RxOutlook®

1st Quarter 2024

Optum Rx®

Welcome to the first quarter RxOutlook Report of 2024. Optum Rx closely monitors and evaluates the drug development pipeline to identify noteworthy upcoming drug approvals and reports the essential findings here in RxOutlook.

Recap of 2023

In 2023, the FDA's Center for Drug Evaluation and Research (CDER) approved 55 novel drugs. Of the 55 approvals, 20 drugs (36%) were considered first-in-class and 36 (65%) used one or more expedited FDA programs (ie, Fast Track, Breakthrough Therapy, Priority Review, or Accelerated Approval). For the fifth year out of the last six, the number of novel therapies approved with Orphan Drug status exceeded non-Orphan Drugs (51% were Orphan Drugs).

In addition to these 55 novel drugs, the FDA's Center for Biologics Evaluation and Research (CBER) approved 5 gene therapies, including two for sickle cell disease, **Lyfgenia™ (lovotibeglogene autotemcel)** and **Casgevy™ (exagamglogene autotemcel)**. Casgevy is the first FDA approved therapy utilizing CRISPR/Cas9, a type of genome editing technology. The other key CBER approvals for 2023 were **Abrysvo™** and **Arexvy**, the first RSV vaccines for older adults.

Looking Ahead to 2024

As of February 2, the number of novel drugs approved or with an FDA submission currently under review for 2024 is 45. The final number of FDA approvals will depend on how many more new drug applications are filed with the FDA over the next several months and how many drugs are rejected by the FDA.

In this edition of RxOutlook, we highlight 6 key pipeline products with an approval decision by the end of the 2nd quarter 2024. The first product discussed in the report, Pfizer's **fidanacogene elaparvovec**, is potentially the second gene therapy approved for hemophilia B, and a direct competitor to CSL Behring's Hemgenix® (etranacogene dezaparvovec). Hemgenix was approved at the end of 2022 and is the most expensive gene therapy on the market ($3.5 million for a one-time dose).

Moderna's **mRNA-1345** is expected to be the third RSV vaccine approved in older adults (≥ 60 years). Unlike Pfizer's Abrysvo and GSK's Arexvy, mRNA-1345 is a mRNA-based vaccine using similar technology as Moderna's previous COVID-19 vaccines, whereas Arexvy and Abrysvo use a more traditional protein-based vaccine approach.

Verona's Pharma's **ensifentrine** is a first-in-class phosphodiesterase 3 and 4 inhibitor for chronic obstructive pulmonary disease (COPD). COPD is one of the most common respiratory conditions in the U.S. but there have been few new treatment modalities over the last 10 to 20 years. Ensifentrine is likely the first of several new treatments that could be approved in the next 2 to 3 years for severe COPD.

Elafibranor, **imetelstat**, and **danicopan** would each represent novel treatments for different orphan conditions. Elafibranor is potentially the second drug approved for secondary treatment of primary biliary cholangitis, a rare liver disease. Imetelstat could be an additional treatment option in patients with myelodysplastic syndromes, a group of rare disorders affecting the bone marrow. Finally, danicopan would be the first add-on therapy to C5 complement inhibitors for treatment of paroxysmal nocturnal hemoglobinuria, a rare disease characterized by red blood cell destruction.

Approval decisions for other key novel therapies are expected by the end of the 2nd 2024 but are not reviewed in this report because they were covered in previous editions of RxOutlook. This includes: resmetirom for nonalcoholic steatohepatitis; aprocitentan for treatment-resistant hypertension; sotatercept for pulmonary arterial hypertension; and donanemab for Alzheimer's disease.

Key pipeline drugs with FDA approval decisions expected by end of the 2nd quarter 2024

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Fidanacogene elaparvovec	Pfizer	Hemophilia B*	2Q 2024
mRNA-1345 vaccine	Moderna	Respiratory syncytial virus (RSV)	4/2024
Elafibranor	Ipsen/Genfit	Primary biliary cholangitis*	6/10/2024
Imetelstat	Geron	Myelodysplastic syndromes*	6/16/2024
Ensifentrine	Verona Pharma	Chronic obstructive pulmonary disease	6/26/2024
Danicopan	AstraZeneca	Paroxysmal nocturnal hemoglobinuria*	1H 2024

* Orphan Drug Designation

Detailed Drug Insights

This section reviews the important characteristics (eg, therapeutic use, clinical profile, competitive environment and regulatory timeline) for key pipeline drugs with potential FDA approvals by the end of the 1st quarter 2024.

Read more

Extended Brand Pipeline Forecast

This supplemental table provides a summary of developmental drugs, including both traditional and specialty medications that may be approved in the upcoming two years.

Read more

Key Pending Indication Forecast

This supplemental table provides a summary of key new indications that are currently under review by the FDA and may be approved in the upcoming 12 months.

Read more

Extended Generic Pipeline Forecast

This section provides a summary of upcoming first-time generic drugs and biosimilars that may be approved in the upcoming two years.

Please note that RxOutlook highlights select near-term approvals. Some drugs may not appear in this issue because they have been reviewed in previous editions of RxOutlook. Drugs of interest that are earlier in development or with expected approvals beyond 2nd quarter 2024 may appear in future reports; however, for those who need an initial look at the larger pipeline, please refer to the Brand Pipeline Forecast Table found later in this report.

Read more

Getting acquainted with pipeline forecast terms

Clinical trial phases

Phase I trials	Researchers test an experimental drug or treatment in a small group of people for the first time to evaluate its safety, determine a safe dosage range, and identify side effects.
Phase II trials	The experimental study drug or treatment is given to a larger group of people to see if it is effective and to further evaluate its safety.
Phase III trials	The experimental study drug or treatment is given to large groups of people to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, and collect information that will allow the experimental drug or treatment to be used safely.
Phase IV trials	Post marketing studies delineate additional information including the drug's risks, benefits, and optimal use.

Pipeline acronyms

ANDA	Abbreviated New Drug Application
BLA	Biologic License Application
CRL	Complete Response Letter
FDA	Food and Drug Administration
MOA	Mechanism of Action
NME	New Molecular Entity
NDA	New Drug Application
sBLA	Supplemental Biologic License Application
sNDA	Supplemental New Drug Application
OTC Drugs	Over-the-Counter Drugs
PDUFA	Prescription Drug User Fee Act
REMS	Risk Evaluation and Mitigation Strategy

Detailed Drug Insights

Fidanacogene elaparvovec (Brand Name: To be determined)

Manufacturer: Pfizer
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 2Q 2024

Therapeutic use

Fidanacogene elaparvovec is under review for the treatment of adults with moderately severe to severe hemophilia B.

Hemophilia B is an inherited bleeding disorder in which the blood does not clot properly. Blood contains many proteins called clotting factors that can help to stop bleeding. People with hemophilia B have low levels of factor IX (FIX). Hemophilia B is classified as mild, moderate, or severe based upon the activity level of FIX. In mild cases, bleeding symptoms may occur only after surgery, injury, or a dental procedure. In some moderate and most severe cases, bleeding symptoms may occur after a minor injury or spontaneously.

Hemophilia B occurs in approximately 1 in 25,000 male births. It is less prevalent than hemophilia A which occurs in approximately 1 in 5,000 male births. Overall, hemophilia affects about 33,000 people in the U.S., and hemophilia B represents about 15% of patients with hemophilia (approximately 5,000 patients).

About 50% of patients with hemophilia B have a severe form of the disease.

What you need to know:

Proposed Indication: Treatment of adults with moderately severe to severe hemophilia B

Mechanism: Gene therapy

Efficacy: Annualized bleeding rate: 1.3 from Month 3 to 15 post-infusion vs. 4.43 during lead-in pre-treatment period (71% reduction)

Safety: Limited safety data

Dosing: IV as a one-time dose

Why it Matters: Eliminates or reduces the need for chronic and as-needed FIX replacement therapy

Important to Note: Second gene therapy to market for hemophilia B (CSL Behring's Hemgenix approved in November 2022), unclear durability of response and long-term safety, small target population

Estimated Cost: $3.5 million for a one-time dose (based on pricing for Hemgenix)

Clinical profile

Fidanacogene elaparvovec is an in vivo gene therapy that contains an adeno-associated virus (AAV) capsid and a high-activity variant of human coagulation FIX gene. FIX is the primary deficiency in individuals with hemophilia B, and the goal of this gene therapy is to enable patients to produce their own FIX and limit need for exogenous blood factors.

Pivotal trial data:
The efficacy of fidanacogene elaparvovec was evaluated in BENEGENE-2, a Phase 3, open-label, single-arm study in adult male patients (age 18 to 65) with moderately severe to severe hemophilia B (defined as FIX circulating activity of ≤ 2%). Eligible study participants (N = 45) completed a minimum 6 months of routine exogeneous FIX prophylaxis therapy during a lead-in study and then received one intravenous (IV) dose of fidanacogene elaparvovec. The main objective of the study was to compare the annualized bleeding rate (ABR) post-infusion with fidanacogene elaparvovec vs. the ABR during the lead-in period with FIX prophylaxis therapy.

Fidanacogene elaparvovec *(continued...)*

The study met the primary endpoint of non-inferiority and superiority in the ABR of total bleeds post-fidanacogene elaparvovec infusion vs. FIX prophylaxis. The mean ABR for all bleeds was 1.3 for the 12 months from Month 3 to Month 15 post-infusion with fidanacogene elaparvovec vs. an ABR of 4.43 during the lead-in pre-treatment period, resulting in a 71% reduction in ABR ($p < 0.0001$).

Key secondary endpoints demonstrated a 78% reduction in treated ABR ($p = 0.0001$) and a 92% reduction in annualized FIX usage ($p < 0.0001$).

Safety:
The safety data for fidanacogene elaparvovec is limited but no deaths, serious adverse events associated with infusion reactions, thrombotic events, or FIX inhibitors were reported.

Dosing:
In the pivotal trial, fidanacogene elaparvovec was administered via intravenous (IV) infusion as a one-time dose.

Competitive environment

The standard of care for patients with severe hemophilia B is chronic FIX prophylactic treatment. In November 2022, the FDA approved Hemgenix® (etranacogene dezaparvovec), the first gene therapy for hemophilia B. Similar to Hemgenix, the goal of treatment with fidanacogene elaparvovec is to reduce, and hopefully eliminate, the need for chronic maintenance and as-needed FIX replacement therapy. FIX replacement therapy has a high treatment burden and can be very costly particularly in severe patients requiring high doses or prophylactic use of FIX.

Like Hemgenix and other gene therapies, the primary limitation or question for fidanacogene elaparvovec is the unknown durability of response and long-term safety. Fidanacogene elaparvovec did significantly reduce the need for FIX replacement therapy but it did not completely eliminate the risk of bleeding events. Over time, FIX activity may decrease post-infusion which could result in reduced efficacy in terms of reductions in bleeding events. Sustained efficacy is especially important with gene therapies because of the high projected cost for a one-time dose.

Compared indirectly, the efficacy of fidanacogene elaparvovec appears similar to Hemgenix, although cross-trial comparisons are difficult.

For reference, the Wholesale Acquisition Cost (WAC) for Hemgenix is $3.5 million for a one-time dose.

mRNA-1345 (Brand Name: To be determined)

Manufacturer: Moderna
Regulatory designations: Breakthrough Therapy, Fast Track
Expected FDA decision: April 2024

Therapeutic use

mRNA-1345 is under review for prevention of respiratory syncytial virus (RSV)-associated lower respiratory tract disease (RSV-LRTD) and acute respiratory disease (ARD) in adults age 60 years or older.

RSV is a common respiratory virus that usually causes mild, cold-like symptoms, lasting about 1 to 2 weeks. However, some patients may develop severe RSV infection, including bronchiolitis and pneumonia, which may result in a hospitalization. Infants, young children, and older adults are most at risk for severe RSV infection. In the U.S. and other areas with similar climates, RSV circulation generally starts during the fall and peaks in the winter, but the timing and severity of RSV season in a given community can vary from year to year.

Each year, it is estimated that between 60,000 to 160,000 older adults in the U.S. are hospitalized and 6,000 to 10,000 die due to infection.

Clinical profile

mRNA-1345 is a RSV vaccine that consists of a single mRNA sequence encoding for a stabilized prefusion F glycoprotein. The vaccine uses the same lipid nanoparticles as in the Moderna COVID-19 vaccines. The F glycoprotein is on the surface of RSV and is required for infection by helping the virus to enter host cells.

