RxOutlook®

3rd Quarter 2024

Optum Rx®

Welcome to the third 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 First Half 2024 - Where Are We Today?

As of August 5th, the FDA's Center for Drug Evaluation and Research (CDER) has approved 23 new molecular entities in 2024. Since the previous quarter's report, notable drug approvals have included: **Ohtuvayre™ (ensifentrine)**, a first-in-class phosphodiesterase 3 and 4 (PDE3/4) inhibitor for chronic obstructive pulmonary disease (COPD); **Kisunla™ (donanemab-azbt)**, the third amyloid beta-directed antibody for Alzheimer's disease; and **Leqselvi™ (deuruxolitinib)**, the third Janus kinase (JAK) inhibitor approved for alopecia areata.

Looking Ahead to 4Q 2024

In this edition of RxOutlook, we will be discussing 8 key products with an approval decision by the end of the 4th quarter 2024. This includes 5 drugs with an Orphan Designation: marstacimab, acoramidis, olezarsen, garadacimab, and crinecerfont.

Marstacimab is a first-in-class, subcutaneously (SC) administered therapy for both hemophilia A and B. Marstacimab will be a competitor to factor replacement therapies for hemophilia A and B as well as Genentech's Hemlibra® (emicizumab-kxwh) for hemophilia A. **Acoramidis** would be the second transthyretin stabilizer approved for cardiomyopathy associated with transthyretin amyloidosis, and a direct competitor to Pfizer's Vyndamax® (tafamidis). **Garadacimab** is a first-in-class, SC administered therapy for prophylactic treatment of hereditary angioedema (HAE) and a competitor to other preventative HAE treatments such as Cinryze® (C1 esterase inhibitor), Takhzyro® (lanadelumab-flyo), and Orladeyo® (berotralstat).

Olezarsen would be the first therapy approved for familial chylomicronemia syndrome (FCS), an ultra-rare genetic disease that causes severely elevated triglyceride levels. Patients with FCS have poor response to conventional lipid-lowering therapies. **Crinecerfont**, would be the first non-steroidal treatment approved for congenital adrenal hyperplasia (CAH), a genetic disease in which the adrenal glands do not produce enough cortisol. Crinecerfont is intended to be used in combination with glucocorticoids.

Two drugs highlighted in the report are **inavolisib** and **datopotamab deruxtecan**, new oncology drugs: one for breast cancer and one for breast or non-small cell lung cancer. Both of these cancers are very common in the U.S. and these new drugs would provide additional therapies for patients with advanced and metastatic disease.

The other drug discussed in this report is **sulopenem etzadroxil/probenecid**, a new antibiotic for uncomplicated urinary tract infection. If approved, sulopenem etzadroxil would be the first oral carbapenem, a class of broad-spectrum antibiotics that have only been available via injection.

Approval decisions for other key novel therapies are expected by the end of the 4th quarter 2024 but are not reviewed in this report because they were covered in previous editions of RxOutlook. This includes: xanomeline/trospium for schizophrenia; and tradipitant for gastroparesis.

Key pipeline drugs with FDA approval decisions expected by the end of the 4th quarter 2024

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Marstacimab	Pfizer	Hemophilia A and B*	4Q 2024
Sulopenem etzadroxil/ probenecid	Iterum Therapeutics	Urinary tract infection	10/25/2024
Inavolisib	Genentech	Breast cancer	11/27/2024
Acoramidis	BridgeBio Pharma	Transthyretin amyloid cardiomyopathy*	11/29/2024
Olezarsen	Ionis Pharmaceuticals	Familial chylomicronemia syndrome*	12/19/2024
Datopotamab deruxtecan	Daiichi Sankyo/ AstraZeneca	Non-small cell lung cancer/breast cancer	12/20/2024 (NSCLC) 1/29/2025 (Breast cancer)
Garadacimab	CSL	Hereditary angioedema*	12/2024
Crinecerfont	Neurocrine Biosciences	Congenital adrenal hyperplasia*	12/29/2024

* 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 4th 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 4th 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

Marstacimab (Brand Name: To be determined)

Manufacturer: Pfizer
Regulatory designations: Orphan Drug, Fast Track
Expected FDA decision: 4Q 2024

Therapeutic use

Marstacimab is under review for prophylactic treatment to prevent or reduce the frequency of bleeding episodes in individuals with hemophilia A or hemophilia B without inhibitors.

Hemophilia is a genetic bleeding disorder in which the blood does not clot properly due to a clotting factor deficiency. People with hemophilia A or B have low levels of either factor VIII (FVIII) or factor IX (FIX), respectively. Hemophilia can lead to spontaneous bleeding as well as excessive bleeding following injuries or surgery. Additionally, bleeding within the joints can lead to chronic joint disease and pain.

As many as 33,000 males are estimated to be living with hemophilia in the U.S., and hemophilia A is three to four times as common as hemophilia B.

The most common treatment approach for hemophilia is factor replacement therapy, which replaces missing clotting factors. However, about 1 in 5 people with hemophilia A and about 3 in 100 people with hemophilia B will develop an inhibitor (or antibody) to factor replacement therapy and these patients typically require higher doses of factor for treatment, are twice as likely to be hospitalized for a bleeding complication, and they are at increased risk of death.

What you need to know:

Proposed Indication: To prevent or reduce the frequency of bleeding episodes in individuals with hemophilia A or hemophilia B without inhibitors

Mechanism: anti-TFPI

Efficacy: Annualized bleeding rate:

- Reduced by 35.2% vs. routine prophylaxis
- Reduced by 91.6% vs. on-demand therapy

Common AEs: Hemorrhage, hepatic disorder, hypersensitivity, hypertension, injection site reaction

Dosing: SC once weekly

Why it Matters: Novel mechanism of action, first SC administered treatment for hemophilia B, potential future use in patients with inhibitors

Important to Note: Alternatives available including SC administered Hemlibra for hemophilia A, lack of data in patients with inhibitors (expected later this year), potential future competition (eg, Novo Nordisk's concizumab, Sanofi's fitusiran)

Estimated Cost: ~$440,000 per year (based on pricing for Hemlibra)

Clinical profile

Marstacimab is a monoclonal antibody that targets tissue factor pathway inhibitor (TFPI), a natural anticoagulation protein that prevents the formation of blood clots.

Pivotal trial data:
The efficacy of marstacimab was evaluated in BASIS, a Phase 3, open-label study in 145 adolescent and adult participants ages 12 to < 75 years with severe hemophilia A or moderately severe to severe hemophilia B. The study compared the annualized bleeding rate (ABR) during a run-in period where patients received factor replacement therapy with a 12-month active treatment phase, during which patients received prophylactic marstacimab. During the run-in period, patients could receive factor replacement therapy as routine prophylaxis or on-demand therapy. The data currently available is limited to patients without inhibitors.

Marstacimab *(continued...)*

Compared to routine prophylaxis during the run-in period, treatment with marstacimab resulted in a 35.2% mean reduction (95% CI: 5.6, 55.6; p = 0.0376) in ABR over 12 months (mean of 7.85 to 5.08). Additionally, marstacimab significantly reduced ABR by 91.6% (95% CI: 88.1, 94.1; p < 0.0001) compared to on-demand therapy over 12 months (mean of 38.00 to 3.18).

Safety:
The most common adverse events with marstacimab use were hemorrhage, hepatic disorder, hypersensitivity, hypertension, and injection site reaction.

Dosing:
In the pivotal trial, marstacimab was administered subcutaneously (SC) once weekly.

Competitive environment

If approved, marstacimab would offer a first-in-class treatment option for the prophylactic treatment of both hemophilia A and B. The current standard of care includes intravenously (IV) administered factor replacement therapy, which can be used for both hemophilia A and B, and Genentech's Hemlibra® (emicizumab), a SC administered factor IXa- and factor X-directed antibody, which is used for hemophilia A. For hemophilia B, marstacimab would represent the first once weekly SC administered treatment and for hemophilia A it would provide a competitor to Hemlibra. Of note, Hemlibra is dosed once weekly to once monthly.

The data for marstacimab in both hemophilia A and B patients without inhibitors is promising and data in patients with inhibitors is expected later this year. While it represents a small population, hemophilia B patients with inhibitors are difficult to manage and there is an unmet need for additional treatment options.

Other non-factor replacement therapies are currently in development for hemophilia, including Novo Nordisk's anti-TFPI, concizumab, and Sanofi's RNAi therapeutic, fitusiran. Both products could be approved by the end of the first quarter 2025 and would be potential competitors to marstacimab.

The Wholesale Acquisition Cost (WAC) for Hemlibra is approximately $440,000 per year.

Sulopenem etzadroxil/probenecid (Brand Name: To be determined)

Manufacturer: Iterum Therapeutics
Regulatory designation: Fast Track
Expected FDA decision: October 25, 2024 *(FDA Advisory Committee scheduled for September 9, 2024)*

Therapeutic use

Sulopenem etzadroxil/probenecid is under review for the treatment of uncomplicated urinary tract infection (UTI) in adult women.

Uncomplicated UTIs are bacterial infections of the bladder in females with no structural abnormalities of their urinary tract. Approximately one-half of all women experience at least one UTI in their lifetime. Uncomplicated UTI accounts for approximately 30 million prescriptions annually.

Clinical profile

Sulopenem etzadroxil is a carbapenem antibiotic with activity against multidrug-resistant gram-negative pathogens, including those that produce extended-spectrum β-lactamases (ESBLs). Probenecid is included in the combination to extend the half-life of sulopenem and to increase concentrations of it in the urine.

Pivotal trial data:
The efficacy of sulopenem etzadroxil/probenecid was evaluated in REASSURE, a Phase 3, randomized, double-blind, active-control study in 2,222 adult women with uncomplicated UTI. Patients were randomized to sulopenem etzadroxil/ probenecid or amoxicillin/ clavulanate for 5 days. The primary endpoint was the overall response (clinical and microbiologic combined response) at Day 12 (+/- 1 day).

What you need to know:

Proposed Indication: Treatment of uncomplicated UTI in adult women

Mechanism: carbapenem antibiotic

Efficacy: Overall response rate:

- REASSURE trial: 61.7% vs. 55.0% with amoxicillin/ clavulanate (in amoxicillin/ clavulanate susceptible population)
- SURE-1 trial: 65.6% vs. 67.9% with ciprofloxacin (in all patients)

Common AEs: Diarrhea

Dosing: Orally twice daily for 5 days

Why it Matters: Potentially the first oral carbapenem, additional broad-spectrum antibiotic for outpatient treatment of uncomplicated UTI

Important to Note: First-line antibiotics for uncomplicated UTI are available generically, use likely to be reserved for patients with known resistance or contraindications to existing antibiotics, additional treatment options expected later this year or 2025 (Utility Therapeutics' Pivya and GSK's gepotidacin)

In the amoxicillin/clavulanate-susceptible population, sulopenem etzadroxil/probenecid was statistically superior to amoxicillin/clavulanate. The overall response rate was 61.7% in the sulopenem arm vs. 55.0% in the amoxicillin/ clavulanate arm (difference 6.7, 95% CI: 0.3, 13.0; p = 0.019).

