PharmNotes

Monthly Communications

February 2025

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Drug Safety Alert Notification

The Drug Safety Communications are provided by the U.S. Food and Drug Administration and are intended to offer important information to patients and health care providers about new safety issues regarding certain medications. This helps prescribers and health care professionals be informed so that decisions regarding the treatment of patients are made accordingly.

No Drug Safety Alert Notification was released during February.

New FDA-Approved Drug Products

New Molecular Entity

Orphan Drug | Specialty

Gomekli™ (mirdametinib) tablets and capsules for oral use

FDA-Approved Indication

For the treatment of adult and pediatric patients 2 years of age and older with neurofibromatosis type 1 (NF1) who have symptomatic plexiform neurofibromas (PN) not amenable to complete resection.

Dosage & Administration

2 mg/m^2 orally twice daily, with or without food, for the first 21 days of each 28-day cycle.

Dosage Forms & Strengths

- Capsules: 1 mg and 2 mg
- Tablets for Oral Suspension: 1 mg

Contraindications

None

Common Adverse Reactions

Rash, diarrhea, nausea, musculoskeletal pain, vomiting, fatigue, abdominal pain, headache, paronychia, left ventricular dysfunction, laboratory abnormality: decreased neutrophil count and increased creatine phosphokinase.

Warnings & Precautions

- Ocular Toxicity
- Left Ventricular Dysfunction
- Dermatologic Adverse Reactions
- Embryo-Fetal Toxicity

Use in Specific Population

- Lactation: Advise not to breastfeed
- Infertility: May impair fertility in females.

Clinical Studies

The approval of Gomekli by the FDA is supported by findings from the Phase 2b ReNeu trial, which involved 114 patients with NF1-PN aged 2 years and older (58 adults and 56 /58) in adults, while 50% and 48%, respectively, experienced a response lasting at least 24 months.

Place in Therapy

Gomekli will face direct competition with Koselugo in this treatment area, with Gomekli targeting a wider patient population that includes both adults and children, while Koselugo has a more limited indication, focused solely on pediatric patients.

New FDA-Approved Drug Products

New Molecular Entity

Specialty

Romvimza™ (vimseltinib) capsules, for oral use

FDA-Approved Indication

For treatment of adult patients with symptomatic tenosynovial giant cell tumor (TGCT) for which surgical resection will potentially cause worsening functional limitation or severe morbidity.

Dosage & Administration

30 mg orally twice weekly, with a minimum of 72 hours between doses as described in the blister package.

Dosage Forms & Strengths

Capsules: 14 mg, 20 mg, 30 mg.

Contraindications

None

Common Adverse Reactions

Increased AST, periorbital edema, fatigue, rash, increased cholesterol, peripheral edema, face edema, decreased neutrophils, decreased leukocytes, pruritus, and increased ALT.

Warnings & Precautions

- Hepatotoxicity
- Embryo-fetal toxicity
- Allergic Reactions to FD&C Yellow No. 5 (tartrazine) and No. 6 (Sunset Yellow FCF)
- Increased serum creatinine without affecting renal function

Drug Interactions

- P-glycoprotein (P-gp) substrates
- Breast Cancer Resistance Protein (BCRP) substrates
- Organic Cation Transporter 2 (OCT) substrates

Use in Specific Populations

Lactation: Advise not to breastfeed.

Clinical Studies

The approval of Romvimza was based on findings from the Phase 3 MOTION trial which included 123 patients with TGCT who were not candidates for surgery and had no prior treatment with anti-CSF1/CSF1R therapies. Patients were randomly assigned (2:1) to receive either Romvimza 30 mg twice a week or a placebo for 24 weeks (Part 1). At Week 25, the primary efficacy measure, overall response rate (ORR), was 40% in the Romvimza group and 0% (no responses) in the placebo group. The median duration of response (DOR) was not reached in the Romvimza group. After an additional 6 months of follow-up, 85% of responders had a DOR of at least 6 months, and 58% had a DOR of at least 9 months.

Place in Therapy

Romvimza is the second kinase inhibitor approved for the treatment of TGCT, following Turalio (pexidartinib). Systemic therapies that have been used off-label for recurrent disease include Gleevec (imatinib), Sutent (sunitinib), Bayer's Nexavar (sorafenib), and Tasigna (nilotinib).

New FDA-Approved Drug Products

New Vaccines

Vimkunya™ (chikungunya vaccine, recombinant) injectable suspension, for intramuscular use

FDA-Approved Indication

Indicated for the prevention of disease caused by chikungunya virus in individuals 12 years of age and older.

