RxOutlook®

2nd Quarter 2023

Optum Rx®

Introduction

Welcome to the second quarterly Optum Rx 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 First Half 2023

As of May 23, the FDA has approved 19 new molecular entities in 2023, including 10 drugs with an Orphan Drug designation. Consistent with historical trends, oncology remains the largest area of drug development with 4 of the 19 drugs being approved for an oncology indication. Aside from oncology and rare disease, notable drug approvals included **Leqembi™ (lecanemab),** the second beta-amyloid targeted therapy for Alzheimer's disease; **Veozah™ (fezolinetant),** a first-in-class nonhormonal therapy for menopause; and **Zavzpret™ (zavegepant),** the first intranasal formulation of a calcitonin gene-related peptide (CGRP) receptor antagonist for migraine.

In addition to these drugs, the FDA approved the first vaccine for RSV infection, GSK's **Arexvy,** for individuals 60 years of age and older, and the first topical gene therapy, **Vyjuvek™ (beremagene geperpavec-svdt),** for dystrophic epidermolysis bullosa.

Looking Ahead to the 3rd Quarter 2023

In this edition of RxOutlook, we highlight 10 key pipeline products with an expected approval decision by the end of the third quarter 2023. This includes two new treatments for major depressive disorder, **gepirone extended-release** and **zuranolone. Zuranolone** is also under FDA review for treatment of postpartum depression and notably, is administered as a 14-day treatment course.

Zimura (avacincaptad pegol), an intravitreal injection, is currently under review for treatment of geographic atrophy, a severe form of age-related macular degeneration (AMD). Until recently, there were no FDA approved treatments for geographic atrophy but earlier this year the FDA approved Apellis Pharmaceuticals' Syfovre™ (pegcetacoplan).

Nirsevimab, an RSV monoclonal antibody, would potentially be the first preventative RSV treatment for all infants. The only preventative treatment currently available for pediatric patients is Synagis® (palivizumab), but Synagis is only approved for patients at high-risk for serious lower respiratory tract disease. Unlike Synagis which requires monthly doses during the RSV reason, nirsevimab is administered as a single-dose.

Lebrikizumab is a monoclonal antibody that targets interleukin-13 (IL-13) and would provide an additional biologic injectable for treatment of atopic dermatitis. Lebrikizumab would be competing with Dupixent® (dupilumab) and Adbry™ (tralokinumab-ldrm).

Several Orphan Drugs will be discussed in this report: two novel bispecific monoclonal antibodies for multiple myeloma – **talquetamab** and **elranatamab;** two additional treatments for generalized myasthenia gravis – **zilucoplan** and **rozanolixizumab;** and the second treatment for primary hyperoxaluria – **nedosiran.**

Approval decisions for other key novel therapies are expected by the end of third quarter 2023 but are not reviewed in this report because they were covered in previous editions of RxOutlook. These include: **bimekizumab** for plaque psoriasis; **delandistrogene moxeparvovec** for Duchenne muscular dystrophy; **obeticholic acid** for nonalcoholic steatohepatitis; **ritlecitinib** for alopecia areata; and **valoctocogene roxaparvovec** for hemophilia A.

Key pipeline drugs with FDA approval decisions expected by the end of the 3rd quarter 2023

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Exxua™ (gepirone)	Fabre-Kramer Pharmaceuticals	Major depressive disorder	6/23/2023
Zuranolone	Biogen	Major depressive disorder and postpartum depression	8/5/2023
Rozanolixizumab	UCB	Generalized myasthenia gravis*	2Q 2023
Zilucoplan	UCB	Generalized myasthenia gravis*	9/2023
Zimura® (avacincaptad pegol)	Iveric Bio	Geographic atrophy	8/19/2023
Talquetamab	Janssen	Multiple myeloma*	8/11/2023
Elranatamab	Pfizer	Multiple myeloma*	8/22/2023
Beyfortus® (nirsevimab)	AstraZeneca/Sanofi	Respiratory syncytial virus	3Q 2023
Lebrikizumab	Eli Lilly	Atopic dermatitis	3Q 2023
Nedosiran	Novo Nordisk	Primary hyperoxaluria*	9/2023

* Orphan Drug Designation

The report is organized in the following manner:

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 3rd quarter 2023.

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 3rd quarter 2023 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

Gepirone extended-release (ER) (Brand Name: **Exxua™**)

Manufacturer: Fabre-Kramer Pharmaceuticals
Expected FDA decision: June 23, 2023

Therapeutic use

Gepirone ER is under review for the treatment of major depressive disorder (MDD).

Depression is one of the most common mental disorders in the U.S. The prevalence of a major depressive episode among U.S. adults aged 18 or older in 2020 was 21 million (8.4% of all adults). Of adults with a major depressive episode, an estimated 66% received treatment in the past year.

Clinical profile

Gepirone is a serotonin single receptor (1A) agonist. Modulation of serotonergic neurotransmission has been the primary mechanism for treating depression. Gepirone's selectivity for 1A receptors reduces overstimulation of other serotonin receptor subtypes.

Pivotal trial data:

The FDA submission for gepirone ER was based on two pivotal studies: 134001 and FKGBE007. Study 134001 was a randomized, double-blind, placebo-controlled study in 208 patients with MDD. Patients were randomized to gepirone ER or placebo. The primary endpoint was the change from baseline on the 17-item Hamilton Depression Rating Scale (HAM-D; scores range from 0 to 52, with higher scores indicating more severe depression) at Week 8 or last visit. Gepirone ER demonstrated a statistically significant reduction in HAM-D total scores vs. placebo at Week 8. The change from baseline in HAM-D total scores was -9.04 and -6.75 for gepirone ER and placebo, respectively (difference of -2.47, 95% CI: -4.41, -0.53; p = 0.013).

Study FKGBE007 was a randomized, double-blind, placebo-controlled study in 248 patients with MDD. Patients were randomized to gepirone ER or placebo. The primary endpoint was the change from baseline on the HAM-D at Week 8 or last visit. Gepirone ER demonstrated a statistically significant reduction in HAM-D total scores vs. placebo at Week 8. The change from baseline in HAM-D total scores was -10.22 and -7.96 for gepirone ER and placebo, respectively (difference of -2.45, 95% CI: -4.47, -0.43; p = 0.018).

Safety:

The most common adverse events with gepirone ER use were dizziness, nausea, and headache.

Dosing:

In the pivotal trials, gepirone ER was administered orally once daily.

What you need to know:

Proposed Indication: Treatment of MDD

Mechanism: Serotonin single receptor (1A) agonist

Efficacy: HAM-D17 change from baseline at Week 8: -9.04 to -10.22 vs. -6.75 to -7.96 with placebo

Common AEs: Dizziness, nausea, headache

Dosing: Oral once daily

Why it Matters: Novel MOA for treatment of MDD, potential for reduced side effects vs. other serotonergic agents (eg, less weight gain or sexual dysfunction)

Important to Note: Alternatives available with significant generic utilization, lack of head-to-head trial data, conflicting historical efficacy data for gepirone

Estimated Cost: ~$5,700 per year (based on current pricing for Trintellix)

Exxua™ *(continued...)*

Competitive environment

If approved, gepirone would offer a novel mechanism of action (MOA) for the treatment of MDD. Unlike other serotonergic agents (eg, selective serotonin reuptake inhibitors [SSRIs]), gepirone selectively works on serotonin 1A receptors. By sparing other serotonin receptor subtypes, gepirone may reduce the risk of side effects associated with other commonly used antidepressants (eg, sexual dysfunction and weight gain). Gepirone has similarities in mechanism to BuSpar® (buspirone), which has been available for decades. Buspirone is FDA approved for treatment of anxiety but has been used off-label for adjunctive treatment of MDD.

