RxOutlook®

4th Quarter 2023

Optum Rx®

Welcome to the fourth quarterly RxOutlook Report of 2023. Optum Rx closely monitors and evaluates the drug development pipeline to identify noteworthy upcoming drug approvals and reports the essential findings here in RxOutlook.

Recap of 2023

As of November 20th, the FDA's Center for Drug Evaluation and Research (CDER) has approved 51 novel drugs in 2023. Chronic inflammatory disease, which is already a leading category of drug spend, saw significant activity since the third quarter RxOutlook report. This included the approval of three novel drugs: **Velsipity™ (etrasimod),** the second S1P modulator for ulcerative colitis; **Bimzelx® (bimekizumab-bkzx),** the first dual IL-17A and IL-17F antagonist for plaque psoriasis; and **Omvoh™ (mirikizumab-mrkz),** the first selective IL-23 antagonist for ulcerative colitis.

In addition to these novel drugs, the FDA approved the first self-administered, subcutaneous (SC) formulations of **vedolizumab (Entyvio®),** for ulcerative colitis, and **infliximab (Zymfentra™),** for ulcerative colitis and Crohn's disease. Both drugs were previously only available as intravenous (IV) products. The new SC formulations are notable because they will potentially shift drug spend for these products from the Medical to Pharmacy Benefit.

Looking Ahead to the 1st Quarter 2024

In this edition of RxOutlook, we highlight seven key pipeline products with an approval decision by the end of the first quarter 2024.

Resmetirom is a novel thyroid hormone receptor (THR)-β agonist under FDA review for the treatment of nonalcoholic steatohepatitis (NASH). The approval decision for resmetirom will be one of the most watched FDA decisions of next year because there are currently no approved treatments for NASH, a condition with high prevalence in the U.S.

Sotatercept, a first-in-class activin receptor type IIA fusion protein, would provide an add-on treatment option for patients with pulmonary arterial hypertension (PAH). The current standard of care for PAH includes endothelin receptor antagonists, phosphodiesterase-5 inhibitors, and drugs targeting the prostacyclin pathway; however, the disease continues to be associated with high morbidity and mortality despite currently available therapies.

Continuing the wave of approvals that began in 2022, two more gene therapies for ultra-rare diseases cold be approved at the beginning of 2024 - **Libmeldy (atidarsagene autotemcel)** for metachromatic leukodystrophy, and **marnetegragene autotemcel** for leukocyte adhesion deficiency type I. Both genetic diseases are associated with a poor prognosis and the only treatment currently available is hematopoietic stem cell transplant (HSCT). HSCT can slow disease progression in MLD and can be curative for LAD-I but not all affected patients have a matched donor.

Roluperidone is a novel antipsychotic and if approved, would be the first treatment specifically for negative symptoms associated with schizophrenia. Negative symptoms are symptoms that are abnormally absent, such as a lack of motivation or inability to feel pleasure. Unlike atypical antipsychotics, roluperidone avoids direct blockade of dopaminergic receptors.

Iptacopan is a first-in-class factor B complement inhibitor for paroxysmal nocturnal hemoglobinuria (PNH). Aside from a novel mechanism of action, iptacopan is notable because it is potentially the first oral therapy approved for PNH, and it demonstrated efficacy in patients who failed existing injectable complement inhibitors (eg, Soliris®, Ultomiris®).

Finally, **zolbetuximab** is a novel monoclonal antibody for HER2-negative advanced or metastatic gastric cancer. Zolbetuximab works by binding to Claudin 18.2 (CLDN18.2), a biomarker expressed on gastric epithelial cells. Gastric cancer is associated with poor 5-year survival so there is a high unmet need for additional treatment options, particularly patients with the HER2-negative subtype.

Approval decisions for other key novel therapies are expected by the end of the 1st quarter 2024 but are not reviewed in this report because they were covered in previous editions of RxOutlook. This includes **donanemab** for Alzheimer's disease and **vadadustat** for chronic kidney disease-associated anemia.

Key pipeline drugs with FDA approval decisions expected by end of the 1st quarter 2024

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Iptacopan	Novartis	Paroxysmal nocturnal hemoglobinuria*	4Q 2023
Zolbetuximab	Astellas	Gastric cancer*	1/12/2024
Roluperidone	Minerva Neurosciences	Schizophrenia	2/26/2024
Resmetirom	Madrigal Pharmaceuticals	Nonalcoholic steatohepatitis	3/14/2024
Libmeldy (atidarsagene autotemcel)	Orchard Therapeutics	Metachromatic leukodystrophy*	3/18/2024
Sotatercept	Merck	Pulmonary arterial hypertension*	3/26/2024
Marnetegragene autotemcel	Rocket Pharmaceuticals	Leukocyte adhesion deficiency-I*	3/31/2024

* Orphan Drug Designation

Detailed Drug Insights

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

Read more

Extended Brand Pipeline Forecast

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

Read more

Key Pending Indication Forecast

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

Read more

Extended Generic Pipeline Forecast

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

Please note that RxOutlook highlights select near-term approvals. Some drugs may not appear in this issue because they have been reviewed in previous editions of RxOutlook. Drugs of interest that are earlier in development or with expected approvals beyond 1st 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

Iptacopan (Brand Name: To be determined)

Manufacturer: Novartis
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 4Q 2023

Therapeutic use

Iptacopan is under review for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH).

PNH is a rare, complement-mediated blood disorder in which an acquired mutation in a patient's hematopoietic stem cells causes the production of defective red blood cells (RBCs). These defective RBCs are susceptible to premature destruction (hemolysis) by a patient's own immune system (complement system). In addition to hemolysis, PNH can result in potentially life-threatening blood clots and bone marrow dysfunction.

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

Clinical profile

Iptacopan is a targeted factor B inhibitor of the alternative complement pathway. It acts upstream of the C5 terminal pathway, preventing not only intravascular but also extravascular hemolysis in PNH.

Pivotal trial data:
The efficacy of iptacopan was evaluated in APPLY-PNH, a Phase 3, randomized, open-label, active-controlled study in 97 adult patients with PNH and residual anemia, despite treatment with an intravenous anti-C5 antibody. Patients were randomized to iptacopan or to continue their current standard of care (SoC) (eg, Soliris® [eculizumab] or Ultomiris® [ravulizumab-cwvz]). The co-primary endpoints were the proportion of patients with a ≥ 2 g/dL hemoglobin (Hb) increase from baseline and the proportion of patients with Hb ≥ 12 g/dL, each in the absence of RBC transfusions. Both endpoints were assessed at Week 24.

An estimated 82.3% of iptacopan-treated vs. 2.0% of SoC-treated patients had a ≥ 2 g/dL Hb increase from baseline (difference: 80.3, 95% CI: 71.3, 87.6; $p < 0.0001$); an estimated 68.8% vs. 1.8% achieved Hb ≥ 12 g/dL, respectively (difference: 67.0, 95% CI: 56.3, 76.9; $p < 0.0001$).

What you need to know:

Proposed Indication: Treatment of patients with PNH

Mechanism: Factor B inhibitor

Efficacy:
Treatment-experienced patients:

- Hb increase ≥ 2 g/dL at Week 24: 82.3% with iptacopan vs. 2.0% with SoC
- Hb ≥ 12 g/dL at Week 24: 68.8% with iptacopan vs. 1.8% with SoC

Treatment-naïve patients:

- Hb increase ≥ 2 g/dL at Week 24: 92.2% with iptacopan
- Hb ≥ 12 g/dL at Week 24: 62.8% with iptacopan

Common AEs: Headache, diarrhea

Dosing: Oral twice daily

Why it Matters: Novel mechanism of action, oral administration (current standard of care is injectable biologics), promising efficacy (particularly in treatment-experienced patients), potential future indications (eg, IgA nephropathy, C3 glomerulopathy)

Important to Note: Lack of robust head-to-head data vs. complement inhibitors in treatment-naïve patients, risk of serious infections

Estimated Cost: ~$460,000 per year (based on pricing for Empaveli)

Iptacopan *(continued...)*

In addition to APPLY-PNH, iptacopan was evaluated in APPOINT-PNH, a Phase 3, open-label, single-arm study in adult PNH patients who were naïve to complement inhibitor therapy. The primary endpoint was the proportion of patients with a ≥ 2 g/dL Hb increase from baseline in the absence of RBC transfusions at 24 weeks. A key secondary endpoint was the proportion of patients achieving sustained Hb levels of ≥ 12 g/dL in the absence of RBC transfusions.

With iptacopan treatment, an estimated 92.2% of patients (95% CI: 82.5, 100) achieved ≥ 2 g/dL Hb level increase from baseline. An estimated 62.8% (95% CI: 47.5, 77.5) of patients achieved Hb levels of ≥ 12 g/dL.

Safety:
The most common adverse events with iptacopan use were headache and diarrhea.

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

Competitive environment

If approved, iptacopan would provide a first-in-class, oral alternative to the current injectable standard of care. Complement inhibitors currently approved for PNH include intravenously (IV) administered Soliris and Ultomiris, and subcutaneously (SC) administered Empaveli® (pegcetacoplan).

The results from the APPLY-PNH trial demonstrate that iptacopan could provide an additional treatment option in patients who continue to experience anemia despite prior complement therapy. Additionally, the efficacy data from APPOINT-PNH in treatment-naïve patients were promising with most patients achieving the primary endpoint. However, there is a lack of robust data comparing iptacopan vs. complement inhibitors in treatment-naïve patients.

Iptacopan appears to be well tolerated but long-term safety data will be needed to assess the risk of serious infections, which is a current boxed warning for complement inhibitors and could be a risk with iptacopan given its mechanism of action.

PNH is a rare disorder so the initial target population for iptacopan will be relatively small. Novartis is currently evaluating iptacopan for several other indications (eg, C3 glomerulopathy, IgA nephropathy, atypical hemolytic uremic syndrome), and if these future studies are positive, that would significantly increase patients eligible for treatment with iptacopan.

For reference, the Wholesale Acquisition Cost (WAC) for Empaveli is approximately $460,000 per year.

Zolbetuximab (Brand Name: To be determined)

Manufacturer: Astellas
Regulatory designation: Orphan Drug
Expected FDA decision: January 12, 2024

Therapeutic use

Zolbetuximab is under review for first-line treatment of patients with locally advanced unresectable or metastatic human epidermal growth factor receptor 2 (HER2)-negative gastric or gastroesophageal junction (GEJ) adenocarcinoma whose tumors are Claudin 18.2 (CLDN18.2)-positive.

In the U.S., an estimated 26,500 people will be diagnosed with gastric cancer and 11,130 will die from the disease annually.

About 80% of gastric cancer cases are HER2-negative and in the Phase 3 studies for zolbetuximab, approximately 38% of patients screened had tumors that were CLDN18.2-positive.

Clinical profile

Zolbetuximab acts by binding to CLDN18.2 on the cancer cell surface of gastric epithelial cells. In pre-clinical studies, this binding interaction then induces cancer cell death by activating two distinct immune system pathways – antibody-dependent cellular cytotoxicity and complement-dependent cytotoxicity.