The efficacy of sulopenem etzadroxil/probenecid was also evaluated in SURE-1, a Phase 3, randomized, double-blind, active-control study in 1,671 adult women with uncomplicated UTI. In this study, patients were randomized to sulopenem etzadroxil/probenecid for 5 days or ciprofloxacin for 3 days. The primary endpoint was the overall response (clinical and microbiologic combined response) at Day 12 (+/- 1 day). In patients with ciprofloxacin-nonsusceptible baseline pathogens, sulopenem etzadroxil/probenecid was compared for superiority over ciprofloxacin; in patients with ciprofloxacin-susceptible pathogens, the agents were compared for noninferiority.

In the nonsusceptible population, sulopenem etzadroxil/probenecid was superior to ciprofloxacin, with an overall response rate of 62.6% vs. 36.0%, respectively (difference 26.6; 95% CI: 15.1, 7.4; p < 0.001). In the susceptible population, sulopenem etzadroxil/probenecid was not noninferior to ciprofloxacin, with a response rate of 66.8% vs. 78.6%, respectively (difference -11.8; 95% CI: -18.0, 5.6). The difference was driven by a higher rate of asymptomatic bacteriuria post-treatment in patients on sulopenem etzadroxil/probenecid. In the combined analysis, sulopenem etzadroxil/ probenecid was noninferior to ciprofloxacin, with an overall response rate of 65.6% vs. 67.9%, respectively (difference -2.3; 95% CI: -7.9, 3.3).

Sulopenem etzadroxil/probenecid *(continued…)*

Safety:
The most common adverse event with sulopenem etzadroxil/probenecid use was diarrhea.

Dosing:
In the pivotal trials, sulopenem etzadroxil/probenecid was administered orally twice daily for 5 days.

Competitive environment

Sulopenem etzadroxil/probenecid would be the first approved oral carbapenem and it would provide a broad-spectrum alternative for outpatient treatment of uncomplicated UTI. The current standard of care for treatment of uncomplicated UTIs includes different antibiotics across various mechanisms of action, including: β-lactams (eg, amoxicillin), quinolones (eg, ciprofloxacin), trimethoprim–sulfamethoxazole, fosfomycin, and nitrofurantoin; however, the resistance rate is over 20% for some of these drugs/classes.

In addition to existing treatment options, sulopenem etzadroxil/probenecid will be competing with other pipeline drugs for UTI. In April 2024, the FDA approved Utility Therapeutics' Pivya™ (pivmecillinam), a β-lactam antibiotic that has been widely used outside of the U.S. for uncomplicated UTI since it has a low risk of selecting for resistant isolates. Pivya is expected to launch sometime in 2025. Additionally, GSK's gepotidacin, a first-in-class triazaacenaphthylene antibiotic, could also be approved in 2025 for uncomplicated UTI.

The FDA has scheduled an Advisory Committee to discuss sulopenem etzadroxil/probenecid, with two primary questions: (1) antimicrobial stewardship issues raised by potential approval and subsequent use of what would be the first oral carbapenem in the U.S.; and (2) the most appropriate target patient population(s) for treatment of uncomplicated UTI with sulopenem etzadroxil/probenecid.

Inavolisib (Brand Name: To be determined)

Manufacturer: Genentech
Regulatory designation: Breakthrough Therapy
Expected FDA decision: November 27, 2024

Therapeutic use

Inavolisib is under review, in combination with Ibrance® (palbociclib) and fulvestrant, for the treatment of patients with PIK3CA-mutated, hormone receptor (HR)-positive, human epidermal growth factor receptor 2 (HER2)-negative, locally advanced or metastatic breast cancer, following recurrence on or within 12 months of completing adjuvant endocrine treatment.

Breast cancer is the second most common cancer in women in the U.S., behind only skin cancers. In 2024, about 310,720 new cases of invasive breast cancer will be diagnosed in women and about 42,250 women will die from breast cancer. Nearly 70% of all breast cancer cases are of the HR-positive/HER2-negative subtype. The PIK3CA mutation is found in approximately 40% of HR-positive metastatic breast cancers.

Clinical profile

Inavolisib is a phosphatidylinositol 3-kinase (PI3K) alpha inhibitor. PI3K is part of a signaling pathway that plays a role in mediating cell growth, survival, and angiogenesis.

Pivotal trial data:
The efficacy of inavolisib was evaluated in INAVO120, a Phase 3, randomized, double-blind, placebo-controlled study in 325 patients with PIK3CA-mutant, HR-positive, HER2-negative, locally advanced or metastatic breast cancer whose disease progressed during treatment or within 12 months of completing adjuvant endocrine therapy. Patients were required to have not received prior systemic therapy for metastatic disease. Patients were randomized to receive inavolisib or placebo in combination with Ibrance (CDK4/6 inhibitor) and fulvestrant (endocrine therapy). The primary endpoint was progression-free survival (PFS).

Median PFS was 15.0 months in the inavolisib arm vs. 7.3 months in the placebo arm (hazard ratio [HR] 0.43, 95% CI: 0.32, 0.59; $p < 0.0001$). This represented a 57% reduction in the risk of disease worsening or death with inavolisib combination therapy vs. Ibrance and fulvestrant alone.

Safety:
The most common adverse events with inavolisib use were hyperglycemia, diarrhea, rash, and stomatitis.

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

What you need to know:

Proposed Indication: In combination with Ibrance (palbociclib) and fulvestrant, for the treatment of patients with PIK3CA-mutated, HR-positive, HER2-negative, locally advanced or metastatic breast cancer, following recurrence on or within 12 months of completing adjuvant endocrine treatment

Mechanism: PI3K alpha inhibitor

Efficacy: PFS:

- 15.0 months with inavolisib + Ibrance + fulvestrant vs. 7.3 months with placebo + Ibrance + fulvestrant

Common AEs: Hyperglycemia, diarrhea, rash, stomatitis

Dosing: Orally once daily

Why it Matters: Promising PFS results vs. standard of care therapy, reasonable safety profile vs. other PI3K inhibitors

Important to Note: Alternatives available targeting PIK3CA (eg, Piqray, Truqap), use will be limited to patients with PIK3CA-mutations, lack of robust overall survival data

Estimated Cost: ~$287,000 per year (based on pricing for Piqray)

Inavolisib *(continued...)*

Competitive environment

Endocrine therapy, with either an aromatase inhibitor or fulvestrant, plus a CDK4/6 inhibitor (eg, Ibrance), is the recommended first-line treatment for advanced or metastatic HR-positive/HER2-negative breast cancer. With the promising PFS data and superiority demonstrated vs. Ibrance/fulvestrant, inavolisib triplet therapy could become a potential first-line standard of care in this setting.

Other drugs that have targeted PIK3CA mutations (ie, Piqray® [alpelisib] and Truqap® [capivasertib]) have been plagued with tolerability issues, including side effects like hyperglycemia and diarrhea. These side effects appear to occur at a lower rate with inavolisib, so it may also have a best-in-class adverse event profile. Piqray is approved as a second-line treatment for this subset of breast cancer patients and Truqap is approved both in the first- and second-line setting.

While the PFS data is encouraging, overall survival data at this point is still not mature (a positive trend has been observed).

The WAC for Piqray is approximately $287,000 per year.

Acoramidis (Brand Name: To be determined)

Manufacturer: BridgeBio Pharma
Regulatory designation: Orphan Drug
Expected FDA decision: November 29, 2024

Therapeutic use

Acoramidis is under review for the treatment of cardiomyopathy due to transthyretin-mediated amyloidosis (ATTR-CM).

Transthyretin is a protein that normally transports thyroid hormone and vitamin A. In ATTR-CM, transthyretin proteins misfold and deposit in different organs of the body, including the heart. When amyloid deposits build up in the heart, it can cause dysfunction of the heart muscles (cardiomyopathy) and ultimately lead to symptoms of heart failure.

Two major forms of ATTR-CM exist. Hereditary ATTR-CM is caused by a genetic mutation in the transthyretin gene which produces transthyretin proteins that are predisposed to misfolding and deposition into muscles. Wild-type ATTR-CM is caused by the deposition of misfolded normal transthyretin through an unknown mechanism.

The exact prevalence of ATTR-CM is unknown since it is underdiagnosed, but it is estimated to affect over 100,000 people in the U.S.

What you need to know:

Proposed Indication: Treatment of ATTR-CM

Mechanism: Transthyretin stabilizer

Efficacy:

- Composite cardiovascular outcome (primary endpoint): 63.7% of pairwise comparisons favoring acoramidis and 35.9% favoring placebo
- On-treatment survival at Month 30 (secondary endpoint): 81% vs. 74% with placebo (25% relative risk reduction in death)

Safety: Incidence of AEs were similar in the acoramidis and placebo groups

Dosing: Orally twice daily

Why it Matters: Potential competitor to Pfizer's Vyndamax (only other drug currently approved for ATTR-CM)

Important to Note: Lack of head-to-head trial data vs. Vyndamax, potential future competition (eg, Alnylam's Amvuttra)

Estimated Cost: ~$270,000 per year (based on pricing for Vyndamax)

Clinical profile

Acoramidis is a transthyretin stabilizer. It binds to and stabilizes transthyretin, preventing misfolding of the protein and inhibiting the formation of transthyretin amyloid fibrils.

Pivotal trial data:
The efficacy of acoramidis was evaluated in ATTRibute-CM, a Phase 3, randomized, double-blind, placebo-controlled study in 632 patients with ATTR-CM. Patients received acoramidis or placebo. The four-step primary hierarchical analysis included death from any cause, cardiovascular-related hospitalization, the change from baseline in the N-terminal pro-B-type natriuretic peptide (NT-proBNP) level, and the change from baseline in the 6-minute walk distance (6-MWD). A win ratio was calculated when comparing acoramidis vs. placebo. The stratified win ratio can be expressed as the proportion of pairwise comparisons for which active treatment wins over placebo divided by the proportion of pairwise comparisons for which placebo wins, taking into account both the hierarchical ordering of the comparisons and the strata in which the comparisons are performed.