Gepirone has been in the pipeline for some time, and development has been hindered by inconsistent efficacy results among the trials. It remains unclear if the current data set is enough for FDA to overcome these past issues. The current FDA resubmission for approval is supported by the two positive 8-week trials, but both were placebo-controlled so there is a lack of data comparing the efficacy and safety of gepirone vs. standard of care antidepressants.

The distinct MOA may confer some tolerability benefits, but gepirone would be entering the marketplace when almost all current first-line antidepressants are available as lower cost generics. Other pipeline antidepressants are also often evaluated in specific subsets of patients (eg, treatment-resistant MDD or postpartum depression), where there may be a higher unmet need than the general MDD population.

For reference, the Wholesale Acquisition Cost (WAC) for Trintellix® (vortioxetine), a branded antidepressant, is approximately $5,700 per year.

Zuranolone (Brand Name: To be determined)

Manufacturer: Biogen/Sage Therapeutics
Regulatory designations: Breakthrough Therapy, Fast Track
Expected FDA decision: August 5, 2023

Therapeutic use

Zuranolone is under review for the treatment of major depressive disorder (MDD) and postpartum depression (PPD).

PPD is one of the most common medical complications during and after pregnancy. PPD is estimated to affect approximately 13% of women who have given birth in the U.S. or approximately 500,000 women annually.

Clinical profile

Zuranolone is a neuroactive steroid, GABA-A receptor positive allosteric modulator. Alterations in GABA levels and GABA-A receptor expression are thought to contribute to development of depression and GABAergic dysregulation in depression has been linked to altered stress responses and changes in neurotransmission.

Pivotal trial data:

MDD

The efficacy of zuranolone was evaluated in the LANDSCAPE program, which includes four randomized studies in adults with MDD (MDD-201B, MOUNTAIN, WATERFALL, CORAL). In each trial, patients received zuranolone as a 14-day treatment course.

What you need to know:

Proposed Indication: Treatment of MDD and PPD

Mechanism: GABA-A receptor positive allosteric modulator

Efficacy: Primary endpoint met in 2 of 3 MDD and 2 of 2 PPD trials (refer to full text for study results)

Common AEs: Headache, somnolence, dizziness, nausea, sedation

Dosing: Oral once daily (14-day treatment course)

Why it Matters: Faster-acting antidepressant, administered orally as a 14-day treatment, unmet need for PPD

Important to Note: Alternatives available with significant generic utilization, lack of head-to-head trial data, lack of robust data showing sustained benefit beyond day 15 for MDD, lack of infant safety data with use while breastfeeding, likely a controlled substance

MDD-201B was a Phase 2, randomized, double-blind, placebo-controlled study in 89 patients. Patients received zuranolone 30 mg or placebo. The primary endpoint was the change from baseline to Day 15 in the score on the 17-item Hamilton Depression Rating Scale (HAM-D; scores range from 0 to 52, with higher scores indicating more severe depression). Zuranolone 30 mg demonstrated a statistically significant reduction in HAM-D total scores vs. placebo at Day 15 (difference of -7.1, $p < 0.001$).

MOUNTAIN was a Phase 3, randomized, double-blind, placebo-controlled study in 581 patients. Patients were randomized to zuranolone 20 mg, zuranolone 30 mg, or placebo. The primary endpoint was the change from baseline to Day 15 in the score on the 17-item HAM-D scale. Zuranolone 30 mg did not demonstrate a statistically significant reduction in HAM-D total scores vs. placebo at Day 15 (difference of -1.4, $p = 0.115$). A statistically significant difference was noted vs. placebo at all visits during the treatment period leading up to Day 15 (Day 3, Day 8, and Day 12). Zuranolone 20 mg did not demonstrate a significant improvement vs. placebo at any measured timepoint.

WATERFALL was a Phase 3, randomized, double-blind, placebo-controlled study in 543 patients. Patients were randomized to zuranolone 50 mg or placebo. The primary endpoint was the change from baseline to Day 15 in the score on the 17-item HAM-D scale. Zuranolone 50 mg demonstrated a statistically significant reduction in HAM-D total scores vs. placebo at Day 15 (difference of -1.7, $p = 0.0141$).

Zuranolone *(continued…)*

CORAL was a Phase 3, randomized, double-blind, placebo-controlled study in 440 patients. Patients were randomized to zuranolone 50 mg co-initiated with an open-label standard of care antidepressant (ADT) or standard of care ADT co-initiated with placebo. The primary endpoint was the change from baseline to Day 3 in the score on the 17-item HAM-D scale. Zuranolone 50 mg demonstrated a statistically significant reduction in HAM-D total scores vs. placebo at Day 3 (difference of -1.9, p = 0.0004).

PPD

The efficacy of zuranolone was evaluated in the NEST program, which includes two randomized studies in adult women with PPD (ROBIN and SKYLARK). In each trial, patients received zuranolone as a 14-day treatment course.

ROBIN was a Phase 3, randomized, double-blind, placebo-controlled study in 153 adult women with PPD. Patients were randomized to zuranolone 30 mg or placebo. The primary endpoint was the change from baseline to Day 15 in the score on the 17-item HAM-D scale. Zuranolone 30 mg demonstrated a statistically significant reduction in HAM-D total scores vs. placebo at Day 15 (difference of -4.2, p = 0.003).

SKYLARK was a Phase 3, randomized, double-blind, placebo-controlled study in 195 adult women with PPD. Patients were randomized to zuranolone 50 mg or placebo. The primary endpoint was the change from baseline to Day 15 in the score on the 17-item HAM-D scale. Zuranolone 50 mg demonstrated a statistically significant reduction in HAM-D total scores vs. placebo at Day 15 (difference of -4.0, p = 0.007).

Safety:

The most common adverse events with zuranolone use were headache, somnolence, dizziness, nausea, and sedation.

Dosing:

In the pivotal trials, zuranolone was administered orally once daily for 14 days.

Zuranolone *(continued…)*

Competitive environment

If approved, zuranolone would offer a "faster acting" antidepressant, with benefit seen in clinical trials as early as day 3. On average, most antidepressants typically require several weeks for patients to see benefit. This is particularly noteworthy in PPD where there is a higher unmet need. Symptom improvement as early as possible can be especially important for PPD to ensure better early bonding between a mother and a newborn or young infant.

Zuranolone would potentially be the second approved drug with an indication specific for PPD. Zulresso® (brexanolone), also by Sage, has a similar MOA to zuranolone, but must be administered via continuous intravenous (IV) infusion for 60 hours, a major barrier to use in the postpartum setting.

Like gepirone, zuranolone will be entering a crowded marketplace with significant generic utilization. Even for PPD, SSRIs have been used for years off-label and have more long-term observational safety data with use during breastfeeding. In the zuranolone trials, women were not permitted to breastfeed during the 14-day treatment and 7 days after the treatment course.

The other big question, particularly for treatment of MDD, is durability of response. While zuranolone met its primary endpoint at day 3 or 15 in 2 of the 3 trials, there was diminished benefit by week 6. Durability is important for MDD because patients are typically treated for a depressive episode for around 4 to 6 weeks to produce a response or remission, and then treatment is continued for several months to consolidate the response and prevent a relapse. In the case of zuranolone, there is a lack of robust long-term randomized data.

Finally, because of zuranolone's mechanism as a GABA modulator, it will likely be a controlled substance (Zulresso is a scheduled IV controlled substance).

