) FOR INJECTION, FOR SUBCUTANEOUS USE	BIOLINERX, LTD.	09/08/2023

THERAPEUTIC CLASS
Hematopoietic Agents

FDA-APPROVED INDICATION(S)
Aphexda™, a hematopoietic stem cell mobilizer, is indicated in combination with filgrastim (G-CSF) to mobilize hematopoietic stem cells to the peripheral blood for collection and subsequent autologous transplantation in patients with multiple myeloma.

DOSAGE AND ADMINISTRATION
- Recommended dosage is 1.25 mg/kg actual body weight by subcutaneous injection 10 to 14 hours prior to initiation of apheresis.
- Initiate Aphexda™ treatment after filgrastim has been administered daily for 4 days.
- A second dose of Aphexda™ can be administered 10 to 14 hours prior to a third apheresis.

DOSAGE FORMS AND STRENGTHS
For Injection: 62 mg as a lyophilized powder in a single-dose vial for reconstitution.

Orphan status: Yes

SAFETY PROFILE

CONTRAINDICATIONS
- History of serious hypersensitivity reaction to Aphexda™.

WARNINGS AND PRECAUTIONS
- *Anaphylactic Shock and Hypersensitivity Reactions:* Premedicate all patients with a combination of an H1-antihistamine, an H2 blocker, and a leukotriene inhibitor prior to each Aphexda™ dose. Administer Aphexda™ in a setting where personnel and therapies are available for immediate treatment. Observe for signs and symptoms and manage promptly.
- *Injection Site Reactions*: The addition of analgesic premedication (e.g., acetaminophen) is recommended.
- *Tumor Cell Mobilization in Patients with Leukemia*: Aphexda™ may mobilize leukemic cells and should not be used in leukemia patients.
- *Leukocytosis*: Increased circulating leukocytes have been observed. Monitor white blood cell counts during Aphexda™ use.
- *Potential for Tumor Cell Mobilization:* Tumor cells may be released from marrow during HSC mobilization with Aphexda™ and filgrastim. Effect of reinfusion of tumor cells is unknown.
- *Embryo-fetal Toxicity*: Can cause fetal harm. Advise women of reproductive potential of the potential risk to a fetus and to use effective contraception.

ADVERSE REACTIONS
- Most common adverse reactions (incidence >20%) are injection site reactions, injection site pain, injection site erythema, injection site pruritus, pruritus, flushing, and back pain.

USE IN SPECIFIC POPULATIONS
- *Pregnancy*: Aphexda™ can cause fetal harm when administered to a pregnant woman.
- *Lactation*: Because of the potential serious adverse reactions in the breastfed child, advise females that breastfeeding is not recommended during the treatment with Aphexda™ treatment and for 8 days after the final dose.
- *Females and Males of Reproductive Potential:* Verify pregnancy status in females of reproductive potential prior to initiating Aphexda™. Advise females of reproductive potential to use effective contraception during treatment with Aphexda™ and for 8 days after the final dose.

New Molecular Entity

DRUG NAME	MANUFACTURER	APPROVAL DATE
OJJAARA™ (MOMELOTINIB) TABLETS FOR ORAL USE	GLAXOSMITHKLINE LLC	09/15/2023

THERAPEUTIC CLASS
Antineoplastics

FDA-APPROVED INDICATION(S)
Ojjaara™ is a kinase inhibitor indicated for the treatment of intermediate or high-risk myelofibrosis (MF), including primary MF or secondary MF [post polycythemia vera (PV) and post-essential thrombocythemia (ET)], in adults with anemia.

DOSAGE AND ADMINISTRATION
- Recommended dosage: 200 mg orally once daily with or without food
- Obtain the following blood tests prior to starting treatment, periodically during treatment, and as clinically indicated: Complete blood count (CBC) with platelets and hepatic panel.

DOSAGE FORMS AND STRENGTHS
Tablets: 100 mg, 150 mg, 200 mg

Orphan status: Yes

SAFETY PROFILE

CONTRAINDICATIONS
None.