Acoramidis *(continued...)*

The primary analysis favored acoramidis over placebo with a corresponding win ratio of 1.8 (95% CI: 1.4, 2.2; p < 0.001), with 63.7% of pairwise comparisons favoring acoramidis and 35.9% favoring placebo. Of note at Month 30, on-treatment survival rate was 81% with acoramidis vs. 74% with placebo, and this represented a 25% relative risk reduction in death.

Safety:
The overall incidence of adverse events was similar in the acoramidis group and the placebo group (98.1% and 97.6%, respectively).

Dosing:
In the pivotal trial, acoramidis was administered orally twice daily.

Competitive environment

If approved, acoramidis would be the second transthyretin stabilizer available for ATTR-CM and a direct competitor to Pfizer's Vyndamax® (tafamidis), the only other drug currently approved for the disease. The primary endpoints were different for the two trials evaluating acoramidis and Vyndamax which makes indirect comparisons difficult; however, the results for acoramidis appear robust across a range of clinical outcomes, including death from any cause and cardiovascular-related hospitalization.

Both acoramidis and Vyndamax may face additional competition for ATTR-CM with Alnylam's Amvuttra® (vutrisiran), a transthyretin-directed small interfering RNA therapy. Amvuttra is currently approved for polyneuropathy of hereditary ATTR but recently Alnylam announced that their Phase 3 trial for ATTR-CM achieved the primary endpoint. Unlike Vyndamax and acoramidis which require daily oral administration, Amvuttra is administered via SC injection once every 3 months. Alnylam is expected to submit for approval for Amvuttra for the ATTR-CM indication in late 2024.

The WAC for Vyndamax is approximately $270,000 per year.

Olezarsen (Brand Name: To be determined)

Manufacturer: Ionis Pharmaceuticals
Regulatory designation: Orphan Drug, Fast Track, Breakthrough Therapy
Expected FDA decision: December 19, 2024

Therapeutic use

Olezarsen is under review for the treatment of adults with familial chylomicronemia syndrome (FCS).

FCS is an ultra-rare inherited disease caused by impaired function of the lipoprotein lipase (LPL) enzyme. Because of limited LPL production or function, people with FCS cannot effectively break down chylomicrons, which are lipoprotein particles that help carry triglycerides to different parts of the body. As a result, patients experience severely elevated triglyceride levels which can lead to a variety of downstream consequences, including increased risk of acute pancreatitis.

FCS is estimated to impact 1 to 13 people per million in the U.S.

Clinical profile

Olezarsen is an antisense oligonucleotide therapy designed to lower the body's production of apolipoprotein C-III, a protein produced in the liver that regulates triglyceride metabolism in the blood.

What you need to know:

Proposed Indication: Treatment of adults with FCS

Mechanism: Antisense oligonucleotide

Efficacy: Triglyceride levels at Month 6:

• Reduced by 43.5% with olezarsen 80 mg vs. placebo

Safety: Incidence of AEs were similar in the olezarsen and placebo group

Dosing: SC once every 4 weeks

Why it Matters: Potentially the first approved therapy for FCS, significant unmet need, in development for broader severe hypertriglyceridemia populations

Important to Note: Small initial target population, potential future competition with Arrowhead Pharmaceuticals' plozasiran (FDA approval possible by mid-2025)

Pivotal trial data:
The efficacy of olezarsen was evaluated in BALANCE, a Phase 3, randomized, double-blind, placebo-controlled study in 66 patients with FCS. Patients received olezarsen 50 mg, olezarsen 80 mg, or placebo. The primary endpoint was the percent change in the fasting triglyceride level from baseline to 6 months between the olezarsen groups and the placebo group. A key secondary endpoint was acute pancreatitis.

At baseline, the mean triglyceride level among the patients was 2,630 mg/dL, and 71% had a history of acute pancreatitis within the previous 10 years. Triglyceride levels at 6 months were significantly reduced with the 80 mg dose of olezarsen as compared with placebo (−43.5%; 95% CI: −69.1, −17.9; $p < 0.001$) but not with the 50 mg dose (−22.4%, 95% CI: −47.2, 2.5; $p = 0.08$).

By 53 weeks, 11 episodes of acute pancreatitis had occurred in the placebo group, and 1 episode had occurred in each olezarsen group (rate ratio 0.12, 95% CI: 0.02 to 0.66).

Safety:
The most common adverse events with olezarsen use were COVID-19, abdominal pain, and diarrhea, and the rates were lower than those observed with placebo.

Dosing:
In the pivotal trial, olezarsen was administered via SC injection every 4 weeks.

Olezarsen *(continued...)*

Competitive environment

Olezarsen would potentially be the first treatment for FCS, a condition with a high unmet need. Patients with FCS have a poor response to conventional triglyceride- and lipid-lowering therapies (eg, fibrates, omega-3 fatty acids, and statins).

FCS is an ultra-rare disease and so the initial target population for olezarsen is expected to be very small. However, olezarsen is also being evaluated across three pivotal studies in patients with severe hypertriglyceridemia. There is an estimated 3 million people in the U.S. with severe hypertriglyceridemia. Results from these trials are expected in the second half of 2025, and if positive, this would significantly expand the eligible population for olezarsen.

For the FCS indication, Ionis may face fairly immediate competition – Arrowhead Pharmaceuticals' plozasiran is in late-stage development with a potential approval by mid-2025.

Datopotamab deruxtecan (Brand Name: To be determined)

Manufacturer: Daiichi Sankyo/AstraZeneca
Expected FDA decision: December 20, 2024 (NSCLC); January 29, 2025 (Breast Cancer)

Therapeutic use

Datopotamab deruxtecan is under review for two indications: (1) treatment of adult patients with locally advanced or metastatic nonsquamous non-small cell lung cancer (NSCLC) who have received prior systemic therapy; and (2) treatment of adult patients with unresectable or metastatic HR-positive, HER2-negative breast cancer who have received prior systemic therapy for unresectable or metastatic disease.

Lung cancer is a common cancer in both men and women, with about 234,580 new cases expected in 2024 in the U.S. NSCLC is the most common type of lung cancer, affecting about 80% of cases, and 70% of NSCLC tumors are of nonsquamous histology.

Breast cancer is the second most common cancer in women in the U.S., behind only skin cancers. In 2024, about 310,720 new cases of invasive breast cancer will be diagnosed in women and about 42,250 women will die from breast cancer. Nearly 70% of all breast cancer cases are of the HR-positive/HER2-negative subtype.

Clinical profile

Datopotamab deruxtecan is an antibody drug conjugate that targets trophoblast cell-surface antigen 2 (TROP2). The anti-TROP2 antibody targets and binds to TROP2 expressed on tumor cells. Upon cellular uptake and lysosomal degradation of a linker, a chemotherapy drug (deruxtecan) is delivered to these tumor cells causing DNA damage and cell death.

Pivotal trial data:
NSCLC
The efficacy of datopotamab deruxtecan was evaluated in TROPION-Lung01, a Phase 3, randomized, open-label study in 604 patients with locally advanced or metastatic NSCLC with and without actionable genomic alterations previously treated with at least one prior line of therapy. Patients were randomized to datopotamab deruxtecan or chemotherapy (docetaxel). The dual primary endpoints were progression-free survival (PFS) and overall survival (OS).

What you need to know:

Proposed Indication:

- Treatment of adult patients with locally advanced or metastatic nonsquamous NSCLC who have received prior systemic therapy
- Treatment of adult patients with unresectable or metastatic HR-positive, HER2-negative breast cancer who have received prior systemic therapy for unresectable or metastatic disease

Mechanism: TROP2 directed antibody drug conjugate

Efficacy: PFS:

- Nonsquamous NSCLC: 5.6 months vs. 3.7 months with docetaxel
- Breast cancer: 6.9 months vs. 4.9 with chemotherapy

Common AEs: Nausea, stomatitis, anemia

Dosing: IV once every 3 weeks

Why it Matters: Additional second- or third-line treatment option for both NSCLC and breast cancer, potentially better safety profile vs. other antibody drug conjugates (eg, Trodelvy), treatment for NSCLC was not limited to specific mutation

Important to Note: Lack of head-to-head trial data vs. non-chemotherapy treatments, targeted therapies likely preferred for both cancers, lack of robust overall survival data

Estimated Cost: ~$20,000 per 21-day cycle (based on pricing for Trodelvy)

Datopotamab deruxtecan *(continued...)*

In the overall population, median PFS was 4.4 months in patients treated with datopotamab deruxtecan vs. 3.7 months with docetaxel; datopotamab deruxtecan reduced the risk of disease progression or death by 25% (hazard ratio [HR] 0.75, 95% CI: 0.62, 0.91; p = 0.004). In patients with nonsquamous NSCLC, datopotamab deruxtecan reduced the risk of disease progression or death by 37% compared to docetaxel (HR 0.63, 95% CI: 0.51, 0.78). In this population, median PFS was 5.6 months in patients treated with datopotamab deruxtecan vs. 3.7 months with docetaxel. For the dual primary endpoint of OS, results did not reach statistical significance at the time of this data cut-off.

Breast cancer
The efficacy of datopotamab deruxtecan was evaluated in TROPION-Breast01, a Phase 3, randomized, open-label study in 732 patients with unresectable or metastatic HR-positive, HER2-negative breast cancer who have progressed on and are not suitable for endocrine therapy and have received at least one additional systemic therapy for unresectable or metastatic disease. Patients were randomized to datopotamab deruxtecan or investigator's choice of single-agent chemotherapy (eribulin, capecitabine, vinorelbine, or gemcitabine). The dual primary endpoints were PFS and OS.

Median PFS was 6.9 months in patients treated with datopotamab deruxtecan vs. 4.9 with chemotherapy, with datopotamab deruxtecan reducing the risk of disease progression or death by 37% compared to chemotherapy (HR 0.63, 95% CI: 0.52, 0.76; p < 0.001). For OS, results did not reach statistical significance at the time of this data cut-off.

Safety:
The most common adverse events with datopotamab deruxtecan use were nausea, stomatitis, and anemia.

Dosing:
In the pivotal trials, datopotamab deruxtecan was administered intravenously (IV) once every 3 weeks.

Competitive environment

If approved, datopotamab deruxtecan would offer an additional second- or third-line treatment option for advanced or metastatic NSCLC and breast cancer. Other antibody drug conjugates are available in similar settings, but these drugs are typically associated with safety concerns. In its pivotal studies, datopotamab deruxtecan was associated with relatively low rates of serious adverse events and drug discontinuations. Additionally, unlike other recently approved drugs, particularly for NSCLC, treatment with datopotamab deruxtecan is not limited to a specific mutational status. The PFS data is promising for heavily pretreated patients, especially those with nonsquamous NSCLC; however, overall survival data is not yet mature.