What you need to know:

Proposed Indication: Prevention of RSV-associated lower respiratory tract disease (LRTD) and acute respiratory disease (ARD) in adults aged 60 years or older

Mechanism: mRNA-based vaccine

Vaccine Efficacy:

- 83.7% for prevention of RSV-associated LRTD with ≥ 2 signs or symptoms
- 82.4% for prevention of RSV-associated LRTD with ≥ 3 signs or symptoms

Common AEs: Injection-site pain, fatigue, headache, myalgia, arthralgia

Dosing: IM injection as a one-time dose

Why it Matters: Comparable efficacy to protein-based vaccines (GSK's Arexvy and Pfizer's Abrysvo), appears well tolerated

Important to Note: Data limited to a single RSV season, frequency of revaccination not yet known, initial indication limited to adults ≥ 60 years

Estimated Cost: $280 to $295 for a one-time dose (based on pricing for Arexvy and Abrysvo)

Pivotal trial data:
The efficacy of mRNA-1345 was evaluated in ConquerRSV, a Phase 3, randomized, double-blind, placebo-controlled study in 35,541 adults 60 years of age or older. Patients were randomized to receive one dose of mRNA-1345 or placebo. The two primary efficacy endpoints were the prevention of RSV-associated LRTD with at least two signs or symptoms and with at least three signs or symptoms. The median follow-up was 112 days (range: 1 to 379). A key secondary endpoint was the prevention of RSV-associated ARD.

Vaccine efficacy was 83.7% (95.88% CI: 66.0, 92.2) against RSV-associated LRTD with at least two signs or symptoms and 82.4% (96.36% CI: 34.8, 95.3) against the disease with at least three signs or symptoms. Vaccine efficacy was 68.4% (95% CI: 50.9, 79.7) against RSV-associated ARD.

Safety:
The most common adverse events with mRNA-1345 use were injection-site pain, fatigue, headache, myalgia, and arthralgia.

Dosing:
In the pivotal trial, mRNA-1345 was administered as an intramuscular (IM) injection as a one-time dose.

mRNA-1345 *(continued...)*

Competitive environment

RSV infection is one of the most common respiratory infections. While typically mild in disease course, severe infections are still a significant driver of morbidity and mortality in patients at elevated risk, particularly the elderly population. Prior to 2023, there were no preventative vaccines or treatments for RSV infection in older adults. This changed in 2023 when the FDA approved two RSV vaccines: GSK's Arexvy and Pfizer's Abrysvo. Unlike Moderna's mRNA-based vaccine, Arexvy and Abrysvo use the more traditional protein-based approach.

While it is difficult to compare across clinical trials, the efficacy of mRNA-1345 appears similar to Arexvy and Abrysvo for RSV protection during one RSV season. GSK and Pfizer's vaccines have data supporting protection into a second RSV season; however, longer-term data is lacking for Moderna's vaccine. There were no cases of acute disseminated encephalomyelitis or Guillain–Barré syndrome in the mRNA-1345 pivotal study; these were rare adverse events with the GSK and Pfizer vaccines.

In addition to patients 60 years and older, Abrysvo is approved for active immunization of pregnant individuals to provide protection for newborns and young infants and Arexvy could be approved in patients 50 years and older by the end of 2024. Compared to these vaccines, mRNA-1345's initial use will be limited to patients 60 years and older.

For reference, the WAC for Arexvy and Abrysvo are $280 and $295, respectively, for a one-time dose.

Elafibranor (Brand Name: To be determined)

Manufacturer: Ipsen/Genfit
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: June 10, 2024

Therapeutic use

Elafibranor is under review for the second-line treatment of patients with primary biliary cholangitis (PBC).

PBC is a chronic, progressive liver disorder that leads to inflammation and scarring of the small bile ducts. The damage to bile ducts can inhibit the liver's ability to get rid of toxins in the body and can lead to cirrhosis (scarring of liver tissue). The exact cause of PBC is unknown, but it is thought that it is likely due to a combination of factors such as autoimmune, genetic, and environmental factors.

PBC mostly occurs in women over the age of 40, and the overall prevalence in the U.S. is estimated to be about 50,000 people.

What you need to know:

Proposed Indication: Second-line treatment of patients with primary biliary cholangitis

Mechanism: PPAR alpha/delta agonist

Efficacy: Biochemical response: 51% vs. 4% with placebo

Common AEs: Abdominal pain, diarrhea, nausea, vomiting

Dosing: Oral once daily

Why it Matters: Novel MOA, potential improved tolerability (eg, less itching) vs. Intercept's Ocaliva (only other FDA approved second-line agent)

Important to Note: Ocaliva has been approved for second-line treatment since 2016, potential future competition (eg, seladelpar), lack of head-to-head trial data

Estimated Cost: ~$110,000 per year (based on pricing for Ocaliva)

Clinical profile

Elafibranor is a dual peroxisome activated receptor (PPAR) alpha/delta agonist. Activation of PPAR alpha/delta targets multiple cell types and biological processes involved in the pathophysiology of PBC, including cholestasis (impairment of bile flow in the liver), bile toxicity, inflammation and fibrosis and bile acid output.

Pivotal trial data:
The efficacy of elafibranor was evaluated in ELATIVE, a Phase 3, randomized, double-blind, placebo-controlled study in 161 patients with PBC who had an inadequate response to or unacceptable side effects with ursodeoxycholic acid (UDCA). Patients were randomized to elafibranor or placebo. The primary endpoint was a biochemical response. Response was defined as an alkaline phosphatase (ALP) level of < 1.67 x the upper limit of the normal (ULN), with a reduction of ≥ 15% from baseline, and normal total bilirubin levels at Week 52. Both ALP and total bilirubin are key biomarkers in PBC, and elevated levels are signs of liver damage. Key secondary endpoints were normalization of the ALP level at Week 52 and a change in pruritus intensity from baseline through Week 52 and through Week 24, as measured on the Worst Itch Numeric Rating Scale (WI-NRS; scores range from 0 [no itch] to 10 [worst itch imaginable]).

A biochemical response was achieved in 51% of the patients who received elafibranor vs. 4% who received placebo (treatment difference of 47%, 95% CI: 32, 57; p < 0.001).

ALP normalized in 15% of the patients in the elafibranor group vs. none of the patients in the placebo group at Week 52 (difference of 15%, 95% CI: 6, 23; p = 0.002). Among patients who had moderate-to-severe pruritus, the least-squares mean change from baseline through Week 52 on the WI-NRS did not differ significantly between the groups (-1.93 vs. -1.15; difference of -0.78; 95% CI: -1.99, 0.42; p = 0.20).

Elafibranor *(continued...)*

Safety:
The most common adverse events with elafibranor use were abdominal pain, diarrhea, nausea, and vomiting.

Dosing:
In the pivotal trial, elafibranor was administered orally once daily.

Competitive environment

First-line treatment of PBC is UDCA, which is available generically. UDCA has been shown to slow disease progression; however, some patients may need additional treatment if liver tests remain high. For second-line treatment, the only approved treatment is Intercept Pharmaceuticals' Ocaliva® (obeticholic acid).

If approved, elafibranor would offer a novel mechanism of action (MOA) for the treatment of PBC and would be a direct competitor to Ocaliva for second-line treatment of the disease. Compared indirectly, the efficacy data for elafibranor appears similar to Ocaliva. The primary differentiator for elafibranor is that it was not associated with increased pruritus, which is a disease manifestation of PBC, and a common side effect associated with Ocaliva.

Additional treatment options are in the pipeline for PBC, including CymaBay Therapeutics' PPAR delta agonist, seladelpar. CymaBay announced an FDA filing for seladelpar on December 15, 2023, which puts them on track for a potential approval as early as August 2024.

For reference, the WAC for Ocaliva is approximately $110,000 per year.

Imetelstat (Brand Name: To be determined)

Manufacturer: Geron
Regulatory designations: Orphan Drug, Fast Track
Expected FDA decision: June 16, 2024

Therapeutic use

Imetelstat is under review for the treatment of transfusion-dependent anemia in adult patients with low- to intermediate-1 risk myelodysplastic syndromes (MDS) who have failed to respond or have lost response to or are ineligible for erythropoiesis-stimulating agents (ESAs).

MDS are conditions that can occur when the blood-forming cells in the bone marrow become abnormal. This leads to low overall blood cell counts, with red blood cells being most commonly affected. Patients who develop anemia often require red blood cell (RBC) transfusions. In some affected patients, MDS may progress to life-threatening failure of the bone marrow or develop into acute leukemia. Patients are stratified, from low to high risk, based on risk of mortality and developing acute leukemia.

MDS affect males slightly more often than females and occurs more commonly in older adults. Approximately 20,000 new patients are diagnosed each year in the U.S.

What you need to know:

Proposed Indication: Treatment of transfusion-dependent anemia in adult patients with low- to intermediate-1 risk MDS who have failed to respond or have lost response to or are ineligible for ESAs

Mechanism: Telomerase inhibitor

Efficacy: RBC-transfusion independence: 40% vs. 15% with placebo

Common AEs: Neutropenia, thrombocytopenia

Dosing: IV once every 4 weeks

Why it Matters: Novel MOA, benefit demonstrated across subgroups of MDS, also in development for other hematologic indications (eg, myelofibrosis)

Important to Note: Initial indication will be limited to second-line therapy behind ESAs, competing with Reblozyl as a second-line treatment, IV administration

Estimated Cost: ~$250,000 per year (based on pricing for Reblozyl)

Clinical profile

Imetelstat is a telomerase inhibitor. By targeting telomerase, imetelstat is believed 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.

Pivotal trial data:
The efficacy of imetelstat was evaluated in IMerge, a randomized, double-blind, placebo-controlled study in 178 adults with ESA-relapsed, ESA-refractory, or ESA-ineligible low- to intermediate-1 risk MDS. Patients were randomized to imetelstat or placebo. The primary endpoint was 8-week red blood cell transfusion independence (RBC-TI), defined as the proportion of patients without RBC transfusions for at least 8 consecutive weeks starting on the day of randomization until subsequent anti-cancer therapy, if any.

In the imetelstat group, 40% of patients had an RBC-TI of at least 8 weeks vs. 15% in the placebo group (rate difference 25, 95% CI: 9.9, 36.9; p = 0.0008).

Safety:
The most common adverse events with imetelstat use were neutropenia and thrombocytopenia.

Dosing:
In the pivotal trial, imetelstat was administered via IV infusion once every 4 weeks.

Imetelstat *(continued...)*

Competitive environment

Treatment for MDS depends on different factors such as the subtype, prognosis (risk group), and the patient's overall health status. Generally, hematopoietic stem cell transplant is the only curative treatment but not all patients are candidates or have a matched donor. Supportive treatments to correct low RBCs are commonly used in MDS, including blood transfusions or pharmacotherapy. Historically that was with ESA treatment, but Bristol Myers Squibb's red blood cell maturation agent, Reblozyl® (luspatercept), was approved as a first-line treatment and alternative to ESAs in August 2023.

Imetelstat is a first-in-class therapy that could be used as an alternative to existing treatment options for MDS. The data for transfusion independence were promising with efficacy demonstrated across different subgroups of MDS patients. However, its initial use will be limited to second-line treatment, where it will also be competing with Reblozyl. Unlike imetelstat, Reblozyl is delivered via subcutaneous (SC) injection that must be administered by a healthcare provider.

Imetelstat is in development for other hematologic indications, including myelofibrosis. An interim analysis of the Phase 3 myelofibrosis study (IMpactMF) is expected in the first half of 2025.

For reference, the WAC for Reblozyl is approximately $250,000 per year.

Ensifentrine (Brand Name: To be determined)

Manufacturer: Verona Pharma
Expected FDA decision: June 26, 2024

Therapeutic use

Ensifentrine is under review for the maintenance treatment of patients with chronic obstructive pulmonary disease (COPD).

COPD is a group of lung diseases that cause abnormal limitations in airflow and lead to chronic and progressive breathing-related problems such as shortness of breath, coughing, and increased lung mucous. It includes emphysema and chronic bronchitis.

Chronic lower respiratory disease, primarily COPD, was the fourth leading cause of death in the U.S. in 2018. Almost 15.7 million people (6.4%) in the U.S. reported that they have been diagnosed with COPD.

Clinical profile

Ensifentrine is a dual inhibitor of the phosphodiesterase 3 (PDE3) and phosphodiesterase 4 (PDE4) enzymes. Dual inhibition of PDE3 and PDE4 has shown enhanced or synergistic effects with contraction of airway smooth muscle and suppression of the inflammatory response.