Rozanolixizumab (Brand Name: To be determined)

Manufacturer: UCB
Regulatory designation: Orphan Drug
Expected FDA decision: 2Q 2023

Therapeutic use

Rozanolixizumab is under review for the treatment of generalized myasthenia gravis (gMG) in adult patients who are acetylcholine receptor autoantibody-positive (AChR-Ab+) or muscle-specific tyrosine kinase autoantibody-positive (MuSK-Ab+).

Myasthenia gravis (MG) is a chronic, fluctuating, autoantibody-mediated disease that results in motor weakness involving ocular, bulbar, respiratory and limb muscles. MG is classified as either ocular MG or gMG, in which the weakness extends beyond the ocular muscles. Patients with MG commonly present with ocular symptoms first, but up to 85% of patients progress to gMG within 2 years.

The prevalence of MG in the U.S. is estimated to be 14 to 20 per 100,000 people. The mechanisms of pathology are best understood for AChR-Ab+ and MuSK-Ab+ MG. Most MG patients, approximately 85%, are AChR-Ab+, and only 6% are MuSK-Ab+. Rare cases of concurrent AChR-MuSK antibody seropositivity have been reported. The remaining patients are either autoantibody seronegative or low-density lipoprotein receptor-related protein 4 autoantibody-positive (anti-LRP4-Ab+).

What you need to know:

Proposed Indication: Treatment of gMG in adults with AChR-Ab+ or MuSK-Ab+

Mechanism: FcRn antagonist

Efficacy: MG-ADL reduction of 3.37 to 3.40 points vs. placebo

Common AEs: Headache, diarrhea, pyrexia, nausea

Dosing: SC infusion; administered as 6 infusions per cycle (one infusion per week) and repeated as needed depending on clinical response

Why it Matters: SC administration, also being studied for other pathogenic IgG-driven autoimmune diseases, inclusion of MuSK-Ab+ patients in the pivotal study

Important to Note: Healthcare provider-administered, alternatives available, no head-to-head trial data vs. standard of care, study not powered for MuSK-Ab+ subgroup analysis

Estimated Cost: ~$225,000 per year (based on current pricing for Vyvgart)

Clinical profile

Rozanolixizumab is a monoclonal antibody that binds with high affinity to neonatal Fc receptor (FcRn), which is widely expressed throughout the body and plays a central role in rescuing immunoglobulin (IgG) antibodies from degradation. By blocking FcRn, rozanolixizumab inhibits IgG recycling and induces the removal of pathogenic IgG autoantibodies against both AChR and MuSK.

Pivotal trial data:

The efficacy of rozanolixizumab was evaluated in MycarinG, a Phase 3, randomized, double-blind, placebo-controlled study in 200 patients with AChR-Ab+ or MuSK-Ab+ gMG. Patients received either rozanolixizumab 7 mg/kg, rozanolixizumab 10 mg/kg, or placebo for 6 weeks, followed by 8 weeks of observation. The primary endpoint was the change from baseline to Day 43 in myasthenia gravis activities of daily living (MG-ADL), which is an 8-item patient-reported scale that measures MG symptoms and functional status. The total MG-ADL score ranges from 0 to 24, with larger numbers indicating more severe disease and a clinically meaningful difference defined as at least a 2-point reduction.

Rozanolixizumab *(continued...)*

Both rozanolixizumab 7 mg/kg (least square (LS) mean -3.37) and rozanolixizumab 10 mg/kg (-3.40) showed greater reductions from baseline in MG-ADL score compared to placebo (-0.78). The LS mean difference from placebo was -2.59 (95% CI: -4.09, -1.25, p < 0.0001) for rozanolixizumab 7 mg/kg and -2.62 (95% CI: -3.99, -1.16, p < 0.0001) for rozanolixizumab 10 mg/kg. For patients with MuSK-Ab+ gMG, the LS mean difference from placebo was -6.30 for rozanolixizumab 7 mg/kg and -4.20 for rozanolixizumab 10 mg/kg, but the study was not powered for this statistical subgroup analysis.

Safety:

The most common adverse events with rozanolixizumab use were headache, diarrhea, pyrexia, and nausea.

Dosing:

In the pivotal trials, rozanolixizumab was administered subcutaneously (SC) as 6 infusions per cycle (one infusion per week) and repeated as needed depending on clinical response.

Competitive environment

The current standard of care for gMG includes acetylcholinesterase inhibitors (eg, pyridostigmine), steroids, and nonsteroidal immunosuppressants (such as azathioprine, tacrolimus, mycophenolate mofetil). For AChR-Ab+ gMG patients who require additional steroid-sparing treatment, biologic immunomodulators, such as AstraZeneca's Ultomiris® (ravulizumab-cwvz) and Soliris® (eculizumab), and Argenx's Vyvgart® (efgartigimod alfa-fcab), are available for the treatment of. In MG with life-threatening signs, plasma exchange (PLEX) or IV immunoglobulin (IVIg) may be used as short-term treatments.

Rozanolixizumab would be a new SC addition to the biologic option for gMG with a broader proposed indication than treatment of adults with AChR-Ab+. However, the additional MuSK-Ab+ subpopulation is expected to be very small since MuSK-Ab is found in less than 10% of MG cases. Although reduction in MG-ADL was observed in MuSK-Ab+ patients, the study was not powered for the subgroup statistical analysis. Rozanolixizumab was not compared head-to-head against existing treatment options, but when compared indirectly, the efficacy appears similar.

Similar to Vyvgart, another FcRn antagonist, which is IV infused once weekly for 4 weeks per cycle, rozanolixizumab is dosed with 6 weekly cyclic treatments. Rozanolixizumab is a SC infusion, but it is administered by healthcare professionals via an infusion pump.

Finally, rozanolixizumab is in Phase 3 development for another IgG-driven autoimmune disease: myelin oligodendrocyte glycoprotein antibody-associated disease. There is a high unmet need for treatments for this disease, as there are currently no FDA approved treatments.

For reference, the WAC for Vyvgart is approximately $225,000 per year.

Zilucoplan (Brand Name: To be determined)

Manufacturer: UCB
Regulatory designation: Orphan Drug
Expected FDA decision: September 2023

Therapeutic use

Zilucoplan is under review for the treatment of generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor antibody positive (AChR-Ab+).

Clinical profile

Zilucoplan is a macrocyclic peptide that inhibits complement component 5 (C5) via a dual MOA. The complement cascade, when activated by AChR autoantibodies resulting in the formation of membrane attack complex (MAC), is one of the main causes of neuromuscular junction destruction and abnormal signaling function. Zilucoplan blocks MAC formation by inhibiting C5 cleavage to C5a and C5b, and blocks residual C5b-mediated activation of MAC by binding to pre-existing C5b.

What you need to know:

Proposed Indication: Treatment of gMG in adults with AChR-Ab+

Mechanism: Complement C5 inhibitor

Efficacy: MG-ADL reduction of 2.1 points vs. placebo

Common AEs: Injection site reactions, non-serious infections, diarrhea, increased lipase

Dosing: SC once daily

Why it Matters: Self-administered SC administration

Important to Note: Once daily administration, alternatives available, no head-to-head trial data vs. standard of care, boxed warning and REMS for increased risk of meningococcal infections

Estimated Cost: ~$225,000 per year (based on current pricing for Vyvgart)

Pivotal trial data:

The efficacy of zilucoplan was evaluated in RAISE, a Phase 3, randomized, double-blind, placebo-controlled study in 174 patients with AChR-Ab+ gMG. Patients received zilucoplan or placebo for 12 weeks. The primary endpoint was the change from baseline to Week 12 in myasthenia gravis activities of daily living (MG-ADL), which is an 8-item patient-reported scale with the total score ranging from 0 to 24, with larger numbers indicating more severe disease and a clinically meaningful difference defined as at least a 2-point reduction.