Dosage & Administration

Administered as a single 0.8 mL dose.

Dosage Forms & Strengths

A single dose injectable suspension (0.8mL).

Contraindications

Do not administer to anyone with a history of a severe allergic reaction (e.g., anaphylaxis) to any component of Vimkunya.

Common Adverse Reactions

Injection site pain, fatigue, headache and myalgia

Warnings & Precautions

- Appropriate medical treatment must be immediately available to manage potential anaphylactic reactions following administration of Vimkunya.
- Immunocompromised individuals, including individuals receiving immunosuppressive therapy, may have a diminished immune response to Vimkunya.
- Syncope (fainting) may occur in association with administration of injectable vaccines including Vimkunya. Procedures should be in place to avoid injury from fainting.

Clinical Studies

Vimkunya was assessed in two Phase 3 clinical studies, which compared a single dose of the vaccine to a placebo. In both trials, all primary objectives, including seroresponse and geometric mean titer on Day 22 post-vaccination, were successfully achieved. These positive outcomes were maintained through Day 183 after vaccination.

Place in Therapy

Vimkunya is the second chikungunya virus vaccine to receive FDA approval, following Ixchiq, which was approved in November 2023. The treatment for chikungunya virus infection is supportive, focusing on rest, hydration, and symptom management for joint pain, inflammation, and fever.

New FDA-Approved Drug Products

New Vaccines

Penmenvy™ (meningococcal groups A, B, C, W and Y vaccine) injectable suspension, for intramuscular use

FDA-Approved Indication

Indicated for active immunization to prevent invasive disease caused by *Neisseria meningitidis* serogroups A, B, C, W, and Y in individuals 10 through 25 years of age.

Dosage & Administration

For intramuscular use: Administer 2 doses (approximately 0.5 mL each) of Penmenvy 6 months apart.

Dosage Forms & Strengths

For injectable suspension. A single dose after reconstitution is approximately 0.5 Ml.

Contraindications

Severe allergic reaction (e.g., anaphylaxis) to a previous dose of Penmenvy, to any component of this vaccine, or to any other diphtheria toxoid-containing vaccine.

Common Adverse Reactions

Pain in the injection site, fatigue, headache, myalgia, nausea, erythema and swelling.

Warnings & Precautions

- Syncope

Clinical Studies

The approval of Penmenvy was based on findings from two Phase 3 trials that assessed the immune response, safety, and tolerability in over 2200 participants. In these trials, Penmenvy achieved all noninferiority endpoints for the MenACWY component and for two of the four MenB strains tested in Study 1. Penmenvy's safety profile seems to be similar to that of Bexsero, Menveo, and its predecessor, Penbraya.

Place in Therapy

Penmenvy is the second pentavalent meningococcal vaccine to receive approval, following Penbraya, which was approved in October 2023. Other available meningococcal vaccines in the United States are quadrivalent, covering serotypes A, C, W, and Y (such as MenQuadfi and Menveo), or targeting serotype B alone (like Bexsero and Trumenba).

New FDA-Approved Drug Products

New Biosimilar Product

Specialty

Ospomvy™ (denosumab-dssb) injection, for subcutaneous use

FDA-Approved Indication

[1] Postmenopausal women with osteoporosis at high risk for fracture; [2] To increase bone mass in men with osteoporosis at high risk for fracture; [3] Glucocorticoid-induced osteoporosis in men and women at high risk for fracture; [4] To increase bone mass in men at high risk for fracture receiving androgen deprivation therapy for nonmetastatic prostate cancer; [5] To increase bone mass in women at high risk for fracture receiving adjuvant aromatase inhibitor therapy for breast cancer.

Dosage & Administration

60 mg every 6 months as a subcutaneous injection administered by a healthcare provider.

Dosage Forms & Strengths

Injection: 60 mg/mL solution in single-dose prefilled syringe.

Contraindications

- Hypocalcemia
- Pregnancy
- Know hypersensitivity to denosumab products

Warnings & Precautions

- ***BBW:*** Severe hypocalcemia in patients with chronic kidney disease
- Patients receiving Ospomyv should not receive other denosumab products concomitantly
- Hypersensitivity including anaphylactic reactions may occur
- Osteonecrosis of the jaw
- Atypical femoral fractures
- Multiple vertebral fractures have been reported following treatment discontinuation
- Serious infections including skin infections
- Dermatological reactions
- Severe bone, joint, muscle pain may occur
- Suppression of bone turnover

Common Adverse Reactions

Back pain, pain in extremity, hypercholesterolemia, musculoskeletal pain, cystitis, arthralgia, nasopharyngitis, hypertension, bronchitis, and headache.