WARNINGS AND PRECAUTIONS
- *Risk of Infections*: Do not initiate Ojjaara™ in patients with an active infection. Monitor for signs and symptoms of infection, including reactivation of hepatitis B, and initiate appropriate treatment promptly.
- *Thrombocytopenia and Neutropenia*: Manage by dose reduction or interruption.
- *Hepatotoxicity*: Obtain liver tests before initiation of and periodically throughout treatment with Ojjaara™.
- *Major Adverse Cardiovascular Events (MACE)*: Monitor for symptoms, evaluate and treat promptly.
- *Thrombosis*: Evaluate and treat symptoms of thrombosis promptly.
- *Malignancies*: Monitor for development of secondary malignancies, particularly in current or past smokers.

ADVERSE REACTIONS
- Most common adverse reactions (incidence >20%) are injection site reactions, injection site pain, injection site erythema, injection site pruritus, pruritus, flushing, and back pain.

DRUG INTERACTIONS
- *Organic Anion Transporting Polypeptide (OATP)1B1/B3 inhibitors:* Monitor for adverse reactions.
- *Breast Cancer Resistance Protein (BCRP) substrates:* Reduce rosuvastatin (BCRP substrate) dosage. Follow approved product information recommendations for other BCRP substrates.

USE IN SPECIFIC POPULATIONS
- *Pregnancy*: May cause fetal harm. Should only be used during pregnancy if the expected benefits to the mother outweigh the potential risks to the fetus.
- *Lactation*: Because of the potential serious adverse reactions in the breastfed child, patients should not breastfeed during treatment with Ojjaara™ and for at least 1 week after the last dose of Ojjaara™.
- *Females and Males of Reproductive Potential*: Advise females of reproductive potential who are not pregnant to use highly effective contraception during therapy and for at least 1 week after the last dose.
- *Hepatic Impairment*: Momelotinib is extensively metabolized. The recommended starting dose of Ojjaara™ in patients with severe hepatic impairment (Child-Pugh C) is 150 mg orally once daily. No dose modification is recommended for patients with mild hepatic impairment (Child-Pugh A) or moderate hepatic impairment (Child-Pugh B).

New Molecular Entity

DRUG NAME	MANUFACTURER	APPROVAL DATE
EXXUA™ (GEPIRONE) EXTENDED-RELEASE TABLETS FOR ORAL USE	FABRE-KRAMER PHARMACEUTICALS, INC.	09/22/2023

THERAPEUTIC CLASS
Antidepressants

FDA-APPROVED INDICATION(S)
Exxua™ is indicated for the treatment of major depressive disorder (MDD) in adults.

DOSAGE AND ADMINISTRATION
- Recommended starting dosage: 18.2 mg once daily.
- Based on clinical response and tolerability, the dosage may be increased to 36.3 mg orally once daily on Day 4 and further titrated to 54.5 mg orally once daily after Day 7 and to 72.6 mg orally once daily after an additional week.
- Maximum recommended daily dosage: 72.6 mg once daily. Take Exxua™ orally with food at approximately the same time each day.

DOSAGE FORMS AND STRENGTHS
Extended-release tablets: 18.2 mg, 36.3 mg, 54.5 mg, and 72.6 mg

Orphan status: No

SAFETY PROFILE

CONTRAINDICATIONS
- Known hypersensitivity to gepirone or components of Exxua™.
- Prolonged QTc interval > 450 msec at baseline.
- Congenital long QT syndrome
- Concomitant use of strong CYP3A4 inhibitors
- Severe hepatic impairment
- Use with an MAOI or within 14 days of stopping treatment with Exxua™
- Do not use Exxua™ within 14 days of discontinuing an MAOI

WARNINGS AND PRECAUTIONS
- **BLACK BOX WARNING: SUICIDAL THOUGHTS AND BEHAVIORS**
  - Increased risk of suicidal thinking and behavior in pediatric and young adult patients taking antidepressants. Closely monitor for worsening and emergence of suicidal thoughts and behaviors. Exxua™ is not approved for use in pediatric patients.
- *QT Interval Prolongation*: Exxua™ prolongs the QTc. Correct electrolyte abnormalities. Perform ECGs prior to initiation, during dose titration, and periodically during treatment with Exxua™. Monitor ECGs more frequently when Exxua™ is used concomitantly with drugs known to prolong the QT interval, in patients who develop QTc ≥ 450 msec during treatment or are at significant risk of developing torsade de pointes. Do not escalate dosage if QTc > 450 msec.