Overall, the initial use for datopotamab deruxtecan will likely be reserved in heavily pretreated patients for both cancers, especially since it was not compared against other non-chemotherapy treatment approaches. The current standard of care targeted therapies are likely to be used ahead of datopotamab deruxtecan until additional data accrues for datopotamab deruxtecan.

The WAC for Trodelvy® (sacituzumab govitecan-hziy), another TROP2 directed antibody drug conjugate, is approximately $20,000 per 21-day treatment cycle.

Garadacimab (Brand Name: To be determined)

Manufacturer: CSL
Regulatory designations: Orphan Drug, Fast Track
Expected FDA decision: December 2024

Therapeutic use

Garadacimab is under review for the prophylactic treatment of hereditary angioedema (HAE).

HAE is an inherited disorder characterized by recurrent episodes of the accumulation of fluids outside of the blood vessels, causing rapid swelling of tissues in the hands, feet, limbs, face, intestinal tract, or airway. Swelling of the airway may lead to obstruction, a potentially very serious complication. The severity of the disease varies greatly among affected individuals.

HAE affects about 1 in every 50,000 individuals and the estimated prevalence in the U.S. is about 6,000 people.

Clinical profile

Garadacimab is a factor XIIa (FXIIa)-inhibitory monoclonal antibody. When FXII is activated, it initiates the cascade of events leading to edema formation. By targeting activated FXII (FXIIa), garadacimab inhibits this cascade and reduces the likelihood of edema formation.

What you need to know:

Proposed Indication: Prophylactic treatment of HAE

Mechanism: FXIIa-inhibitory monoclonal antibody

Efficacy: HAE attacks per month:

• 0.27 vs. 2.01 with placebo (87% reduction)

Common AEs: Upper respiratory tract infections, nasopharyngitis, headaches

Dosing: SC once every month

Why it Matters: Novel mechanism of action, appears well tolerated, administered once monthly

Important to Note: Alternatives available (eg, Cinryze, Takhzyro, Orladeyo), lack of head-to-head trial data vs. competitors, SC administration

Estimated Cost: ~$500,000 per year (based on pricing for Orladeyo)

Pivotal trial data:
The efficacy of garadacimab was evaluated in VANGUARD, a Phase 3, randomized, double-blind, placebo-controlled study in 65 patients with HAE. Patients were randomized to garadacimab or placebo. The primary endpoint was the time-normalized number of HAE attacks (number of hereditary angioedema attacks per month) during the 6-month treatment period (Day 1 to Day 182).

During the 6-month treatment period, the mean number of HAE attacks per month was significantly lower in the garadacimab group (0.27) than in the placebo group (2.01), corresponding to a percentage difference of −87% (95% CI: −96, −58; $p < 0.0001$).

Safety:
The most common adverse events with garadacimab use were upper respiratory tract infections, nasopharyngitis, and headaches.

Dosing:
In the pivotal trial, garadacimab was administered via SC injection once every month.

Garadacimab *(continued...)*

Competitive environment

The current standard of care for prophylaxis treatment of HAE includes injectable C1 inhibitor concentrates (eg, Cinryze®); Takhzyro® (lanadelumab-flyo), a SC administered plasma kallikrein inhibitor; and Orladeyo® (berotralstat), an orally administered plasma kallikrein inhibitor. Garadacimab would offer a first-in-class mechanism for the treatment of HAE, that works upstream of other HAE treatments.

Compared indirectly, the efficacy data for garadacimab appear similar to other injectable treatment options and it was well tolerated in the pivotal study. However, there is a lack of head-to-head trial data compared to other well-established treatment options. Garadacimab's primary differentiator is that it can be administered once monthly in all patients while existing treatment options are generally administered more frequently.

The WAC for Orladeyo is approximately $500,000 per year.

Crinecerfont (Brand Name: To be determined)

Manufacturer: Neurocrine Biosciences
Regulatory designation: Orphan Drug, Breakthrough Therapy
Expected FDA decision: December 29, 2024

Therapeutic use

Crinecerfont is under review for the treatment of adolescents and adults with classic congenital adrenal hyperplasia (CAH) due to 21-hydroxylase (21-OHD) enzyme deficiency.

CAH is an inherited disease of the adrenal glands, characterized by a deficiency in production of key hormones. In about 95% of CAH cases, patients lack the 21-hydroxylase (21-OHD) enzyme, which is essential for making cortisol and in most cases, aldosterone. The absence of cortisol also leads the body to produce excessive androgen hormones, which can lead to a variety of symptoms including abnormal bone growth and development in pediatric patients and female health problems such as excess hair growth and menstrual irregularities.

The standard of care for patients with CAH are glucocorticoid steroids to treat cortisol deficiency and reduce androgens, but long-term treatment with these drugs is associated with their own adverse events (eg, weight gain, diabetes, cardiovascular disease).

The estimated prevalence of classic CAH due to 21-OHD deficiency is about 30,000 in the U.S.

What you need to know:

Proposed Indication: Treatment of adolescents and adults with classic CAH due to 21-OHD enzyme deficiency

Mechanism: CRF1 receptor antagonist

Efficacy: Androstenedione level at Week 4:

- Pediatric study: –197 ng/dL vs. +71 ng/dL with placebo
- Adult study: –299 ng/dL vs. +45.5 ng/dL with placebo

Common AEs: Headache, pyrexia, vomiting

Dosing: Orally twice daily

Why it Matters: First-in-class, non-glucocorticoid treatment for CAH, potential to lower the dose of glucocorticoid therapy, thereby improving safety/tolerability of treatment

Important to Note: Does not replace glucocorticoid therapy, improvement in long-term outcomes (eg, glucocorticoid therapy adverse events) has not been established

Clinical profile

Crinecerfont is a selective corticotropin-releasing factor type 1 (CRF1) receptor antagonist. Blocking CRF1 receptors in the pituitary gland has been shown to decrease adrenocorticotropic hormone (ACTH) levels, which in turn decreases the production of adrenal androgens.

Pivotal trial data:
The efficacy of crinecerfont was evaluated in CAHtalyst Pediatric, a Phase 3, randomized, double-blind, placebo-controlled study in 103 patients ages 2 to 17 years with CAH due to 21-OHD deficiency and with stable glucocorticoid therapy. Patients were randomized to crinecerfont or placebo. The primary endpoint was the change in the androstenedione (a key adrenal androgen) level from baseline to Week 4. A secondary endpoint was the percent change in the glucocorticoid dose from baseline to Week 28 while androstenedione control was maintained.

Crinecerfont *(continued...)*

At Week 4, the androstenedione level was substantially reduced in the crinecerfont group (−197 ng/dL) but increased in the placebo group (71 ng/dL) (least-squares mean difference −268 ng/dL; $p < 0.001$). At Week 28, the mean glucocorticoid dose had decreased (while androstenedione control was maintained) by 18.0% with crinecerfont but increased by 5.6% with placebo (least-squares mean difference −23.5; $p < 0.001$).

In addition to the pediatric study, crinecerfont was also evaluated in CAHtalyst, a Phase 3, randomized, double-blind, placebo-controlled study in 182 adults with CAH due to 21-OHD deficiency and with stable glucocorticoid therapy. Patients were randomized to crinecerfont or placebo. The primary endpoint was the percent change in the daily glucocorticoid dose from baseline to Week 24 with maintenance of androstenedione control. A key secondary endpoint was the change in the androstenedione level from baseline to Week 4.

At Week 24, the change in the glucocorticoid dose (with androstenedione control) was −27.3% in the crinecerfont group and −10.3% in the placebo group (least-squares mean difference −17.0; $p < 0.001$). A physiologic glucocorticoid dose (with androstenedione control) was reported in 63% of the patients in the crinecerfont group and in 18% in the placebo group ($p < 0.001$). At Week 4, androstenedione levels decreased with crinecerfont (−299 ng/dL) but increased with placebo (45.5 ng/dL) (least-squares mean difference −345 ng/dL; $p < 0.001$).

Safety:
The most common adverse events with crinecerfont use were headache, pyrexia, and vomiting.

Dosing:
In the pivotal trials, crinecerfont was administered orally twice daily.

Competitive environment

If approved, crinecerfont would be a first-in-class, non-glucocorticoid treatment for CAH. The current standard of care for CAH is treatment with glucocorticoids, which can improve cortisol levels and reduce androgen hormone production. However, high doses of glucocorticoids are generally required to control androgen production, which can lead to glucocorticoid-related adverse events.

The use of crinecerfont would not replace glucocorticoids but allow for lower dosing to improve the safety and tolerability of treatment for CAH. Both the pediatric and adult studies were promising, with crinecerfont reducing average glucocorticoid dosages without compromising androgen production. These studies did not find any difference in long-term glucocorticoid therapy adverse events (eg, body weight, insulin resistance); these outcomes would require a longer-term trial to evaluate.