Use in Specific Populations

- Pregnant women and females of reproductive potential: May cause fetal harm.
- Pediatric patients: Ospomyv is not approved for use in pediatric patients.
- Renal impairment: No dose adjustment is necessary in patients with renal impairment.

Clinical Studies

The FDA's decision is based on a comprehensive data package and the totality of evidence, including the results from a phase III study demonstrating biosimilarity between Ospomyv and reference Prolia.

Place in Therapy

Ospomyv is the second FDA-approved biosimilar to Prolia, following Jubbonti (denosumab-bbdz), which was the first biosimilar to receive approval. Additionally, Samsung Bioepis obtained FDA approval for the denosumab-dssb, which is not labeled for specific indications.

New FDA-Approved Drug Products

New Biosimilar Product

Specialty

Stoboclo™ (denosumab-bmwo) injection, for subcutaneous use

FDA-Approved Indication

[1] Postmenopausal women with osteoporosis at high risk for fracture; [2] To increase bone mass in men with osteoporosis at high risk for fracture; [3] Glucocorticoid-induced osteoporosis in men and women at high risk for fracture; [4] To increase bone mass in men at high risk for fracture receiving androgen deprivation therapy for nonmetastatic prostate cancer; [5] To increase bone mass in women at high risk for fracture receiving adjuvant aromatase inhibitor therapy for breast cancer.

Dosage & Administration

60 mg every 6 months as a subcutaneous injection administered by a healthcare provider.

Dosage Forms & Strengths

Injection: 60 mg/mL solution in single-dose prefilled syringe.

Contraindications

- Hypocalcemia
- Pregnancy
- Know hypersensitivity to denosumab products

Warnings & Precautions

- ***BBW:*** Severe hypocalcemia in patients with chronic kidney disease
- Patients receiving Stoboclo should not receive other denosumab products concomitantly
- Hypersensitivity including anaphylactic reactions may occur
- Osteonecrosis of the jaw
- Atypical femoral fractures
- Multiple vertebral fractures have been reported following treatment discontinuation
- Serious infections including skin infections
- Dermatological reactions
- Severe bone, joint, muscle pain may occur
- Suppression of bone turnover

Common Adverse Reactions

Back pain, pain in extremity, hypercholesterolemia, musculoskeletal pain, cystitis, arthralgia, nasopharyngitis, hypertension, bronchitis, and headache.

Use in Specific Populations

- Pregnant women and females of reproductive potential: May cause fetal harm.
- Pediatric patients: Stoboclo is not approved for use in pediatric patients.
- Renal impairment: No dose adjustment is necessary in patients with renal impairment.

Clinical Studies

The FDA's decision is based on a comprehensive data package and the totality of evidence, including the results from a phase III study demonstrating biosimilarity between Stoboclo and reference Prolia.

Place in Therapy

Stoboclo is the third FDA-approved biosimilar to Prolia. Jubbonti (denosumab-bbdz) and Ospomyv (denosumab-dssb) were the first and second biosimilars to Prolia, respectively, to receive approval.

New FDA-Approved Drug Products

New Biosimilar Product

Specialty

Xbryk™ (denosumab-dssb) injection, for subcutaneous use

FDA-Approved Indication

[1] Prevention of skeletal-related events in patients with multiple myeloma and in patients with bone metastases from solid tumors; [2] Treatment of adults and skeletally mature adolescents with giant cell tumor of bone that is unresectable or where surgical resection is likely to result in severe morbidity; [3] Treatment of hypercalcemia of malignancy refractory to bisphosphonate therapy.

Dosage & Administration

- Multiple Myeloma and Bone Metastasis from Solid Tumors: 120 mg every 4 weeks as a subcutaneous injection.
- Giant Cell Tumor of Bone: 120 mg every 4 weeks with additional 120 mg doses on Days 8 and 15 of the first month of therapy, administered subcutaneously.
- Hypercalcemia of Malignancy: 120 mg every 4 weeks with additional 120 mg doses on Days 8 and 15 of the first month of therapy administered subcutaneously.

Dosage Forms & Strengths

Injection: 120 mg/1.7 mL (70 mg/mL) solution in a single-dose vial.