WARNINGS AND PRECAUTIONS (cont.)
- *Serotonin Syndrome*: Increased risk when co-administered with other serotonergic agents. If serotonin syndrome occurs, discontinue Exxua™ and initiate supportive measures.
- *Activation of Mania/Hypomania*: Screen patients for bipolar disorder

ADVERSE REACTIONS
- Most common adverse reactions (incidence of ≥5% and at least twice incidence of placebo) were dizziness, nausea, insomnia, abdominal pain, and dyspepsia.

DRUG INTERACTIONS
- *Strong CYP3A4 inducers*: Reduces Exxua™ exposure. Avoid concomitant use.

USE IN SPECIFIC POPULATIONS
- *Pregnancy:* Third trimester use may increase the risk for persistent pulmonary hypertension and symptoms of poor adaptation (respiratory distress, temperature instability, feeding difficulty, hypotonia, irritability) in the neonate.
- *Renal Impairment:* maximum recommended daily dosage of Exxua™ in patients with a creatinine clearance.
- *Hepatic Impairment:* maximum recommended dosage of Exxua™ in patients with moderate hepatic impairment is lower than in patients with normal hepatic function.

New Molecular Entity

DRUG NAME	MANUFACTURER	APPROVAL DATE
POMBILITI™ (CIPAGLUCOSIDASE ALFA-ATGA) FOR INJECTION, FOR INTRAVENOUS USE	AMICUS THERAP US	09/28/2023

THERAPEUTIC CLASS
Endocrine-Metabolic Agents

FDA-APPROVED INDICATION(S)
Pombiliti™ is a hydrolytic lysosomal glycogen-specific enzyme indicated, in combination with Opfolda, an enzyme stabilizer, for the treatment of adult patients with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency) weighing ≥40 kg and who are not improving on their current enzyme replacement therapy (ERT)

DOSAGE AND ADMINISTRATION
- Administer Pombiliti™ in combination with Opfolda™.
- Consider administering antihistamines, antipyretics, and/or corticosteroids prior to Pombiliti™ administration.
- Recommended dosage: is 20 mg/kg (of actual body weight) administered every other week as an intravenous infusion over approximately 4 hours.
- Initiate the Pombiliti™ infusion approximately 1 hour after oral administration of Opfolda™. If the Pombiliti™ infusion cannot be started within 3 hours of oral administration of Opfolda™, reschedule Pombiliti™ in combination with Opfolda at least 24 hours after Opfolda was last taken. If Pombiliti™ in combination with Opfolda™ are both missed, re-start treatment as soon as possible.

DOSAGE FORMS AND STRENGTHS
For injection: 105 mg of cipaglucosidase alfa-atga as a lyophilized powder in a single-dose vial for reconstitution

SAFETY PROFILE

CONTRAINDICATIONS
- Pregnancy

WARNINGS AND PRECAUTIONS
- **BLACK BOX WARNING: SEVERE HYPERSENSITIVITY REACTIONS, INFUSION-ASSOCIATED REACTIONS, AND RISK OF ACUTE CARDIORESPIRATORY FAILURE IN SUSCEPTIBLE PATIENTS**
  - *Hypersensitivity Reactions Including Anaphylaxis:* Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available. If a severe hypersensitivity reaction occurs, Pombiliti™ should be discontinued immediately, and appropriate medical treatment should be initiated.
  - *Infusion-Associated Reactions (IARs):* If severe IARs occur, immediately discontinue Pombiliti™ and initiate appropriate medical treatment.
  - *Risk of Acute Cardiorespiratory Failure in Susceptible Patients:* Patients susceptible to fluid volume overload, or those with acute underlying respiratory illness or compromised cardiac or respiratory function, may be at risk of serious exacerbation of their cardiac or respiratory status during Pombiliti™ infusion.