Pivotal trial data:
The efficacy of ensifentrine was evaluated in ENHANCE-1 (N = 760) and ENHANCE-2 (N = 789), two Phase 3, randomized, double-blind, placebo-controlled studies in adults with moderate to severe symptomatic COPD. Patients were receiving no long-acting maintenance therapy or were receiving long-acting β2-agonists (LABA) with or without inhaled corticosteroids (ICS) or long-acting muscarinic antagonists (LAMA) with or without ICS. The primary endpoint, average forced expiratory volume in the first second (FEV_1) area under the curve $(AUC)_{0-12\,h}$, was selected to assess bronchodilatory effects over 12 hours and was calculated as the AUC (pre-dose and 30 min and 1, 2, 4, 6, 8, and 12 hour post-dose), divided by the 12 hours. This endpoint is a standard method for evaluating lung function.

What you need to know:

Proposed Indication: Treatment of patients with COPD

Mechanism: PDE3/4 inhibitor

Efficacy: Mean change from baseline at Week 12 in the FEV1 AUC0–12 h:

- ENHANCE-1 trial: 62 mL vs. –26 with placebo
- ENHANCE-2 trial: 48 mL vs. –46 mL with placebo

Safety: Adverse events were similar between ensifentrine and placebo

Dosing: Inhaled (via nebulizer) twice daily

Why it Matters: Novel MOA, unmet need, potential future indications/uses (eg, cystic fibrosis, asthma)

Important to Note: Likely reserved for patients who are symptomatic despite existing standard of care, lack of data in patients receiving dual bronchodilators (LAMA/LABA), must be administered via nebulizer, potential future competition (eg, Dupixent)

Estimated Cost: ~$7,600 per year (based on pricing for Breztri Aerosphere)

In ENHANCE-1, the least-squares (LS) mean change from baseline in FEV_1 $AUC_{0-12\,h}$ at 12 weeks was 62 mL and –26 with ensifentrine and placebo, respectively (difference 87, 95% CI: 55, 119; $p < 0.001$).

In ENHANCE-2, the LS mean change from baseline in FEV_1 $AUC_{0-12\,h}$ was 48 mL and –46 with ensifentrine and placebo, respectively (difference 94, 95% CI: 65, 124; $p < 0.001$).

Additionally, ensifentrine treatment reduced the rate of moderate or severe exacerbations over 24 weeks by 36% ($p = 0.050$) and 43% ($p = 0.009$) vs. placebo in ENHANCE-1 and -2, respectively.

Safety:
The adverse event rates were similar for ensifentrine and placebo.

Ensifentrine *(continued...)*

Dosing:
In the pivotal trials, ensifentrine was administered twice daily via nebulizer.

Competitive environment

The current standard of care for treatment of COPD includes inhaled short- and long-acting bronchodilators (muscarinic antagonists and β2-agonists) and corticosteroids. In patients who continue to be symptomatic, there are limited treatment options available and there remains an unmet need. It's estimated that up to 50% of COPD patients are currently symptomatic despite their current treatment.

Ensifentrine would provide a novel MOA as an add-on therapy to patients who require additional treatment to manage their COPD. In the pivotal studies, ensifentrine demonstrated significant improvements in lung function; however, ensifentrine was not studied in all possible current combinations of medications that could be used to treat COPD and specifically excluded patients receiving concurrent dual LAMA/LABA or triple (LAMA/LABA/ICS) therapy.

Ensifentrine is expected to be the first of several new treatments for COPD over the next 2 to 3 years. Most notably, Sanofi/Regeneron's Dupixent® (dupilumab), a SC administered interleukin (IL)-4 receptor alpha antagonist, could be approved for COPD in the second half of 2024 and would compete with ensifentrine as a back-line treatment in certain patients with severe COPD.

The use of ensifentrine could be limited initially since it will only be available as a solution for use with a nebulizer. Verona is developing a dry powder inhaler and metered-dose inhaler, but it is unknown when those formulations could be on the market.

Aside from COPD, ensifentrine is being evaluated for several other diseases including non-cystic fibrosis bronchiectasis, cystic fibrosis, and asthma.

For reference, the WAC for Breztri Aerosphere® (budesonide/glycopyrrolate/formoterol fumarate), a combination inhaler for COPD, is approximately $7,600 per year.

Danicopan (Brand Name: To be determined)

Manufacturer: AstraZeneca
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 1H 2024

Therapeutic use

Danicopan is under review for the treatment of paroxysmal nocturnal hemoglobinuria (PNH) in patients who have clinically evident extravascular hemolysis (EVH).

PNH is a rare, complement-mediated blood disorder in which an acquired mutation in a patient's hematopoietic stem cells causes the production of defective red blood cells (RBCs). These defective RBCs are susceptible to premature destruction by a patient's own immune system (complement system). Some patients treated with C5 complement inhibitors for their PNH can experience EVH (removal of RBCs outside of the blood vessels). These patients can have clinically significant EVH, which can result in continued symptoms of anemia.

The incidence of clinically significant PNH is estimated to be at least 1 to 10 cases per million in the general population and approximately 6,000 people are living with PNH in the U.S.

What you need to know:

Proposed Indication: Treatment of paroxysmal nocturnal hemoglobinuria in patients who have clinically evident extravascular hemolysis

Mechanism: Factor D inhibitor

Efficacy: Mean change from baseline at Week 12 in Hb: 2.94 g/dL vs. 0.50 g/dL with placebo

Common AEs: Headache, nausea, arthralgia, diarrhea

Dosing: Oral three times daily

Why it Matters: Novel MOA, synergy when used in combination with a standard of care therapy

Important to Note: Must be used as add-on therapy with C5 inhibitors, competing with injectable Empaveli (C3 inhibitor) and recently approved oral Fabhalta (Factor B inhibitor) in patients who fail C5 inhibitors (used as monotherapy), small target population

Clinical profile

Danicopan is a factor D inhibitor. Factor D is a complement system protein that plays a key role in the amplification of the complement system response.

Pivotal trial data:
The efficacy of danicopan was evaluated in ALPHA, a Phase 3, randomized, double-blind, placebo-controlled study in patients with PNH who experience clinically significant EVH. Patients were randomized to danicopan or placebo, in addition to their ongoing C5 inhibitor (Soliris® or Ultomiris®). The protocol-prespecified interim efficacy analysis set included the first 63 participants. The primary endpoint was change in hemoglobin (Hb) concentration from baseline to week 12.

At week 12, the least squares mean (LSM) change from baseline in Hb was 2.94 g/dL with danicopan vs. 0.50 g/dL with placebo (LSM difference 2.44 g/dL, 95% CI: 1.69, 3.20); $p < 0.0001$).

Additionally, significantly more patients treated with danicopan avoided transfusion (remaining transfusion-free and not requiring a transfusion as per protocol) through week 12 (83.3% vs. 38.1%; $p = 0.0004$).

Safety:
The most common adverse events with danicopan use were headache, nausea, arthralgia, and diarrhea.

Dosing:
In the pivotal trial, danicopan was administered orally three times daily.

Danicopan *(continued...)*

Competitive environment

If approved, danicopan would offer a novel, oral treatment option in patients who need additional treatment after C5 complement inhibitors. The efficacy and safety data for danicopan appear promising; however, it must be used in combination with injectable C5 inhibitors. Other alternatives to C5 inhibitors, including SC administered Empaveli® (pegcetacoplan) (C3 inhibitor) and the recently approved oral Fabhalta® (iptacopan) (factor B inhibitor) are administered as monotherapies.

PNH is a rare disorder so the initial target population for danicopan will be relatively small. It will be competing with Empaveli and Fabhalta in the subset of patients who require treatment beyond C5 inhibitors.

AstraZeneca is also evaluating danicopan for geographic atrophy, a severe form of wet age-related macular degeneration (AMD). A Phase 2 study for this indication is currently ongoing.

For reference, the WAC for Fabhalta is approximately $550,000 per year. However, because danicopan is intended to be used as add-on therapy rather than replacing existing treatment options, it will likely have differentiated pricing.