Pivotal trial data:
The efficacy of zolbetuximab was evaluated in two Phase 3, randomized, double-blind studies: SPOTLIGHT and GLOW.

What you need to know:

Proposed Indication: First-line treatment of patients with locally advanced unresectable or metastatic HER2-negative gastric or GEJ adenocarcinoma whose tumors are CLDN18.2-positive

Mechanism: Monoclonal antibody targeting CLDN18.2

Efficacy: Median PFS:

- SPOTLIGHT trial: 10.61 months with zolbetuximab plus mFOLFOX6 vs. 8.67 months with placebo plus mFOLFOX6
- GLOW trial: 8.21 months with zolbetuximab plus CAPOX vs. 6.80 months with placebo plus CAPOX

Common AEs: Nausea, vomiting, decreased appetite

Dosing: IV once every 3 weeks

Why it Matters: First targeted therapy for CLDN18.2-positive gastric cancer, demonstrated superiority in combination with chemotherapy vs. chemotherapy alone, high unmet need

Important to Note: Narrow initial indication, competing with established therapies (eg, Opdivo) in HER2-negative subset of patients

Estimated Cost: ~$187,000 per year (based on pricing for Opdivo)

SPOTLIGHT compared zolbetuximab plus mFOLFOX6 (a combination regimen that includes oxaliplatin, leucovorin and fluorouracil) vs. placebo plus mFOLFOX6 as a first-line treatment in 565 patients with locally advanced unresectable or metastatic HER2-negative gastric or GEJ adenocarcinoma whose tumors were CLDN18.2-positive. The primary endpoint was progression-free survival (PFS). A key secondary endpoint was overall survival (OS).

Zolbetuximab treatment demonstrated a reduction in the risk of disease progression or death compared with placebo (hazard ratio [HR] 0.75, 95% CI: 0.60, 0.94; p = 0.0066). The median progression-free survival was 10.61 months (95% CI: 8.90, 12.48) in the zolbetuximab group vs. 8.67 months (95% CI: 8.21, 10.28) in the placebo group. Zolbetuximab treatment also showed a reduction in the risk of death vs. placebo (HR 0.75, 95% CI: 0.60, 0.94; p = 0.0053).

GLOW compared zolbetuximab plus CAPOX (a combination chemotherapy regimen which includes capecitabine and oxaliplatin) vs. placebo plus CAPOX as a first-line treatment in 507 patients with locally advanced unresectable or metastatic HER2-negative gastric or GEJ cancer whose tumors were CLDN18.2 positive. The primary endpoint was PFS. A key secondary endpoint was OS.

Zolbetuximab *(continued...)*

Zolbetuximab treatment demonstrated a reduction in the risk of disease progression or death compared with placebo (HR 0.687, 95% CI: 0.544, 0.866; p = 0.0007). Median PFS was 8.21 months (95% CI: 7.46, 8.84) in the zolbetuximab group vs. 6.80 months (95% CI: 6.14, 8.08) in the placebo group. Zolbetuximab treatment also demonstrated a reduction in the risk of death by 22.9% vs. placebo (HR 0.771, 95% CI: 0.615, 0.965; p = 0.0118).

Safety:
The most common adverse events with zolbetuximab use were nausea, vomiting, and decreased appetite.

In VISION-DMD, height percentile declined in prednisone-treated (not vamorolone-treated) participants (change from baseline: prednisone, -1.88 percentile vs. vamorolone 6 mg/kg per day, +3.86 percentile; p = 0.02). Additionally, bone turnover markers declined with prednisone but not with vamorolone.

Dosing:
In the pivotal trials, zolbetuximab was administered once as a loading dose and then once every 3 weeks via IV infusion.

Competitive environment

Zolbetuximab is a novel monoclonal antibody and the first treatment specifically targeting CLDN18.2 in gastric cancer cells. There is a high unmet need for additional treatment options for gastric cancer – overall 5-year relative survival is 35.7% and falls to only 6.6% once patients have metastatic disease. Unlike many investigational oncology drugs where data is limited to overall response rates, the FDA submission for zolbetuximab includes both PFS and OS data.

The target population for zolbetuximab is expected to be small given the narrow initial indication; however, this could grow if future studies evaluating additional uses for zolbetuximab are positive. While treatment options are limited for HER2-negative gastric cancer, Opdivo® (nivolumab), a PD-1 targeted therapy, is approved for a similar indication and is a more established cancer treatment.

For reference, the WAC for Opdivo is approximately $187,000 per year.

Roluperidone (Brand Name: To be determined)

Manufacturer: Minerva Neurosciences
Expected FDA decision: February 26, 2024

Therapeutic use

Roluperidone is under review for the treatment of negative symptoms in patients with schizophrenia.

Schizophrenia is a mental disorder characterized by disruptions in thought processes, emotional responsiveness, and social interactions. People with the disorder may not be able to distinguish between real and unreal experiences. Schizophrenia symptoms vary from patient to patient, but two major categories include positive and negative symptoms. Positive symptoms involve distortions of reality and include hallucinations, delusions, paranoia, and exaggerated perceptions. Negative symptoms involve the absence or reduction of normal processes such as a loss or a decrease in the ability to initiate plans, speak, express emotion, or find pleasure.

Schizophrenia is typically diagnosed in the late teen years to early thirties. Estimates of the prevalence of schizophrenia and related psychotic disorders in the U.S. range between 0.25% and 0.64%.

What you need to know:

Proposed Indication: Treatment of negative symptoms in patients with schizophrenia

Mechanism: Serotonin (5-HT2A), sigma2, adrenergic-α1A receptor antagonist

Efficacy: Change from baseline in the PANSS-derived NSFS or PANSS negative factor score:

- Phase 3 trial: −4.0 to −4.3 with roluperidone vs. −3.5 with placebo (not statistically significant)
- Phase 2b trial: −3.07 to −3.50 with roluperidone vs. −1.53 with placebo

Common AEs: Headache, anxiety, asthenia, nausea, somnolence

Dosing: Oral once daily

Why it Matters: Potentially the first approved drug for negative symptoms of schizophrenia, unique mechanism that may reduce risk of some adverse events associated with atypical antipsychotics

Important to Note: Mixed trial results, lack of data with combination use with other antipsychotics

Estimated Cost: ~$19,000 per year (based on pricing for Caplyta)

Clinical profile

Roluperidone avoids a direct blockade of dopaminergic receptors (the key pharmacological target for current first- and second-generation antipsychotics), while maintaining blockade of a specific subtype of serotonin receptor called 5-HT2A (an additional key target of second-generation antipsychotics) as well as additional pharmacological targets (sigma2 and adrenergic-α1A).

Pivotal trial data:
The efficacy of roluperidone was evaluated in a Phase 3, randomized, double-blind, placebo-controlled study in 513 patients with schizophrenia with moderate to severe negative symptoms. Patients were randomized to roluperidone 32 mg daily, roluperidone 64 mg daily, or placebo. The primary endpoint was the change from baseline in the Positive and Negative Syndrome Scale (PANSS)-derived negative symptom factor score (NSFS) at Week 12.

NSFS were numerically lower with roluperidone treatment, however the difference was not statistically significant vs. placebo. The change from baseline in the least squares mean (LSM) of the NSFS was −4.0 with roluperidone 32 mg, −4.3 with 64 mg roluperidone, and −3.5 with placebo.

Roluperidone was also evaluated in a Phase 2b study with a similar design and patient population as the Phase 3 study. The primary endpoint was the PANSS negative factor score. Both doses of roluperidone demonstrated a statistically significant improvement in the primary endpoint vs. placebo. The change from baseline in the PANSS negative factor score LSM was −3.07 with roluperidone 32 mg ($p = 0.024$ vs. placebo), −3.50 with roluperidone 64 mg ($p = 0.004$ vs. placebo), and −1.53 with placebo.

Roluperidone *(continued...)*

Safety:
The most common adverse events with roluperidone use were headache, anxiety, asthenia, nausea, and somnolence.

Dosing:
In the pivotal trials, roluperidone was administered orally once daily.

Competitive environment

The standard of care for schizophrenia is atypical antipsychotics, which primarily interfere with dopamine neurotransmission. These drugs can provide significant benefit for the positive symptoms associated with schizophrenia but have a more marginal impact on negative symptoms. Atypical antipsychotic use can also be limited due to safety and tolerability.

Roluperidone would be the first drug FDA approved specifically for negative symptoms of schizophrenia. Due to its unique mechanism, it may reduce the risk of common adverse events associated with atypical antipsychotics (eg, weight gain). However, the trial results for roluperidone were mixed, with the lone Phase 3 trial failing to meet its primary endpoint.

In addition to the modest efficacy results, there were several limitations in the pivotal trials, which could jeopardize the approval of roluperidone or limit its use in practice. First, it was only evaluated in patients who were stable for their positive symptoms of schizophrenia. Roluperidone was only evaluated as a monotherapy and so it is unknown how the drug would interact with current atypical antipsychotics. Finally, the Phase 2b pivotal study, which did meet its primary endpoint, was conducted exclusively in Europe, so the results may not be generalizable to a U.S. population.

For reference, the WAC for Caplyta® (lumateperone), an atypical antipsychotic available only as a brand, is approximately $19,000 per year.

Sotatercept (Brand Name: To be determined)

Manufacturer: Merck
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: March 26, 2024

Therapeutic use

Sotatercept is under review for the treatment of adult patients with pulmonary arterial hypertension (PAH) (World Health Organization [WHO] Group 1).

PAH is a rare, progressive disease characterized by high blood pressure in the arteries of the lungs, usually due to an unknown cause. High blood pressure in the lungs causes the heart to work harder to pump blood, which can eventually lead to heart failure.

PAH is most common in women between the ages of 30 to 60 years. Approximately 500 to 1,000 new cases of PAH are diagnosed each year in the U.S., and it is estimated that 40,000 people are living with the disease.

Clinical profile

Sotatercept is an activin receptor type IIA fusion protein. PAH is caused by hyperproliferation of cells in the arterial walls in the lung, leading to narrowing and abnormal constriction. Sotatercept works by modulating vascular cell proliferation and reversing vascular and right ventricle remodeling.

What you need to know:

Proposed Indication: Treatment of adult patients with PAH (WHO Group 1)

Mechanism: Activin receptor type IIA fusion protein

Efficacy: Median change from baseline in 6-MWD: 34.4 meters vs. 1.0 meters with placebo

Common AEs: Epistaxis, dizziness, telangiectasia, increased hemoglobin levels, thrombocytopenia, increased blood pressure

Dosing: SC once every 3 weeks

Why it Matters: Novel mechanism of action, promising efficacy results with improvements in function and symptoms, high unmet need

Important to Note: Likely reserved as a third- or fourth-line therapy, SC administration, lack of long-term data

Estimated Cost: ~$260,000 per year (based on pricing for Uptravi)

Pivotal trial data:
The efficacy of sotatercept was evaluated in STELLAR, a phase 3, randomized, double-blind, placebo-controlled study in 323 adults with PAH (WHO functional class II or III) who were receiving stable background therapy. Patients were randomized to receive sotatercept or placebo every 3 weeks. The primary endpoint was the change from baseline at Week 24 in the 6-minute walk distance (6-MWD). A key secondary endpoint was time to death or clinical worsening, which was assessed when the last patient completed the Week 24 visit.