Warnings & Precautions

- Patients receiving Xbryk should not receive other denosumab products concomitantly
- Hypersensitivity reactions including anaphylaxis may occur
- Hypocalcemia
- Osteonecrosis of the jaw
- Atypical femoral fracture
- Hypercalcemia Following Treatment Discontinuation in Patients with Giant Cell Tumor of Bone and in Patients with Growing Skeletons
- Multiple Vertebral Fractures (MVF) Following Treatment Discontinuation
- Embryo-Fetal Toxicity

Contraindications

- Hypocalcemia
- Known clinically significant hypersensitivity to denosumab products

Common Adverse Reactions

Fatigue/asthenia, hypophosphatemia, diarrhea, nausea, anemia, back pain, thrombocytopenia, peripheral edema, hypocalcemia, upper respiratory tract infection, rash, headache, arthralgia, dyspnea, decreased appetite, vomiting and constipation.

Use in Specific Populations

- Pediatric patients: Recommended only for treatment of skeletally mature adolescents with giant cell tumor of bone.
- Renal impairment: Patients with creatinine clearance less than 30 mL/min or receiving dialysis are at risk for hypocalcemia.

Clinical Studies

The FDA's decision is based on a comprehensive data package and the totality of evidence, including the results from a phase III study demonstrating biosimilarity between Xbryk and reference Xgeva.

Place in Therapy

Xbryk is the second FDA-approved biosimilar to Xgeva, following Wyost (denosumab-bbdz), which was the first biosimilar to receive approval for Xgeva.

New FDA-Approved Drug Products

New Biosimilar Product

Specialty

Osenvelt™ (denosumab-bmwo) injection, for subcutaneous use

FDA-Approved Indication

[1] Prevention of skeletal-related events in patients with multiple myeloma and in patients with bone metastases from solid tumors; [2] Treatment of adults and skeletally mature adolescents with giant cell tumor of bone that is unresectable or where surgical resection is likely to result in severe morbidity; [3] Treatment of hypercalcemia of malignancy refractory to bisphosphonate therapy.

Dosage & Administration

- Multiple Myeloma and Bone Metastasis from Solid Tumors: 120 mg every 4 weeks as a subcutaneous injection.
- Giant Cell Tumor of Bone: 120 mg every 4 weeks with additional 120 mg doses on Days 8 and 15 of the first month of therapy, administered subcutaneously.
- Hypercalcemia of Malignancy: 120 mg every 4 weeks with additional 120 mg doses on Days 8 and 15 of the first month of therapy administered subcutaneously.

Dosage Forms & Strengths

Injection: 120 mg/1.7 mL (70 mg/mL) solution in a single-dose vial.

Warnings & Precautions

- Patients receiving Osenvelt should not receive other denosumab products concomitantly
- Hypersensitivity reactions including anaphylaxis may occur
- Hypocalcemia
- Osteonecrosis of the jaw
- Atypical femoral fracture
- Hypercalcemia Following Treatment Discontinuation in Patients with Giant Cell Tumor of Bone and in Patients with Growing Skeletons
- Multiple Vertebral Fractures (MVF) Following Treatment Discontinuation
- Embryo-Fetal Toxicity

Contraindications

- Hypocalcemia
- Known clinically significant hypersensitivity to denosumab products

Common Adverse Reactions

Fatigue/asthenia, hypophosphatemia, diarrhea, nausea, anemia, back pain, thrombocytopenia, peripheral edema, hypocalcemia, upper respiratory tract infection, rash, headache, arthralgia, dyspnea, decreased appetite, vomiting and constipation.

Use in Specific Populations

- Pediatric patients: Recommended only for treatment of skeletally mature adolescents with giant cell tumor of bone.
- Renal impairment: Patients with creatinine clearance less than 30 mL/min or receiving dialysis are at risk for hypocalcemia.

Clinical Studies

The FDA's decision is based on a comprehensive data package and the totality of evidence, including the results from a phase III study demonstrating biosimilarity between Osenvelt and reference Xgeva.

Place in Therapy

Osenvelt is the third FDA-approved biosimilar to Xgeva. Wyost (denosumab-bbdz) and Xbryk (denosumab-dssb) were the first and second biosimilars to Xgeva, respectively, to receive approval.

New FDA-Approved Drug Products

New Biosimilar Product

Merilog™ (insulin aspart-szjj) / Merilog™ Solostar injection for subcutaneous use

FDA-Approved Indication

To improve glycemic control in adults and pediatric patients with diabetes mellitus.

Dosage & Administration

Individualize and adjust the dosage based on the individual's metabolic needs, blood glucose monitoring results and glycemic control goal. Should generally be used in regimens with an intermediate- or long-acting insulin.