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									
RG-1594	ocrelizumab/ hyaluronidase	Genentech	CD20-directed cytolytic antibody	Multiple sclerosis	SC	Filed BLA	09/13/2024	Yes	No
Tecentriq SC	atezolizumab	Roche	programmed death-ligand 1 blocking antibody	Cancers (mirroring indications to IV formulation)	SC	Filed BLA	09/15/2024	Yes	No
LY-686017	tradipitant	Vanda Pharmaceuticals	neurokinin 1 receptor antagonist	Gastroparesis	PO	Filed NDA	09/18/2024	No	No
arimoclomol	arimoclomol	Zevra Therapeutics	cytoprotectives	Niemann-Pick disease	PO	Filed NDA	09/21/2024	Yes	Yes
IBI-1000	acetylleucine	IntraBio	modified amino acid	Niemann-Pick Disease type C	PO	Filed NDA	09/24/2024	Yes	Yes
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
PF-06741086	marstacimab	Pfizer	anti-tissue factor pathway inhibitor	Hemophilia A and B	SC	Filed BLA	10/11/2024	Yes	Yes
CAM-2029	octreotide	Camurus	somatostatin analogue	Acromegaly	SC	Filed NDA	10/21/2024	Yes	Yes
sulopenem	sulopenem	Iterum Therapeutics	carbapenem	Urinary tract infections	PO	Filed NDA	10/25/2024	No	No
Ovastat	treosulfan	Medexus Pharmaceuticals	alkylating agent	Hematopoietic stem cell transplantation	IV	Filed NDA	10/30/2024	Yes	Yes
MILR-1444A	lebrikizumab	Eli Lilly	interleukin-13 inhibitor	Atopic dermatitis	SC	Filed BLA	10/2024	Yes	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	11/04/2024	No	No
MCLA-128	zenocutuzumab	Merus	neuregulin/HER3 inhibitor	Non-smell cell lung cancer/ pancreatic cancer	IV	Filed BLA	11/06/2024	Yes	Yes
iMAB-362	zolbetuximab	Astellas	GC182 monoclonal antibody	Gastric cancer	IV	Filed BLA	11/09/2024	Yes	Yes
Humacyl	human acellular vessel	Humacyte	cellular therapy	End-stage renal disease	Implant	Filed BLA	11/10/2024	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
PTC-AADC	eladocagene exuparvovec	PTC Therapeutics	gene therapy	Aromatic L-amino acid decarboxylase deficiency	Intracerebral	Filed BLA	11/13/2024	Yes	Yes
Obe-cel	obecabtagene autoleucel	Autolus Therapeutics	autologous chimeric antigen receptor T-cells	Acute lymphoblastic leukemia	IV	Filed BLA	11/16/2024	Yes	Yes
RG-6114	inavolisib	Roche	phosphatidylinositol 3-kinase alpha inhibitor	Breast cancer	PO	Filed NDA	11/27/2024	Yes	No
AT-007	govorestat	Applied Therapeutics	aldose reductase inhibitor	Galactosemia	PO	Filed NDA	11/28/2024	Yes	Yes
ZW-25	zanidatamab	Jazz Pharmaceuticals	HER2 monoclonal antibody	Biliary tract cancer	IV	Filed BLA	11/29/2024	Yes	Yes
AG-10 (AG10)	acoramidis	BridgeBio	tetrameric transthyretin stabilizer	Transthyretin amyloid cardiomyopathy	PO	Filed NDA	11/29/2024	Yes	No
SH-201	SH-201	Shorla Oncology	Unknown	Leukemias	PO	Filed NDA	11/30/2024	Yes	No
CSL-312	garadacimab	CSL Limited	anti-factor XIIa monoclonal antibody	Hereditary angioedema	SC	Filed BLA	12/14/2024	Yes	Yes
IONIS-APOCIII-LRx (ISIS-678354)	olezarsen	Ionis	antisense drug	Familial chylomicronemia syndrome	SC	Filed BLA	12/19/2024	Yes	Yes
DS-1062	datopotamab deruxtecan	Daiichi Sankyo/ AstraZeneca	trop-2 antibody-drug conjugate	Non-small cell lung cancer; breast cancer	IV	Filed BLA	12/20/2024	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
Rybrevant SC	amivantamab-vmjw/ hyaluronidase	Johnson & Johnson	bispecific EGF receptor-directed and MET receptor-directed antibody	Non-small cell lung cancer	SC	Filed BLA	12/20/2024	Yes	No
ZP-1848	glepaglutide	Zealand Pharma	glucagon peptide-2 agonist	Short bowel syndrome	SC	Filed NDA	12/22/2024	Yes	Yes
SNDX-5613	revumenib	Syndax	Menin-mixed lineage leukemia 1 inhibitor	Acute myelogenous leukemia	PO	Filed NDA	12/26/2024	Yes	Yes
X-396	ensartinib	Xcovery	anaplastic lymphoma kinase inhibitor	Non-small cell lung cancer	PO	Filed NDA	12/28/2024	Yes	No
CK-301	cosibelimab	Checkpoint Therapeutic	anti programmed cell death ligand 1	Cutaneous squamous cell carcinoma	IV	Filed NDA	12/28/2024	Yes	No
NBI-74788	crinecerfont	Neurocrine Biosciences	CRF receptor antagonist	Congenital adrenal hyperplasia	PO	Filed NDA	12/29/2024	Yes	Yes
Opdivo SC	nivolumab/ hyaluronidase	Bristol Myers Squibb	programmed death receptor-1-blocking antibody	Various cancers	SC	Filed BLA	12/29/2024	Yes	No
2025 Possible launch date									
VX-121/ tezacaftor/ deutivacaftor	vanzacaftor/ tezacaftor/ deutivacaftor	Vertex	CF transmembrane conductance modulators	Cystic fibrosis	PO	Filed NDA	01/02/2025	Yes	Yes
Subvenite	lamotrigine	OWP Pharmaceuticals	anticonvulsant	Epilepsy/ bipolar disorder	PO	Filed NDA	01/03/2025	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
Prochymal	remestemcel-L	Mesoblast	mesenchymal stem cells	Graft vs. host disease	IV	Filed BLA	01/08/2025	Yes	Yes
EBV-CTL (ATA-129)	tabelecleucel	Atara Biotherapeutics	cell therapy	Lymphoproliferative disorder	IV	Filed BLA	01/15/2025	Yes	Yes
MTP-131 (SS-31)	elamipretide	Stealth Biotherapeutics	mitochondrial permeability transition pore inhibitor	Barth syndrome	SC	Filed NDA	01/29/2025	Yes	Yes
VX-548	suzetrigine	Vertex	selective NaV1.8 inhibitor	Acute pain	PO	Filed NDA	01/30/2025	No	No
NN-7415	concizumab	Novo Nordisk	anti-tissue factor pathway inhibitor	Hemophilia A and B	SC	Filed BLA	01/2025	Yes	Yes
SPN-830	apomorphine	Supernus Pharmaceuticals	non-ergoline dopamine agonist	Parkinson's disease	SC infusion	Filed NDA	02/01/2025	Yes	No
PB-2452	bentracimab	SFJ Pharmaceuticals	antiplatelet monoclonal antibody	Antiplatelet drug toxicity	IV	Filed BLA	02/02/2025	No	No
MenABCWY	meningococcal vaccine	GSK	vaccine	Meningococcal disease	IM	Filed BLA	02/14/2025	No	No
DCC-3014	vimseltinib	Deciphera	CSF1R inhibitor	Tenosynovial giant cell tumor	PO	Filed NDA	02/17/2025	Yes	No
KVD-900	sebetralstat	KalVista Pharmaceuticals	plasma kallikrein inhibitor	Hereditary angioedema	PO	Filed NDA	02/18/2025	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
DCCR	diazoxide choline controlled-release	Soleno Therapeutics	vasodilator	Prader-Willi syndrome	PO	Filed NDA	02/28/2025	Yes	Yes
PD-0325901	mirdametinib	SpringWorks Therapeutics	MEK 1/2 inhibitor	Neurofibromatosis	PO	Filed NDA	03/01/2025	Yes	Yes
Hernicore (SI-6603)	condoliase	Seikagaku	glycosaminoglycan-degrading enzyme	Pain	Intrathecal	Filed BLA	03/14/2025	Yes	No
MSP-2017	etripamil	Milestone	calcium channel blocker	Arrhythmia	Intranasal	Filed NDA	03/26/2025	TBD	No
ALN-APC (ALN-AT3)	fitusiran	Sanofi/ Alnylam	RNAi therapeutic	Hemophilia A and B	SC	Filed BLA	03/28/2025	Yes	Yes
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	CRL	1Q2025	Yes	Yes
EB-101	prademagene zamikeracel	Abeona Therapeutics	gene therapy	Epidermolysis Bullosa	TOP	CRL	1Q2025	Yes	Yes
PDP-716	brimonidine	Visiox Pharma	alpha-2 agonist	Glaucoma	OPH	Not Approved	1Q2025	No	No
UGN-102	mitomycin	UroGen	alkylating drug	Bladder cancer	Intravesical	Filed NDA	04/15/2025	Yes	No
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension; interstitial lung disease	INH	Tentative Approval	05/23/2025	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
Xinlay	atrasentan	Novartis	selective endothelin-A receptor antagonist	IgA nephropathy	PO	Filed NDA	06/2025	Yes	No
chenodeoxycholic acid	chenodeoxycholic acid	Mirum Pharmaceuticals	farnesoid X receptor agonist	Cerebrotendinous xanthomatosis	PO	Filed NDA	06/28/2025	Yes	Yes
Leqembi SC	lecanemab	Eisai/Biogen	beta-amyloid targeted therapy	Alzheimer's disease	SC	InTrial	2Q2025	Yes	No
ABBV-951	foscarbidopa/ foslevodopa	AbbVie	aromatic amino acid decarboxylation inhibitor/ aromatic amino acid	Parkinson's disease	SC	CRL	1H2025	Yes	No
YN-96D1	rivoceranib (apatinib)	Elevar Therapeutics	vascular endothelial growth factor receptor antagonist	Hepatocellular carcinoma	PO	CRL	1H2025	Yes	Yes
SHR-1210	camrelizumab	Elevar Therapeutics	programmed death receptor-1-blocking antibody	Hepatocellular carcinoma	IV	CRL	1H2025	Yes	Yes
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
S-217622	ensitrelvir fumaric acid	Shionogi	Protease inhibitor	COVID-19 treatment	PO	InTrial	1H2025	No	No
NS-2 (ALDX-1E1, ADX-102)	reproxalap	Aldeyra Therapeutics	aldehyde antagonist	Dry eye disease	OPH	CRL	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
SDN-037	difluprednate	Visiox	corticosteroid	Ocular inflammation/pain	OPH	InTrial	Mid-2025	No	No
PTC-743	vatiquinone	PTC Therapeutics	undisclosed	Friedreich's ataxia	PO	InTrial	Mid-2025	Yes	Yes
INS-1007	brensocatib	Insmed	dipeptidyl peptidase 1 inhibitor	Bronchiectasis	PO	InTrial	Mid-2025	No	No
GZ-402671 (SAR-402671)	venglustat (ibiglustat)	Sanofi	glucosylceramide synthase inhibitor	M2 Gangliosidosis	PO	InTrial	Mid-2025	Yes	Yes
RP-L102 (RPL-102)	RP-L102	Rocket Pharmaceuticals	gene therapy	Fanconi anemia	IV	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
ADI-PEG20	pegargiminase	Polaris	pegylated arginine deiminase	Mesothelioma	IM	InTrial	Mid-2025	Yes	Yes
PRN-1008	rilzabrutinib	Sanofi	BTK inhibitor	Chronic immune thrombocytopenia	PO	InTrial	Mid-2025	No	Yes
ALZ-801	valiltramiprosate	Alzheon	amyloid beta-protein inhibitor	Alzheimer's disease	PO	InTrial	Mid-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
SEL-212	SVP-rapamycin/ pegsiticase	Selecta Biosciences/ 3SBio	synthetic vaccine particle/ enzyme combination	Gout	IV	InTrial	Mid-2025	Yes	No
CK-274	aficamten	Cytokinetics	cardiac myosin inhibitor	Obstructive hypertrophic cardiomyopathy	PO	InTrial	Mid-2025	Yes	Yes
RP-L201	marnetegragene autotemcel	Rocket Pharmaceuticals	gene therapy	Leukocyte adhesion deficiency-I	IV	CRL	Mid-2025	Yes	Yes
RGX-121	RGX-121	Regenxbio	gene therapy	Mucopolysaccharidosis Type II	Intracisternal	InTrial	Mid-2025	Yes	Yes
LIB-003	lerodalcibep	LIB Therapeutics	PCSK9 inhibitor	Hypocholesteremia	SC	InTrial	Mid-2025	No	No
UX-111 (ABO-102)	UX-111	Ultragenyx Pharmaceutical	gene therapy	Sanfilippo syndrome type A	IV	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