The median change from baseline at Week 24 in the 6-MWD was 34.4 meters (95% CI: 33.0, 35.5) in the sotatercept group and 1.0 meters (95% CI: −0.3, 3.5) in the placebo group. The estimate of the difference between the sotatercept and placebo groups in the change from baseline at Week 24 in the 6-MWD was 40.8 meters (95% CI: 27.5, 54.1; $p < 0.001$).

There was a significant difference in the distribution of time to first occurrence of death or nonfatal clinical worsening event in the sotatercept and placebo groups ($p < 0.001$). After a median follow-up of 32.7 weeks across the groups, the hazard ratio in the sotatercept group as compared with the placebo group was 0.16 (95% CI: 0.08, 0.35).

Safety:
The most common adverse events with sotatercept use were epistaxis, dizziness, telangiectasia, increased hemoglobin levels, thrombocytopenia, and increased blood pressure.

Dosing:
In the pivotal trial, sotatercept was administered SC every 3 weeks.

Sotatercept *(continued...)*

Competitive environment

The current standard of care for PAH treatment includes endothelin receptor antagonists (ERAs), phosphodiesterase-5 (PDE-5) inhibitors, soluble guanylate cyclase stimulators, and drugs targeting the prostacyclin pathway. These treatments can improve patient function and progression-free survival, but do not cure the condition. Despite available therapy, median survival ranges from 5 to 7 years after diagnosis.

Sotatercept would offer a first-in-class treatment for PAH that could be used in addition to existing treatment options. Improvements in exercise capacity and other secondary endpoints were demonstrated in the overall population and across most prespecified subgroups in the pivotal study, including patients receiving triple background therapy with SC or intravenous (IV) prostacyclin analogues.

Sotatercept's initial place in therapy will be limited based on the population studied in the pivotal STELLAR study. STELLAR only included PAH patients with functional class II or III with certain forms of PAH and the overwhelmingly majority (95%) were already on dual or triple combination therapy. Additionally, sotatercept requires SC injection whereas most front-line treatments for PAH are now available as oral or inhaled therapies. The trial results for sotatercept appear promising in treatment-experienced patients; however, long-term durability of response is still unknown.

For reference, the WAC for Uptravi® (selexipag), a prostacyclin receptor agonist, is approximately $260,000 per year.

Resmetirom (Brand Name: To be determined)

Manufacturer: Madrigal Pharmaceuticals
Regulatory designations: Breakthrough Therapy, Fast Track
Expected FDA decision: March 14, 2024 (Accelerated Approval Pathway)

Therapeutic use

Resmetirom is under review for the treatment of patients with nonalcoholic steatohepatitis (NASH) with liver fibrosis.

NASH is a progressive liver disease caused by excessive fat accumulation in the liver that leads to inflammation and liver injury. Progressive liver scarring (fibrosis) can lead to cirrhosis, liver failure, cancer, and death. Additionally, patients with NASH, especially those with other comorbidities (eg, hypertension, type 2 diabetes), are at increased risk for adverse cardiovascular events.

The prevalence of NASH in the general population is between 1.5% to 6.45%.

Clinical profile

Resmetirom is a selective thyroid hormone receptor (THR)-β agonist. Selectivity for THR-β may provide metabolic benefits of thyroid hormone that are mediated by the liver, including reduction of excess hepatic fat, atherogenic lipids, and lipoproteins, while avoiding systemic actions of excess thyroid hormone in heart and bone that are largely mediated through THR-α.

Pivotal trial data:
The efficacy of resmetirom was evaluated in MAESTRO-NASH, a Phase 3, randomized, double-blind, placebo-controlled study in 966 patients with liver biopsy-confirmed NASH. Most patients (95%) had moderate or advanced fibrosis (F2/F3 fibrosis score). Patients received resmetirom 80 mg, resmetirom 100 mg, or placebo once daily. The dual primary surrogate endpoints on biopsy were (1) NASH resolution with ≥ 2-point reduction in NAFLD Activity Score (NAS), and with no worsening of fibrosis; and (2) a 1-point decrease in fibrosis with no worsening of NAS after 52 weeks of treatment. A key secondary endpoint was LDL cholesterol level.

NASH resolution was achieved in 30%, 26%, and 10% of patients receiving resmetirom 100 mg, resmetirom 80 mg, and placebo, respectively ($p < 0.0001$ for both resmetirom doses vs. placebo).

Fibrosis improvement was achieved in 26%, 24%, and 14% of patients receiving resmetirom 100 mg, resmetirom 80 mg, and placebo, respectively ($p < 0.0001$ for both resmetirom doses vs. placebo).

LDL cholesterol was reduced by 14% and 16% with resmetirom 100 mg, resmetirom 80 mg vs. no change with placebo ($p < 0.0001$ for both resmetirom doses vs. placebo).

What you need to know:

Proposed Indication: Treatment of patients with NASH with liver fibrosis

Mechanism: Selective THR-β agonist

Efficacy:

- NASH resolution: 30%, 26%, and 10% of patients with resmetirom 100 mg, resmetirom 80 mg, and placebo, respectively
- Fibrosis improvement: 26%, 24%, and 14% of patients with resmetirom 100 mg, resmetirom 80 mg, and placebo, respectively

Common AEs: Diarrhea, nausea

Dosing: Oral once daily

Why it Matters: Potentially the first FDA approved therapy for NASH, large potential target population, appears well tolerated, oral and once daily administration

Important to Note: Lack of long-term data (eg, all-cause mortality, significant liver events), potential future competition (eg, GLP-1 receptor agonists)

Resmetirom *(continued...)*

Safety:
The most common adverse events with resmetirom use were diarrhea and nausea.

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

Competitive environment

Resmetirom is a novel THR-β selective agonist and potentially the first FDA approved treatment for NASH. NASH is a very common chronic condition in the U.S. with millions of patients potentially eligible for treatment. The current first line treatment for NASH is lifestyle modifications – primarily weight loss. A reduction in weight can not only reduce inflammation in the liver but also potentially improve fibrosis. However, only a small subset of patients with NASH can achieve adequate weight loss. Off-label use of glucagon-like peptide-1 (GLP-1) receptor agonists (eg, semaglutide), insulin-sensitizing agents (eg, pioglitazone), and vitamin E can be used in select patients but the data for these therapies in NASH is limited and none have been shown to improve fibrosis.

In June 2023, the FDA declined to approve Intercept Pharmaceuticals' obeticholic acid for NASH, based on efficacy and safety concerns. Unlike obeticholic acid, resmetirom met both of its dual primary efficacy endpoints and it appears to have a better safety and tolerability profile. Notably, obeticholic acid treatment caused a transient increase in LDL cholesterol whereas resmetirom reduces LDL cholesterol levels.

The initial FDA approval decision for resmetirom is through the accelerated approval pathway and while the available efficacy data is promising, the outcomes are considered surrogate endpoints. A long-term outcomes trial is ongoing that will evaluate the impact of resmetirom on all-cause mortality, liver transplant, and significant hepatic events, including potential hepatic decompensation events. This trial is not expected to complete until second half 2025.

Other drugs are currently under development for treatment of NASH, including GLP-1 receptor agonists such as tirzepatide and semaglutide. A Phase 3 trial for semaglutide is expected to complete sometime in 2025 and if positive, would be a potential competitor to resmetirom.

Atidarsagene autotemcel (Brand Name: Libmeldy)

Manufacturer: Orchard Therapeutics
Regulatory designations: Orphan Drug
Expected FDA decision: March 18, 2024

Therapeutic use

Atidarsagene autotemcel is under review for the treatment of early-onset metachromatic leukodystrophy (MLD).

MLD is a rare genetic disease characterized by accumulation of fats called sulfatides in cells. MLD is primarily caused by mutations in the ARSA gene which causes a deficiency of the enzyme arylsulfatase A and a decreased ability to break down sulfatides.

Sulfatide accumulation in myelin-producing cells causes progressive destruction of white matter (leukodystrophy) throughout the nervous system. Symptoms vary by MLD subtype but can include difficulty talking, seizures, difficulty walking, personality changes, and behavior and personality changes.

MLD is estimated to occur in approximately one in every 100,000 live births.

What you need to know:

Proposed Indication: Treatment of early-onset MLD

Mechanism: Gene therapy delivering ARSA gene

Efficacy: Treatment resulted in statistically significant improvement in sMFS (refer to the pivotal trial data section for complete details)

Safety: Data is limited

Dosing: IV as a one-time dose

Why it Matters: Promising preservation of motor function, significant unmet need

Important to Note: Small target population, complex patient journey including intensive myeloablative conditioning therapy

Estimated Cost: $3 million for a one-time dose (based on pricing for Skysona)

Clinical profile

Libmeldy is an ex vivo gene therapy that contains autologous (patient-derived) hematopoietic stem cells that have been genetically modified with a lentiviral vector to deliver a functional copy of the ARSA gene.

Pivotal trial data:
The efficacy of Libmeldy for MLD was evaluated in two prospective non-randomized clinical studies (N = 30) or treated under expanded access frameworks (N = 9) and compared with natural history data from 49 untreated patients. The primary composite endpoint was severe motor impairment-free survival (sMFS), defined as the interval from birth to the first occurrence of loss of locomotion and loss of sitting without support or death.

At the time of the updated integrated analysis (median follow-up 6.76 years, range 0.64, 12.19), treatment with Libmeldy resulted in statistically significant improvement in sMFS in the pre-symptomatic late infantile ($p < 0.001$), pre-symptomatic early juvenile ($p = 0.042$) and early-symptomatic early juvenile ($p < 0.001$) MLD subgroups compared to matched untreated natural history patients.

Of the 18 pre-symptomatic late infantile patients, 17 maintained the ability to walk at last assessment, in contrast to untreated late infantile natural history patients, all of whom lost all locomotion by a median age of 2.6 years.

All 7 surviving pre-symptomatic early juvenile patients maintained the ability to walk without support with quality and performance normal for age at last assessment, and 7 of 9 surviving early-symptomatic early juvenile patients maintained the ability to sit without support and/or crawl/roll at last assessment, in contrast with untreated early juvenile natural history patients, all of whom lost all locomotion by a median age of 6.4 years.

Atidarsagene autotemcel *(continued...)*

Safety:
Safety data is limited, but no Libmeldy-related serious adverse events have been reported to date.

Dosing:
In the pivotal trial, patient's hematopoietic stem cells (HSCs) were collected via apheresis. Myeloablative conditioning with busulfan preceded IV infusion with Libmeldy.