Zilucoplan demonstrated a statistically significant reduction in MG-ADL score compared with placebo. At Week 12, the change from baseline in the MG-ADL score was -4.4 with zilucoplan and -2.3 with placebo (LS mean difference of -2.1; $p < 0.001$). Greater reductions in MG-ADL score were seen as early as Week 1 for the zilucoplan group and the effect was maintained up to Week 12.

Safety:

The most common adverse events with zilucoplan use were injection site reactions, non-serious infections, diarrhea, and increased lipase.

Since zilucoplan is a C5 inhibitor, it is likely to have a boxed warning and a Risk Evaluation and Mitigation Strategy (REMS) program for the increased risk of meningococcal infections like the other complement inhibitor drugs (Soliris, Ultomiris).

Dosing:

In the pivotal trial, zilucoplan was administered SC once daily.

Zilucoplan *(continued...)*

Competitive environment

Like rozanolixizumab, zilucoplan would be another new SC administered addition to the biologic treatments for gMG, but it would offer a dosing convenience advantage over the current biologic immunomodulators and rozanolixizumab because zilucoplan is a self-administered injection. While it does require daily injections, currently available biologic alternatives are all IV infused products (dosed less frequently, once every week to once every 8 weeks). Currently, a SC formulation of Vyvgart is also under FDA review for gMG, so the dosing convenience advantage for zilucoplan may not be unique by the time it comes to market.

Zilucoplan was not compared in head-to-head trials against existing treatment options; compared indirectly, the efficacy results appear to be similar.

For reference, the WAC for Vyvgart is approximately $225,00 per year.

Avacincaptad pegol (Brand Name: **Zimura®**)

Manufacturer: IVERIC bio
Regulatory designations: Breakthrough Therapy, Fast Track
Expected FDA decision: August 19, 2023

Therapeutic use

Avacincaptad pegol is under review for the treatment of geographic atrophy secondary to age-related macular degeneration (AMD).

Geographic atrophy is an advanced form of dry AMD caused by destruction of retinal cells through irreversible lesion growth. Geographic atrophy is a progressive disease that typically starts in the perifoveal region and expands to involve the fovea, eventually leading to permanent loss of visual acuity.

Approximately 1 million people are affected with geographic atrophy, making it is one of the leading causes of blindness in the U.S..

Clinical profile

Avacincaptad pegol is a complement C5 protein inhibitor. Overactivity of the complement system and the C5 protein are suspected to play a role in the development and growth of scarring and vision loss associated GA. By targeting C5, avacincaptad pegol has the potential to decrease activity of the complement system that causes the degeneration of retinal cells.

What you need to know:

Proposed Indication: Treatment of geographic atrophy secondary to AMD

Mechanism: Complement C5 inhibitor

Efficacy: Reduction in GA lesion growth at Month 12: 14.3% to 27.4% with 2 mg monthly injections vs. sham

Common AEs: Conjunctival hemorrhage, increased IOP, choroidal neovascularization

Dosing: Intravitreal injection once every month

Why it Matters: Second FDA approved treatment for GA, large potential target population

Important to Note: Alternative now available (Syfovre), increased risk of new-onset exudations (ie, wet AMD) and serious eye infections, initial dosing is expected to be monthly intravitreal injections

Estimated Cost: $2,190 per month (based on current pricing for Syfovre)

Pivotal trial data:

The efficacy of avacincaptad pegol was evaluated in GATHER1 and GATHER2, two Phase 3, randomized, double-masked, sham-controlled studies in patients with GA secondary to AMD. GATHER1 (N = 286) consisted of two parts: in Part 1, patients were randomized to receive avacincaptad pegol 1 mg, avacincaptad pegol 2 mg, and sham. Subsequently in Part 2, patients were randomized to receive avacincaptad pegol 2 mg, avacincaptad pegol 4 mg, and sham. All doses were given monthly. The primary endpoint was the rate of GA lesion area growth at Month 12. The reduction in the mean rate of GA growth over 12 months was 27.4% (p = 0.0072) for the avacincaptad pegol 2 mg cohort and 27.8% (p = 0.0051) for the avacincaptad pegol 4 mg cohort compared with their corresponding sham cohorts.

In GATHER2 (N = 448), patients were randomized to receive either avacincaptad pegol 2 mg or sham once every month for 12 months. At 12 months, patients in the avacincaptad pegol arm were re-randomized to either receive avacincaptad pegol 2 mg once monthly or every other month until Month 23. The final evaluation will take place at Month 24. The primary endpoint was based on GA lesion area at Month 12. The reduction in the mean rate of GA growth over 12 months was 14.3% (p = 0.0064) compared to the sham.

Zimura® *(continued...)*

Safety:

The most common adverse events with avacincaptad pegol use were conjunctival hemorrhage, increased intraocular pressure (IOP), and choroidal neovascularization.

Dosing:

In the pivotal trials, avacincaptad pegol was administered via intravitreal injection once every month.

Competitive environment

Up until this year, there were no FDA approved treatments for GA. Existing vascular endothelial growth factor (VEGF) inhibitors (eg, Eylea®, Lucentis®) used for "wet" AMD are ineffective for GA due to differences in pathophysiology. However, in February 2023, the FDA approved Apellis Pharmaceuticals' Syfovre™ (pegcetacoplan), a complement C3 inhibitor for GA. If approved, avacincaptad pegol would be a direct competitor to Syfovre.

Avacincaptad pegol and Syfovre have a similar MOA, although Syfovre blocks an earlier step in the complement cascade. The clinical significance of this difference is unknown and there are no direct head-to-head trials comparing the two products. From a dosing perspective, Syfovre is approved for use as a monthly or every other month injection whereas data for avacincaptad pegol is only available for a monthly injection.

A concern with both avacincaptad pegol and Syfovre is the development of new-onset exudations (ie, wet AMD). Even in the absence of any treatment, patients with GA can develop wet AMD and vice versa, but in clinical trials, the risk of exudations increases with these complement inhibitors. A higher rate of exudations with patients treated with either of these drugs could result in more patients needing to be treated with VEGF inhibitors.

For reference, the WAC for Syfovre is $2,190 per month.

Talquetamab (Brand Name: To be determined)

Manufacturer: Janssen
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: August 11, 2023 (Accelerated Approval pathway)

Therapeutic use

Talquetamab is under review for the treatment of patients with relapsed or refractory multiple myeloma who were previously treated with a proteasome inhibitor, an immunomodulatory agent, and an anti-CD38 monoclonal antibody.

Multiple myeloma is a blood cancer of plasma cells in the bone marrow. In patients with multiple myeloma, the plasma cells make an antibody that stimulates overgrowth of plasma cells. Overgrowth of plasma cells and abnormal plasma cells can lead to a variety of complications, including low blood counts, infections, and kidney problems.

The American Cancer Society estimates that there are 35,730 new cases of multiple myeloma diagnosed annually and about 12,590 deaths.

Clinical profile

Talquetamab is a G protein–coupled receptor, family C, group 5, member D (GPRC5D) CD-3 targeted bispecific antibody. Talquetamab is designed to bind to two different targets simultaneously: GPRC5D, which is highly expressed on the surface of multiple myeloma cells, and CD3 receptors found on the surface of T-cells. By binding both targets, talquetamab bridges the 2 cells together and activates the T-cells to kill the myeloma cell.