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
INO-3107	INO-3107	Inovio Pharmaceuticals	immunotherapy	Recurrent respiratory papillomatosis	IM	InTrial	Mid-2025	Yes	Yes
DZD-9008	sunvozertinib	Dizal	EGFR inhibitor	Non-small cell lung cancer	PO	InTrial	Mid-2025	Yes	No
PTC-923	sepiapterin	PTC Therapeutics	phenylalanine hydroxylase activator	Phenylketonuria	PO	Filed NDA	07/30/2025	Yes	Yes
mRNA-1283	COVID-19 vaccine, mRNA	Moderna	messanger RNA	COVID-19 prevention	IM	InTrial	2025	No	No
BAY-342	elinzanetant	Bayer	neurokinin-1,3 receptor antagonist	Vasomotor symptoms	PO	Filed NDA	08/01/2025	No	No
LNZ-100	aceclidine	Lenz Therapeutics	acetylcholine receptor agonist	Treatment of presbyopia	OPH	Filed NDA	08/12/2025	No	No
AR-15512	AR-15512	Aerie Pharmaceuticals	TRPM8 agonist	Dry eye disease	OPH	InTrial	3Q2025	No	No
MT-1621	deoxythymidine/ deoxycytidine	UCB	deoxynucleoside	Thymidine kinase 2 deficiency	PO	InTrial	3Q2025	Yes	Yes
ARO-APOC3	plozasiran	Arrowhead Pharmaceuticals	RNAi targeting apolipoprotein C-III	Familial chylomicronemia syndrome	SC	InTrial	3Q2025	Yes	Yes
ANB-019	imsidolimab	AnaptysBio	interleukin-36 receptor antagonist	Generalized pustular psoriasis	IV	InTrial	3Q2025	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
SAR-442168	tolebrutinib	Sanofi	Bruton's tyrosine kinase inhibitor	Multiple sclerosis	PO	InTrial	4Q2025	Yes	No
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
PAX-101	suramin	PaxMedica	unknown	trypanosomiasis	IV	InTrial	2H2025	No	No
ND-0612H	levodopa/ carbidopa	Mitsubishi Tanabe	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	SC	CRL	2H2025	Yes	No
SPR-001	tildacerfont	Spruce Biosciences	corticotropin-releasing factor type-1 receptor antagonist	Congenital adrenal hyperplasia	PO	InTrial	2H2025	Yes	Yes
CORT-125134	relacorilant	Corcept Therapeutics	glucocorticoid receptor II antagonist	Cushing's syndrome	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
AXS-12	reboxetine	Axsome Therapeutics	norepinephrine reuptake inhibitor	Narcolepsy	PO	InTrial	2H2025	No	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
OX-124	naloxone	Orexo	opioid antagonist	Opioid overdose	Intranasal	CRL	2H2025	No	No
SPI-014	lanthanum dioxycarbonate	Unicycive	phosphate binder	Hyperphosphatemia	PO	InTrial	2H2025	No	No
EB-1020	centanafadine	Otsuka	norepinephrine, dopamine and serotonin reuptake inhibitor	Attention deficit hyperactivity disorder	PO	InTrial	2H2025	No	No
PF-06939926	fordadistrogene movaparvovec	Pfizer	gene therapy	Duchenne muscular dystrophy	IV	InTrial	2H2025	Yes	Yes
SPK-8011	dirloctocogene samoparvovec	Roche/ Spark Therapeutics	gene therapy	Hemophilia A	IV	InTrial	2H2025	Yes	Yes
NRX-100	ketamine	NeuroRx	NMDA antagonist	Depression	PO	InTrial	2H2025	No	No
AXS-14	S-reboxetine	Axsome Therapeutics	selective noradrenaline reuptake inhibitor	Fibromyalgia	PO	InTrial	2H2025	No	No
SLS-001 (WT-1)	galinpepimut-S	Sellas Life Sciences Group	vaccine	Acute myeloid leukemia	SC	InTrial	2H2025	Yes	Yes
RPC-4046 (ABT-308)	cendakimab	Bristol Myers Squibb	interleukin-13 inhibitor	Eosinophilic esophagitis	SC	InTrial	2H2025	Yes	Yes
Revascor (NeoFuse, Replicart, MPC-150-IM, MPC-25,	rexlemestrocel-L	Mesoblast	allogeneic autologous mesenchymal precursor cell	Heart failure	IV	InTrial	2H2025	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
MPC-150, MPC-300, CEP-41750)									
GSK-3511294	depemokimab	GlaxoSmithKline	interleukin-5 antagonist	Eosinophilic asthma	SC	InTrial	2H2025	Yes	No
LOU-064	remibrutinib	Novartis	Bruton's tyrosine kinase inhibitor	Chronic spontaneous urticaria	PO	InTrial	2H2025	Yes	No
IONIS-PKK-LRx (ISIS-721744)	donidalorsen	Ionis	antisense drug	Hereditary angioedema	SC	InTrial	2H2025	Yes	Yes
CPI-0610	pelabresib	MorphoSys	BET inhibitor	Myelofibrosis	PO	InTrial	2H2025	Yes	Yes
D-PLEX100	doxycycline	PolyPid	tetracycline	Surgical site infections	IMPLANT	InTrial	2H2025	No	No
XEN-1101	XEN-1101	Xenon Pharmaceuticals	Kv7 potassium channel opener	Focal epilepsy	PO	InTrial	2H2025	TBD	No
CT-041	CT-041	CARsgen Therapeutics	chimeric antigen receptor T cell therapy	Gastric cancer	IV	InTrial	2H2025	Yes	Yes
CRN-00808	paltusotine	Crinetics Pharmaceuticals	somatostatin receptor 2 agonist	Acromegaly	PO	InTrial	2H2025	Yes	Yes
DTX-401	pariglasgene brecaparvovec	Ultragenyx Pharmaceutical	gene therapy	Glycogen storage disease type Ia	IV	InTrial	2H2025	Yes	Yes
TransCon CNP	navepegritide	Ascendis Pharma	C-type natriuretic peptide	Achondroplasia	SC	InTrial	2H2025	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
MCO-010	sonpiretigene Isteparvovec	Nanoscope Therapeutics	gene therapy	Retinitis pigmentosa	Intravitreal	InTrial	2H2025	Yes	Yes
HLX-10	serplulimab	Henlius	anti-PD-1	Small cell lung cancer	IV	InTrial	2H2025	Yes	Yes
IcoSema	insulin icodec/ semaglutide	Novo Nordisk	long-acting insulin analog/ glucagon-like peptide 1 receptor agonist	Diabetes	SC	InTrial	2H2025	No	No
BNT161+ BNT162b2	influenza and COVID-19 vaccine	Pfizer/BioNTech	mRNA	Prevention of influenza and COVID-19 infection	IM	InTrial	2025	No	No
LAI-287	insulin icodec	Novo Nordisk	ultra-long-acting basal insulin	Diabetes mellitus	SC	CRL	2025	No	No
RTT-01	tiratricol	Egetis Therapeutics	thyroid-stimulating hormone receptor	Monocarboxylate transporter 8 deficiency	PO	InTrial	2025	Yes	Yes
APN-311	dinutuximab beta	Recordati	anti-GD2 antigen	Neuroblastoma	IV	InTrial	2025	Yes	Yes
AXS-07	meloxicam/rizatriptan	Axsome Therapeutics	non-steroidal anti-inflammatory drug/triptan	Migraine	PO	CRL	2025	No	No
M-281	nipocalimab	J&J	FcRn antagonist	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
FCX-007 (GM-HDF-COL7, INXN-3002)	dabocemagene autoficel	Castle Creek Pharmaceutical	gene therapy	Epidermolysis bullosa	Intradermal	InTrial	2025	Yes	Yes
pIL-12 (DNA IL-12)	tavokinogene telsaplasmid	OncoSec Medical	gene therapy	Melanoma	Intratumoral	InTrial	2025	Yes	Yes
NRX-101 (Cyclurad)	d-cycloserine/ lurasidone	NeuroRx	N-methyl-D-aspartate receptor modulator/ 5-HT2A receptor antagonist	Bipolar disorder	PO	InTrial	2025	No	No
Hepcludex	bulevirtide	Gilead	HBV receptor binder	Hepatitis delta virus	SC	CRL	2025	Yes	Yes
VNRX-5133	cefepime/ taniborbactam	VenatoRx Pharmaceuticals	cephalosporin/ beta-lactamase inhibitor	Bacterial infections	IV	CRL	2025	No	No
OMS-721	narsoplimab	Omeros	anti-MASP-2 monoclonal antibody	Hematopoietic stem cell transplant-associated thrombotic microangiopathy	IV	CRL	2025	Yes	Yes
STS-101	dihydroergotamine	Satsuma Pharmaceuticals	ergotamine	Migraine	Intranasal	CRL	2025	No	No
MOR-202	felzartamab	I-Mab	anti-CD38 monoclonal antibody	Multiple myeloma	IV	InTrial	2025	Yes	No
REGN-5458	linvoseltamab	Regeneron	BCMA and CD3 bispecific antibody inhibitor	Multiple myeloma	IV	CRL	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
PXT-3003	baclofen/ naltrexone/ sorbitol	Pharnext	GABA-ergic agonist/ opioid receptor antagonist/ sorbitol combination	Charcot-Marie Tooth disease	PO	InTrial	2025	No	Yes
AEB-1102	pegzilarginase	Aeglea BioTherapeutics	enzyme replacement/ arginase-I stimulator	Arginase 1 deficiency	IV	InTrial	2025	Yes	Yes
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
Dasynoc	dasatinib	Xspray Pharma	kinase inhibitor	Chronic myeloid leukemia	PO	CRL	2025	Yes	Yes
CUTX-101	copper histidinate	Fortress Biotech	copper replacement	Menkes Disease	SC	InTrial	2025	Yes	Yes
RG-7433 (ABT-263)	navitoclax	AbbVie	Bcl-2 inhibitor	Myelofibrosis	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
GSK-2330672	linerixibat	GlaxoSmithKline	ileal bile acid transfer inhibitor	Primary biliary cholangitis	PO	InTrial	2025	Yes	Yes
TAK-935	soticlestat	Takeda	cholesterol 24-hydroxylase inhibitor	Lennox-Gastaut syndrome/ Dravet syndrome	PO	InTrial	2025	Yes	Yes
TAVT-45	abiraterone acetate	Tavanta Therapeutics	CYP17 inhibitor	Prostate cancer	PO	InTrial	2025	Yes	No
F-901318	olorofim	F2G	orotomide antifungal	Aspergillosis	PO/IV	CRL	2025	No	Yes
Dihydroergotamine autoinjector	dihydroergotamine	Amneal Pharmaceuticals	ergot derivative	Migraine	SC	InTrial	2025	No	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
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
Sarconeos	BIO-101	Biophytis	MAS G-protein coupled receptor agonist	COVID-19 treatment	PO	InTrial	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
PRGN-2012	PRGN-2012	Precigen	immunotherapy	Respiratory papillomatosis	SC	InTrial	2025	Yes	Yes
Lydolyte	lidocaine	MEDRx	anesthetic agent	Neuropathic pain	TOP	CRL	2025	No	No
HER3-DXd	patritumab deruxtecan	Daiichi Sankyo/ Merck	antibody drug conjugate	Non-small cell lung cancer	IV	CRL	2025	Yes	No
mRNA-1083	influenza and COVID-19 vaccine	Moderna	mRNA	Prevention of influenza and COVID-19	IM	InTrial	2025	No	No
mRNA-1010	mRNA-1010	Moderna	vaccine	Influenza	IM	InTrial	2025	No	No
FE-203799	apraglutide	Ironwood	glucagon-like peptide-2 analog	Short bowel syndrome	SC	InTrial	2025	Yes	Yes
SY-1425	tamibarotene	Syros Pharmaceuticals	retinoic acid receptor alpha agonist	Myelodysplastic syndrome	PO	InTrial	2025	Yes	Yes
AZD-0914	zoliflodacin	Innoviva	DNA gyrase inhibitor	Gonorrhea	PO	InTrial	Late 2025	No	No
CAP-1002	deramiocel	Capricor Therapeutics	cellular therapy	Duchenne muscular dystrophy	IV	InTrial	Late 2025	Yes	Yes
SB-525	giroctocogene fitelparvovec	Pfizer/ Sangamo Therapeutics	gene therapy	Hemophilia A	IV	InTrial	Late 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
RG-6058	tiragolumab	Roche	TIGIT monoclonal antibody	Non-small cell lung cancer/ esophageal cancer	IV	InTrial	Late 2025	Yes	No
AQST-109	epinephrine	Aquestive Therapeutics	non-selective alpha/ beta-adrenergic receptor agonist	Anaphylaxis	PO	InTrial	Late 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	Projected Approval Date
2024 Possible launch date							
Filspari	sparsentan	Travere Therapeutics	endothelin/angiotensin II receptor antagonist	Accelerated to Full Approval	To reduce proteinuria in adults with primary IgA nephropathy at risk of rapid disease progression, generally a urine protein-to-creatinine ratio (UPCR) ≥1.5 g/g	PO	09/05/2024
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 inhibitor	New	Add-on maintenance treatment for adolescents aged 12 to 17 years with inadequately controlled chronic rhinosinusitis with nasal polyposis	SC	09/15/2024
Rybrevant	amivantamab-vmjw	Janssen	bispecific EGF receptor-directed and MET receptor-directed antibody	Revised	In combination with chemotherapy (carboplatin and pemetrexed) for the treatment of patients with locally advanced or metastatic non-small cell lung cancer with EGFR exon 19 deletions or L858R substitution after disease progression on or after osimertinib	PO	09/20/2024
Keytruda	pembrolizumab	Merck	programmed death receptor-1-blocking antibody	New	In combination with chemotherapy, for the first-line treatment of patients with unresectable advanced or metastatic malignant pleural mesothelioma	IV	09/25/2024