Extended brand pipeline forecast

Optum Rx brand pipeline forecast

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
2024 Possible launch date									
NVK-002	atropine	Vyluma	anticholinergic	Myopia	OPH	Filed NDA	01/31/2024	No	No
VNRX-5133	cefepime/ taniborbactam	VenatoRx Pharmaceuticals	cephalosporin/ beta-lactamase inhibitor	Bacterial infections	IV	Filed NDA	02/22/2024	No	No
LN-144	lifileucel	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Melanoma	IV	Filed BLA	02/24/2024	Yes	Yes
MIN-101	roluperidone	Minerva Neurosciences	sigma-2 and 5HT-2A receptor antagonist	Schizophrenia	PO	Filed NDA	02/26/2024	No	No
AAI-101	cefepime/ enmetazobactam	Allecra Therapeutics	beta-lactam/b-lactamase inhibitor	Urinary tract infection	IV	Filed NDA	02/27/2024	No	No
Botulax	letibotulinumtoxinA	Hugel Pharma	botulinum toxins	Wrinkles	IM	Filed BLA	03/01/2024	Yes	No
APP-13007	clobetasol propionate	Formosa Pharmaceuticals	corticosteroid	Eye inflammation/ pain	OPH	Filed NDA	03/04/2024	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
glatiramer acetate depot	glatiramer acetate long-acting	Viatris	immunomodulator	Multiple sclerosis	IM	Filed NDA	03/08/2024	Yes	No
MGL-3196	resmetirom	Madrigal Pharmaceuticals	beta-selective thyroid hormone receptor agonist	Nonalcoholic steatohepatitis	PO	Filed NDA	03/14/2024	Yes	No
OTL-200	atidarsagene autotemcel	Orchard Therapeutics	gene therapy	Leukodystrophy	IV	Filed BLA	03/18/2024	Yes	Yes
ACT-132577	aprocitentan	Idorsia Pharmaceuticals	endothelin receptor antagonist	Hypertension	PO	Filed NDA	03/19/2024	No	No
TAK-721 (SHP-621)	budesonide	Takeda	corticosteroid	Eosinophilic esophagitis	PO	Filed NDA	03/20/2024	Yes	Yes
ITF-2357	givinostat	Italfarmaco S.p.A.	histone deacetylase inhibitor	Duchenne muscular dystrophy	PO	Filed NDA	03/21/2024	Yes	Yes
ACE-011	sotatercept	Merck	activin receptor type IIA-Fc fusion protein	Pulmonary arterial hypertension	SC	Filed BLA	03/26/2024	Yes	Yes
AKB-6548	vadadustat	Otsuka Pharmaceutical	hypoxia-inducible factor-prolyl hydroxylase inhibitor	Chronic kidney disease-related anemia	PO	Filed NDA	03/27/2024	No	No
LY-3002813	donanemab	Eli Lilly	beta-amyloid monoclonal antibody	Alzheimer's disease	IV	Filed BLA	03/2024	Yes	No
Opsynvi	macitentan/ tadalafil	Janssen	endothelin receptor antagonist/	Pulmonary arterial hypertension	PO	Filed NDA	03/30/2024	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
			phosphodiesterase 5 inhibitor						
REGN-1979	odronextamab	Regeneron	CD20/CD3 monoclonal antibody	Follicular lymphoma/ diffuse large b-cell lymphoma	IV	Filed BLA	03/31/2024	Yes	Yes
RP-L201	marnetegragene autotemcel	Rocket Pharmaceuticals	gene therapy	Leukocyte adhesion deficiency-I	IV	Filed BLA	03/31/2024	Yes	Yes
LTX-03	hydrocodone bitartrate/ acetaminophen	Acura Pharmaceuticals	opioid analgesic	Pain	PO	Filed NDA	1Q2024	No	No
Zeftera	ceftobiprole	Basilea	cephalosporin antibiotic	Bacterial infections	IV	Filed NDA	04/03/2024	No	No
SPN-830	apomorphine	Supernus Pharmaceuticals	non-ergoline dopamine agonist	Parkinson's disease	SC infusion	Filed NDA	04/05/2024	Yes	No
ALT-803	nogapendekin alfa inbakicept	ImmunityBio	interleukin-15 (IL-15) super agonist/ IL-15R alpha-Fc fusion complex	Bladder cancer	Intravesical	Filed BLA	04/23/2024	Yes	No
pivmecillinam	pivmecillinam	Utility Therapeutics	amidinopenicillin	Urinary tract infections	PO	Filed NDA	04/24/2024	No	No
Ingrezza oral granules	valbenazine	Neurocrine Bioscience	vesicular monoamine transporter 2 inhibitor	Tardive dyskinesia/ Huntington's disease	PO	Filed NDA	04/24/2024	Yes	Yes
PF-06838435 (SPK-9001)	fidanacogene elaparvovec	Pfizer/ Spark Therapeutics	gene therapy	Hemophilia B	IV	Filed BLA	04/27/2024	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
X4P-001 (X-4P-001, X4-136, X4P-001-RD)	mavorixafor	X4 Pharma	CXC receptor type 4 inhibitor	WHIM syndrome	PO	Filed NDA	04/30/2024	Yes	Yes
mRNA-1345	mRNA-1345	Moderna	vaccine	Respiratory syncytial virus	IM	Filed BLA	04/2024	No	No
DAY-101	tovorafenib	Day One Biopharmaceuticals	pan-Raf kinase inhibitor	Brain cancer	PO	Filed NDA	05/12/2024	Yes	Yes
TransCon PTH	palopegteriparatide	Ascendis Pharma	parathyroid hormone	Hypoparathyroidism	SC	Filed NDA	05/14/2024	Yes	Yes
SHR-1210	camrelizumab	Elevar Therapeutics	programmed death receptor-1-blocking antibody	Hepatocellular carcinoma	IV	Filed BLA	05/14/2024	Yes	Yes
YN-96D1	rivoceranib (apatinib)	Elevar Therapeutics	vascular endothelial growth factor receptor antagonist	Hepatocellular carcinoma	PO	Filed NDA	05/16/2024	Yes	Yes
EB-101	prademagene zamikeracel	Abeona Therapeutics	gene therapy	Epidermolysis Bullosa	TOP	Filed BLA	05/25/2024	Yes	Yes
GFT-505	elafibranor	Ipsen/ Genfit	selective peroxisome proliferator-activated receptor modulator	Primary biliary cholangitis	PO	Filed NDA	06/10/2024	Yes	Yes
BBI-4000	sofpironium bromide	Botanix Pharmaceuticals	anticholinergic	Hyperhidrosis	TOP	Filed NDA	06/10/2024	No	No
AMG-757	tarlatamab	Amgen	bispecific T cell engager antibody	Small cell lung cancer	IV	Filed BLA	06/12/2024	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
GRN-163L	imetelstat	Geron	telomerase inhibitor	Myelodysplastic syndrome	IV	Filed NDA	06/16/2024	Yes	Yes
V-116	pneumococcal conjugate vaccine	Merck	vaccine	Pneumococcal vaccine	IM	Filed BLA	06/17/2024	No	No
arimoclomol	arimoclomol	Zevra Therapeutics	cytoprotectives	Niemann-Pick disease	PO	Filed NDA	06/21/2024	Yes	Yes
RPL-554	ensifentrine	Verona Pharma	phosphodiesterase-3 and phosphodiesterase-4 inhibitor	Chronic obstructive pulmonary disease	INH	Filed NDA	06/26/2024	No	No
HER3-DXd	patritumab deruxtecan	Daiichi Sankyo	antibody drug conjugate	Non-small cell lung cancer	IV	Filed BLA	06/26/2024	Yes	No
ALXN-2040	danicopan	AstraZeneca	complement factor D inhibitor	Paroxysmal nocturnal hemoglobinuria	PO	Filed NDA	2Q2024	Yes	Yes
Tecentriq SC	atezolizumab	Roche	programmed death-ligand 1 blocking antibody	Cancers (mirroring indications to IV formulation)	SC	CRL	2Q2024	Yes	No
BT-595	immune globulin	Biotest	immune globulin	Primary immunodeficiency	IV	Filed BLA	06/29/2024	Yes	No
LAI-287	insulin icodec	Novo Nordisk	ultra-long-acting basal insulin	Diabetes mellitus	SC	Filed BLA	1H2024	No	No
BGB-A317 (BGB-A-317)	tislelizumab	BeiGene	programmed death-1 inhibitor	Esophageal squamous cell carcinoma	IV	Filed BLA	1H2024	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
OMS-721	narsoplimab	Omeros	anti-MASP-2 monoclonal antibody	Hematopoietic stem cell transplant-associated thrombotic microangiopathy	IV	CRL	Mid-2024	Yes	Yes
I/Ontak	denileukin diftitox	Citius	CD25-directed cytotoxin	Cutaneous T-cell lymphoma	IV	CRL	Mid-2024	Yes	Yes
Lydolyte	lidocaine	MEDRx	anesthetic agent	Neuropathic pain	TOP	CRL	Mid-2024	No	No
RG-6107	crovalimab	Roche	C5 inhibitor	Paroxysmal nocturnal hemoglobinuria	IV/SC	Filed BLA	07/07/2024	Yes	Yes
ALPHA-1062	galantamine prodrug	Alpha Cognition	acetylcholinesterase inhibitor	Alzheimer's disease	PO	Filed NDA	07/27/2024	No	No
ADP-A2M4 (MAGE-A4)	afamitresgene autoleucel	Adaptimmune	SPEAR T-cell therapy	Sarcoma	IV	Filed BLA	08/06/2024	Yes	Yes
CTP-543	deuruxolitinib	Sun Pharma	janus kinase inhibitor	Alopecia areata	PO	Filed NDA	08/06/2024	Yes	No
IPX-203	carbidopa/ levodopa	Amneal	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	PO	Filed NDA	08/08/2024	No	No
Humacyl	human acellular vessel	Humacyte	cellular therapy	End-stage renal disease	Implant	Filed BLA	08/12/2024	Yes	No
MDMA	midomafetamine	MAPS Public Benefit Corporation	psychoactive drug	Post-traumatic stress disorder	PO	Filed NDA	08/12/2024	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
MBX-8025 (RWJ-800025)	seladelpar	CymaBay Therapeutics	peroxisome proliferator-activated receptor delta agonist	Primary biliary cholangitits	PO	Filed NDA	08/15/2024	Yes	Yes
ZP-1848	glepaglutide	Zealand Pharma	glucagon peptide-2 agonist	Short bowel syndrome	SC	Filed NDA	08/22/2024	Yes	Yes
JNJ-1937	lazertinib	Janssen	kinase inhibitor	Non-small cell lung cancer	PO	Filed NDA	08/22/2024	Yes	No
SNDX-6352	axatilimab	Syndax Pharmaceuticals	colony stimulating factor 1 receptor monoclonal antibody	Graft vs. host disease	IV	Filed BLA	08/28/2024	Yes	Yes
SNDX-5613	revumenib	Syndax	Menin-mixed lineage leukemia 1 inhibitor	Acute myelogenous leukemia	PO	Filed NDA	08/29/2024	Yes	Yes
AT-007	govorestat	Applied Therapeutics	aldose reductase inhibitor	Galactosemia	PO	Filed NDA	08/2024	Yes	Yes
REGN-5458	linvoseltamab	Regeneron	BCMA and CD3 bispecific antibody inhibitor	Multiple myeloma	IV	Filed BLA	08/2024	Yes	No
LY-686017	tradipitant	Vanda Pharmaceuticals	neurokinin 1 receptor antagonist	Gastroparesis	PO	Filed NDA	09/18/2024	No	No
KarXT	xanomeline/ trospium	Karuna Therapeutics	muscarinic acetylcholine receptor agonist/ muscarinic receptor antagonist	Schizophrenia	PO	Filed NDA	09/26/2024	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
VYD222	VYD222	Invivyd	Monoclonal antibody	Prevention of COVID-19	IV	Filed BLA	3Q2024	No	No
IBI-1000	acetylleucine	IntraBio	modified amino acid	Niemann-Pick Disease type C	PO	Filed NDA	09/2024	Yes	Yes
Leqembi SC	lecanemab	Eisai/Biogen	beta-amyloid targeted therapy	Alzheimer's disease	SC	InTrial	3Q2024	Yes	No
OX-125	nalmefene	Orexo	opioid receptor antagonist	Opioid use disorder	Intranasal	InTrial	3Q2024	No	No
LY-03010	paliperidone	Luye Pharma	atypical antipsychotic	Schizophrenia	IM	Filed NDA	10/09/2024	No	No
PF-06741086	marstacimab	Pfizer	tissue factor pathway inhibitor	Hemophilia	IV/SC	Filed BLA	10/11/2024	Yes	Yes
CAM-2029	octreotide	Camurus	somatostatin analogue	Acromegaly	SC	Filed NDA	10/21/2024	Yes	Yes
Trogarzo	ibalizumab-uiyk	Theratechnologies	viral entry inhibitor	Maintenance treatment of HIV	IM	Filed sBLA	11/2/2024	No	No
BH-009	docetaxel	Zhuhai Beihai Biotechnology	microtubule inhibitor	Breast cancer/ non-small cell lung cancer/ prostate cancer/ gastric cancer	IV	Filed NDA	11/03/2024	Yes	No
DFD-29	minocycline	Journey Medical/ Dr. Reddy's	tetracycline	Rosacea	PO	Filed NDA	11/05/2024	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
Obe-cel	obecabtagene autoleucel	Autolus Therapeutics	autologous chimeric antigen receptor T-cells	Acute lymphoblastic leukemia	IV	Filed BLA	11/16/2024	Yes	Yes
AG-10 (AG10)	acoramidis	BridgeBio	tetrameric transthyretin stabilizer	Transthyretin amyloid cardiomyopathy	PO	Filed NDA	11/29/2024	Yes	No
CSL-312	garadacimab	CSL Limited	anti-factor XIIa monoclonal antibody	Hereditary angioedema	SC	Filed BLA	12/14/2024	Yes	Yes
PTC-AADC	eladocagene exuparvovec	PTC Therapeutics	gene therapy	Aromatic L-amino acid decarboxylase deficiency	Intracerebral	InTrial	4Q2024	Yes	Yes
STS-101	dihydroergotamine	Satsuma Pharmaceuticals	ergotamine	Migraine	Intranasal	CRL	4Q2024	No	No
sulopenem	sulopenem	Iterum Therapeutics	carbapenem	Urinary tract infections	PO	CRL	4Q2024	No	No
IONIS-APOCIII-LRx (ISIS-678354)	olezarsen	Ionis	antisense drug	Familial chylomicronemia syndrome	SC	InTrial	4Q2024	Yes	No
PB-2452	bentracimab	SFJ Pharmaceuticals	antiplatelet monoclonal antibody	Antiplatelet drug toxicity	IV	InTrial	2H2024	No	No
AXS-07	meloxicam/rizatriptan	Axsome Therapeutics	non-steroidal anti-inflammatory drug/triptan	Migraine	PO	CRL	2H2024	No	No
OX-124	naloxone	Orexo	opioid antagonist	Opioid overdose	Intranasal	CRL	2H2024	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
ZW-25	zanidatamab	Jazz Pharmaceuticals	HER2 monoclonal antibody	Biliary tract cancer	IV	InTrial	2H2024	Yes	Yes
ADI-PEG20	pegargiminase	Polaris	pegylated arginine deiminase	Mesothelioma	IM	InTrial	2H2024	Yes	Yes
CK-301	cosibelimab	Checkpoint Therapeutic	anti programmed cell death ligand 1	Cutaneous squamous cell carcinoma	IV	CRL	2H2024	Yes	No
Dasynoc	dasatinib	Xspray Pharma	kinase inhibitor	Chronic myeloid leukemia	PO	CRL	2H2024	Yes	Yes
CUTX-101	copper histidinate	Fortress Biotech	copper replacement	Menkes Disease	SC	InTrial	2H2024	Yes	Yes
RG-6058	tiragolumab	Roche	TIGIT monoclonal antibody	Non-small cell lung cancer/ esophageal cancer	IV	InTrial	2H2024	Yes	No
ARS-1	epinephrine	ARS Pharmaceuticals	non-selective alpha/ beta-adrenergic receptor agonist	Anaphylaxis	Intranasal	CRL	2H2024	No	No
F-901318	olorofim	F2G	orotomide antifungal	Aspergillosis	PO/IV	CRL	2H2024	No	Yes
XMT-1536	upifitamab rilsodotin	Mersana Therapeutics	antibody-drug conjugate	Ovarian cancer	IV	InTrial	2H2024	Yes	No
HP-5000	diclofenac	Hisamitsu Pharmaceutical	non-steroidal anti-inflammatory drug	Osteoarthritis	Transdermal	InTrial	2H2024	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
TC-002	latanoprost	TearClear	prostaglandin analog	Glaucoma	OPH	InTrial	2H2024	No	No
AG-881	vorasidenib	Servier	isocitrate dehydrogenase-1 and -2 inhibitor	Brain cancer	PO	InTrial	2H2024	Yes	Yes
UX-111 (ABO-102)	UX-111	Ultragenyx Pharmaceutical	gene therapy	Sanfilippo syndrome type A	IV	InTrial	2H2024	Yes	Yes
Oral semaglutide (weight loss)	semaglutide	Novo Nordisk	glucagon-like peptide 1 receptor agonist	Chronic weight management	PO	InTrial	2H2024	No	No
Multikine	leukocyte interleukin (CS-001P3)	CEL-SCI	immunomodulator	Head and Neck cancer	SC	InTrial	2024	Yes	Yes
NRX-101 (Cyclurad)	d-cycloserine/ lurasidone	NeuroRx	N-methyl-D-aspartate receptor modulator/ 5-HT2A receptor antagonist	Bipolar disorder	PO	InTrial	2024	No	No
Hepcludex	bulevirtide	Gilead	HBV receptor binder	Hepatitis delta virus	SC	CRL	2024	Yes	Yes
MILR-1444A	lebrikizumab	Eli Lilly	interleukin-13 inhibitor	Atopic dermatitis	SC	CRL	2024	Yes	No
MT-7117	dersimelagon	Mitsubishi Tanabe Pharma	undisclosed	Erythropoietic protoporphyria	PO	InTrial	2024	Yes	No
MOR-202	felzartamab	I-Mab	anti-CD38 monoclonal antibody	Multiple myeloma	IV	InTrial	2024	Yes	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension; interstitial lung disease	INH	Tentative Approval	2024	Yes	No
Risvan	risperidone	Laboratorios Farmacéuticos Rovi	atypical antipsychotic	Schizophrenia	IM	CRL	2024	Yes	No