WARNINGS AND PRECAUTIONS
- *Embryo-Fetal Toxicity*: May cause embryo-fetal harm. Advise females of reproductive potential of the potential risk to a fetus and to use effective contraception during treatment and for at least 60 days after the last dose.
- *Risks Associated with Opfolda*™: Refer to Opfolda™ Package Insert.

ADVERSE REACTIONS
- Most common adverse reactions ≥ 5% are headache, diarrhea, fatigue, nausea, abdominal pain, and pyrexia.

USE IN SPECIFIC POPULATIONS
- *Pregnancy:* may cause embryo-fetal harm when administered to a pregnant female.
- *Lactation*: Breastfeeding not recommended.
- *Females and Males of Reproductive Potential:* Verify the pregnancy status in females of reproductive potential prior to initiating treatment with Pombiliti™ in combination with Opfolda™. Advise females of reproductive potential to use effective contraception during treatment with Pombiliti™ in combination with Opfolda™ and for at least 60 days after the last dose.

Orphan status: Yes

New Molecular Entity

DRUG NAME	MANUFACTURER	APPROVAL DATE
RIVFLOZA™ (NEDOSIRAN) INJECTION, FOR SUBCUTANEOUS USE	NOVO NORDISK INC.	09/29/2023

THERAPEUTIC CLASS
Hyperoxaluria Agents

FDA-APPROVED INDICATION(S)
Rivfloza™ is an *LDHA*-directed small interfering RNA indicated to lower urinary oxalate levels in children 9 years of age and older and adults with primary hyperoxaluria type 1 (PH1) and relatively preserved kidney function, e.g., eGFR ≥ 30 mL/min/1.73 m^2.

DOSAGE AND ADMINISTRATION
Recommended dosage is shown below and is administered subcutaneously once monthly.

Age	Body Weight	Dosing Regimen
Adults and adolescents 12 years and older	Greater than or equal to 50 kg	160 mg once monthly (Pre-filled Syringe, 1 mL)
	Less than 50 kg	128 mg once monthly (Pre-filled Syringe, 0.8 mL)
Children 9 to 11 years	Greater than or equal to 50 kg	160 mg once monthly (Pre-filled Syringe, 1 mL)
	Less than 50 kg	3.3 mg/kg once monthly, not to exceed 128 mg (Vial, dose volume rounded to nearest 0.1 mL)

DOSAGE FORMS AND STRENGTHS
Injection: 160 mg/mL is a clear, colorless-to-yellow solution available as follows: 80 mg (0.5 mL) single-dose vial; 128 mg (0.8 mL) single-dose Pre-filled Syringe; 160 mg (1 mL) single-dose Pre-filled Syringe

SAFETY PROFILE

CONTRAINDICATIONS
None.

ADVERSE REACTIONS
Most common adverse reaction (reported in ≥20% of patients) is injection site reactions.

Orphan status: Yes

New Biosimilar Products

Drug Name and Manufacturer	Date	Therapeutic Class	Indication(s)	Additional Information
Tofidence™ (tociizumab-bavi) injection, for intravenous use / Biogen MA Inc.	9/29/2023	Immunological Agents	[1] Treatment of adult patients with moderately to severely active rheumatoid arthritis who have had an inadequate response to one or more disease-modifying anti-rheumatic drugs (DMARDs) [2] Treatment of patients 2 years of age and older with active polyarticular juvenile idiopathic arthritis [3] Treatment of patients 2 years of age and older with active systemic juvenile idiopathic arthritis	Tofidence™ is the first intravenous formulation of tocilizumab biosimilar approved in the United States. Pricing and launch date still pending. Reference Product: Actemra™ (tocilizumab) Orphan: No