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Projected Approval Date
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 inhibitor	New	Maintenance treatment in adult patients with uncontrolled chronic obstructive pulmonary disease with type 2 inflammation	SC	09/27/2024
Sarclisa	isatuximab-irfc	Sanofi	CD38-directed cytolytic antibody	Revised	In combination with bortezomib, lenalidomide and dexamethasone for the treatment of patients with transplant-ineligible newly diagnosed multiple myeloma	IV	09/27/2024
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	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
Tagrisso	osimertinib	AstraZeneca	kinase inhibitor	Revised	Treatment of adult patients with unresectable, stage III epidermal growth factor receptor-mutated non-small cell lung cancer after chemoradiotherapy	PO	10/10/2024
Ocaliva	obeticholic acid	Intercept Pharmaceuticals	farnesoid X receptor	Accelerated to Full Approval	Treatment of adult patients with primary biliary cholangitis without cirrhosis or with compensated cirrhosis who do not have evidence of portal hypertension, either in combination with ursodeoxycholic acid (UDCA) with an inadequate response to UDCA or as monotherapy in patients unable to tolerate UDCA	PO	10/15/2024
Bimzelx	bimekizumab	UCB	interleukin-17A and F antagonist	New	Treatment of hidradenitis suppurativa	SC	12/04/2024

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Projected Approval Date
Nemluvio	nemolizumab-ilto	Galderma	interleukin-31 receptor antagonist	New	Treatment of moderate-to-severe atopic dermatitis	SC	12/12/2024
Vtama	tapinarof	Dermavant Sciences	aryl hydrocarbon receptor agonist	New	Treatment of moderate-to-severe atopic dermatitis in patients 2 years of age and older	TOP	12/14/2024
Imfinzi	durvalumab	AstraZeneca	programmed death-ligand 1 blocking antibody	New	Treatment of patients with limited-stage small cell lung cancer whose disease has not progressed following platinum-based concurrent chemoradiotherapy	IV	12/15/2024
Inpefa	sotagliflozin	Lexicon Pharmaceuticals	sodium-glucose cotransporter 2 inhibitor	New	As an adjunct to insulin therapy for glycemic control in patients with type 1 diabetes and chronic kidney disease	PO	12/20/2024
Bimzelx	bimekizumab	UCB	interleukin-17A and F antagonist	New	Treatment of psoriatic arthritis	SC	12/2024
Bimzelx	bimekizumab	UCB	interleukin-17A and F antagonist	New	Treatment of non-radiographic axial spondyloarthritis	SC	12/2024
Bimzelx	bimekizumab	UCB	interleukin-17A and F antagonist	New	Treatment of ankylosing spondylitis	SC	12/2024
Tevimbra	tislelizumab	BeiGene	programmed death receptor-1–blocking antibody	New	In combination with fluoropyrimidine- and platinum-containing chemotherapy, for the treatment of patients with locally advanced unresectable or metastatic gastric or gastroesophageal junction adenocarcinoma	IV	12/2024
Imcivree	setmelanotide	Rhythm Pharmaceuticals	melanocortin 4 receptor agonist	Revised	Chronic weight management in adult and pediatric patients 2 years of age and older with monogenic or syndromic obesity due to:	SC	4Q2024

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Projected Approval Date
					(1) Pro-opiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1), or leptin receptor (LEPR) deficiency as determined by an FDA-approved test demonstrating variants in POMC, PCSK1, or LEPR genes that are interpreted as pathogenic, likely pathogenic, or of uncertain significance (VUS); or (2) Bardet-Biedl syndrome		
Enhertu	fam-trastuzumab deruxtecan-nxki	AstraZeneca	HER2-directed antibody and topoisomerase inhibitor conjugate	Revised	Third-line treatment of advanced/refractory, metastatic HER2+ breast cancer	IV	2H2024
Fasenra	benralizumab	AstraZeneca	interleukin-5 receptor antibody	New	Treatment of eosinophilic granulomatosis with polyangiitis	SC	2H2024
Zepbound	tirzepatide	Eli Lilly	GIP/GLP-1 receptor agonist	New	Treatment of obstructive sleep apnea in patients with obesity	SC	Late 2024
2025 Possible launch date							
Tremfya	guselkumab	Janssen	interleukin-23 inhibitor	New	Treatment of adults with moderately to severely active ulcerative colitis	IV/SC	01/11/2025
Gemtesa	vibegron	Sumitomo Pharma America	beta-3 adrenergic receptor agonist	Revised	Treatment of men with overactive bladder symptoms receiving pharmacological therapy for benign prostatic hyperplasia	PO	01/13/2025
Scemblix	asciminib	Novartis	kinase inhibitor	Revised	Treatment of newly diagnosed adult patients with Philadelphia chromosome-positive chronic myelogenous leukemia in chronic phase	PO	01/29/2025

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Projected Approval Date
Ozempic	semaglutide	Novo Nordisk	glucagon-like peptide-1 receptor agonist	New	Prevention of progression of renal impairment and risk of renal and cardiovascular mortality in people with type 2 diabetes and chronic kidney disease	SC	01/2025
Rexulti	brexpiprazole	Otsuka/ Lundbeck	atypical antipsychotic	New	In combination with sertraline for the treatment of post-traumatic stress disorder in adults	PO	02/08/2025
Furoscix	furosemide	scPharmaceuticals	diuretic	Revised	Treatment of edema due to fluid overload in patients with chronic kidney disease	SC	03/06/2025
Adcetris	brentuximab vedotin	Pfizer	CD30-directed antibody-drug conjugate	Revised	In combination with lenalidomide and rituximab for patients with relapsed/refractory large B-cell lymphoma	IV	03/2025
Omvoh	mirikizumab-mrkz	Eli Lilly	interleukin-23 antagonist	New	Treatment of adults with moderately to severely active Crohn's disease	IV/SC	1Q2025
Cabometyx	cabozantinib	Exelixis	kinase inhibitor	New	Treatment of adults with previously treated, locally advanced/unresectable or metastatic, well- or moderately differentiated pancreatic neuroendocrine tumors (pNET), and the treatment of adults with previously treated, locally advanced/unresectable or metastatic, well- or moderately differentiated extra-pancreatic NET (epNET)	PO	04/03/2025
Prezcobix	darunavir/ cobicistat	Johnson & Johnson	HIV protease inhibitor/ CYP3A inhibitor	Revised	Treatment of HIV-1 infection in treatment-naïve and treatment-experienced adults and pediatric patients weighing at least 25 kg with no darunavir resistance-associated substitutions	PO	04/04/2025