DS-100	dehydrated alcohol	Eton	undisclosed	Methanol poisoning	SC	CRL	2024	No	Yes
ABBV-951	foscarbidopa/ foslevodopa	AbbVie	aromatic amino acid decarboxylation inhibitor/ aromatic amino acid	Parkinson's disease	SC	CRL	2024	Yes	No
ITCA-650 (sustained release exenatide)	exenatide sustained-release	Intarcia	glucagon-like peptide-1 receptor agonist	Diabetes mellitus	SC implant	Filed NDA	2024	No	No
RG-7433 (ABT-263)	navitoclax	AbbVie	Bcl-2 inhibitor	Myelofibrosis	PO	InTrial	2024	Yes	Yes
Iomab-B	iodine I 131 monoclonal antibody BC8	Actinium	anti-CD45 monoclonal antibody	Acute myeloid leukemia	IV	InTrial	2024	Yes	Yes
Dihydroergotamine autoinjector	dihydroergotamine	Amneal Pharmaceuticals	ergot derivative	Migraine	SC	InTrial	2024	No	No
D-PLEX100	doxycycline	PolyPid	tetracycline	Surgical site infections	IMPLANT	InTrial	2024	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
DS-1062	datopotamab deruxtecan	Daiichi Sankyo/ AstraZeneca	trop-2 antibody-drug conjugate	Non-small cell lung cancer; breast cancer	IV	InTrial	2024	Yes	No
AZD-5156	AZD-5156	AstraZeneca	monoclonal antibody	COVID-19	IM	InTrial	2024	TBD	No
nemolizumab	nemolizumab	Galderma	interleukin-31 receptor antagonist	Atopic dermatitis	SC	InTrial	Late 2024	Yes	No
iMAB-362	zolbetuximab	Astellas	GC182 monoclonal antibody	Gastric cancer	IV	CRL	Late 2024	Yes	Yes
APN-311	dinutuximab beta	Recordati	anti-GD2 antigen	Neuroblastoma	IV	InTrial	Late 2024	Yes	Yes
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	CRL	Late 2024	Yes	Yes
RP-L102 (RPL-102)	RP-L102	Rocket Pharmaceuticals	gene therapy	Fanconi anemia	IV	InTrial	Late 2024	Yes	Yes
AEB-1102	pegzilarginase	Aeglea BioTherapeutics	enzyme replacement/ arginase-I stimulator	Arginase 1 deficiency	IV	InTrial	Late 2024	Yes	Yes
SLS-001 (WT-1)	galinpepimut-S	Sellas Life Sciences Group	vaccine	Acute myeloid leukemia	SC	InTrial	Late 2024	Yes	Yes
Ovastat	treosulfan	Medexus Pharmaceuticals	alkylating agent	Hematopoietic stem cell transplantation	IV	InTrial	Late 2024	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
MT-1621	deoxythymidine/ deoxycytidine	UCB	deoxynucleoside	Thymidine kinase 2 deficiency	PO	InTrial	Late 2024	Yes	Yes
NN-7415	concizumab	Novo Nordisk	anti-tissue factor pathway inhibitor	Hemophilia A and hemophilia B	SC	CRL	Late 2024	Yes	Yes
RG-1594	ocrelizumab	Genentech	CD20-directed cytolytic antibody	Multiple sclerosis	SC	InTrial	Late 2024	Yes	No
NBI-74788	crinecerfont	Neurocrine Biosciences	CRF receptor antagonist	Congenital adrenal hyperplasia	PO	InTrial	Late 2024	Yes	Yes
2025 Possible launch date									
S-217622	ensitrelvir fumaric acid	Shionogi	Protease inhibitor	COVID-19 treatment	PO	InTrial	1Q2025	No	No
EBV-CTL (ATA-129)	tabelecleucel	Atara Biotherapeutics	cell therapy	Lymphoproliferative disorder	IV	InTrial	1Q2025	Yes	Yes
AXS-14	S-reboxetine	Axsome Therapeutics	selective noradrenaline reuptake inhibitor	Fibromyalgia	PO	InTrial	1Q2025	No	No
PDP-716	brimonidine	Visiox Pharma	alpha-2 agonist	Glaucoma	OPH	Not Approved	1Q2025	No	No
PD-0325901	mirdametinib	SpringWorks Therapeutics	MEK 1/2 inhibitor	Neurofibromatosis	PO	InTrial	1Q2025	Yes	Yes
UGN-102	mitomycin	UroGen	alkylating drug	Bladder cancer	Intravesical	InTrial	1Q2025	Yes	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
Sarconeos	BIO-101	Biophytis	MAS G-protein coupled receptor agonist	COVID-19 treatment	PO	InTrial	1H2025	No	No
NS-2 (ALDX-1E1, ADX-102)	reproxalap	Aldeyra Therapeutics	aldehyde antagonist	Dry eye disease	OPH	CRL	1H2025	No	No
RTT-01	tiratricol	Egetis Therapeutics	thyroid-stimulating hormone receptor	Monocarboxylate transporter 8 deficiency	PO	InTrial	1H2025	Yes	Yes
Xinlay	atrasentan	Novartis	selective endothelin-A receptor antagonist	IgA nephropathy	PO	InTrial	1H2025	Yes	No
CORT-125134	relacorilant	Corcept Therapeutics	glucocorticoid receptor II antagonist	Cushing's syndrome	PO	InTrial	1H2025	Yes	Yes
DCCR	diazoxide choline controlled-release	Soleno Therapeutics	vasodilator	Prader-Willi syndrome	PO	InTrial	1H2025	Yes	Yes
cytisine	cytisine	Achieve Life Sciences	nicotinic acetylcholine receptor antagonist	Smoking cessation	PO	InTrial	1H2025	No	No
SPI-014	lanthanum dioxycarbonate	Unicycive	phosphate binder	Hyperphosphatemia	PO	InTrial	1H2025	No	No
NRX-100	ketamine	NeuroRx	NMDA antagonist	Depression	PO	InTrial	1H2025	No	No
OPT-302	sozinibercept	Opthea	dual VEGF-C and VEGF-D inhibitor	Wet age-related macular degeneration	Intravitreal	InTrial	1H2025	Yes	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
SEL-212	SVP-rapamycin/ pegsiticase	Selecta Biosciences/ 3SBio	synthetic vaccine particle/ enzyme combination	Gout	IV	InTrial	1H2025	Yes	No
KVD-900	sebetralstat	KalVista Pharmaceuticals	plasma kallikrein inhibitor	Hereditary angioedema	PO	InTrial	1H2025	Yes	Yes
LIB-003	lerodalcibep	LIB Therapeutics	PCSK9 inhibitor	Hypocholesteremia	SC	InTrial	1H2025	No	No
REN-001	mavodelpar	Reneo Pharmaceuticals	PPARd agonist	Primary mitochondrial myopathies	PO	InTrial	1H2025	Yes	Yes
VX-121/ tezacaftor/ deutivacaftor	vanzacaftor/ tezacaftor/ deutivacaftor	Vertex	CF transmembrane conductance modulators	Cystic fibrosis	PO	InTrial	1H2025	Yes	Yes
DCC-3014	vimseltinib	Deciphera	CSF1R inhibitor	Tenosynovial giant cell tumor	PO	InTrial	1H2025	Yes	No
VS-6063	defactinib	Verastem	focal adhesion kinase inhibitor	Ovarian cancer	PO	InTrial	1H2025	Yes	Yes
VS-6766	avutometinib	Verastem	RAF/MEK clamp	Ovarian cancer	PO	InTrial	1H2025	Yes	No
PAX-101	suramin	PaxMedica	unknown	trypanosomiasis	IV	InTrial	Mid-2025	No	No
SDN-037	difluprednate	Visiox	corticosteroid	Ocular inflammation/pain	OPH	InTrial	Mid-2025	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
GZ-402671 (SAR-402671)	venglustat (ibiglustat)	Sanofi	glucosylceramide synthase inhibitor	M2 Gangliosidosis	PO	InTrial	Mid-2025	Yes	Yes
K-127	pyridostigmine	Amneal	cholinesterase inhibitor	Myasthenia gravis	PO	InTrial	Mid-2025	No	No
ONS-5010	bevacizumab-vikg	Outlook Therapeutics	anti-VEGF antibody	Wet age-related macular degeneration	Intravitreal	CRL	Mid-2025	Yes	No
AR-15512	AR-15512	Aerie Pharmaceuticals	TRPM8 agonist	Dry eye disease	OPH	InTrial	Mid-2025	No	No
ALZ-801	tramiprosate	Alzheon	amyloid beta-protein inhibitor	Alzheimer's disease	PO	InTrial	Mid-2025	Yes	No
SB-525	giroctocogene fitelparvovec	Pfizer/ Sangamo Therapeutics	gene therapy	Hemophilia A	IV	InTrial	Mid-2025	Yes	Yes
GSK-3511294	depemokimab	GlaxoSmithKline	interleukin-5 antagonist	Eosinophilic asthma	SC	InTrial	Mid-2025	Yes	No
LOU-064	remibrutinib	Novartis	Bruton's tyrosine kinase inhibitor	Chronic spontaneous urticaria	PO	InTrial	Mid-2025	Yes	No
ALVR-105	posoleucel	AlloVir	allogeneic T-cell therapy	BK viremia	IV	InTrial	Mid-2025	Yes	Yes
ARO-APOC3	plozasiran	Arrowhead Pharmaceuticals	RNAi targeting apolipoprotein C-III	Familial chylomicronemia syndrome	SC	InTrial	Mid-2025	Yes	Yes
RGX-121	RGX-121	Regenxbio	gene therapy	Mucopolysaccharidosis Type II	Intracisternal	InTrial	Mid-2025	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
PTC-923	sepiapterin	PTC Therapeutics	phenylalanine hydroxylase activator	Phenylketonuria	PO	InTrial	Mid-2025	Yes	Yes
RP-1	vusolimogene oderparepvec	Replimune	oncolytic immunotherapy	Cutaneous skin cell cancer	Intratumoral	InTrial	Mid-2025	Yes	No
AT-527	bemnifosbuvir	Atea Pharmaceuticals	HCV NS5B polymerase inhibitor	Treatment of COVID-19	PO	InTrial	Mid-2025	No	No
AGEN-1181	botensilimab	Agenus	anti-CTLA-4 antibody	Colorectal cancer	IV	InTrial	Mid-2025	Yes	No
ICP-022	orelabrutinib	InnoCare	Bruton's tyrosine kinase inhibitor	Mantle cell lymphoma	PO	InTrial	Mid-2025	Yes	Yes
VX-548	suzetrigine	Vertex	selective NaV1.8 inhibitor	Pain	PO	InTrial	Mid-2025	No	No
INO-3107	INO-3107	Inovio Pharmaceuticals	immunotherapy	Recurrent respiratory papillomatosis	IM	InTrial	Mid-2025	Yes	Yes
ANB-019	imsidolimab	AnaptysBio	interleukin-36 receptor antagonist	Generalized pustular psoriasis	IV	InTrial	3Q2025	Yes	Yes
resiniferatoxin	resiniferatoxin	Sorrento Therapeutics	TRPV-1 inhibitor	Osteoarthritis pain/ cancer pain	Intrathecal/ Intraarticular	InTrial	4Q2025	TBD	Yes
Donesta	estetrol	Mithra Pharmaceuticals	estrogen	Vasomotor symptoms	PO	InTrial	4Q2025	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
SPR-001	tildacerfont	Spruce Biosciences	corticotropin-releasing factor type-1 receptor antagonist	Congenital adrenal hyperplasia	PO	InTrial	2H2025	Yes	Yes
Tonmya	cyclobenzaprine	Tonix	muscle relaxant	Fibromyalgia	PO	InTrial	2H2025	No	No
GSK-2140944	gepotidacin	GlaxoSmithKline	bacterial Type II topoisomerase inhibitor	Bacterial infections	PO/IV	InTrial	2H2025	No	No
IdeS (immunoglobulin G-degrading enzyme of Streptococcus pyogenes)	imlifidase	Hansa Medical	bacterial enzyme	Kidney transplant	IV	InTrial	2H2025	Yes	Yes
RPC-4046 (ABT-308)	cendakimab	Bristol Myers Squibb	interleukin-13 inhibitor	Eosinophilic esophagitis	SC	InTrial	2H2025	Yes	Yes
BAY-342	elinzanetant	Bayer	neurokinin-1,3 receptor antagonist	Vasomotor symptoms	PO	InTrial	2H2025	No	No
CK-274	aficamten	Cytokinetics	cardiac myosin inhibitor	Obstructive hypertrophic cardiomyopathy	PO	InTrial	2H2025	Yes	Yes
CPI-0610	pelabresib	MorphoSys	BET inhibitor	Myelofibrosis	PO	InTrial	2H2025	Yes	Yes
CTX-1301	dexmethylphenidate	Cingulate	CNS stimulant	Attention deficit hyperactivity disorder	PO	InTrial	2H2025	TBD	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
XEN-1101	XEN-1101	Xenon Pharmaceuticals	Kv7 potassium channel opener	Focal epilepsy	PO	InTrial	2H2025	TBD	No
CRN-00808	paltusotine	Crinetics Pharmaceuticals	somatostatin receptor 2 agonist	Acromegaly	PO	InTrial	2H2025	Yes	Yes
AQST-109	epinephrine	Aquestive Therapeutics	non-selective alpha/ beta-adrenergic receptor agonist	Anaphylaxis	PO	InTrial	2H2025	No	No
XS-003	nilotinib	Xspray Pharma	kinase inhibitor	Chronic myeloid leukemia	PO	InTrial	2H2025	Yes	No
TransCon CNP	navepegritide	Ascendis Pharma	C-type natriuretic peptide	Achondroplasia	SC	InTrial	2H2025	Yes	Yes
mRNA-1083	influenza and COVID-19 vaccine	Moderna	mRNA	Prevention of influenza and COVID-19	IM	InTrial	2025	No	No
BNT161+BNT162b2	influenza and COVID-19 vaccine	Pfizer/BioNTech	mRNA	Prevention of influenza and COVID-19 infection	IM	InTrial	2025	No	No
ND-0612H	levodopa/ carbidopa	Mitsubishi Tanabe/ NeuroDerm	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	SC	InTrial	2025	Yes	No
M-281	nipocalimab	J&J	IgG1 antibody	Warm autoimmune hemolytic anemia/ generalized myasthenia gravis	IV	InTrial	2025	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
P2B-001	pramipexole/ rasagiline	Pharma Two B	dopamine agonist/ monoamine oxidase B inhibitor	Parkinson's disease	PO	InTrial	2025	No	No
FCX-007 (GM-HDF-COL7, INXN-3002)	dabocemagene autoficel	Castle Creek Pharmaceutical	gene-modified autologous fibroblast	Epidermolysis bullosa	Intradermal	InTrial	2025	Yes	Yes
pIL-12 (DNA IL-12)	tavokinogene telsaplasmid	OncoSec Medical	gene therapy	Melanoma	Intratumoral	InTrial	2025	Yes	Yes
ALN-APC (ALN-AT3)	fitusiran	Sanofi/ Alnylam	RNAi therapeutic	Hemophilia A and B	SC	InTrial	2025	Yes	Yes
AXS-12	reboxetine	Axsome Therapeutics	norepinephrine reuptake inhibitor	Narcolepsy	PO	InTrial	2025	No	Yes
EB-1020	centanafadine	Otsuka	norepinephrine, dopamine and serotonin reuptake inhibitor	Attention deficit hyperactivity disorder	PO	InTrial	2025	No	No
MSC-2364447 (M-2951)	evobrutinib	Merck	Bruton tyrosine kinase inhibitor	Multiple sclerosis	PO	InTrial	2025	Yes	No
PF-06939926	fordadistrogene movaparvovec	Pfizer	gene therapy	Duchenne muscular dystrophy	IV	InTrial	2025	Yes	Yes
SAR-442168	tolebrutinib	Sanofi	Bruton’s tyrosine kinase inhibitor	Multiple sclerosis	PO	InTrial	2025	Yes	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
ASP-1929 (RM-1929)	ASP-1929	Rakuten	EGFR inhibitor	Head and neck squamous cell carcinoma	IV	InTrial	2025	Yes	No
PRN-1008	rilzabrutinib	Sanofi	BTK inhibitor	Immune thrombocytopenia	PO	InTrial	2025	No	Yes
SPK-8011	dirloctocogene samoparvovec	Roche/ Spark Therapeutics	gene therapy	Hemophilia	IV	InTrial	2025	Yes	Yes
PXT-3003	baclofen/ naltrexone/ sorbitol	Pharnext	GABA-ergic agonist/ opioid receptor antagonist/ sorbitol combination	Charcot-Marie Tooth disease	PO	InTrial	2025	No	Yes
CF-101	piclidenoson	Can-Fite BioPharma	A3 adenosine receptor agonist	Plaque psoriasis	PO	InTrial	2025	Yes	No
CNM-Au8	CNM-Au8	Clene	gold nanocrystal	Amyotrophic lateral sclerosis	PO	InTrial	2025	Yes	Yes
KN-035	envafolimab	TRACON Pharmaceuticals	programmed death-ligand 1 inhibitor	Sarcoma	SC	InTrial	2025	Yes	Yes
Mino-Lok	minocycline-EDTA-ETOH	Citrus	tetracyclines	Bacterial infection	Intracatheter	InTrial	2025	No	No
REGN-2477	garetosmab	Regeneron	Activin A antibody	Fibrodysplasia ossificans progressiva	IV/SC	InTrial	2025	Yes	Yes
GSK-2330672	linerixibat	GlaxoSmithKline	ileal bile acid transfer inhibitor	Primary biliary cholangitis	PO	InTrial	2025	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
TAK-935	soticlestat	Takeda	cholesterol 24-hydroxylase inhibitor	Lennox-Gastaut syndrome/ Dravet syndrome	PO	InTrial	2025	Yes	Yes
RG-6114	inavolisib	Roche	phosphatidylinositol 3-kinase alpha inhibitor	Breast cancer	PO	InTrial	2025	Yes	No
TAVT-45	abiraterone acetate	Tavanta Therapeutics	CYP17 inhibitor	Prostate cancer	PO	InTrial	2025	Yes	No
CT-053 (Zevor-cel)	CT-053	CARsgen Therapeutics	B-cell maturation antigen-directed genetically modified autologous T cell immunotherapy	Multiple myeloma	IV	InTrial	2025	Yes	Yes
CT-041	CT-041	CARsgen Therapeutics	chimeric antigen receptor T cell therapy	Gastric cancer	IV	InTrial	2025	Yes	Yes
ABBV-399	telisotuzumab	AbbVie	antibody (anti-c-Met)-drug conjugate	Non-small cell lung cancer	IV	InTrial	2025	Yes	No
MTX-005	MTX-005	Memo Therapeutics	monoclonal antibody	BKV infection	IV	InTrial	2025	TBD	No
PRGN-2012	PRGN-2012	Precigen	immunotherapy	Respiratory papillomatosis	SC	InTrial	2025	Yes	Yes
mRNA-1010	mRNA-1010	Moderna	vaccine	Influenza	IM	InTrial	2025	No	No