Dosage Forms & Strengths

Injection: 100 units/mL (U-100) of insulin aspart-szjj available as:

- 10 mL multiple-dose vial
- 3 mL single-patient-use Merilog SoloStar prefilled pen

Contraindications

- During episodes of hypoglycemia
- Hypersensitivity to insulin aspart products or any of the excipients in Merilog

Common Adverse Reactions

Hypoglycemia, allergic reactions, local injection site reactions, lipodystrophy, rash, and pruritus.

Warnings & Precautions

- Never share a Merilog SoloStar prefilled pen between patients, even if the needle is changed
- Hyperglycemia or hypoglycemia with changes in insulin regimen
- Medication Errors
- Hypersensitivity reactions
- Hypokalemia
- Fluid retention and heart failure with concomitant use of thiazolidinediones (TZDs)

Drug Interactions

- Drugs that may increase the risk of hypoglycemia
- Drugs that may decrease the blood glucose lowering effect
- Drugs that may increase or decrease the blood glucose lowering effect
- Drugs that may blunt the signs and symptoms of hypoglycemia

Clinical Studies

The approval of Merilog is based on review of a comprehensive data package and totality of evidence demonstrating a high degree of similarity to its reference product, Novolog.

Place in Therapy

Merilog is the first rapid-acting insulin biosimilar product approved by the FDA.

New FDA-Approved Drug Products

New Formulations, Combinations, and Line Extensions

Orphan Drug | Specialty

Onapgo™ (apomorphine hydrochloride) injection for subcutaneous use

FDA-Approved Indication

For the treatment of motor fluctuations in adults with advanced Parkinson's disease.

Dosage & Administration

For subcutaneous use by infusion only.

Refer to the package insert for more information.

Dosage Forms & Strengths

Injection: 98 mg/20 mL (4.9 mg/mL) of apomorphine hydrochloride in single-dose cartridges

Contraindications

- Concomitant use with 5HT3 antagonists, including antiemetics
- Hypersensitivity to apomorphine or the excipients of Onapgo, including sulfite

Warnings & Precautions

- Subcutaneous use only (thrombus formation and pulmonary embolism have followed intravenous administration)
- May cause nausea and vomiting
- Falling asleep during activities of daily living and daytime somnolence may occur
- May cause hypotension/orthostatic hypotension and syncope may occur
- May cause or increase the risk of falls.
- May cause infusion site reactions and infections.
- May cause hallucinations and psychotic-like behavior
- May cause dyskinesia or exacerbate pre-existing dyskinesia
- May cause hemolytic anemia
- May cause impulse control/ compulsive and impulsive behaviors
- May cause cardiac events
- May prolong QTc and cause torsades de pointes or sudden death

Common Adverse Reactions

Infusion site nodule, nausea, somnolence, infusion site erythema, dyskinesia, headache, and insomnia.

Drug Interactions

- Concomitant use of antihypertensive medications and vasodilators
- Dopamine antagonists such as neuroleptics or metoclopramide, may diminish the effectiveness of Onapgo

Use in Specific Populations

Pregnancy: May cause fetal harm

Clinical Studies

The effectiveness of Onapgo was demonstrated in a randomized, double-blind, placebo-controlled Phase 3 TOLEDO trial involving 104 patients with Parkinson's disease who experienced motor fluctuations despite receiving carbidopa/levodopa and other medications for Parkinson's disease. Participants were randomly assigned to receive either Onapgo or a placebo. The primary endpoint was the change in total daily OFF time, measured from baseline to the end of the 12-week treatment period using patient diaries. The least squares mean change in OFF time from baseline was -0.90 ± 0.416 with placebo compared to -2.55 ± 0.487 with Onapgo (a difference of -1.65, 95% CI: -2.91, -0.38, p = 0.0114).

Place in Therapy

Onapgo is the only FDA-approved continuous subcutaneous apomorphine infusion device for treating Parkinson's disease in the United States. It offers an alternative treatment option for patients with Parkinson's disease who are not responding to their current therapy (including levodopa [LD]) and who may otherwise be candidates for invasive surgical treatments, such as deep brain stimulation (DBS) or focused ultrasound.

New FDA-Approved Drug Products

New Formulations, Combinations, and Line Extensions

Emblaveo™ (avicbactam sodium; aztreonam) injection for subcutaneous use

FDA-Approved Indication

When used in combination with metronidazole, it is indicated in patients 18 years and older who have limited or no alternative options for the treatment of complicated intra-abdominal infections (cIAI) including those caused by the following susceptible gram-negative microorganisms: *Escherichia coli, Klebsiella pneumoniae, Klebsiella oxytoca, Enterobacter cloacae complex, Citrobacter freundii* complex, and Serratia marcescens

Dosage & Administration

Refer to the package insert for more information.