Competitive environment

There is no cure for MLD, and bone marrow transplantation can only delay progression of the disease in some infantile-onset cases. Most children with the infantile form die by age 5. Symptoms of the juvenile form progress with death occurring 10 to 20 years following onset. People affected by the adult form typically die within 6 to 14 years following onset of symptoms.

Libmeldy would be the first approved treatment for MLD. The data available are promising, particularly in pediatric patients who have not yet developed symptoms. Relative to many gene therapies pursuing FDA approval, the duration of follow-up for Libmeldy is relatively long with a median follow-up of nearly 7 years as of the last data cut-off.

Libmeldy, as an ex vivo gene therapy, is complex to prepare and administer. The process from collecting a patient's own cells to administering the final genetically modified product will take several months and requires myeloablative conditioning and extensive monitoring. The eligible target population will be very small given the rarity of the disease and because gene therapy will likely be reserved for patients with late infantile or early juvenile forms of the condition.

For reference, the WAC for Skysona™ (elivaldogene autotemcel), a recently approved gene therapy for another ultra-rare disease (cerebral adrenoleukodystrophy), is $3 million for a one-time dose.

Marnetegragene autotemcel (Brand Name: To be determined)

Manufacturer: Rocket Pharmaceuticals
Regulatory designations: Orphan Drug, Fast Track
Expected FDA decision: March 31, 2024

Therapeutic use

Marnetegragene autotemcel is under review for the treatment of severe leukocyte adhesion deficiency-I (LAD-I).

LAD-I is a rare, genetic immunodeficiency caused by mutations in the ITGB2 gene, which results in deficient levels or defective CD18. CD18 facilitates leukocyte adhesion and extravasation from blood vessels to combat infections. Patients with severe LAD-I suffer from recurrent life-threatening bacterial and fungal infections starting from infancy. Without a successful bone marrow transplant, survival beyond childhood is rare.

LAD-I is estimated to impact 800 to 1,000 patients in the U.S. and Europe and the annual incidence is 50 to 75 individuals.

What you need to know:

Proposed Indication: Treatment of severe LAD-I

Mechanism: Gene therapy delivering ITGB2 gene

Efficacy: Overall survival: 100% (9/9) at 12 months post-infusion

Safety: Data is limited

Dosing: IV as a one-time dose

Why it Matters: Promising short-term data, alternative to HSCT, significant unmet need

Important to Note: Long-term safety and efficacy unknown, small target population, complex patient journey including intensive myeloablative conditioning therapy

Estimated Cost: $3 million for a one-time dose (based on pricing for Skysona)

Clinical profile

Marnetegragene autotemcel is an ex vivo gene therapy that contains autologous (patient-derived) hematopoietic stem cells that have been genetically modified with a lentiviral vector to deliver a functional copy of the ITGB2 gene.

Pivotal trial data:
The efficacy of marnetegragene autotemcel was evaluated in a Phase 1/2, single-arm study in patients ≥ 3 months old with severe LAD-I. The primary endpoint was overall survival without hematopoietic stem cell transplantation.

As of the November 2022 data cut-off, marnetegragene autotemcel demonstrated 100% overall survival at 12 months post-infusion (and for the entire duration of follow-up) for all 9 LAD-I patients with 12 to 24 months of available follow-up. Data also showed large decreases compared with pre-treatment history in the incidences of significant infections.

Safety:
Safety data is limited, but no marnetegragene autotemcel-related serious adverse events have been reported to date.

Dosing:
In the pivotal trial, patient's (HSCs) were collected via apheresis. Myeloablative conditioning with busulfan preceded IV infusion with marnetegragene autotemcel.

Marnetegragene autotemcel *(continued...)*

Competitive environment

The only curative treatment for severe LAD-I currently is hematopoietic stem cell transplant (HSCT); however, this is a limited option because not all patients have a compatible donor (particularly a matched related donor). HSCT carries its own risks such as graft failure/rejection and graft-versus-host disease (GvHD). In the absence of HSCT and due to repeat infections, the life expectancy of patients with severe LAD1 is significantly shorted.

Marnetegragene autotemcel would be the first approved treatment for LAD-I and an alternative to HSCT, particularly in patients without a compatible donor. The short-term overall survival data for marnetegragene autotemcel are promising with all 9 patients alive at the last data cut-off.

Like Libmeldy, marnetegragene autotemcel is an ex vivo gene therapy, so it is complex to prepare and administer. The eligible target population will also be very small given the low prevalence of LAD-I.

Lastly, like other gene therapies, the durability of response and long-term safety has not yet been established.

For reference, the WAC for Skysona™ (elivaldogene autotemcel), a recently approved gene therapy for another ultra-rare disease (cerebral adrenoleukodystrophy), is $3 million for a one-time dose.

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
2023 Possible launch date					
NEUPRO	rotigotine	UCB	Parkinson's Disease; Restless Legs Syndrome	External	2023
FORTEO	teriparatide	Eli Lilly	Osteoporosis	Injection	2023
DYLOJECT	diclofenac	Hospira/Pfizer/Javelin	Mild to Moderate Pain	Intravenous	2023
DULERA	formoterol fumarate/mometasone furoate	Organon	Asthma	Inhalation	2023
NASCOBAL	cyanocobalamin	Par/Endo	Pernicious Anemia Patients	Intranasal	2023
TEFLARO	ceftaroline fosamil	Allergan	Community Acquired Pneumonia; Skin and Skin Structure Infections	Intravenous	2023
THALOMID	thalidomide	Celgene	Multiple Myeloma; Erythema Nodosum Leprosum	Oral	2023
GATTEX	teduglutide recombinant	Takeda	Short Bowel Syndrome	Subcutaneous	2H-2023
PROLENSA	bromfenac	Bausch Health	Postoperative Ocular Inflammation and Ocular Pain Following Cataract Surgery	Ophthalmic	4Q-2023
RAYOS	prednisone	Amgen	Anti-inflammatory and Immunosuppressive to Treat a Variety of Conditions	Oral	12-2023
2024 Possible launch date					
NEULASTA ONPRO KIT	pegfilgrastim	Amgen/Insulet	Prophylaxis of Neutropenia in Cancer Patients	Subcutaneous	2024
TYSABRI	natalizumab	Biogen	Multiple Sclerosis; Crohn's Disease	Intravenous	2024
VESICARE LS	solifenacin	Astellas	Neurogenic Detrusor Overactivity	Oral	1H-2024
NYMALIZE	nimodipine	Arbor	Subarachnoid Hemorrhage	Oral	1H-2024
GIAZO	balsalazide disodium	Bausch Health	Ulcerative Colitis in Male Patients	Oral	01-2024
GRALISE	gabapentin	Assertio Therapeutics	Postherpetic Neuralgia	Oral	01-2024

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
TASIGNA	nilotinib	Novartis	Philadelphia Chromosome-Positive Chronic Myeloid Leukemia	Oral	01-2024
NATESTO	testosterone	Acerus	Replacement Therapy in Males with Deficiency of Endogenous Testosterone	Nasal	02-2024
EMFLAZA	deflazacort	PTC Therapeutics	Duchenne Muscular Dystrophy	Oral	02-2024
ISENTRESS HD	raltegravir	Merck	Human Immunodeficiency Virus-1 Infection	Oral	04-2024
ISENTRESS	raltegravir	Merck	Human Immunodeficiency Virus-1 Infection	Oral	04-2024
RADICAVA	edaravone	Mitsubishi Tanabe	Amyotrophic Lateral Sclerosis	Intravenous	05-2024
DUAVEE	conjugated estrogens/bazedoxifene acetate	Pfizer/Ligand Pharmaceuticals	Treatment of Moderate to Severe Vasomotor Symptoms Associated with Menopause; Prevention of Postmenopausal Osteoporosis	Oral	05-2024
PROBUPHINE	buprenorphine	Titan Pharmaceuticals/Braeburn Pharmaceuticals	Maintenance Treatment of Opioid Dependence	Subdermal	06-2024
VICTOZA	liraglutide	Novo Nordisk	Type 2 Diabetes Mellitus (T2DM); Reduce the Risks of Cardiovascular Events in T2DM	Subcutaneous	06-2024
TWYNEO	tretinoin/benzoyl peroxide	Galderma	Acne Vulgaris	External	07-2024
SLYND	drospirenone	Exeltis/Insud	Prevention of Pregnancy	Oral	08-2024
OXTELLAR XR	oxcarbazepine	Supernus	Partial Seizures	Oral	09-2024
SPRYCEL	dasatinib	Bristol-Myers Squibb	Chronic Myeloid Leukemia; Acute Lymphoblastic Leukemia	Oral	09-2024
SUSTOL	granisetron	Heron Therapeutics	Chemotherapy-Induced Nausea and Vomiting	Subcutaneous	09-2024
PRIALT	ziconotide acetate	TerSera Therapeutics	Severe Pain	Intrathecal	10-2024
LAZANDA	fentanyl citrate	Depomed	Breakthrough Pain in Cancer Patients	Intranasal	10-2024
RYDAPT	midostaurin	Novartis	Acute Myeloid Leukemia; Systemic Mastocytosis; Mast Cell Leukemia	Oral	10-2024
VUITY	pilocarpine	AbbVie	Presbyopia	Ophthalmic	10-2024
STENDRA	avanafil	Metuchen 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
PRADAXA	dabigatran etexilate mesylate	Boehringer Ingelheim	Venous Thromboembolic Events in Pediatric Patients	Oral	12-2024

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
2025 Possible launch date					
ACTEMRA	tocilizumab	Roche/Chugai	Juvenile Idiopathic Arthritis; Rheumatoid Arthritis; Giant Cell Arteritis; Cytokine Release Syndrome; Systemic Sclerosis-Associated Interstitial Lung Disease	Intravenous; subcutaneous	2025
BOSULIF	bosutinib	Pfizer	Chronic Myelogenous Leukemia	Oral	2025
COMPLERA	emtricitabine/rilpivirine/tenofovir disoproxil fumarate	Gilead/Janssen	Human Immunodeficiency Virus-1 Infection	Oral	2025
EYLEA	aflibercept	Regeneron	Wet Age-Related Macular Degeneration; Diabetic Macular Edema; Macular Edema Following Retinal Vein Occlusion; Diabetic Retinopathy in Patients with Diabetic Macular Edema; Retinopathy of Prematurity	Intravitreal	2025
XOLAIR	omalizumab	Roche/Genentech	Asthma; Idiopathic Urticaria; Nasal Polyps	Intravenous	2025
NAMZARIC	memantine/donepezil	Allergan/Adamas	Moderate to Severe Dementia of the Alzheimer's Type	Oral	01-2025
TRACLEER	bosentan	Actelion/Janssen	Pulmonary Arterial Hypertension	Oral	01-2025
RISPERDAL CONSTA	risperidone	Janssen	Psychosis; Schizophrenia	Injection	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	01-2025
HALAVEN	eribulin	Eisai	Metastatic Breast Cancer; Liposarcoma	Intravenous	01-2025
CORLANOR	ivabradine	Amgen	Heart Failure	Oral	01-2025
PHOSLYRA	calcium acetate	Fresenius	Phosphate Binder	Oral	01-2025
FINACEA	azelaic acid	LEO Pharma	Rosacea	External	01-2025
SANCUSO	granisetron	Kyowa Hakko Kirin/ProStrakan	Prevention of Nausea and Vomiting in Patients Receiving Moderately and/or Highly Emetogenic Chemotherapy	External	01-2025
PROLIA	denosumab	Amgen	Postmenopausal Osteoporosis; Bone Loss in Men and Women at Risk of Fracture	Subcutaneous	02-2025
XGEVA	denosumab	Amgen	Prevention of Fractures in Bone Malignancies and Multiple Myeloma; Giant Cell Tumor in Bone; Hypercalcemia	Subcutaneous	02-2025