IM = intramuscular, INH = inhalation, INJ = injection, IUD = intrauterine device, IV = intravenous, OPH = ophthalmic, PO = oral, SC = subcutaneous, TOP = topical

Key pending indication forecast

Optum Rx key pending indication forecast

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Estimated Approval Date
Onivyde	irinotecan	Ipsen	topoisomerase inhibitor	Revised	In combination with fluorouracil/leucovorin and oxaliplatin as first-line treatment for metastatic pancreatic ductal adenocarcinoma	IV	02/13/2024
Ixinity	coagulation factor IX (recombinant)	Medexus Pharmaceuticals	human blood coagulation factor	Revised	On-demand, prophylactic, and perioperative treatment of pediatric patients under 12 years of age with hemophilia B	IV	02/15/2024
Tagrisso	osimertinib	AstraZeneca	kinase inhibitor	Revised	In combination with chemotherapy for the treatment of adult patients with locally advanced or metastatic epidermal growth factor receptor-mutated (EGFRm) non-small cell lung cancer	PO	02/16/2024
Rybrevant	amivantamab-vmjw	Janssen	bispecific EGF receptor-directed and MET receptor-directed antibody	New	In combination with chemotherapy (carboplatin-pemetrexed) for the first-line treatment of patients with locally advanced or metastatic non-small cell lung cancer with EGFR exon 20 insertion mutations	IV	02/28/2024
Hetlioz	tasimelteon	Vanda Pharmaceuticals	melatonin receptor agonist	New	Treatment of insomnia characterized by difficulties with sleep initiation	PO	03/04/2024
Livmarli	maralixibat	Mirum Pharmaceuticals	ileal bile acid transporter inhibitor	New	Treatment of pruritus in patients 2 years of age and older with progressive familial intrahepatic cholestasis	PO	03/14/2024

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Estimated Approval Date
Breyanzi	lisocabtagene maraleucel	Bristol Myers Squibb	CD19-directed genetically modified autologous T cell immunotherapy	Revised	Treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia or small lymphocytic lymphoma who received a prior Bruton tyrosine kinase inhibitor and B-cell lymphoma 2 inhibitor	IV	03/14/2024
Xhance	fluticasone	Optinose	corticosteroid	New	Treatment of chronic sinusitis	Intranasal	03/16/2024
Ofev	nintedanib	Boehringer Ingelheim	tyrosine kinase inhibitor	New	Treatment for children and adolescents between 6 to 17 years old with fibrosing interstitial lung disease	PO	03/25/2024
Xolair	omalizumab	Genentech	IgE antagonist	New	Reduction of allergic reactions, including anaphylaxis, that may occur with an accidental exposure to one or more foods in adult and pediatric patients aged 1 year and older with food allergy	SC	1Q2024
Brukinsa	zanubrutinib	BeiGene	kinase inhibitor	New	In combination with obinutuzumab for the treatment of adult patients with relapsed or refractory follicular lymphoma after at least two prior lines of therapy	PO	1Q2024
Wegovy	semaglutide	Novo Nordisk	glucagon-like peptide-1 receptor agonist	New	To reduce the risk of major adverse cardiovascular events in overweight and obese adults	SC	1Q2024
Nexletol	bempedoic acid	Esperion	adenosine triphosphate-citrate lyase inhibitor	New	To reduce the risk of cardiovascular events in statin intolerant patients	PO	04/01/2024
Fanapt	iloperidone	Vanda	atypical antipsychotic	New	Treatment of acute manic and mixed episodes associated with bipolar I disorder in adults	PO	04/02/2024