What you need to know:

Proposed Indication: Treatment of patients with relapsed or refractory multiple myeloma who were previously treated with a proteasome inhibitor, an immunomodulatory agent, and an anti-CD38 monoclonal antibody

Mechanism: GPRC5D CD-3 targeted bispecific antibody

Efficacy: ORR: 62.7% to 74.1%

Common AEs: Anemia, neutropenia, lymphopenia, thrombocytopenia, leukopenia, skin-related events, dysgeusia, CRS

Dosing: SC once every week or every other week (maintenance dose)

Why it Matters: Novel MOA, SC administration, potential future use in earlier treatment settings

Important to Note: Narrow initial indication, lack of late-stage trial data, requires healthcare provider administration and monitoring

Estimated Cost: ~$39,500 per month (based on current pricing for Tecvayli)

Pivotal trial data:

The efficacy of talquetamab was evaluated in MonumenTAL-1, a Phase 1/2, open-label, single-arm study in multiple myeloma patients who are refractory to at least one proteasome inhibitor, one immunomodulatory drug and one anti-CD38 monoclonal antibody. The study included patients who were T-cell redirection therapy-naïve or previously exposed to T-cell redirection therapy (with another bispecific antibody or CAR T cell therapy). Two doses of talquetamab were evaluated for efficacy: 0.4 mg/kg weekly and 0.8 mg/kg every other week. The primary endpoint was the objective response rate (ORR).

In T-cell redirection therapy-naïve patients (N = 288), the ORR was 74.1% to 73.1% with the 0.4 mg/kg and 0.8 mg/kg doses, respectively. The median duration of response (DOR) was 9.3 months (range: 6.6, 12.7) and 13.0 months (range: 10.6, not estimable), respectively.

In patients previously exposed to T-cell redirection therapy (N = 51), the ORR was 62.7%. The data are still immature, but the median DOR was 12.7 months (range: 3.7, not estimable).

Talquetamab *(continued…)*

Safety:

The most common adverse events with talquetamab use were anemia, neutropenia, lymphopenia, thrombocytopenia, leukopenia, skin-related events, dysgeusia, and cytokine release syndrome (CRS).

CRS is a condition that may occur after treatment with some types of immunotherapy, such as monoclonal antibodies and CAR T cells. Signs and symptoms of CRS include fever, nausea, headache, rash, rapid heartbeat, low blood pressure, and trouble breathing. Most patients have a mild reaction, but sometimes, the reaction may be severe or life threatening.

Dosing:

In the pivotal trial, talquetamab was administered SC once weekly or once every other week.

Competitive environment

If approved, talquetamab would provide an additional option with a novel MOA for treatment for multiple myeloma in the fourth line setting. While several new agents have been approved for multiple myeloma, including CAR T cell therapies, there is still an unmet need because multiple myeloma is generally incurable and associated with a high relapse rate with conventional drugs and patients are likely to need multiple lines of therapy over the course of the disease.

The data available for talquetamab is limited to early-stage trials and the initial accelerated approval would be based on ORR. Additional trials will be required to confirm clinical benefit (ie, overall survival). The initial indication is expected to be narrow but talquetamab is being evaluated in other trials and in earlier settings of multiple myeloma so the target population could increase over time depending on the success of those trials.

While talquetamab is a SC injection, it will require administration by a healthcare provider because of post-administration monitoring.

For reference, the WAC for Tecvayli™ (teclistamab), another bispecific antibody for multiple myeloma, is approximately $39,500 per month.

Elranatamab (Brand Name: To be determined)

Manufacturer: Pfizer
Regulatory designations: Orphan Drug, Breakthrough Therapy, Fast Track
Expected FDA decision: August 22, 2023 (Accelerated Approval pathway)

Therapeutic use

Elranatamab is under review for the treatment of patients with relapsed or refractory multiple myeloma who were previously treated with a proteasome inhibitor, an immunomodulatory agent, and an anti-CD38 monoclonal antibody.

Clinical profile

Elranatamab is a B-cell maturation antigen (BCMA) CD3-targeted bispecific antibody. BCMA is highly expressed on the surface of multiple myeloma cells, and CD3 receptors found on the surface of T-cells, bridging them together and activating the T-cells to kill the myeloma cell.

Pivotal trial data:

The efficacy of elranatamab was evaluated in MagnetisMM, a Phase 2, open-label, single-arm study in patients with multiple myeloma who are refractory to at least one proteasome inhibitor, one immunomodulatory drug and one anti-CD38 monoclonal antibody. The FDA submission is primarily based on Cohort A of the study, which included 123 patients who had not received prior BCMA-directed therapy. The primary endpoint was objective response rate (ORR).

What you need to know:

Proposed Indication: Treatment of patients with relapsed or refractory multiple myeloma who were previously treated with a proteasome inhibitor, an immunomodulatory agent, and an anti-CD38 monoclonal antibody

Mechanism: BCMA CD3-targeted bispecific antibody

Efficacy: ORR: 61%

Common AEs: Anemia, neutropenia, thrombocytopenia, lymphopenia, CRS

Dosing: SC once weekly (maintenance dose)

Why it Matters: More tolerable safety profile than CAR T cell therapies, SC administration, potential future use in earlier treatment settings

Important to Note: Narrow initial indication, lack of late-stage trial data, requires healthcare provider administration and monitoring

Estimated Cost: ~$39,500 per month (based on current pricing for Tecvayli)

As of the data cut-off on October 14, 2022, the median follow-up was 10.4 months. Patients who received elranatamab achieved an ORR of 61%. In patients achieving a response, there was an 84% probability of maintaining response at 9 months.

Safety:

The most common adverse events with elranatamab use were anemia, neutropenia, thrombocytopenia, lymphopenia, and cytokine release syndrome (CRS).

Dosing:

In the pivotal trial, elranatamab was administered SC weekly (76 mg dose) on a 28-day cycle with a step-up priming dose regimen, 12 mg and 32 mg administered on day 1 and day 4, respectively, during cycle 1.

Elranatamab *(continued…)*

Competitive environment

Elranatamab would provide an additional SC administered, BCMA targeted therapy for multiple myeloma. As discussed previously, while several new agents have been approved for multiple myeloma over the years, there is still an unmet need for additional options because patients with the disease often need multiple lines of therapy.

Elranatamab would be the second BCMA targeted bispecific antibody – Janssen's Tecvayli™ (teclistamab-cqyv), was approved in October 2022. Other BCMA targeted therapies are CAR T cell therapies (Carvykti® and Abecma®). Bispecific antibodies such as elranatamab provide an "off-the-shelf' T-cell redirection therapy and an alternative to CAR T cell therapies. While demonstrating promising response rates, the manufacturing process for CAR T cell therapies is cumbersome and expensive because they are individually manufactured for each patient. Additionally, CAR T cell therapies have reported high rates of CRS and neurotoxicity. Compared indirectly to CAR T cell therapies, elranatamab does appear to have a more tolerable safety profile.

Like talquetamab, this initial FDA submission for elranatamab is through the accelerated approval pathway and confirmatory trials will be required to demonstrate clinical benefit. Additionally, elranatamab will need to be administered by a healthcare provider because of post-administration monitoring.

For reference, the WAC for Tecvayli is approximately $39,500 per month.

Nirsevimab (Brand Name: **Beyfortus®**)

Manufacturer: AstraZeneca/Sanofi
Regulatory designations: Breakthrough Therapy, Fast Track
Expected FDA decision: 3Q 2023

Therapeutic use

Nirsevimab is under review for the prevention of lower respiratory tract disease (LRTD) caused by respiratory syncytial virus (RSV) in neonates and infants entering or during their first RSV season, as well as in children up to 24 months of age who remain vulnerable to severe RSV disease through their second RSV season.

RSV is a common respiratory virus that usually causes mild, cold-like symptoms that typically last between 1 to 2 weeks. However, RSV infection can be serious and is the leading cause of bronchiolitis and pneumonia in both infants and the elderly. In the U.S. and other regions with similar climates, the RSV season typically begins in November and lasts through April, but it may begin earlier or last longer in certain communities.