Dosage Forms & Strengths

Injection: Lyophilized powder in a single-dose vial containing 2 g (1.5 grams aztreonam and 0.5 grams avibactam).

Contraindications

Known hypersensitivity to the components of Emblaveo (aztreonam and avibactam).

Common Adverse Reactions

Hepatic adverse reactions, anemia, diarrhea, hypokalemia, and pyrexia

Warnings & Precautions

- Hypersensitivity Reactions
- Serious Skin Disorders
- Hepatic Adverse Reactions
- *Clostridioides Difficile*-Associated Diarrhea

Clinical Studies

The approval of Emblaveo was based on the Phase 3 REVISIT trial, which included 412 hospitalized patients with complicated intra-abdominal infections (cIAI) in the safety population. Patients were randomly assigned in a 2:1 ratio to receive either ATM-AVI with MTZ or meropenem (MER), with or without colistin, for a treatment duration of 5–14 days. In patients with cIAIs, the adjudicated clinical cure rate was 76.4% (159 out of 208) for the ATM-AVI group and 74.0% (77 out of 104) for the MER group.

Place in Therapy

It is the first and only monobactam/β-lactamase inhibitor combination approved in the United States. The dosing schedule and extended infusion time required for Emblaveo may restrict its use in hospital settings.

New FDA-Approved Drug Products

New Formulations, Combinations, and Line Extensions

Orphan Drug | Specialty

Evrysdi™ (risdiplan) tablets for oral use

FDA-Approved Indication

For the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients.

Dosage & Administration

5mg once daily with or without food.

Dosage Forms & Strengths

Tablets: 5 mg

Contraindications

None

Common Adverse Effects

Fever, diarrhea, rash, respiratory tract infection, lower respiratory tract infection, constipation, vomiting, and cough.

Drug Interactions

Avoid coadministration with drugs that are substrates of multidrug and toxin extrusion (MATE) transporters.

Use in Specific Populations

Pregnancy: May cause fetal harm.

Clinical Studies

The approval of the Evrysdi tablet was supported by the results of a bioequivalence study, which showed that the 5 mg tablet, when either swallowed whole or dissolved in non-chlorinated drinking water (e.g., filtered water), provides similar exposure to risdiplam as the original oral solution.

Place in Therapy

Previously, Evrysdi was only available as an oral solution. The tablets offer the convenience of being stored at room temperature, unlike the solution, which needs to be refrigerated between 36°F and 46°F for up to 64 days. While Evrysdi oral solution can be kept at room temperature up to 104°F, it is only for a total of 5 days. The Evrysdi oral solution will continue to be available for patients on different doses of Evrysdi and for those who may prefer the oral solution form.

New FDA-Approved Drug Products

New Formulations, Combinations, and Line Extensions

Specialty

Nilotinib, capsules for oral use

FDA-Approved Indication

For the treatment of: [1] Adults with newly diagnosed Philadelphia chromosome positive chronic myeloid leukemia (Ph+ CML) in chronic phase; [2] Adult patients with chronic phase (CP) and accelerated phase (AP) Ph+ CML resistant to or intolerant to prior therapy that included imatinib

Dosage & Administration

Recommended Adult Dose: Newly diagnosed Ph+ CML-CP: 300 mg orally twice daily. Resistant or intolerant Ph+ CML-CP and CML-AP: 400 mg orally twice daily.

Dosage Forms & Strengths

Capsules: 50 mg, 150 mg, and 200 mg of nilotinib

Contraindications

- Hypokalemia
- Hypomagnesemia
- Long QT syndrome

Common Adverse Effects

Nausea, rash, headache, fatigue, pruritus, vomiting, diarrhea, cough, constipation, arthralgia, nasopharyngitis, pyrexia, night sweats, thrombocytopenia, neutropenia, and anemia.

Warnings & Precautions

- ***BBW***: QT prolongation and sudden deaths
- Myelosuppression
- Cardiac and Arterial Vascular Occlusive Events
- Pancreatitis and Elevated Serum Lipase
- Hepatotoxicity
- Electrolyte Abnormalities
- Tumor Lysis Syndrome
- Hemorrhage
- Fluid Retention
- Effects on Growth and Development in Pediatric Patients
- Embryo-Fetal Toxicity
- Treatment Discontinuation

Drug Interactions

- Strong CYP3A Inhibitors
- Strong CYP3A Inducers
- Proton Pump Inhibitors

Use in Specific Populations

Lactation: Advise women not to breastfeed

Clinical Studies

The effectiveness was established based on adequate and well-controlled studies of Tasigna capsules.