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
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
BENLYSTA	belimumab	GSK	Systemic Lupus Erythematosus; Lupis Nephritis	Intravenous; subcutaneous	03-2025
AURYXIA	ferric citrate	Keryx/Akebia Therapeutics	Control of Serum Phosphorus Levels in Chronic Kidney Disease (CKD) on Dialysis; Iron Deficiency Anemia in Adult Patients with CKD Not on Dialysis	Oral	03-2025
YERVOY	ipilimumab	Bristol-Myers Squibb	Melanoma; Renal Cell Cancer; Colorectal Cancer; Hepatocellular Cancer; Non-Small Cell Lung Cancer; Mesothelioma	Intravenous	03-2025
HORIZANT	gabapentin enacarbil	Arbor	Restless Legs Syndrome; Postherpetic Neuralgia	Oral	04-2025
JYNARQUE	tolvaptan	Otsuka	Polycystic Kidney Disease	Oral	04-2025
BRILINTA	ticagrelor	AstraZeneca	To Reduce the Risk of Cardiovascular Death, Myocardial Infarction (MI), and Stroke in Patients with Acute Coronary Syndrome, History of MI, Coronary Artery Disease, or Acute Ischemic Stroke or Transient Ischemic Attack	Oral	05-2025
APTIOM	eslicarbazepine	Sunovion/Bial	Partial-Onset Seizures	Oral	05-2025
TIROSINT-SOL	levothyroxine	IBSA Institut Biochemique	Hypothyroidism; Thyrotropin-Dependent Thyroid Cancer	Oral	05-2025
FYCOMPA	perampanel	Eisai	Partial-Onset Seizures; Primary Generalized Tonic-Clonic Seizures	Oral	05-2025
NULOJIX	belatacept	Bristol-Myers Squibb	Prophylaxis of Organ Rejection in Kidney Transplant	Intravenous	06-2025
NUCYNTA	tapentadol	Collegium	Moderate to Severe Acute Pain	Oral	06-2025
NUCYNTA ER	tapentadol	Collegium	Moderate to Severe Chronic Pain	Oral	06-2025
PERJETA	pertuzumab	Genentech	HER-2 Positive Breast Cancer	Intravenous	2H-2025
CARDENE IV	nicardipine	Chiesi	Short-Term Treatment of Hypertension When Oral Therapy is Not Possible	Intravenous	07-2025
RAVICTI	glycerol phenylbutyrate	Amgen	Urea Cycle Disorders	Oral	07-2025

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
RYANODEX	dantrolene	Eagle Pharmaceuticals	Malignant Hyperthermia	Intravenous	07-2025
SOLIQUA 100/33	insulin glargine/lixisenatide	Sanofi	Type 2 Diabetes Mellitus	Subcutaneous	07-2025
RYTARY	carbidopa/levodopa	Impax/Amneal	Parkinson's Disease	Oral	07-2025
DIACOMIT	stiripentol	Biocodex	Dravet Syndrome	Oral	08-2025
ADZENYS XR-ODT	amphetamine polistirex	Neos Therapeutics	Attention Deficit Hyperactivity Disorder	Oral	09-2025
OFEV	nintedanib	Boehringer Ingelheim	Idiopathic Pulmonary Fibrosis; Systemic Sclerosis-Associated Interstitial Lung Disease (ILD); Chronic Fibrosing ILD	Oral	10-2025
XIGDUO XR	dapagliflozin/metformin	AstraZeneca	Type 2 Diabetes Mellitus; Reduce the Risk of Hospitalizations with Heart Failure; Chronic Kidney Disease	Oral	10-2025
FARXIGA	dapagliflozin	AstraZeneca	Type 2 Diabetes Mellitus; Reduce the Risk of Hospitalization with Heart Failure; Chronic Kidney Disease	Oral	10-2025
QTERN	dapagliflozin/saxagliptin	AstraZeneca	Type 2 Diabetes Mellitus	Oral	10-2025
FUROSCIX	furosemide	scPharmaceuticals	Chronic Heart Failure	Subcutaneous	10-2025
ELELYSO	taliglucerase alfa	Pfizer	Gaucher Disease	Intravenous	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
PICATO	ingenol mebutate	LEO Pharma	Actinic Keratosis	External	12-2025
OPSUMIT	macitentan	Janssen	Pulmonary Arterial Hypertension	Oral	12-2025
2026 Possible launch date					
CIMZIA	certolizumab pegol	UCB/Royalty Pharma	Psoriatic Arthritis; Rheumatoid Arthritis; Ankylosing Spondylitis; Crohn's Disease; Plaque Psoriasis; Axial Spondyloarthritis	Subcutaneous	2026
BRYHALI	halobetasol	Bausch Health	Plaque Psoriasis	External	2026
ABILIFY MAINTENA	aripiprazole	Otsuka/Lundbeck	Schizophrenia; Bipolar Disorder	Intramuscular	2026
MAVENCLAD	cladribine	Serono	Multiple Sclerosis	Oral	2026

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
POMALYST	pomalidomide	Celgene	Multiple Myeloma; Kaposi Sarcoma	Oral	1Q-2026
MOTEGRITY	prucalopride	Takeda	Chronic Idiopathic Constipation	Oral	01-2026
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
BROMSITE	bromfenac	Sun	Treatment of Postoperative Inflammation and Prevention of Ocular Pain in Patients Undergoing Cataract Surgery	Ophthalmic	01-2026
MYRBETRIQ	mirabegron	Astellas	Overactive Bladder; Neurogenic Detrusor Overactivity	Oral	01-2026
ELLA	ulipristal	Afaxys/Perrigo	Emergency Contraception	Oral	01-2026
TYVASO	treprostinil	United Therapeutics	Pulmonary Arterial Hypertension; Pulmonary Hypertension with Interstitial Lung Disease	Inhalation	01-2026
PROMACTA	eltrombopag	Novartis	Thrombocytopenia	Oral	01-2026
QBRELIS	lisinopril	Silvergate	Hypertension; Heart Failure; Acute Myocardial Infarction	Oral	01-2026
CYRAMZA	ramucirumab	Eli Lilly	Gastric Cancer; Gastroesophageal Cancer; Metastatic Gastric Cancer; Non-Small Cell Lung Cancer	Intravenous	01-2026
BRIVIACT	brivaracetam	UCB	Epilepsy	Oral; intravenous	02-2026
XELJANZ XR	tofacitinib	Pfizer	Rheumatoid Arthritis; Psoriatic Arthritis; Ulcerative Colitis; Ankylosing Spondylitis	Oral	2Q-2026
XELJANZ	tofacitinib	Pfizer	Rheumatoid Arthritis; Ulcerative Colitis; Psoriatic Arthritis; Juvenile Idiopathic Arthritis; Ankylosing Spondylitis	Oral	2Q-2026
JANUVIA	sitagliptan	Merck	Type 2 Diabetes Mellitus	Oral	05-2026
JANUMET	sitagliptan/metformin	Merck	Type 2 Diabetes Mellitus	Oral	05-2026

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
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
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	Subcutaneous	10-2026
TRINTELLIX	vortioxetine	Takeda/Lundbeck	Major Depressive Disorder	Oral	12-2026
1st Half 2027 Possible launch date					
KYPROLIS	carfilzomib	Amgen	Multiple Myeloma	Intravenous	2027
ENTRESTO	sacubitril/valsartan	Novartis	Heart Failure	Oral	2027
SAXENDA	liraglutide	Novo Nordisk	Chronic Weight Management	Subcutaneous	2027
IBRANCE	palbociclib	Pfizer	Breast Cancer	Oral	1Q-2027
MINOCIN	minocycline	Rempex/Melinta Therapeutics	Infections	Intravenous	01-2027

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
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
CUBICIN RF	daptomycin	Cubist/Merck	Complicated Skin and Skin Structure Infections; Staphylococcus aureus Bloodstream Infections	Intravenous	01-2027
JUBLIA	efinaconazole	Bausch Health	Onychomycosis of the Toenail	External	01-2027
VIBATIV	telavancin	Theravance	Infections	Intravenous	01-2027
ENVARSUS XR	tacrolimus	Veloxis	Prophylaxis of Organ Rejection in Kidney Transplant Patients	Oral	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	NPS/Nycomed	Hypoparathyroidism	Subcutaneous	01-2027
SUBSYS	fentanyl	BTcP Pharma	Breakthrough Pain in Cancer Patients	Oral	01-2027
ALTABAX	retapamulin	Aqua Pharmaceuticals/Almirall	Impetigo	External	02-2027
BYDUREON	exenatide	AstraZeneca	Type 2 Diabetes Mellitus	Subcutaneous	02-2027
VITEKTA	elvitegravir	Gilead	Human Immunodeficiency Virus-1 Infection	Oral	02-2027
CRESEMBA	isavuconazonium	Astellas	Invasive Aspergillosis; Invasive Mucormycosis	Oral; Intravenous	03-2027
TUDORZA PRESSAIR	aclidinium	AstraZeneca	Chronic Obstructive Pulmonary Disease	Inhalation	04-2027
DUAKLIR PRESSAIR	aclidinium/formoterol fumarate	Covis Pharma	Chronic Obstructive Pulmonary Disease	Inhalation	04-2027
LUMIGAN	bimatoprost	Allergan	Glaucoma; Ocular Hypertension	Ophthalmic	06-2027
ORENITRAM	treprostinil diethanolamine	Supernus/United Therapeutics	Pulmonary Arterial Hypertension	Oral	06-2027