More than 90% of all children will be infected with RSV by the age of 2. Each year in the U.S., RSV leads to 58,000 to 80,000 hospitalizations and 100 to 300 deaths among children under the age of 5.

What you need to know:

Proposed Indication: Prevention of LRTD caused by RSV in neonates and infants entering or during their first RSV season, as well as in children up to 24 months of age who remain vulnerable to severe RSV disease through their second RSV season

Mechanism: Monoclonal antibody

Efficacy: 79.5% against MA RSV LRTIs

Common AEs: Pyrexia, nasal congestion

Dosing: Single-dose IM injection

Why it Matters: High unmet need, broader indication vs. Synagis, single dose vs. Synagis's monthly doses

Important to Note: Potential future competition (eg, Pfizer's maternal vaccine), efficacy beyond Day 150 unknown

Clinical profile

Nirsevimab is a recombinant neutralizing human immunoglobulin type 1 (IgG1) long-acting monoclonal antibody that binds to a highly conserved epitope on the prefusion F protein of RSV, inhibiting the essential membrane fusion step in the viral entry into cells lining the respiratory tract, neutralizing the virus and blocking cell-to-cell fusion.

Pivotal trial data:

The FDA submission for nirsevimab was based on MELODY, MEDLEY, and a prespecified pooled analysis of the MELODY and Phase 2b trials looking at healthy preterm and term infants who received the optimized dose of nirsevimab compared to placebo.

The Phase 2b trial was a randomized, double-blind, placebo-controlled study that included 1,453 healthy preterm infants who were randomized in a 2:1 ratio to receive nirsevimab or placebo. The primary endpoint was medically attended (MA) RSV-associated lower respiratory tract infections (LRTIs) through 150 days post dose. The secondary efficacy endpoint was hospitalization for RSV-associated lower respiratory tract infection through 150 days post dose. The incidence of MA RSV-associated LRTIs was 70.1% lower with nirsevimab prophylaxis than with placebo (95% CI: 52.3, 81.2; $p < 0.001$). The incidence of hospitalization for RSV-associated LRTIs was 78.4% lower with nirsevimab than with placebo (95% CI: 51.9, 90.3; $p < 0.001$).

Beyfortus® *(continued…)*

MELODY was a Phase 3, randomized, double-blind, placebo-controlled study that included 1,490 healthy late preterm and term infants who were randomized to receive a single dose of nirsevimab or placebo. The primary endpoint was the incidence of MA RSV LRTIs for 150 days post dose. A key secondary endpoint was the incidence of hospitalization due to RSV confirmed LRTIs, 150 days post dose. The overall vaccine efficacy was 74.5% (95% CI: 49.6, 87.1; $p < 0.001$) against RSV LRTIs. However, the efficacy of 62.1% (95% CI: -8.6, 86.6; $p = 0.07$) against hospitalization due to RSV LRTIs was not statistically significant.

A prespecified pooled analysis of the MELODY and Phase 2b trials (n = 2,350) was conducted to assess the efficacy of nirsevimab in infants receiving the optimized dosing regimen of nirsevimab. The overall vaccine efficacy was 79.5% (95% CI: 65.9, 87.7; $p < 0.0001$) against MA RSV LRTIs and 77.3% (95% CI: 50.3, 89.7; $p < 0.001$) against hospitalizations for RSV LRTIs.

MEDLEY was a Phase 2/3, randomized, double-blind, Synagis® (palivizumab)-controlled study evaluating the safety, pharmacokinetics, and descriptive efficacy of nirsevimab in 925 infants eligible to receive palivizumab when entering their first or second RSV season. The primary endpoint was safety and tolerability, but a key secondary endpoint was incidence of MA RSV LRTIs. The incidence of treatment emergent adverse events (TEAEs) through 360 days was similar between nirsevimab and palivizumab treatment groups (66.0% vs. 65.0% for the preterm cohort and 71.2% vs. 73.5% for the congenital heart disease (CHD)/chronic lung disease (CLD) cohort, respectively). The incidence of MA RSV LRTIs at Day 151 was low in both the nirsevimab group (0.6%) and the palivizumab group (1.0%).

Safety:

The most common adverse events with nirsevimab use were pyrexia and nasal congestion.

Dosing:

In the pivotal trails, nirsevimab was administered as a single dose via intramuscular (IM) injection.

Competitive environment

Currently, Sobi's monoclonal antibody Synagis is the only available preventive RSV treatment for pediatric patients, but its use is restricted to a niche group of infants at high risk for RSV-related complications. Therefore, there is still a very high unmet need for RSV protection in healthy newborns and infants. If approved, nirsevimab would be the second preventive RSV treatment with a broader proposed indication in all infants. Unlike Synagis, which requires monthly doses that continue throughout the RSV season, nirsevimab is administered as a single dose. The timing and setting of nirsevimab dosing would differ for infants born during October to March and those born during April through September because the efficacy of nirsevimab beyond Day 150 is unknown.

In pivotal, placebo-controlled trials, nirsevimab appeared well tolerated and showed statistically significant efficacy against LRTIs caused by RSV. In the prespecified pooled analysis, nirsevimab met both the primary and secondary endpoints, showing efficacy against both MA LRTIs and hospitalization caused by RSV. In a separate Synagis-controlled study, nirsevimab and Synagis had similar safety profiles.

Nirsevimab may see competition later this year with Pfizer's maternal vaccine, which would be given to the mother during pregnancy to protect a newborn from LRTD caused by RSV infection.

The CDC's Advisory Committee on Immunization Practices (ACIP) is expected to meet in June to vote on the recommendations regarding nirsevimab and continue the discussions on other RSV vaccinations. If approved and recommended by ACIP with a broad indication, large population-level adoption of nirsevimab is likely.

For reference, the benchmark price of nirsevimab used in a cost-effectiveness model presented at CDC's ACIP meeting in February was $300, with a range from $50 to $600.

Lebrikizumab (Brand Name: To be determined)

Manufacturer: Eli Lilly
Expected FDA decision: September 2023

Therapeutic use

Lebrikizumab is under review for the treatment of adult and adolescent patients with moderate-to-severe atopic dermatitis.

Atopic dermatitis, also referred to as atopic eczema, is a common and chronic inflammatory skin disorder characterized by dry skin, erythema, oozing, crusting, and severe pruritus. Atopic dermatitis is commonly considered a childhood disease because the majority of affected patients develop symptoms in childhood, but it can continue into adulthood or first develop in adulthood.

In the U.S., the prevalence of atopic dermatitis is estimated to be approximately 16% among children and 7.3% among adults.

Clinical profile

Lebrikizumab is a monoclonal antibody that binds with high affinity to interleukin 13 (IL-13), which is one of the pivotal cytokines involved in the pathophysiology of atopic dermatitis.

Pivotal trial data:

The efficacy of lebrikizumab was evaluated in two Phase 3, identically designed, 52-week, randomized, double-blind, placebo-controlled trials (ADvocate1 and ADvocate2) in a total of 851 adolescent and adult patients with moderate-to-severe atopic dermatitis. Both trials included a 16-week induction period and a 36-week maintenance period. During the induction period, participants were randomized in a 2:1 ratio to either lebrikizumab or placebo every 2 weeks (Q2W). The co-primary endpoints were Investigator's Global Assessment (IGA) score of 0 or 1 at Week 16 and 75% reduction from baseline in Eczema Area and Severity Index (EASI-75) at Week 16. Overall, more patients who received lebrikizumab vs. placebo achieved an IGA score of 0 or 1: 43.1% vs. 12.7% in ADvocate1 ($p < 0.001$) and 33.2% vs. 10.8% in ADvocate2 ($p < 0.001$). A higher percentage of patients also achieved EASI-75: 58.8% vs. 16.2% in ADvocate1 ($p < 0.001$) and 52.1% vs. 18.1% in ADvocate2 ($p < 0.001$).