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Estimated Approval Date
Carvykti	ciltacabtagene autoleucel	J&J	B-cell maturation antigen-directed genetically modified autologous T cell immunotherapy	Revised	Treatment of relapsed and refractory multiple myeloma in patients with 1 to 3 prior lines of therapy	IV	04/05/2024
Elahere	mirvetuximab soravtansine-gynx	ImmunoGen	folate receptor alpha-directed antibody and microtubule inhibitor conjugate	Accelerated to Full Approval	Treatment of adult patients with FRα positive, platinum-resistant epithelial ovarian, fallopian tube, or primary peritoneal cancer, who have received one to three prior systemic treatment regimens	IV	04/05/2024
Opdivo	nivolumab	Bristol Myers Squibb	programmed death receptor-1-blocking antibody	Revised	In combination with cisplatin-based chemotherapy as a first-line treatment for adult patients with unresectable or metastatic urothelial carcinoma	IV	04/05/2024
NexoBrid	anacaulase-bcdb	MediWound	proteolytic enzymes	Revised	Eschar removal in pediatric patients with deep partial thickness and/or full thickness thermal burns	TOP	05/09/2024
Tivdak	tisotumab vedotin-tftv	Anika Therapeutics	tissue factor-directed antibody and microtubule inhibitor conjugate	Accelerated to Full Approval	Treatment of patients with recurrent or metastatic cervical cancer with disease progression on or after first-line therapy	IV	05/09/2024
Breyanzi	lisocabtagene maraleucel	Bristol Myers Squibb	CD19-directed genetically modified autologous T cell immunotherapy	New	Treatment of adult patients with relapsed or refractory follicular lymphoma	IV	05/23/2024
Edurant	rilpivirine	Janssen	non-nucleoside reverse transcriptase inhibitor	Revised	In combination with other antiretroviral agents for the treatment of HIV-1 infection in treatment-naïve patients 2 years of age and older and weighing at least 10 kg with HIV-1 RNA less than or equal to 100,000 copies/mL	PO	05/28/2024

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Estimated Approval Date
Enhertu	trastuzumab deruxtecan	AstraZeneca/ Daiichi Sankyo	HER2-directed antibody and topoisomerase inhibitor conjugate	New	Treatment of adult patients with unresectable or metastatic HER2-positive solid tumors who have received prior treatment or who have no satisfactory alternative treatment options	IV	05/29/2024
Breyanzi	lisocabtagene maraleucel	Bristol Myers Squibb	CD19-directed genetically modified autologous T cell immunotherapy	New	Treatment of relapsed or refractory mantle cell lymphoma	IV	05/31/2024
Arexvy	respiratory syncytial virus vaccine, adjuvanted	GSK	vaccine	Revised	Active immunization for the prevention of lower respiratory tract disease caused by respiratory syncytial virus in individuals 50 years of age and older	IM	06/07/2024
Kevzara	sarilumab	Sanofi	interleukin-6 receptor monoclonal antibody	New	Treatment of polyarticular juvenile idiopathic arthritis	SC	06/10/2024
Blincyto	blinatumomab	Amgen	bispecific CD19-directed CD3 T-cell engager	Revised	Treatment of early-stage, CD19-positive B-cell precursor acute lymphoblastic leukemia	IV	06/21/2024
Elevidys	delandistrogene moxeparvovec-rokl	Sarepta	gene therapy	Accelerated to Full Approval	Treatment of Duchenne muscular dystrophy patients with a confirmed mutation in the DMD gene	IV	06/22/2024
Skyrizi	risankizumab-rzaa	AbbVie	interluekin-23 inhibitor	New	Treatment of ulcerative colitis	SC	06/28/2024
Iclusig	ponatinib	Takeda	kinase inhibitor	Revised	Front line treatment of Ph+ acute lymphoblastic leukemia	PO	2Q2024
Abecma	idecabtagene vicleucel	Bristol Myers Squibb	B-cell maturation antigen-directed genetically modified autologous T cell immunotherapy	Revised	Treatment of adult patients with relapsed and refractory multiple myeloma who have received an immunomodulatory agent, a	IV	2Q2024

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Estimated Approval Date
					proteasome inhibitor, and an anti-CD38 monoclonal antibody		
Sirturo	bedaquiline	Janssen	diarylquinoline antimycobacterial drug	Accelerated to Full Approval	As part of combination therapy in adult and pediatric patients (5 years and older and weighing at least 15 kg) with pulmonary multi-drug resistant tuberculosis (MDR-TB). Reserve SIRTURO for use when an effective treatment regimen cannot otherwise be provided	PO	06/2024
Imfinzi	durvalumab	AstraZeneca	programmed death-ligand 1 blocking antibody	New	Adjuvant treatment of non-small cell lung cancer	IV	1H2024
Zoryve	roflumilast	Arcutis Biotherapeutics	phosphodiesterase-4 inhibitor	New	Treatment of mild-to-moderate atopic dermatitis in patients 6 years and older	TOP	07/07/2024
Voquezna	vonoprazan	Phathom Pharmaceuticals	potassium-competitive acid blocker	New	Treatment of heartburn associated with non-erosive gastroesophageal reflux disease in adults	PO	07/19/2024
Darzalex Faspro	daratumumab/ hyaluronidase-fihj	J&J	humanized anti-CD38 monoclonal antibody	Revised	In combination with bortezomib, lenalidomide and dexamethasone for induction and consolidation treatment and with lenalidomide for maintenance treatment of adult patients who are newly diagnosed with multiple myeloma (NDMM) and are eligible for autologous stem cell transplant	SC	07/30/2024
Rybrevant	amivantamab-vmjw	Janssen	bispecific EGF receptor-directed and MET receptor-directed antibody	Revised	In combination with lazertinib for the first-line treatment of adult patients with locally advanced or metastatic non-small cell lung cancer with epidermal growth factor receptor (EGFR) exon 19 deletions or L858R	IV	08/22/2024

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Estimated Approval Date
					substitution mutations, as detected by an FDA-approved test		
Alecensa	alectinib	Roche	kinase inhibitor	New	Treatment of postoperative adjuvant therapy for ALK fusion gene-positive non-small cell lung cancer	PO	09/2024
Wakix	pitolisant	Harmony Biosciences	histamine-3 receptor antagonist/inverse agonist	Revised	"Treatment of excessive daytime sleepiness or cataplexy in pediatric patients with narcolepsy	PO	3Q2024
Opdivo	nivolumab	Bristol Myers Squibb	programmed death receptor-1-blocking antibody	New	Neoadjuvant treatment with chemotherapy followed by surgery and adjuvant treatment for the perioperative treatment of resectable stage IIA to IIIB non-small cell lung cancer	IV	10/08/2024
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 inhibitor	New	Treatment of chronic obstructive pulmonary disease	SC	10/2024
Wegovy	semaglutide	Novo Nordisk	glucagon-like peptide-1 receptor agonist	New	Treatment of adults with heart failure with preserved ejection fraction and obesity	SC	11/2024

Extended generic and biosimilar pipline forecast

Optum Rx generic and biosimilar pipeline forecast

(Bolded fields are Biosimilar products)

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
2024 Possible launch date					
GIAZO	balsalazide disodium	Bausch Health	Ulcerative Colitis in Male Patients	Oral	2024
NEUPRO	rotigotine	UCB	Parkinson's Disease; Restless Legs Syndrome	External	2024
TEFLARO	ceftaroline fosamil	Allergan	Community Acquired Pneumonia; Skin and Skin Structure Infections	Intravenous	2024
VESICARE LS	solifenacin	Astellas	Neurogenic Detrusor Overactivity	Oral	1H-2024
NYMALIZE	nimodipine	Arbor	Subarachnoid Hemorrhage	Oral	1H-2024
TYSABRI	natalizumab	Biogen	Multiple Sclerosis; Crohn's Disease	Intravenous	1H-2024
NEULASTA ONPRO KIT	pegfilgrastim	Amgen/Insulet	Prophylaxis of Neutropenia in Cancer Patients	Subcutaneous	1Q-2024
ISENTRESS	raltegravir	Merck	Human Immunodeficiency Virus-1 Infection	Oral	04-2024
ACTEMRA	tocilizumab	Roche/Chugai	Juvenile Idiopathic Arthritis; Rheumatoid Arthritis; Giant Cell Arteritis; Cytokine Release Syndrome; Systemic Sclerosis-Associated Interstitial Lung Disease	Intravenous; subcutaneous	05-2024
RADICAVA	edaravone	Mitsubishi Tanabe	Amyotrophic Lateral Sclerosis	Intravenous	05-2024
DUAVEE	conjugated estrogens/bazedoxifene acetate	Pfizer/Ligand Pharmaceuticals	Treatment of Moderate to Severe Vasomotor Symptoms Associated with Menopause; Prevention of Postmenopausal Osteoporosis	Oral	05-2024
PROBUPHINE	buprenorphine	Titan Pharmaceuticals/Braeburn Pharmaceuticals	Maintenance Treatment of Opioid Dependence	Subdermal	06-2024
VICTOZA	liraglutide	Novo Nordisk	Type 2 Diabetes Mellitus (T2DM); Reduce the Risks of Cardiovascular Events in T2DM	Subcutaneous	06-2024
TWYNEO	tretinoin/benzoyl peroxide	Galderma	Acne Vulgaris	External	07-2024
SLYND	drospirenone	Exeltis/Insud	Prevention of Pregnancy	Oral	08-2024

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
OXTELLAR XR	oxcarbazepine	Supernus	Partial Seizures	Oral	09-2024
SPRYCEL	dasatinib	Bristol-Myers Squibb	Chronic Myeloid Leukemia; Acute Lymphoblastic Leukemia	Oral	09-2024
SUSTOL	granisetron	Heron Therapeutics	Chemotherapy-Induced Nausea and Vomiting	Subcutaneous	09-2024
PRIALT	ziconotide acetate	TerSera Therapeutics	Severe Pain	Intrathecal	10-2024
LAZANDA	fentanyl citrate	Depomed	Breakthrough Pain in Cancer Patients	Intranasal	10-2024
VUITY	pilocarpine	AbbVie	Presbyopia	Ophthalmic	10-2024
STENDRA	avanafil	Petros Pharmaceuticals	Erectile Dysfunction	Oral	10-2024
QSYMIA	phentermine/topiramate	Vivus	Chronic Weight Management	Oral	12-2024
SIKLOS	hydroxyurea	Addmedica/Medunik	Sickle Cell Anemia	Oral	12-2024
2025 Possible launch date					
GELNIQUE	oxybutynin	Allergan	Overactive Bladder	External	2025
BOSULIF	bosutinib	Pfizer	Chronic Myelogenous Leukemia	Oral	2025
COMPLERA	emtricitabine/rilpivirine/tenofovir disoproxil fumarate	Gilead/Janssen	Human Immunodeficiency Virus-1 Infection	Oral	2025
EYLEA	aflibercept	Regeneron	Wet Age-Related Macular Degeneration; Diabetic Macular Edema; Macular Edema Following Retinal Vein Occlusion; Diabetic Retinopathy in Patients with Diabetic Macular Edema; Retinopathy of Prematurity	Intravitreal	2025
NAMZARIC	memantine/donepezil	AbbVie	Moderate to Severe Dementia of the Alzheimer's Type	Oral	01-2025
TRACLEER	bosentan	Actelion/Janssen	Pulmonary Arterial Hypertension	Oral	01-2025
LEXETTE	halobetasol	Mayne	Plaque Psoriasis	External	01-2025
IZBA	travoprost	Alcon	Open-Angle Glaucoma; Ocular Hypertension	Ophthalmic	01-2025
STELARA	ustekinumab	Janssen	Plaque Psoriasis; Psoriatic Arthritis; Ulcerative Colitis; Crohn's Disease	Subcutaneous; intravenous	01-2025
HALAVEN	eribulin	Eisai	Metastatic Breast Cancer; Liposarcoma	Intravenous	01-2025
CORLANOR	ivabradine	Amgen	Heart Failure	Oral	01-2025
PHOSLYRA	calcium acetate	Fresenius	Phosphate Binder	Oral	01-2025