Place in Therapy

Additional pediatric use information is approved for Tasigna (nilotinib) capsules. However, due to Novartis Pharmaceuticals Corporation's marketing exclusivity rights, this drug product is not labeled with that pediatric information.

New FDA-Approved Drug Products

New Formulations, Combinations, and Line Extensions

Specialty | Orphan Drug

Ctexli™ (chenodiol) tablets for oral use

FDA-Approved Indication

For the treatment of cerebrotendinous xanthomatosis (CTX) in adults.

Dosage & Administration

250 mg orally three times daily.

Dosage Forms & Strengths

Tablets: 250mg

Contraindications

None

Common Adverse Effects

Diarrhea, headache, abdominal pain, constipation, hypertension, muscular weakness, and upper respiratory tract infection.

Warnings & Precautions

- Hepatotoxicity

Drug Interactions

- Bile acid sequestering agents and aluminum-based antacids
- Coumarin and its derivatives

Clinical Studies

The approval was supported by the findings from the Phase 3 RESTORE trial (NCT04270682), which assessed the safety and effectiveness of Ctexli in 13 patients. Patients were randomized and treated in a crossover withdrawal design to receive either Ctexli or placebo for 4 weeks during 2 double-blind treatment periods. The primary endpoint of the trial was the reduction in bile alcohol (urine 23S-pentol). At the conclusion of the randomized, double-blind withdrawal phase, urine 23S-pentol levels demonstrated a 20-fold difference between the placebo group and those treated with Ctexli.

Place in Therapy

Ctexli is the first FDA-approved treatment for CTX. However, Chenodal (chenodiol), which contains the same active ingredient, was approved in 2009 for the treatment of radiolucent bladder stones, though it has been used off-label for several years to treat CTX.

New FDA-Approved Drug Products

New Formulations, Combinations, and Line Extensions

Miudella (copper intrauterine system)

FDA-Approved Indication

Indicated for prevention of pregnancy in females of reproductive potential for up to 3 years.

Dosage & Administration

Insert a single Miudella at the fundus of the uterine cavity. Miudella must be removed or replaced after 3 years.

Dosage Forms & Strengths

Intrauterine system: 175 mm^2 exposed copper surface area.

Contraindications

Pregnancy or suspicion of pregnancy.

Common Adverse Effects

Heavy menstrual bleeding, dysmenorrhea, intermenstrual bleeding, pelvic discomfort, procedural pain, pelvic pain, post procedural hemorrhage, dyspareunia.

Warnings & Precautions

- ***BBW***: Risk of complications due to improper insertion
- Risk of Ectopic Pregnancy
- Risks with Intrauterine Pregnancy
- Sepsis
- Pelvic Inflammatory Disease
- Perforation resulting in embedment or translocation
- Expulsion
- Bleeding patterns
- MRI Safety Information

Clinical Studies

The effectiveness of Miudella was demonstrated in a single-arm, open-label study involving 1,601 generally healthy women aged 17 to 45 years who had the Miudella device successfully inserted. The primary endpoint was contraceptive efficacy over 3 years of use, measured by the Pearl Index (PI) in women aged 17 to 35. Participants in the study contributed 12,493 evaluable 28-day cycle equivalents in the first year and 27,115 evaluable cycles over the three-year treatment period. The cumulative 3-year PI, or pregnancy rate, was 1.05 (95% CI: 0.66, 1.60).

Place in Therapy

Miudella is a next-generation, hormone-free, low-dose copper intrauterine device (IUD). It contains less than half the amount of copper compared to the currently available copper IUD, Paragard.

New First-Time Generic Approvals

First-Time Generics are the first generic forms of brand name drugs. The generic version is formulated to work in the same way as the brand-name product and provides the same clinical benefit.

Product	Manufacturer	Generic For	Therapeutic Class	Indication(s)
Bosentan Tablets for Oral Suspension 32mg	Natco Pharma USA LLC	Tracleer tablets for suspension	Cardiovascular Agents – Misc	Pulmonary Arterial Hypertension
Gadoteridol Injection 279.3 mg/mL	Hainan Poly Pharm Co. Ltd	ProHance	Diagnostic Products	Magnetic Resonance Imaging
Mercaptopurine Oral Suspension 20mg/mL	Hikma Pharmaceuticals USA Inc	Purixan	Antineoplastic and Adjunctive Therapies	Acute Lymphoblastic Leukemia

New FDA-Approved Indications for Existing Drugs

The following table contains drugs that have gained FDA approval for the treatment of additional diseases or conditions.