Responders at Week 16 were re-randomized to receive lebrikizumab Q2W, lebrikizumab every 4 weeks (Q4W) or placebo Q2W (lebrikizumab withdrawn) for 36 additional weeks. After 52 weeks, an IGA response was maintained by 71.2% of patients treated with lebrikizumab Q2W, 76.9% of patients treated with lebrikizumab Q4W and 47.9% of patients with placebo. EASI-75 was maintained by 78.4% of patients treated with lebrikizumab Q2W, 81.7% of patients treated with lebrikizumab Q4W and 66.4% of patients with placebo.

What you need to know:

Proposed Indication: Treatment of adult and adolescent patients with moderate-to-severe atopic dermatitis

Mechanism: IL-13 monoclonal antibody

Efficacy:

- IGA response (at Week 16): 33.2% to 43.1% vs. 10.8% to 12.7% with placebo
- EASI-75 response (at Week 16): 52.1% to 58.8% vs. 16.2% to 18.1% with placebo

Common AEs: Conjunctivitis, nasopharyngitis, allergic conjunctivitis

Dosing: SC once every 2 or 4 weeks

Why it Matters: Well tolerated, favorable and durable results

Important to Note: Alternatives available (eg, IL-4/IL-13 inhibitors, JAK inhibitors), lack of head-to-head trial data vs. standards of care, potential future competition (injectable and topical agents)

Estimated Cost: ~$41,000 per year (based on current pricing for Dupixent)

Lebrikizumab *(continued…)*

Safety:

The most common adverse events with lebrikizumab use were conjunctivitis, nasopharyngitis and allergic conjunctivitis.

Dosing:

In the pivotal trials, lebrikizumab was administered via SC injection every 2 or 4 weeks.

Competitive environment

If approved, lebrikizumab would add another IL-13 antagonist to the treatment armamentarium for atopic dermatitis. Current pharmacotherapy for atopic dermatitis includes topical treatments such as corticosteroids, calcineurin inhibitors, Eucrisa® (crisaborole) and Opzelura® (ruxolitinib). In patients with moderate-to-severe disease who require systemic therapy, treatment options include oral Janus kinase (JAK) inhibitors such as Cibinqo® (abrocitinib) and Rinvoq® (upadacitinib), or injectables such as Adbry® (tralokinumab-ldrm), an IL-13 antagonist, and Dupixent® (dupilumab), an IL-4/IL-13 antagonist.

JAK inhibitors are dosed daily and have boxed warnings for serious infections, malignancy and thrombosis, whereas lebrikizumab is administered less frequently (once every 2 or 4 weeks) and has a more favorable safety profile.

Lebrikizumab was not compared in head-to-head trials against existing treatment options, but the efficacy data for lebrikizumab appears to be similar to the current market leader in the severe atopic dermatitis subpopulation, Dupixent. Both lebrikizumab with Dupixent target patients 12 years of age and older, whereas Adbry is only approved for adults.

Lebrikizumab will be a relatively late market entry competing not only with existing treatment options, but potential future pipeline agents including Galderma's nemolizumab, a SC administered IL-31 antagonist, and topical agents such as Vtama® (tapinarof), Zoryve ® (roflumilast), and several topical JAK inhibitors. These competitors could be on the market or receive additional approvals for atopic dermatitis as early as second quarter 2024.

For reference, the WAC for Dupixent is approximately $41,000 per year.

Nedosiran (Brand Name: To be determined)

Manufacturer: Novo Nordisk
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: September 2023

Therapeutic use

Nedosiran is under review for the treatment of patients with type 1 primary hyperoxaluria (PH1).

Primary hyperoxaluria (PH) is a family of severe, ultra-rare, inherited metabolic disorders of the liver characterized by defects in glyoxylate metabolism, causing excess production and accumulation of oxalate. Because oxalate is normally eliminated through the kidneys, excess oxalate can lead to progressive kidney damage, end-stage renal disease or kidney failure. Oxalate can also build up and damage other organs including heart, bones, and eyes.

The true prevalence of PH is unknown, but it is estimated that it affects less than 3 per one million individuals. In the U.S., PH affects approximately 8,500 people and PH1 accounts for 80% of the diagnosed PH cases. It is estimated that about 80% of PH patients remain undiagnosed.

What you need to know:

Proposed Indication: Treatment of patients with PH1

Mechanism: RNAi drug that inhibits production of LDH enzyme

Efficacy: Reduction of Uox AUC: 57.5% greater than placebo

Common AEs: Injection site reactions

Dosing: SC once every month

Why it Matters: Self-administered SC injection, generally well tolerated

Important to Note: Alternative available for PH1 (Oxlumo), lack of head-to-head comparison with Oxlumo, monthly injection, small target population

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

Clinical profile

Nedosiran is a ribonucleic acid interfering (RNAi) drug that inhibits the production of hepatic lactate hydrogenase (LDH) enzyme, which is an enzyme that catalyzes the final step in the oxalate pathway that can lead to oxalate overproduction and accumulation in PH.

Pivotal trial data:

The efficacy of nedosiran was evaluated in PHYOX2, a Phase 2/3, randomized, double-blind, placebo-controlled study in 35 patients 6 years of age and older with PH1 or PH2. The primary endpoint was the area under the curve (AUC) of percentage change from baseline in 24-hour urinary oxalate (Uox) excretion between Day 90 and Day 180. The key secondary endpoint was the percentage of patients achieving normalization (or near-normalization) of Uox levels on two or more consecutive visits from Day 90 to Day 180.

Nedosiran resulted in a 57.5% greater daily average reduction in Uox AUC from Day 90 to Day 180 compared to placebo ($p < 0.0001$). The study also met the key secondary endpoint, with 50% of nedosiran-treated patients achieved normal or near-normal Uox on at least 2 consecutive visits compared to 0% with placebo ($p = 0.002$).

Safety:

The most common adverse events with nedosiran use were injection site reactions.

Dosing:

In the pivotal trial, nedosiran was administered via SC injection once every month.

Nedosiran *(continued...)*

Competitive environment

Nedosiran would be the second drug to market for this ultra-rare disease joining Alnylam's Oxlumo ® (lumasiran), another RNAi drug. Like Oxlumo, nedosiran is a SC injection, but the primary differentiator for nedosiran is that it can be self-administered whereas Oxlumo is administered by a healthcare provider. Nedosiran is dosed more frequently than Oxlumo, which is administered every 3 months for the maintenance doses.

In clinical trials, nedosiran appeared to be well tolerated and when compared indirectly to Oxlumo, appears to be similarly effective in reducing Uox levels. Oxlumo does have the first-to-market advantage for PH1, but because nedosiran acts on the final step of the oxalate metabolic pathway, it could theoretically have potential use for all 3 subtypes of PH. With this potential for a future broader indication covering all types of PH, the target population for nedosiran could increase, but only slightly because PH2 and PH3 account for a much smaller percentage of diagnosed cases of PH.

For reference, the WAC for Oxlumo is approximately $500,000 per year.