New Formulations, Combination Products & Line Extensions

Drug Name and Manufacturer	Date	Therapeutic Class	Indication(s)	Additional Information
Xalkori™ (crizotinib) oral pellets / Pfizer	9/7/2023	Antineoplastics	[1] Treatment of adult patients with metastatic non-small cell lung cancer (NSCLC) whose tumors are anaplastic lymphoma kinase (ALK) or ROS1-positive [2] Treatment of pediatric patients 1 year of age and older and young adults with relapsed or refractory, systemic anaplastic large cell lymphoma (ALCL) that is ALK-positive [3] Treatment of adult and pediatric patients 1 year of age and older with unresectable, recurrent or refractory inflammatory myofibroblastic tumor (IMT) that is ALK-positive	The new oral pellets formulation can be administered by one of two options: open shell(s) containing Xalkori and empty the contents directly into the patient's mouth or open shell(s) and empty the contents into a consumer-supplied oral dosing aid that is then administered directly into the patient's mouth. Orphan: Yes
Spikevax™ (COVID-19 Vaccine, mRNA) Suspension for injection, for intramuscular use / Moderna Tx Inc	9/11/2023	Vaccines	For active immunization to prevent coronavirus disease 2019 (COVID-19) caused by severe acute respiratory syndrome coronavirus 2 (SARS-CoV-2) in individuals 12 years of age and older	Moderna's updated COVID-19 vaccine contains an updated monovalent XBB. 1.5 composition for the 2023-2024 vaccination season. Clinical data shows that its updated vaccine resulted in robust immune responses across multiple XBB sublineages including XBB. 1.5 and XBB. 1.16. Orphan: No
Comirnaty™ (COVID-19 Vaccine, mRNA) Suspension for injection, for intramuscular use / BioNTech Manufacturing GmbH	9/11/2023	Vaccines	For active immunization to prevent coronavirus disease 2019 (COVID-19) caused by severe acute respiratory syndrome coronavirus 2 (SARS-CoV-2) in individuals 12 years of age and older	This new formulation of the vaccine shows that it generates improved neutralizing antibody responses against multiple circulating Omicron-related sublineages including XBB.1.5, BA.2.86 (Pirola), and EG.5.1 (Eris), which currently accounts for the largest portion of the U.S. cases. Orphan: No

New Formulations, Combination Products & Line Extensions

Drug Name and Manufacturer	Date	Therapeutic Class	Indication(s)	Additional Information
Likmez™ (metronidazole) oral suspension / Saptalis Pharmaceuticals, LLC	9/22/2023	Antimicrobial	[1] Trichomoniasis in adults [2] Amebiasis in adults and pediatric patients [3] Anaerobic bacterial infections in adults	The first and only FDA approved ready made suspension of metronidazole for the treatment of antimicrobial infections that addresses the unmet need in patients with dysphagia and avoids risks associated with drug compounding, and discontinuation related anti-microbial resistance. Orphan: No
Ryzumvi™ (phentolamine ophthalmic solution) 0.75% for topical ophthalmic use / Ocuphire Pharma, Inc.	9/25/2023	Antihypertensive	Treatment of pharmacologically-induced mydriasis produced by adrenergic agonists (e.g., phenylephrine) or parasympatholytic (e.g., tropicamide) agents	Ryzumvi™ is a preservative-free, stable eye drop solution and it is expected to be commercially available in the U.S. in the first half of 2024. Orphan: No
Bosulif™ (bosutinib) capsules, for oral use / Pfizer Inc.	9/27/2023	Antineoplastics	[1] Treatment of adult and pediatric patients 1 year of age and older with chronic phase Ph + chronic myelogenous leukemia (CML), newly-diagnosed or resistant or decreased, lymphocyte count decreased, platelets decreased, ALT intolerant to prior therapy [2] Adult patients with accelerated , or blast phase Ph + CML with resistance or intolerance to prior therapy	With the FDA approval for the new capsule dosage form available in strengths of 50 mg and 100 mg, a new indication for pediatric patients 1 year of age and older with chronic phase Ph+ chronic myelogenous leukemia (CML), newly-diagnosed or resistant or decreased, lymphocyte count decreased, platelets decreased, ALT intolerant to prior therapy was approved. Orphan: Yes
Empaveli™ (pegcetacoplan) injection for subcutaneous use / Apellis Pharmaceuticals, Inc.	9/28/2023	Hematological Agents	Treatment of adult patients with paroxysmal nocturnal hemoglobinuria	The FDA has approved the Empaveli™ injector. It is a compact, single-use, on-body device designed to enhance self-administration of Empaveli™. Orphan: Yes