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
2023 Possible launch date									
NS-2 (ALDX-1E1, ADX-102)	reproxalap	Aldeyra Therapeutics	aldehyde antagonist	Dry eye disease	OPH	Filed NDA	11/23/2023	No	No
PF-3084014 (PF-03084014)	nirogacestat	SpringWorks Therapeutics	gamma secretase inhibitor	Desmoid tumors	PO	Filed NDA	11/27/2023	Yes	Yes
CTX-001 (Exa-cel)	exagamglogene autotemcel	CRISPR Therapeutics/ Vertex	gene therapy (gene editing CRISPR-Cas9)	Sickle cell disease/ beta-thalassemia	IV	Filed BLA	12/08/2023	Yes	Yes
ARQ-154	roflumilast	Arcutis Biotherapeutics	phosphodiesterase-4 inhibitor	Seborrheic dermatitis	TOP	Filed NDA	12/16/2023	No	No
ACT-132577	aprocitentan	Idorsia Pharmaceuticals	endothelin receptor antagonist	Hypertension	PO	Filed NDA	12/20/2023	No	No
LentiGlobin	lovotibeglogene autotemcel	bluebird bio	gene therapy	Sickle cell disease	IV	Filed BLA	12/20/2023	Yes	Yes
ITF-2357	givinostat	Italfarmaco S.p.A.	histone deacetylase inhibitor	Duchenne muscular dystrophy	PO	Filed NDA	12/21/2023	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
AKCEA-TTR-LRx	eplontersen	AstraZeneca/ Ionis	antisense oligonucleotide	Hereditary transthyretin-mediated amyloid polyneuropathy	SC	Filed BLA	12/22/2023	Yes	Yes
iDose travoprost	travoprost	Glaukos	prostaglandin analog	Glaucoma/ Ocular hypertension	Intraocular	Filed NDA	12/22/2023	No	No
MK-7264	gefapixant	Merck	P2X3 antagonist	Chronic cough	PO	Filed NDA	12/27/2023	No	No
LNP-023	iptacopan	Novartis	factor B inhibitor	Paroxysmal nocturnal hemoglobinuria	PO	Filed NDA	4Q2023	Yes	Yes
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension; interstitial lung disease	INH	Tentative Approval	2H2023	Yes	No
BGB-A317 (BGB-A-317)	tislelizumab	BeiGene	programmed death-1 inhibitor	Esophageal squamous cell carcinoma	IV	Filed BLA	Late 2023	Yes	Yes
ITCA-650 (sustained release exenatide)	exenatide sustained-release	Intarcia	glucagon-like peptide-1 receptor agonist	Diabetes mellitus	SC implant	Filed NDA	Late 2023	No	No
2024 Possible launch date									
CK-301	cosibelimab	Checkpoint Therapeutic	anti programmed cell death ligand 1	Cutaneous squamous cell carcinoma	IV	Filed BLA	01/03/2024	Yes	No
SB-206	berdazimer	Novan Therapeutics	nitric oxide-releasing compound	Molluscum contagiosum	TOP	Filed NDA	01/05/2024	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
iMAB-362	zolbetuximab	Astellas	GC182 monoclonal antibody	Gastric adenocarcinoma	IV	Filed BLA	01/12/2024	Yes	Yes
GC-5107	human immunoglobulin	GC Biopharma	human immunoglobulin	Primary immunodeficiencies	IV	Filed BLA	01/13/2024	Yes	No
DPI-386	scopolamine	Repurposed Therapeutics	anticholinergic	Motion sickness	Intranasal	Filed NDA	01/26/2024	No	No
NVK-002	atropine	Vyluma	anticholinergic	Myopia	OPH	Filed NDA	01/31/2024	No	No
STS-101	dihydroergotamine	Satsuma Pharmaceuticals	ergotamine	Migraine	Intranasal	Filed NDA	01/2024	No	No
VNRX-5133	cefepime/ taniborbactam	VenatoRx Pharmaceuticals	cephalosporin/ beta-lactamase inhibitor	Bacterial infections	IV	Filed NDA	02/22/2024	Yes	No
LN-144	lifileucel	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Melanoma	IV	Filed BLA	02/24/2024	Yes	Yes
MIN-101	roluperidone	Minerva Neurosciences	sigma-2 and 5HT-2A receptor antagonist	Schizophrenia	PO	Filed NDA	02/26/2024	No	No
AAI-101	cefepime/enmetazobactam	Allecra Therapeutics	beta-lactam/b-lactamase inhibitor	Urinary tract infection	IV	Filed NDA	02/27/2024	No	No
Botulax	letibotulinumtoxinA	Hugel Pharma	botulinum toxins	Wrinkles	IM	Filed BLA	03/01/2024	Yes	No
APP-13007	clobetasol propionate	Formosa Pharmaceuticals	corticosteroid	Eye inflammation/ pain	OPH	Filed NDA	03/04/2024	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
glatiramer acetate depot	glatiramer acetate long-acting	Viatris	immunomodulator	Multiple sclerosis	IM	Filed NDA	03/08/2024	Yes	No
MGL-3196	resmetirom	Madrigal Pharmaceuticals	beta-selective thyroid hormone receptor agonist	Nonalcoholic steatohepatitis	PO	Filed NDA	03/14/2024	Yes	No
OTL-200	atidarsagene autotemcel	Orchard Therapeutics	gene therapy	Leukodystrophy	IV	Filed BLA	03/18/2024	Yes	Yes
TAK-721 (SHP-621)	budesonide	Takeda	corticosteroid	Eosinophilic esophagitis	PO	Filed NDA	03/20/2024	Yes	Yes
ACE-011	sotatercept	Merck	activin receptor type IIA-Fc fusion protein	Pulmonary arterial hypertension	SC	Filed BLA	03/26/2024	Yes	Yes
AKB-6548	vadadustat	Otsuka Pharmaceutical	hypoxia-inducible factor-prolyl hydroxylase inhibitor	Chronic kidney disease-related anemia	PO	Filed NDA	03/27/2024	Yes	No
Opsynvi	macitentan/ tadalafil	Janssen	endothelin receptor antagonist/ phosphodiesterase 5 inhibitor	Pulmonary arterial hypertension	PO	Filed NDA	03/30/2024	Yes	Yes
REGN-1979	odronextamab	Regeneron	CD20/CD3 monoclonal antibody	Follicular lymphoma/ diffuse large b-cell lymphoma	IV	Filed BLA	03/31/2024	Yes	Yes
LY-3002813	donanemab	Eli Lilly	beta-amyloid monoclonal antibody	Alzheimer's disease	IV	Filed BLA	1Q2024	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
RP-L201	marnetegragene autotemcel	Rocket Pharmaceuticals	gene therapy	Leukocyte adhesion deficiency-I	IV	Filed BLA	03/31/2024	Yes	Yes
LTX-03	hydrocodone bitartrate/ acetaminophen	Acura Pharmaceuticals	opioid analgesic	Pain	PO	Filed NDA	1Q2024	No	No
Zeftera	ceftobiprole	Basilea	cephalosporin antibiotic	Bacterial infections	IV	Filed NDA	04/03/2024	No	No
SPN-830	apomorphine	Supernus Pharmaceuticals	non-ergoline dopamine agonist	Parkinson's disease	SC infusion	Filed NDA	04/05/2024	Yes	No
TransCon PTH	palopegteriparatide	Ascendis Pharma	parathyroid hormone	Hypoparathyroidism	SC	Filed NDA	04/15/2024	Yes	Yes
ALT-803	nogapendekin alfa inbakicept	ImmunityBio	interleukin-15 (IL-15) super agonist/ IL-15R alpha-Fc fusion complex	Bladder cancer	Intravesical	Filed BLA	04/23/2024	Yes	No
Ingrezza oral granules	valbenazine	Neurocrine Bioscience	vesicular monoamine transporter 2 inhibitor	Tardive dyskinesia/ Huntington's disease	PO	Filed NDA	04/24/2024	Yes	Yes
PF-06838435 (SPK-9001)	fidanacogene elaparvovec	Pfizer/ Spark Therapeutics	gene therapy	Hemophilia B	IV	Filed BLA	04/27/2024	Yes	Yes
X4P-001 (X-4P-001, X4-136, X4P-001-RD)	mavorixafor	X4 Pharma	CXC receptor type 4 inhibitor	WHIM syndrome	PO	Filed NDA	04/30/2024	Yes	Yes
mRNA-1345	mRNA-1345	Moderna	vaccine	Respiratory syncytial virus	IM	Filed BLA	04/2024	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
DAY-101	tovorafenib	Day One Biopharmaceuticals	pan-Raf kinase inhibitor	Brain cancer	PO	Filed NDA	05/12/2024	Yes	Yes
SHR-1210	camrelizumab	Elevar Therapeutics	programmed death receptor-1-blocking antibody	Hepatocellular carcinoma	IV	Filed BLA	05/14/2024	Yes	Yes
YN-96D1	rivoceranib (apatinib)	Elevar Therapeutics	vascular endothelial growth factor receptor antagonist	Hepatocellular carcinoma	PO	Filed NDA	05/16/2024	Yes	Yes
EB-101	EB-101	Abeona Therapeutics	gene therapy	Epidermolysis Bullosa	TOP	Filed BLA	05/26/2024	Yes	Yes
GRN-163L	imetelstat	Geron	telomerase inhibitor	Myelodysplastic syndrome	IV	Filed NDA	06/16/2024	Yes	Yes
RPL-554	ensifentrine	Verona Pharma	phosphodiesterase-3 and phosphodiesterase-4 inhibitor	Chronic obstructive pulmonary disease	INH	Filed NDA	06/26/2024	No	No
LY-686017	tradipitant	Vanda Pharmaceuticals	neurokinin 1 receptor antagonist	Gastroparesis	PO	InTrial	2Q2024	No	No
arimoclomol	arimoclomol	Orphazyme	cytoprotectives	Niemann-Pick disease	PO	CRL	2Q2024	Yes	Yes
Tecentriq SC	atezolizumab	Roche	programmed death-ligand 1 blocking antibody	Cancers (mirroring indications to IV formulation)	SC	Filed BLA	2Q2024	Yes	No
BT-595	immune globulin	Biotest	immune globulin	Primary immunodeficiency	IV	Filed BLA	06/29/2024	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
LAI-287	insulin icodec	Novo Nordisk	ultra-long-acting basal insulin	Diabetes mellitus	SC	Filed BLA	1H2024	No	No
AXS-07	meloxicam/rizatriptan	Axsome Therapeutics	non-steroidal anti-inflammatory drug/triptan	Migraine	PO	CRL	1H2024	No	No
P2B-001	pramipexole/ rasagiline	Pharma Two B	dopamine agonist/ monoamine oxidase B inhibitor	Parkinson's disease	PO	InTrial	1H2024	No	No
Hepcludex	bulevirtide	Gilead	HBV receptor binder	Hepatitis delta virus	SC	CRL	1H2024	Yes	Yes
Risvan	risperidone	Laboratorios Farmacéuticos Rovi	atypical antipsychotic	Schizophrenia	IM	CRL	1H2024	Yes	No
ALXN-2040	danicopan	AstraZeneca	complement factor D inhibitor	Paroxysmal nocturnal hemoglobinuria	PO	Filed NDA	1H2024	Yes	Yes
PB-2452	bentracimab	SFJ Pharmaceuticals	antiplatelet monoclonal antibody	Antiplatelet drug toxicity	IV	InTrial	Mid-2024	No	No
AT-007	govorestat	Applied Therapeutics	aldose reductase inhibitor	Galactosemia	PO	InTrial	Mid-2024	Yes	Yes
ADP-A2M4 (MAGE-A4)	afamitresgene autoleucel	Adaptimmune	SPEAR T-cell therapy	Sarcoma	IV	InTrial	Mid-2024	Yes	Yes
RP-L102 (RPL-102)	RP-L102	Rocket Pharmaceuticals	gene therapy	Fanconi anemia	IV	InTrial	Mid-2024	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
OMS-721	narsoplimab	Omeros	anti-MASP-2 monoclonal antibody	Hematopoietic stem cell transplant-associated thrombotic microangiopathy	IV	CRL	Mid-2024	Yes	Yes
SPI-014	lanthanum dioxycarbonate	Unicycive	phosphate binder	Hyperphosphatemia	PO	InTrial	Mid-2024	No	No
PF-06939926	fordadistrogene movaparvovec	Pfizer	gene therapy	Duchenne muscular dystrophy	IV	InTrial	Mid-2024	Yes	Yes
ADI-PEG20	pegargiminase	Polaris	pegylated arginine deiminase	Mesothelioma	IM	InTrial	Mid-2024	Yes	Yes
CUTX-101	copper histidinate	Fortress Biotech	copper replacement	Menkes Disease	SC	InTrial	Mid-2024	Yes	Yes
I/Ontak	denileukin diftitox	Citius	CD25-directed cytotoxin	Cutaneous T-cell lymphoma	IV	CRL	Mid-2024	Yes	Yes
RG-6058	tiragolumab	Roche	TIGIT monoclonal antibody	Non-small cell lung cancer/ esophageal cancer	IV	InTrial	Mid-2024	Yes	No
SNDX-5613	revumenib	Syndax	Menin-mixed lineage leukemia 1 inhibitor	Acute myelogenous leukemia	PO	InTrial	Mid-2024	Yes	Yes
SNDX-6352	axatilimab	Syndax Pharmaceuticals	colony stimulating factor 1 receptor monoclonal antibody	Graft vs. host disease	IV	InTrial	Mid-2024	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
Obe-cel	obecabtagene autoleucel	Autolus Therapeutics	autologous chimeric antigen receptor T-cells	Acute lymphoblastic leukemia	IV	InTrial	Mid-2024	Yes	Yes
TC-002	latanoprost	TearClear	prostaglandin analog	Glaucoma	OPH	InTrial	Mid-2024	No	No
UX-111 (ABO-102)	UX-111	Ultragenyx Pharmaceutical	gene therapy	Sanfilippo syndrome type A	IV	InTrial	Mid-2024	Yes	Yes
Oral semaglutide (weight loss)	semaglutide	Novo Nordisk	glucagon-like peptide 1 receptor agonist	Chronic weight management	PO	InTrial	Mid-2024	No	No
Leqembi SC	lecanemab	Eisai/Biogen	beta-amyloid targeted therapy	Alzheimer's disease	SC	InTrial	Mid-2024	Yes	No
Lydolyte	lidocaine	MEDRx	anesthetic agent	Neuropathic pain	TOP	CRL	Mid-2024	No	No
RG-6107	crovalimab	Roche	C5 inhibitor	Paroxysmal nocturnal hemoglobinuria	IV/SC	Filed BLA	07/07/2024	Yes	Yes
ALPHA-1062	galantamine prodrug	Alpha Cognition	acetylcholinesterase inhibitor	Alzheimer's disease	PO	Filed NDA	07/27/2024	No	No
CTP-543	deuruxolitinib	Sun Pharma	janus kinase inhibitor	Alopecia areata	PO	Filed NDA	08/06/2024	Yes	No
KarXT	xanomeline/ trospium	Karuna Therapeutics	muscarinic acetylcholine receptor agonist/ muscarinic receptor antagonist	Schizophrenia	PO	Filed NDA	09/28/2024	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
TAVT-45	abiraterone acetate	Tavanta Therapeutics	CYP17 inhibitor	Prostate cancer	PO	InTrial	3Q2024	Yes	No
OX-125	nalmefene	Orexo	opioid receptor antagonist	Opioid use disorder	Intranasal	InTrial	3Q2024	No	No
MSP-2017	etripamil	Milestone	calcium channel blocker	Arrhythmia	Intranasal	Filed NDA	10/24/2024	TBD	No
sulopenem	sulopenem	Iterum Therapeutics	carbapenem	Urinary tract infections	PO	CRL	4Q2024	No	No
IONIS-APOCIII-LRx (ISIS-678354)	olezarsen	Ionis	antisense drug	Familial chylomicronemia syndrome	SC	InTrial	4Q2024	Yes	No
Donesta	estetrol	Mithra Pharmaceuticals	estrogen	Vasomotor symptoms	PO	InTrial	4Q2024	No	No
nemolizumab	nemolizumab	Galderma	interleukin-31 receptor antagonist	Atopic dermatitis	SC	InTrial	2H2024	Yes	No
GFT-505	elafibranor	Genfit	selective peroxisome proliferator-activated receptor modulator	Primary biliary cirrhosis	PO	InTrial	2H2024	Yes	Yes
GSK-2140944	gepotidacin	GlaxoSmithKline	bacterial Type II topoisomerase inhibitor	Bacterial infections	PO/IV	InTrial	2H2024	No	No
OX-124	naloxone	Orexo	opioid antagonist	Opioid overdose	Intranasal	CRL	2H2024	No	No
PTC-AADC	eladocagene exuparvovec	PTC Therapeutics	gene therapy	Aromatic L-amino acid decarboxylase deficiency	Intracerebral	InTrial	2H2024	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
AVB-S6-500	batiraxcept	Aravive Biologics	GAS6/AXL inhibitor	Ovarian cancer	IV	InTrial	2H2024	Yes	No
REGN-5458	linvoseltamab	Regeneron	BCMA and CD3 bispecific antibody inhibitor	Multiple myeloma	IV	InTrial	2H2024	Yes	No
AG-10 (AG10)	acoramidis	BridgeBio	tetrameric transthyretin stabilizer	Transthyretin amyloid cardiomyopathy	PO	InTrial	2H2024	Yes	No
SAR-408701	tusamitamab ravtansine	Sanofi	antibody-drug conjugate	Non-small cell lung cancer	IV	InTrial	2H2024	Yes	No
CF-101	piclidenoson	Can-Fite BioPharma	A3 adenosine receptor agonist	Plaque psoriasis	PO	InTrial	2H2024	Yes	No
ZP-1848	glepaglutide	Zealand Pharma	glucagon peptide-2 agonist	Short bowel syndrome	SC	InTrial	2H2024	Yes	Yes
Dasynoc	dasatinib	Xspray Pharma	kinase inhibitor	Chronic myeloid leukemia	PO	CRL	2H2024	Yes	Yes
MDMA	midomafetamine	MAPS Public Benefit Corporation	psychoactive drug	Post-traumatic stress disorder	PO	InTrial	2H2024	Yes	No
PF-06741086	marstacimab	Pfizer	tissue factor pathway inhibitor	Hemophilia	IV/SC	InTrial	2H2024	Yes	Yes
CSL-312	garadacimab	CSL Limited	anti-factor XIIa monoclonal antibody	Hereditary angioedema	SC	InTrial	2H2024	Yes	Yes
ARS-1	epinephrine	ARS Pharmaceuticals	non-selective alpha/ beta-adrenergic receptor agonist	Anaphylaxis	Intranasal	CRL	2H2024	No	No