Drug Name and Manufacturer	Previous Indication(s)	New Indication
Susvimo (ranibizumab) From: Genetech, Inc.	For the treatment of patients with neurovascular (WET) age -related macular degeneration (AMD) who have previously responded to at least two intravitreal injections of a VEGF inhibitor.	For the treatment of diabetic macular edema (DME).
Adcetris (brentuximab-vedotin) From: Pfizer	[1] Adult patients with previously untreated stage III or IV classical Hodgkin lymphoma (CHL), in combination with doxorubicin, vinblastine, and dacarbazine; [2] Pediatric patients 2 years and older with previously untreated high risk classical Hodgkin lymphoma (CHL), in combination with doxorubicin, vincristine, etoposide, prednisone, and cyclophosphamide; [3] Adult patients with classical Hodgkin lymphoma (CHL) at high risk of relapse or progression as post-autologous hematopoietic stem cell transplantation (AUTO-HSCT) consolidation; [4] Adult patients with classical Hodgkin lymphoma (CHL) after failure of AUTO-HSCT or after failure of at least two prior multi-agent chemotherapy regimens in patients who are not AUTO-HSCT candidates; [5] Adult patients with previously untreated systemic anaplastic large cell lymphoma (SALCL) or other CD30-expressing peripheral T-cell lymphomas (PTCL), including angioimmunoblastic T-cell lymphoma and PTCL not otherwise specified (NOS), in combination with	Adult patients with relapsed or refractory large B-cell lymphoma (LBCL), including diffuse large B-cell lymphoma (DLBCL) NOS, DLBCL arising from indolent lymphoma, or high-grade B-cell lymphoma (HGBL), after two or more lines of systemic therapy who are not eligible for auto-HSCT or CAR T-CELL therapy, in combination with lenalidomide and a rituximab product.

	cyclophosphamide, doxorubicin, and prednisone; [6] Adult patients with systemic anaplastic large cell lymphoma (SALCL) after failure of at least one prior multi-agent chemotherapy regimen; [7] Adult patients with primary cutaneous anaplastic large cell lymphoma (PCALCL) or CD30-expressing mycosis fungoides (MF) who have received prior systemic therapy	
Odefsey (emtricitabine / rilpivirine / tenofovir alafenamide) From: Gilead	For the treatment of HIV-1 infection in adult and pediatric patients weighing at least 35 kg.	For the treatment of HIV-1 infection in adult and pediatric patients weighing at least 25 kg.
Sublocade (buprenorphine extended-release) From: Indivior	For the treatment of moderate to severe OUD in patients who have initiated treatment with buprenorphine-containing product, followed by dose adjustment for a minimum of 7 days	For the treatment of moderate to severe opioid use disorder (OUD) in patients who have initiated treatment with a single dose of a transmucosal buprenorphine product or who are already being treated with buprenorphine
Odactra (House Dust Mite) From: ALK-Abello A/S	To treat house dust mite (HDM)-induced nasal inflammation (allergic rhinitis), with or without eye inflammation (conjunctivitis), in people 18 through 65 years of age.	As immunotherapy for the treatment of house dust mite (HDM)-induced allergic rhinitis, with or without conjunctivitis, confirmed by positive in vitro testing for IGE antibodies to *dermatophagoides farinae* or *dermatophagoides pteronyssinus* house dust mites or by positive skin testing to licensed house dust mite allergen extracts, in individuals 5 through 65 years of age.
TNKase (tislezumab-jsgr) *From: Genetech*	For the treatment of acute ischemic stroke in adults.	To reduce the risk of death associated with acute ST elevation myocardial infarction (STEMI) in adults.

Pipeline

The goals of the NDA (or BLA) are to provide enough information to permit FDA approval of a new pharmaceutical for sale and marketing in the U.S.

Drug Name and Manufacturer	Indication(s)	Additional Information	Impact
Brensocatib From: Insmed Inc.	Bronchiectasis	NDA accepted	High
Lerodalcibep From: LIB Therapeutics Inc.	High Cholesterol	BLA accepted	Moderate
Linvoseltamab From: Regeneron Pharmaceuticals, Inc.	For the treatment of relapsed/refractory multiple myeloma	BLA accepted	High
Troriluzole From: Biohaven Lid.	For the treatment of adult patients with spinocerebellar ataxia	NDA accepted	High
Dordaviprone From: Chimerix, Inc.	For the treatment of patients with recurrent H3 K27M-mutant diffuse glioma	NDA accepted	High
Vatiquinone From: PTC Therapeutics, Inc.	For the treatment of Friedreich's ataxia	NDA accepted	High