New Formulations, Combination Products & Line Extensions

Drug Name and Manufacturer	Date	Therapeutic Class	Indication(s)	Additional Information
Entyvio™ and Entyvio PEN™ (vedolizumab) injection for subcutaneous use / Takeda Pharms USA	9/27/2023	Monoclonal Antibody	Treatment of moderately to severely active ulcerative colitis (UC)	Entyvio™ subcutaneous (SC) formulation is expected to be available in the U.S. as a single-dose pre-filled pen (Entyvio™ Pen) by the end of October. Additionally, a Biologics License Application for an investigational SC administration of Entyvio™ for the treatment of adults with moderately to severely active Crohn's disease is currently under review by the FDA. Orphan: No
Opfolda™ (miglustat) capsules for oral use / Amicus Therapeutics	9/28/2023	Enzyme stabilizer	Treatment of adult patients with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency) weighing ≥40 kg and who are not improving on their current enzyme replacement therapy (ERT).	Opfolda™ capsules are used in combination with Pombiliti™ (cipaglucosidase alfa-atga) infusion to increase the effectiveness and binds Pombiliti™ to stabilize and protect it in the bloodstream after infusion. Orphan: Yes
Technegas™ (kit for the preparation of technetium Tc 99m-labeled carbon inhalation aerosol) for oral inhalation use / Cyclomedica Australia Pty Ltd	9/29/2023	Diagnostic Agent	For use in adults and pediatric patients aged 6 years and older for: • Visualization of pulmonary ventilation • Evaluation of pulmonary embolism when paired with perfusion imaging	The approval of this new formulation of technetium allows for broad use of Technegas®, supporting wider future indications across other respiratory disease states including Chronic Obstructive Pulmonary Disease (COPD), Asthma, Long COVID and lung cancer. Orphan: No

New First-Time Generic Approvals

Product	Manufacturer	Approval Date	Generic For:	Therapeutic Class	Indication(s)	Projected Launch Date
Tofacitinib Citrate oral solution 1mg (base)/mL	Slayback Pharma LLC	9/25/2023	Xeljanz™ oral solution	Janus Kinase (JAK) inhibitors	Polyarticular Course Juvenile Idiopathic Arthritis	6/9/2026

New FDA-Approved Indications for Existing Drugs

New FDA-Approved Indications

Drug Name and Manufacturer	Therapeutic Class	Previous Indication(s)	New Indication(s)	Date
Jardiance™ (empagliflozin) tablets for oral use / Boehringer-Ingelheim	Antidiabetic Agent	[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	To reduce the risk of sustained decline in estimated glomerular filtration rate (eGFR), end-stage kidney disease, cardiovascular death and hospitalization in adults with chronic kidney disease (CKD) at risk of progression	9/21/2023