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
RG-1594	ocrelizumab	Genentech	CD20-directed cytolytic antibody	Multiple sclerosis	SC	InTrial	2H2024	Yes	No
F-901318	olorofim	F2G	orotomide antifungal	Aspergillosis	PO/IV	CRL	2H2024	No	Yes
XMT-1536	upifitamab rilsodotin	Mersana Therapeutics	antibody-drug conjugate	Ovarian cancer	IV	InTrial	2H2024	Yes	No
HP-5000	diclofenac	Hisamitsu Pharmaceutical	non-steroidal anti-inflammatory drug	Osteoarthritis	Transdermal	InTrial	2H2024	No	No
BBP-305	encaleret	BridgeBio	Ca sensing receptor antagonist	Autosomal dominant hypocalcemia type 1	PO	InTrial	2H2024	Yes	Yes
RP-1	vusolimogene oderparepvec	Replimune	oncolytic immunotherapy	Cutaneous skin cell cancer	Intratumoral	InTrial	2H2024	Yes	No
HER3-DXd	patritumab deruxtecan	Daiichi Sankyo	antibody drug conjugate	Non-small cell lung cancer	IV	InTrial	2H2024	Yes	No
ZW-25	zanidatamab	Zymeworks	HER2 monoclonal antibody	Biliary tract cancer	IV	InTrial	2H2024	Yes	Yes
Multikine	leukocyte interleukin (CS-001P3)	CEL-SCI	immunomodulator	Head and Neck cancer	SC	InTrial	2024	Yes	Yes
ND-0612H	levodopa/ carbidopa	NeuroDerm	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	SC	InTrial	2024	Yes	No
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	CRL	2024	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
SDN-037	difluprednate	Visiox	corticosteroid	Ocular inflammation/pain	OPH	InTrial	2024	No	No
SYD-985	[vic-] trastuzumab duocarmazine	Byondis	HER2-targeting antibody-drug conjugate	Breast cancer	IV	CRL	2024	Yes	No
NRX-101 (Cyclurad)	d-cycloserine/ lurasidone	NeuroRx	N-methyl-D-aspartate receptor modulator/ 5-HT2A receptor antagonist	Bipolar disorder	PO	InTrial	2024	No	No
BBI-4000	sofpironium bromide	Brickell	anticholinergic	Hyperhidrosis	TOP	CRL	2024	No	No
MILR-1444A	lebrikizumab	Eli Lilly	interleukin-13 inhibitor	Atopic dermatitis	SC	CRL	2024	Yes	No
MT-7117	dersimelagon	Mitsubishi Tanabe Pharma	undisclosed	Erythropoietic protoporphyria	PO	InTrial	2024	Yes	No
MOR-202	felzartamab	I-Mab	anti-CD38 monoclonal antibody	Multiple myeloma	IV	InTrial	2024	Yes	No
Humacyl	human acellular vessel	Humacyte	cellular therapy	End-stage renal disease	Implant	InTrial	2024	Yes	No
DS-100	dehydrated alcohol	Eton	undisclosed	Methanol poisoning	SC	CRL	2024	No	Yes
Mino-Lok	minocycline-EDTA-ETOH	Citrus	tetracyclines	Bacterial infection	Intracatheter	InTrial	2024	No	No
ABBV-951	foscarbidopa/ foslevodopa	AbbVie	aromatic amino acid decarboxylation inhibitor/ aromatic amino acid	Parkinson's disease	SC	CRL	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
RG-7433 (ABT-263)	navitoclax	AbbVie	Bcl-2 inhibitor	Myelofibrosis	PO	InTrial	2024	Yes	Yes
Iomab-B	iodine I 131 monoclonal antibody BC8	Actinium	anti-CD45 monoclonal antibody	Acute myeloid leukemia	IV	InTrial	2024	Yes	Yes
NN-7415	concizumab	Novo Nordisk	anti-tissue factor pathway inhibitor	Hemophilia A and hemophilia B	SC	CRL	2024	Yes	Yes
Dihydroergotamine autoinjector	dihydroergotamine	Amneal Pharmaceuticals	ergot derivative	Migraine	SC	InTrial	2024	No	No
D-PLEX100	doxycycline	PolyPid	tetracycline	Surgical site infections	IMPLANT	InTrial	2024	No	No
DS-1062	datopotamab deruxtecan	Daiichi Sankyo/ AstraZeneca	trop-2 antibody-drug conjugate	Non-small cell lung cancer; breast cancer	IV	InTrial	2024	Yes	No
LY-03010	paliperidone	Luye Pharma	atypical antipsychotic	Schizophrenia	IM	InTrial	2024	No	No
AZD-5156	AZD-5156	AstraZeneca	monoclonal antibody	COVID-19	IM	InTrial	2024	TBD	No
APN-311	dinutuximab beta	Recordati	anti-GD2 antigen	Neuroblastoma	IV	InTrial	Late 2024	Yes	Yes
AXS-14	S-reboxetine	Axsome Therapeutics	selective noradrenaline reuptake inhibitor	Fibromyalgia	PO	InTrial	Late 2024	No	No
CNM-Au8	CNM-Au8	Clene	gold nanocrystal	Amyotrophic lateral sclerosis	PO	InTrial	Late 2024	Yes	Yes