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
FINACEA	azelaic acid	LEO Pharma	Rosacea	External	01-2025
SANCUSO	granisetron	Kyowa Hakko Kirin/ProStrakan	Prevention of Nausea and Vomiting in Patients Receiving Moderately and/or Highly Emetogenic Chemotherapy	External	01-2025
PROLIA	denosumab	Amgen	Postmenopausal Osteoporosis; Bone Loss in Men and Women at Risk of Fracture	Subcutaneous	02-2025
XGEVA	denosumab	Amgen	Prevention of Fractures in Bone Malignancies and Multiple Myeloma; Giant Cell Tumor in Bone; Hypercalcemia	Subcutaneous	02-2025
XARELTO	rivaroxaban	Bayer/Janssen	Reduce the Risk of Stroke, Myocardial Infarction, Cardiovascular Events and Blood Clots; Prevention and Treatment of Deep Vein Thrombosis and Pulmonary Embolism	Oral	03-2025
SOLIRIS	eculizumab	AstraZeneca	Paroxsymal Nocturnal Hemoglobinuria; Hemolytic Uremic Syndrome; Myasthenia Gravis; Neuromyelitis Optica	Intravenous	03-2025
AURYXIA	ferric citrate	Keryx/Akebia Therapeutics	Control of Serum Phosphorus Levels in Chronic Kidney Disease (CKD) on Dialysis; Iron Deficiency Anemia in Adult Patients with CKD Not on Dialysis	Oral	03-2025
HORIZANT	gabapentin enacarbil	Arbor	Restless Legs Syndrome; Postherpetic Neuralgia	Oral	04-2025
JYNARQUE	tolvaptan	Otsuka	Polycystic Kidney Disease	Oral	04-2025
BRILINTA	ticagrelor	AstraZeneca	To Reduce the Risk of Cardiovascular Death, Myocardial Infarction (MI), and Stroke in Patients with Acute Coronary Syndrome, History of MI, Coronary Artery Disease, or Acute Ischemic Stroke or Transient Ischemic Attack	Oral	05-2025
APTIOM	eslicarbazepine	Sunovion/Bial	Partial-Onset Seizures	Oral	05-2025
TIROSINT-SOL	levothyroxine	IBSA Institut Biochemique	Hypothyroidism; Thyrotropin-Dependent Thyroid Cancer	Oral	05-2025
FYCOMPA	perampanel	Eisai	Partial-Onset Seizures; Primary Generalized Tonic-Clonic Seizures	Oral	05-2025
TASIGNA	nilotinib	Novartis	Philadelphia Chromosome-Positive Chronic Myeloid Leukemia	Oral	06-2025
NUCYNTA	tapentadol	Collegium	Moderate to Severe Acute Pain	Oral	06-2025
NUCYNTA ER	tapentadol	Collegium	Moderate to Severe Chronic Pain	Oral	06-2025

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
PERJETA	pertuzumab	Genentech	HER-2 Positive Breast Cancer	Intravenous	2H-2025
CARDENE IV	nicardipine	Chiesi	Short-Term Treatment of Hypertension When Oral Therapy is Not Possible	Intravenous	07-2025
RAVICTI	glycerol phenylbutyrate	Amgen	Urea Cycle Disorders	Oral	07-2025
RYANODEX	dantrolene	Eagle Pharmaceuticals	Malignant Hyperthermia	Intravenous	07-2025
RYTARY	carbidopa/levodopa	Impax/Amneal	Parkinson's Disease	Oral	07-2025
DIACOMIT	stiripentol	Biocodex	Dravet Syndrome	Oral	08-2025
ADZENYS XR-ODT	amphetamine polistirex	Neos Therapeutics	Attention Deficit Hyperactivity Disorder	Oral	09-2025
OFEV	nintedanib	Boehringer Ingelheim	Idiopathic Pulmonary Fibrosis; Systemic Sclerosis-Associated Interstitial Lung Disease (ILD); Chronic Fibrosing ILD	Oral	10-2025
QTERN	dapagliflozin/saxagliptin	AstraZeneca	Type 2 Diabetes Mellitus	Oral	10-2025
FUROSCIX	furosemide	scPharmaceuticals	Chronic Heart Failure	Subcutaneous	10-2025
EDURANT	rilpivirine	Janssen	Human Immunodeficiency Virus-1 Infection	Oral	10-2025
XOLAIR	omalizumab	Roche/Genentech	Asthma; Idiopathic Urticaria; Nasal Polyps	Intravenous	11-2025
TRADJENTA	linagliptin	Eli Lilly/Boehringer Ingelheim	Type 2 Diabetes Mellitus	Oral	11-2025
JENTADUETO XR	linagliptin/metformin	Boehringer Ingelheim/Eli Lilly	Type 2 Diabetes Mellitus	Oral	11-2025
JENTADUETO	linagliptin/metformin	Boehringer Ingelheim/Eli Lilly	Type 2 Diabetes Mellitus	Oral	11-2025
PICATO	ingenol mebutate	LEO Pharma	Actinic Keratosis	External	12-2025
OPSUMIT	macitentan	Janssen	Pulmonary Arterial Hypertension	Oral	12-2025
2026 Possible launch date					
BRYHALI	halobetasol	Bausch Health	Plaque Psoriasis	External	2026
MAVENCLAD	cladribine	Serono	Multiple Sclerosis	Oral	2026
ABILIFY MAINTENA	aripiprazole	Otsuka/Lundbeck	Schizophrenia; Bipolar Disorder	Intramuscular	2026
POMALYST	pomalidomide	Celgene	Multiple Myeloma; Kaposi Sarcoma	Oral	1Q-2026
YONSA	abiraterone	Sun	Prostate Cancer	Oral	01-2026
VELPHORO	sucroferric oxyhydroxide	Vifor Fresenius Medical Care Renal Pharma (VFMCRP)	Hyperphosphatemia In Patients with Chronic Kidney Disease on Dialysis	Oral	01-2026

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
BYVALSON	nebivolol/valsartan	AbbVie	Hypertension	Oral	01-2026
LUCEMYRA	lofexidine	US Worldmeds	Opioid Withdrawal Symptoms	Oral	01-2026
JEVTANA KIT	cabazitaxel	Sanofi	Hormone-Refractory Metastatic Prostate Cancer	Intravenous	01-2026
EDARBI	azilsartan kamedoxomil	Arbor	Hypertension	Oral	01-2026
SERNIVO	betamethasone dipropionate	Encore Dermatology	Plaque Psoriasis	External	01-2026
MYRBETRIQ	mirabegron	Astellas	Overactive Bladder; Neurogenic Detrusor Overactivity	Oral	01-2026
ELLA	ulipristal	Afaxys/Perrigo	Emergency Contraception	Oral	01-2026
TYVASO	treprostinil	United Therapeutics	Pulmonary Arterial Hypertension; Pulmonary Hypertension with Interstitial Lung Disease	Inhalation	01-2026
PROMACTA	eltrombopag	Novartis	Thrombocytopenia	Oral	01-2026
QBRELIS	lisinopril	Silvergate	Hypertension; Heart Failure; Acute Myocardial Infarction	Oral	01-2026
BRIVIACT	brivaracetam	UCB	Epilepsy	Oral; intravenous	02-2026
XELJANZ XR	tofacitinib	Pfizer	Rheumatoid Arthritis; Psoriatic Arthritis; Ulcerative Colitis; Ankylosing Spondylitis	Oral	2Q-2026
XELJANZ	tofacitinib	Pfizer	Rheumatoid Arthritis; Ulcerative Colitis; Psoriatic Arthritis; Juvenile Idiopathic Arthritis; Ankylosing Spondylitis	Oral	2Q-2026
NULOJIX	belatacept	Bristol-Myers Squibb	Prophylaxis of Organ Rejection in Kidney Transplant	Intravenous	04-2026
JANUVIA	sitagliptan	Merck	Type 2 Diabetes Mellitus	Oral	05-2026
JANUMET	sitagliptan/metformin	Merck	Type 2 Diabetes Mellitus	Oral	05-2026
EVOMELA	melphalan	Acrotech/Aurobindo	Multiple Myeloma; Conditioning for Stem Cell Transplant	Intravenous	06-2026
CERDELGA	eliglustat	Sanofi/Genzyme	Gaucher Disease Type 1	Oral	06-2026
SUPPRELIN LA	histrelin	Endo	Central Precocious Puberty	Subcutaneous	06-2026
COTEMPLA XR-ODT	methylphenidate	Neos Therapeutics	Attention Deficit Hyperactivity Disorder	Oral	07-2026
INJECTAFER	ferric carboxymaltose	American Regent/CSL Limited	Iron Deficiency Anemia	Intravenous	07-2026
JANUMET XR	sitagliptin/metformin	Merck	Type 2 Diabetes Mellitus	Oral	07-2026

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
NUEDEXTA	dextromethorphan/quinidine sulfate	Avanir	Pseudobulbar Affect	Oral	07-2026
COMETRIQ	cabozantinib (S)-malate	Exelixis	Medullary Thyroid Cancer	Oral	08-2026
ADEMPAS	riociguat	Bayer	Pulmonary Arterial Hypertension; Chronic Thromboembolic Pulmonary Hypertension	Oral	4Q-2026
UPTRAVI	selexipag	Janssen	Pulmonary Arterial Hypertension	Oral	10-2026
VEREGEN	sinecatechins	Sandoz	External Genital and Perianal Warts	External	10-2026
CYRAMZA	ramucirumab	Eli Lilly	Gastric Cancer; Gastroesophageal Cancer; Metastatic Gastric Cancer; Non-Small Cell Lung Cancer	Intravenous	10-2026
ADASUVE	loxapine	Alexza	Agitation Associated with Schizophrenia or Bipolar Disorder	Inhalation	10-2026
ILARIS	canakinumab	Novartis	Cryopyrin-Associated Periodic Syndromes; Familial Cold Autoinflammatory Syndrome; Muckle-Wells Syndrome; Tumor Necrosis Factor Receptor Associated Periodic Syndrome; Hyperimmunoglobulin D Syndrome/Mevalonate Kinase Deficiency; Familial Mediterranean Fever; Still's Disease; Gout Flares	Subcutaneous	10-2026
TRINTELLIX	vortioxetine	Takeda/Lundbeck	Major Depressive Disorder	Oral	12-2026
1st Half 2027 Possible launch date					
KYPROLIS	carfilzomib	Amgen	Multiple Myeloma	Intravenous	2027
CIMZIA	certolizumab pegol	UCB/Royalty Pharma	Psoriatic Arthritis; Rheumatoid Arthritis; Ankylosing Spondylitis; Crohn's Disease; Plaque Psoriasis; Axial Spondyloarthritis	Subcutaneous	2027
ENTRESTO	sacubitril/valsartan	Novartis	Heart Failure	Oral	2027
SAXENDA	liraglutide	Novo Nordisk	Chronic Weight Management	Subcutaneous	2027
IBRANCE	palbociclib	Pfizer	Breast Cancer	Oral	1Q-2027
MINOCIN	minocycline	Rempex/Melinta Therapeutics	Infections	Intravenous	01-2027
BONJESTA	doxylamine/pyridoxine	Duchesnay	Nausea and Vomiting Associated with Pregnancy	Oral	01-2027
DIFICID	fidaxomicin	Merck	Treatment of Clostridium difficile-Associated Diarrhea	Oral	01-2027
OSPHENA	ospemifene	Duchesnay	Menopause Symptoms; Dyspareunia	Oral	01-2027

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
BELEODAQ	belinostat	Acrotech/Aurobindo	Relapsed or Refractory Peripheral T-cell Lymphoma	Intravenous	01-2027
VIBATIV	telavancin	Cumberland	Infections	Intravenous	01-2027
CUBICIN RF	daptomycin	Merck	Complicated Skin and Skin Structure Infections; Staphylococcus aureus Bloodstream Infections	Intravenous	01-2027
ENVARSUS XR	tacrolimus	Veloxis	Prophylaxis of Organ Rejection in Kidney Transplant Patients	Oral	01-2027
RYDAPT	midostaurin	Novartis	Acute Myeloid Leukemia; Systemic Mastocytosis; Mast Cell Leukemia	Oral	01-2027
JUBLIA	efinaconazole	Bausch Health	Onychomycosis of the Toenail	External	01-2027
VALTOCO	diazepam	Neurelis	Epilepsy	Intranasal	01-2027
VIVITROL	naltrexone	Alkermes	Alcohol and/or Opioid Dependence	Intramuscular	01-2027
BELBUCA	buprenorphine	BioDelivery Sciences International	Severe Pain	Oral	01-2027
NATPARA	parathyroid hormone 1-84	Takeda	Hypoparathyroidism	Subcutaneous	01-2027
SUBSYS	fentanyl	BTcP Pharma	Breakthrough Pain in Cancer Patients	Oral	01-2027
ALTABAX	retapamulin	Aqua Pharmaceuticals/Almirall	Impetigo	External	02-2027
BYDUREON	exenatide	AstraZeneca	Type 2 Diabetes Mellitus	Subcutaneous	02-2027
VITEKTA	elvitegravir	Gilead	Human Immunodeficiency Virus-1 Infection	Oral	02-2027
TUDORZA PRESSAIR	aclidinium	AstraZeneca	Chronic Obstructive Pulmonary Disease	Inhalation	04-2027
DUAKLIR PRESSAIR	aclidinium/formoterol fumarate	AstraZeneca	Chronic Obstructive Pulmonary Disease	Inhalation	04-2027
RAPIVAB	peramivir	BioCryst	Treatment of Acute Uncomplicated Influenza	Intravenous	05-2027
LUMIGAN	bimatoprost	Allergan/AbbVie	Glaucoma; Ocular Hypertension	Ophthalmic	06-2027
ORENITRAM	treprostinil diethanolamine	Supernus/United Therapeutics	Pulmonary Arterial Hypertension	Oral	06-2027

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