Pipeline

Pipeline

Drug Name and Manufacturer	Date	Indication(s)	Additional Information	Impact
Crovalimab / Genentech	9/4/2023	Treatment of paroxysmal nocturnal hemoglobinuria (PNH)	Crovalimab is an investigational, novel anti-C5 recycling monoclonal antibody that in the phase 3 COMMODORE 2 study showed that it provided disease control and was non-inferior to Soliris™. It was given as a subcutaneous injection every 4 weeks. BLA: Accepted	High
Mavorixafor / X4 Pharmaceuticals	9/5/2023	Treatment of individuals aged 12 and older with Warts, Hypogammaglobulinemia, Infections and Myelokathexis (WHIM) syndrome	Mavorixafor is an investigational small-molecule antagonist of CXCR4. It is being developed as a once-daily oral therapy. It has been granted Breakthrough Therapy Designation, Fast Track Designation, and Rare Pediatric Designation in the U.S., and Orphan Drug Status. NDA: Submitted	High High
Libervant™ (diazepam) buccal film / Aquestive Therapeutics, INC.	9/11/2023	Acute treatment of intermittent, stereotypic episodes of frequent seizure activity (i.e., seizure clusters, acute repetitive seizures) in patients between 2 and 5 years of age	Libervant is a buccally administered film formulation of diazepam. It provides another treatment option for the pediatric population. The FDA has set a Prescription Drug User Fee Act (PDUFA) target goal date set for April 28, 2024. NDA: Accepted	Moderate
Ensifentrine / Verona Pharma Plc	9/11/2023	Maintenance treatment of patients with chronic obstructive pulmonary disease (COPD)	Ensifentrine is a novel, potent, and selective dual inhibitor of phosphodiesterase (PDE) 3 and PDE 4 designed for delivery by inhalation that combines effects on airway inflammation, bronchodilation and ciliary function in bronchial epithelia. If approved, it is expected to be the first novel mechanism available for COPD in more than a decade. The FDA has assigned a PDUFA target action date of June 26, 2024. NDA: Accepted	High

Pipeline

Drug Name and Manufacturer	Date	Indication(s)	Additional Information	Impact
Resmetirom / Madrigal Pharmaceuticals, Inc.	9/13/2023	Treatment of adult patients with nonalcoholic steatohepatitis (NASH)	Resmetirom is a once-daily, oral, thyroid hormone receptor (THR)-β selective agonist designed to target key underlying causes of NASH in the liver. The FDA has granted Priority Review and assigned a PDUFA date for resmetirom of March 14, 2024. NDA: Accepted	High
OTL-200 (atidarsagene autotemcel) / Orchard Therapeutics	9/18/2023	Treatment of metachromatic leukodystrophy (MLD)	MLD is a rare hereditary disease caused by changes in the arylsulfatase A (ARSA) gene. OTL-200 is a gene therapy that involves the extraction of certain stem cells from a patient's bone marrow or blood. It has been granted Priority Review and has a PDUFA date set for March 18, 2024. BLA: Accepted	High
OX124 / Orexo AB	9/18/2023	Treatment of opioid overdose	OX124 is a high-dose intranasal naloxone designed to reverse the effects of powerful synthetic opioids, such as fentanyl. OX124 has shown to have a significantly faster and higher absorption of naloxone compared to intramuscular naloxone. If approved, US launch is expected to be initiated late in 2024 or early 2025. NDA: Submitted	Moderate
DPI-386 (scopolamine) nasal gel / Defender Pharmaceuticals, Inc.	9/26/2023	Prevention of nausea and vomiting associated with motion sickness	DPI-386 is an intranasally-administered scopolamine gel. It has been demonstrated to have rapid absorption. The FDA has granted Priority Review and PDUFA action date set for January 26, 2024. NDA: Accepted	Moderate

Pipeline

Drug Name and Manufacturer	Date	Indication(s)	Additional Information	Impact
EB-101 / Abeona Therapeutics Inc.	9/26/2023	Treatment of recessive dystrophic epidermolysis bullosa	B-101 is an autologous, engineered cell therapy. It has been granted Regenerative Medicine Advanced Therapy, Breakthrough Therapy, Orphan Drug and Rare Pediatric Disease designations by the FDA. BLA: Submitted	High high
ALPHA-1062 / Alpha Cognition Inc.	9/27/2023	Treatment of mild-to-moderate Alzheimer's Disease	ALPHA-1062, a pro-drug of galantamine, is uniquely designed to reduce gastrointestinal adverse effects by remaining inert as it passes through the stomach. Currently approved drugs for Alzheimer's Disease are associated with significant gastrointestinal adverse events resulting in an unmet need for effective and tolerable treatments. NDA: Submitted	Moderate

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