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Projected Approval Date
Tremfya	guselkumab	Janssen	interleukin-23 inhibitor	New	Treatment of adults with moderately to severely active Crohn's disease	IV/SC	04/20/2025
Rinvoq	upadacitinib	AbbVie	janus kinase inhibitor	New	Treatment of adult patients with giant cell arteritis	PO	05/12/2025
Spravato	esketamine	J&J	NMDA receptor antagonist	Revised	Monotherapy for adults living with treatment-resistant depression	Intranasal	05/22/2025
Zoryve	roflumilast	Arcutis Biotherapeutics	phosphodiesterase 4 inhibitor	New	Treatment of adults and adolescents ages 12 and over with scalp and body psoriasis	TOP	05/23/2025
Abrysvo	respiratory syncytial virus vaccine	Pfizer	vaccine	Revised	Active immunization for the prevention of lower respiratory tract disease caused by RSV in individuals 18 years of age and older	IM	1H2025

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					
SANDOSTATIN LAR DEPOT	octreotide acetate	Novartis	Acromegaly; Carcinoid Tumors; Vasoactive Intestinal Peptide Tumors	Subcutaneous	2H-2024
TASIGNA	nilotinib	Novartis	Philadelphia Chromosome-Positive Chronic Myeloid Leukemia	Oral	2H-2024
NYMALIZE	nimodipine	Arbor	Subarachnoid Hemorrhage	Oral	2H-2024
GIAZO	balsalazide disodium	Bausch Health	Ulcerative Colitis in Male Patients	Oral	2H-2024
TEFLARO	ceftaroline fosamil	Allergan	Community Acquired Pneumonia; Skin and Skin Structure Infections	Intravenous	2H-2024
ISENTRESS	raltegravir	Merck	Human Immunodeficiency Virus-1 Infection	Oral	2H-2024
HUMALOG	insulin lispro	Eli Lilly	Type 1 and 2 Diabetes Mellitus	Subcutaneous	08-2024
NOVOLOG	insulin aspart	Novo Nordisk	Type 1 and 2 Diabetes Mellitus	Subcutaneous	08-2024
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
TYSABRI	natalizumab	Biogen	Multiple Sclerosis; Crohn's Disease	Intravenous	4Q-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

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
PRADAXA	dabigatran etexilate mesylate	Boehringer Ingelheim	Venous Thromboembolic Events in Pediatric Patients	Oral	12-2024
2025 Possible launch date					
GELNIQUE	oxybutynin	Allergan	Overactive Bladder	External	2025
BOSULIF	bosutinib	Pfizer	Chronic Myelogenous Leukemia	Oral	2025
PROMACTA	eltrombopag	Novartis	Thrombocytopenia	Oral	2025
SIMPONI	golimumab	Janssen	Ankylosing Spondylitis; Psoriatic Arthritis; Rheumatoid Arthritis; Ulcerative Colitis	Subcutaneous	2025
SIMPONI ARIA	golimumab	Janssen	Rheumatoid Arthritis; Psoriatic Arthritis; Ankylosing Spondylitis; Juvenile Idiopathic Arthritis	Intravenous	2025
COMPLERA	emtricitabine/rilpivirine/tenofovir disoproxil fumarate	Gilead/Janssen	Human Immunodeficiency Virus-1 Infection	Oral	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
PHOSLYRA	calcium acetate	Fresenius	Phosphate Binder	Oral	01-2025
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
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;	Oral	03-2025

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
			Iron Deficiency Anemia in Adult Patients with CKD Not on Dialysis		
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	Catalyst	Partial-Onset Seizures; Primary Generalized Tonic-Clonic Seizures	Oral	05-2025
PROLIA	denosumab	Amgen	Postmenopausal Osteoporosis; Bone Loss in Men and Women at Risk of Fracture	Subcutaneous	05-2025
XGEVA	denosumab	Amgen	Prevention of Fractures in Bone Malignancies and Multiple Myeloma; Giant Cell Tumor in Bone; Hypercalcemia	Subcutaneous	05-2025
NUCYNTA ER	tapentadol	Collegium	Moderate to Severe Chronic Pain	Oral	06-2025
XOLAIR	omalizumab	Roche/Genentech	Asthma; Idiopathic Urticaria; Nasal Polyps; IgE-Mediated Food Allergy	Subcutaneous	2H-2025
PERJETA	pertuzumab	Genentech	HER-2 Positive Breast Cancer	Intravenous	2H-2025
ENTRESTO	sacubitril/valsartan	Novartis	Heart Failure	Oral	3Q-2025
RAVICTI	glycerol phenylbutyrate	Amgen	Urea Cycle Disorders	Oral	07-2025
RYANODEX	dantrolene	Eagle Pharmaceuticals	Malignant Hyperthermia	Intravenous	07-2025
CARDENE IV	nicardipine	Chiesi	Short-Term Treatment of Hypertension When Oral Therapy is Not Possible	Intravenous	07-2025
RYTARY	carbidopa/levodopa	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
QTERN	dapagliflozin/saxagliptin	AstraZeneca	Type 2 Diabetes Mellitus	Oral	10-2025

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
FUROSCIX	furosemide	scPharmaceuticals	Chronic Heart Failure	Subcutaneous	10-2025
EDURANT	rilpivirine	Janssen	Human Immunodeficiency Virus-1 Infection	Oral	10-2025
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
NUCYNTA	tapentadol	Collegium	Moderate to Severe Acute Pain	Oral	11-2025
OPSUMIT	macitentan	Janssen	Pulmonary Arterial Hypertension	Oral	12-2025
2026 Possible launch date					
FIRMAGON	degarelix	Ferring	Prostate Cancer	Subcutaneous	2026
BRYHALI	halobetasol	Bausch Health	Plaque Psoriasis	External	2026
MAVENCLAD	cladribine	Serono	Multiple Sclerosis	Oral	2026
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	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
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
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

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
QBRELIS	lisinopril	Silvergate	Hypertension; Heart Failure; Acute Myocardial Infarction	Oral	01-2026
BRIVIACT	brivaracetam	UCB	Epilepsy	Oral; intravenous	02-2026
SAVELLA	milnacipran	AbbVie	Fibromyalgia	Oral	03-2026
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
OFEV	nintedanib	Boehringer Ingelheim	Idiopathic Pulmonary Fibrosis; Systemic Sclerosis-Associated Interstitial Lung Disease (ILD); Chronic Fibrosing ILD	Oral	04-2026
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
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
HEMADY	dexamethasone	Acrotech Biopharma	Multiple Myeloma	Oral	10-2026

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
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
AVYCAZ	ceftazidime/avibactam	AbbVie	Intra-Abdominal Infections; Urinary Tract Infections, including Pyelonephritis; Pneumonia; Bacterial Pneumonia	Intravenous	11-2026
IWILFIN	eflornithine	US World Meds	Neuroblastoma	Oral	12-2026
TRINTELLIX	vortioxetine	Takeda/Lundbeck	Major Depressive Disorder	Oral	12-2026
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
SAXENDA	liraglutide	Novo Nordisk	Chronic Weight Management	Subcutaneous	2027
ENTYVIO	vedolizumab	Takeda	Ulcerative Colitis; Crohn's Disease	Intravenous; subcutaneous	2027
IBRANCE	palbociclib	Pfizer	Breast Cancer	Oral	1Q-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
BELEODAQ	belinostat	Acrotech/Aurobindo	Relapsed or Refractory Peripheral T-cell Lymphoma	Intravenous	01-2027
VIBATIV	telavancin	Cumberland	Infections	Intravenous	01-2027

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
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
NEVANAC	nepafenac	Harrow Health	Pain and Inflammation Associated with Cataract Surgery	Ophthalmic	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
DUAVEE	conjugated estrogens/bazedoxifene acetate	Pfizer/Ligand Pharmaceuticals	Treatment of Moderate to Severe Vasomotor Symptoms Associated with Menopause; Prevention of Postmenopausal Osteoporosis	Oral	03-2027
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
XTANDI	enzalutamide	Astellas/Pfizer	Prostate Cancer	Oral	3Q-2027
RELISTOR	methylnaltrexone	Bausch Health	Opioid-Induced Constipation	Subcutaneous	07-2027
MYALEPT	metreleptin	Aegerion	Leptin Deficiency in Patients with Lipodystrophy	Subcutaneous	07-2027

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
DOPTELET	avatrombopag	AkaRx	Thrombocytopenia	Oral	07-2027
PIZENSY	lactitol	Braintree/Sebela	Chronic Idiopathic Constipation	Oral	08-2027
CRESEMBA	isavuconazonium	Astellas	Invasive Aspergillosis; Invasive Mucormycosis	Oral; Intravenous	09-2027
SOLOSEC	secnidazole	Lupin	Bacterial Vaginosis; Trichomoniasis	Oral	09-2027
WAKIX	pitolisant	Harmony Biosciences	Narcolepsy	Oral	10-2027
BRONCHITOL	mannitol	Arna Pharma	Cystic Fibrosis	Inhalation	10-2027
TALICIA	amoxicillin/rifabutin/omeprazole	Redhill Biopharma	Helicobacter pylori	Oral	11-2027
FANAPT	iloperidone	Vanda	Schizophrenia; Bipolar Disorder	Oral	11-2027
NUCALA	mepolizumab	GSK	Severe Asthma; Rhinosinusitis with Nasal Polyps; Eosinophilic Granulomatosis with Polyangitis; Hypereosinophilic Syndrome	Subcutaneous	11-2027
ZOKINVY	lonafarnib	Sentynl Therapeutics	Hutchinson-Gilford Progeria Syndrome	Oral	11-2027
MEKINIST	trametinib dimethyl sulfoxide	Novartis/GSK	Melanoma; Non-Small Cell Lung Cancer; Anaplastic Thyroid Cancer; Glioma; Solid Tumors	Oral	11-2027
TRULICITY	dulaglutide	Eli Lilly	Type 2 Diabetes Mellitus	Subcutaneous	12-2027
ZONTIVITY	vorapaxar sulfate	Key Pharma	Reduction of Thrombotic Cardiovascular Events in Patients with a History of Myocardial Infarction or with Peripheral Arterial Disease	Oral	12-2027
ADYNOVATE	antihemophilic factor recombinant pegylated	Takeda	Hemophilia A	Intravenous	12-2027

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