Pipeline Drug Name(s)	Generic Name	Company	Mechanism of Action	Disease State	Route	FDA Status	Projected FDA Approval Decision	Specialty Drug	Orphan Drug
SLS-001 (WT-1)	galinpepimut-S	Sellas Life Sciences Group	vaccine	Acute myeloid leukemia	SC	InTrial	Late 2024	Yes	Yes
Ovastat	treosulfan	Medexus Pharmaceuticals	alkylating agent	Hematopoietic stem cell transplantation	IV	InTrial	Late 2024	Yes	Yes
MT-1621	deoxythymidine/ deoxycytidine	UCB	deoxynucleoside	Thymidine kinase 2 deficiency	PO	InTrial	Late 2024	Yes	Yes
MAT-2203	amphotericin B	Matinas BioPharma	fungicidal agent	Cryptococcal meningitis	PO	InTrial	Late 2024	No	Yes
CAM-2029	octreotide	Camurus	somatostatin analogue	Acromegaly	SC	InTrial	Late 2024	Yes	Yes
NBI-74788	crinecerfont	Neurocrine Biosciences	CRF receptor antagonist	Congenital adrenal hyperplasia	PO	InTrial	Late 2024	Yes	Yes
ABBV-399	telisotuzumab	AbbVie	antibody (anti-c-Met)-drug conjugate	Non-small cell lung cancer	IV	InTrial	Late 2024	Yes	No
UGN-102	mitomycin	UroGen	alkylating drug	Bladder cancer	Intravesical	InTrial	Late 2024	Yes	No

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

Key pending indication forecast

Optum Rx key pending indication forecast

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Estimated Approval Date
Cresemba	isavuconazonium	Astellas	azole antifungal	Revised	Treatment of invasive aspergillosis and invasive mucormycosis in pediatric patients	PO/IV	12/09/2023
Abecma	idecabtagene vicleucel	Bristol Myers Squibb	B-cell maturation antigen-directed genetically modified autologous T cell immunotherapy	Revised	Treatment of adult patients with relapsed and refractory multiple myeloma who have received an immunomodulatory agent, a proteasome inhibitor, and an anti-CD38 monoclonal antibody	IV	12/16/2023
Xhance	fluticasone	Optinose	corticosteroid	New	Treatment of chronic sinusitis	Intranasal	12/16/2023
Tarpeyo	budesonide	Calliditas Therapeutics	corticosteroid	Accelerated to Full Approval	To reduce proteinuria in adults with primary immunoglobulin A nephropathy at risk of rapid disease progression, generally a urine protein-to-creatinine ratio ≥ 1.5 g/g	PO	12/20/2023
Vabysmo	faricimab-svoa	Roche/ Genentech	vascular endothelial growth factor and angiopoietin-2 inhibitor	New	Treatment of macular edema following retinal vein occlusion	Intravitreal	12/22/2023
Wilate	von Willebrand factor/coagulation factor VIII complex	Octapharma	von Willebrand Factor	Revised	Routine prophylaxis to reduce the frequency of bleeding episodes in children and adults with any type of von Willebrand disease	IV	12/23/2023
Lumakras	sotorasib	Amgen	RAS GTPase inhibitor	Accelerated to Full Approval	Treatment of adult patients with KRAS G12C-mutated locally advanced or metastatic non-small cell lung cancer, as	PO	12/24/2023

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Estimated Approval Date
					determined by an FDA-approved test, who have received at least one prior systemic therapy		
Zegalogue	dasiglucagon	Zealand Pharma	antihypoglycemic agent	New	Prevention and treatment of hypoglycemia in pediatric patients 7 days of age or older with congenital hyperinsulinism	SC	12/30/2023
Welireg	belzutifan	Merck	hypoxia-inducible factor inhibitor	Revised	Treatment of adult patients with advanced renal cell carcinoma following immune checkpoint and anti-angiogenic therapies	PO	01/17/2024
Keytruda	pembrolizumab	Merck	programmed death receptor-1-blocking antibody	Revised	In combination with external beam radiotherapy plus concurrent chemotherapy, followed by brachytherapy (also known as concurrent chemoradiotherapy) as treatment with definitive intent for newly diagnosed patients with high-risk locally advanced cervical cancer	IV	01/20/2024
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	01/27/2024
Edurant	rilpivirine	Janssen	non-nucleoside reverse transcriptase inhibitor	Revised	In combination with other antiretroviral agents for the treatment of HIV-1 infection in treatment-naïve patients 2 years of age and older and weighing at least 10 kg with HIV-1 RNA less than or equal to 100,000 copies/mL	PO	01/28/2024
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 inhibitor	Revised	Treatment of adult and pediatric patients aged 1 year and older, with eosinophilic esophagitis	SC	01/31/2024

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Estimated Approval Date
Onivyde	irinotecan	Ipsen	topoisomerase inhibitor	Revised	In combination with fluorouracil/leucovorin and oxaliplatin as first-line treatment for metastatic pancreatic ductal adenocarcinoma	IV	02/13/2024
Ixinity	coagulation factor IX (recombinant)	Medexus Pharmaceuticals	human blood coagulation factor	Revised	On-demand, prophylactic, and perioperative treatment of pediatric patients under 12 years of age with hemophilia B	IV	02/15/2024
Tagrisso	osimertinib	AstraZeneca	kinase inhibitor	Revised	In combination with chemotherapy for the treatment of adult patients with locally advanced or metastatic epidermal growth factor receptor-mutated (EGFRm) non-small cell lung cancer	PO	02/16/2024
Rybrevant	amivantamab-vmjw	Janssen	bispecific EGF receptor-directed and MET receptor-directed antibody	New	In combination with chemotherapy (carboplatin-pemetrexed) for the first-line treatment of patients with locally advanced or metastatic non-small cell lung cancer with EGFR exon 20 insertion mutations	IV	02/28/2024
Balversa	erdafitinib	Janssen	kinase inhibitor	Accelerated to Full Approval	Treatment of adult patients with locally advanced or metastatic urothelial carcinoma that has susceptible FGFR3 genetic alterations, and progressed during or following at least one line of a programmed death receptor-1 (PD-1) or programmed death-ligand 1 (PD-L1) inhibitor in the locally advanced or	PO	02/28/2024

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Estimated Approval Date
					metastatic setting or within 12 months of neoadjuvant or adjuvant therapy		
Livmarli	maralixibat	Mirum Pharmaceuticals	ileal bile acid transporter inhibitor	New	Treatment of pruritus in patients 2 years of age and older with progressive familial intrahepatic cholestasis	PO	03/14/2024
Breyanzi	lisocabtagene maraleucel	Bristol Myers Squibb	CD19-directed genetically modified autologous T cell immunotherapy	Revised	Treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia or small lymphocytic lymphoma who received a prior Bruton tyrosine kinase inhibitor and B-cell lymphoma 2 inhibitor	IV	03/14/2024
Ofev	nintedanib	Boehringer Ingelheim	tyrosine kinase inhibitor	New	Treatment for children and adolescents between 6 to 17 years old with fibrosing interstitial lung disease	PO	03/25/2024
Brukinsa	zanubrutinib	BeiGene	kinase inhibitor	New	In combination with obinutuzumab for the treatment of adult patients with relapsed or refractory follicular lymphoma after at least two prior lines of therapy	PO	1Q2024
Wegovy	semaglutide	Novo Nordisk	glucagon-like peptide-1 receptor agonist	New	To reduce the risk of major adverse cardiovascular events in overweight and obese adults	SC	1Q2024
Nexletol	bempedoic acid	Esperion	adenosine triphosphate-citrate lyase inhibitor	New	To reduce the risk of cardiovascular events in statin intolerant patients	PO	04/01/2024
Carvykti	ciltacabtagene autoleucel	J&J	B-cell maturation antigen-directed genetically	Revised	Treatment of relapsed and refractory multiple myeloma in patients with 1 to 3 prior lines of therapy	IV	04/05/2024

Brand Name	Generic Name	Company	Mechanism of Action	Indication Type	Proposed New/Revised Indication	Route	Estimated Approval Date
			modified autologous T cell immunotherapy				
Kevzara	sarilumab	Sanofi	interleukin-6 receptor monoclonal antibody	New	Treatment of polyarticular juvenile idiopathic arthritis	SC	06/10/2024
Skyrizi	risankizumab-rzaa	AbbVie	interluekin-23 inhibitor	New	Treatment of ulcerative colitis	SC	06/28/2024
Sirturo	bedaquiline	Janssen	diarylquinoline antimycobacterial drug	Accelerated to Full Approval	As part of combination therapy in adult and pediatric patients (5 years and older and weighing at least 15 kg) with pulmonary multi-drug resistant tuberculosis (MDR-TB). Reserve SIRTURO for use when an effective treatment regimen cannot otherwise be provided	PO	06/2024
Gammagard Liquid	immune globulin (human)	Takeda	immune globulin	New	Treatment of chronic inflammatory demyelinating polyneuropathy	IV/SC	1H2024
Imfinzi	durvalumab	AstraZeneca	programmed death-ligand 1 blocking antibody	New	Adjuvant treatment of non-small cell lung cancer	IV	1H2024
Zoryve	roflumilast	Arcutis Biotherapeutics	phosphodiesterase-4 inhibitor	New	Treatment of mild-to-moderate atopic dermatitis in patients 6 years and older	TOP	07/12/2024

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