Extended generic and biosimilar pipeline 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					
PREZISTA	darunavir	Janssen	Human Immunodeficiency Virus-1 Infection	Oral	2023
ONGLYZA	saxagliptin	Astra Zeneca	Type 2 Diabetes Mellitus	Oral	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
ONEXTON	clindamycin/benzoyl peroxide	Bausch Health	Acne Vulgaris	External	2023
ALPHAGAN P	brimonidine	Allergan	Glaucoma; Ocular Hypertension	Ophthalmic	2023
KOMBIGLYZE XR	saxagliptin/metformin	Astra Zeneca	Type 2 Diabetes Mellitus	Oral	2023
NEUPRO	rotigotine	UCB	Parkinson's Disease; Restless Legs Syndrome	External	2023
MYDAYIS	amphetamine mixture/dextroamphetamine mixture	Takeda	Attention Deficit Hyperactivity Disorder	Oral	2023
THALOMID	thalidomide	Celgene	Multiple Myeloma; Erythema Nodosum Leprosum	Oral	2023
NOXAFIL	posaconazole	Merck	Prophylaxis of Invasive Aspergillus and Candida Infections; Treatment of Oropharyngeal Candidiasis	Intravenous	2Q-2023
ACTEMRA	tocilizumab	Roche/Chugai	Juvenile Idiopathic Arthritis; Rheumatoid Arthritis; Giant Cell Arteritis; Cytokine Release Syndrome; Systemic Sclerosis-Associated Interstitial Lung Disease	Intravenous; subcutaneous	2Q-2023
AGGRASTAT	tirofiban	Medicure	Acute Coronary Syndrome	Intravenous	2Q-2023

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
CLINDESSE	clindamycin phosphate	Perrigo	Bacterial Vaginosis in Non-Pregnant Women	Vaginal	05-2023
TYSABRI	natalizumab	Biogen	Multiple Sclerosis; Crohn's Disease	Intravenous	05-2023
TOLAK	fluorouracil	Pierre Fabre	Actinic Keratosis	External	07-2023
MOZOBIL	plerixafor	Sanofi/Genzyme	Autologous Transplant Patients with Non-Hodgkin's Lymphoma or Multiple Myeloma	Subcutaneous	07-2023
VYVANSE	lisdexamfetamine	Shire/Takeda	Attention Deficit Hyperactivity Disorder; Moderate to Severe Binge Eating Disorder	Oral	08-2023
STELARA	ustekinumab	Janssen	Plaque Psoriasis; Psoriatic Arthritis; Ulcerative Colitis; Crohn's Disease	Subcutaneous; intravenous	09-2023
CAROSPIR	spironolactone	CMP Pharma	Edema in Cirrhotic Patients, Heart Failure and/or Hypertension	Oral	09-2023
LEXETTE	halobetasol	Mayne	Plaque Psoriasis	External	09-2023
PROLENSA	bromfenac	Bausch Health	Postoperative Ocular Inflammation and Ocular Pain Following Cataract Surgery	Ophthalmic	4Q-2023
NEULASTA ONPRO	pegfilgrastim	Amgen/Insulet	Prophylaxis of Neutropenia in Cancer Patients	Subcutaneous	10-2023
LIVALO	pitavastatin	Eli Lilly/Kowa Pharmaceuticals	Hyperlipidemia	Oral	11-2023
2024 Possible launch date					
VESICARE LS	solifenacin	Astellas	Neurogenic Detrusor Overactivity	Oral	1H-2024
SPIRIVA HANDIHALER	tiotropium	Boehringer Ingelheim	Chronic Obstructive Pulmonary Disease	Inhalation	01-2024
BALCOLTRA	levonorgestrel/ethinyl estradiol/ferrous bisglycinate	Avion/Albion	Pregnancy Prevention	Oral	01-2024
GIAZO	balsalazide disodium	Bausch Health	Ulcerative Colitis in Male Patients	Oral	01-2024
MYRBETRIQ	mirabegron	Astellas	Overactive Bladder; Neurogenic Detrusor Overactivity	Oral	01-2024
GRALISE	gabapentin	Assertio Therapeutics	Postherpetic Neuralgia	Oral	01-2024
TASIGNA	nilotinib	Novartis	Philadelphia Chromosome-Positive Chronic Myeloid Leukemia	Oral	01-2024
SIMPONI	golimumab	Janssen	Ankylosing Spondylitis; Psoriatic Arthritis; Rheumatoid Arthritis; Ulcerative Colitis	Subcutaneous	02-2024
SIMPONI ARIA	golimumab	Janssen	Rheumatoid Arthritis; Psoriatic Arthritis; Ankylosing Spondylitis; Juvenile Idiopathic Arthritis	Intravenous	02-2024

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
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	raltegravir	Merck	Human Immunodeficiency Virus-1 Infection	Oral	04-2024
DUTREBIS	lamivudine/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
SAXENDA	liraglutide	Novo Nordisk	Chronic Weight Management	Subcutaneous	05-2024
ARANESP	darbepoetin alfa	Amgen/Kirin	Anemia in Cancer and Kidney Disease	Intravenous; subcutaneous	05-2024
NYMALIZE	nimodipine	Arbor	Subarachnoid Hemorrhage	Oral	06-2024
PROBUPHINE	buprenorphine	Titan Pharmaceuticals/Braeburn Pharmaceuticals	Maintenance Treatment of Opioid Dependence	Subdermal	06-2024
HAEGARDA	C1 esterase inhibitor	CSL Behring	Hereditary Angioedema	Subcutaneous	06-2024
VICTOZA	liraglutide	Novo Nordisk	Type 2 Diabetes Mellitus (T2DM); Reduce the Risks of Cardiovascular Events in T2DM	Subcutaneous	06-2024
VIVITROL	naltrexone	Alkermes	Alcohol and/or Opioid Dependence	Intramuscular	2H-2024
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	2H-2024
TWYNEO	tretinoin/benzoyl peroxide	Galderma	Acne Vulgaris	External	07-2024
SLYND	drospirenone	Exeltis/Insud	Prevention of Pregnancy	Oral	08-2024
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

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
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
2025 Possible launch date					
BOSULIF	bosutinib	Pfizer	Chronic Myelogenous Leukemia	Oral	2025
DALVANCE	dalbavancin	AbbVie	Acute Bacterial Skin and Skin Structure Infections	Intravenous	2025
COMPLERA	emtricitabine/rilpivirine/tenofovir disoproxil fumarate	Gilead/Janssen	Human Immunodeficiency Virus-1 Infection	Oral	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
FLOVENT DISKUS	fluticasone propionate	GSK	Asthma	Inhalation	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 Foam	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
SOLIRIS	eculizumab	Alexion	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
TRADJENTA	linagliptin	Eli Lilly/Boehringer Ingelheim	Type 2 Diabetes Mellitus	Oral	05-2025
JENTADUETO XR	linagliptin/metformin	Boehringer Ingelheim/Eli Lilly	Type 2 Diabetes Mellitus	Oral	05-2025
JENTADUETO	linagliptin/metformin	Boehringer Ingelheim/Eli Lilly	Type 2 Diabetes Mellitus	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
PERJETA	pertuzumab	Genentech	HER-2 Positive Breast Cancer	Intravenous	06-2025
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
THYQUIDITY	levothyroxine	Vistapharm	Hypothyroidism; Pituitary Thyrotropin Suppression	Oral	2H-2025

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
CARDENE	nicardipine	Chiesi	Short-Term Treatment of Hypertension When Oral Therapy is Not Possible	Intravenous	07-2025
RAVICTI	glycerol phenylbutyrate	Horizon	Urea Cycle Disorders	Oral	07-2025
RYANODEX	dantrolene	Eagle Pharmaceuticals	Malignant Hyperthermia	Intravenous	07-2025
SOLIQUA	insulin glargine/lixisenatide	Sanofi	Type 2 Diabetes Mellitus	Subcutaneous	07-2025
ENTRESTO	sacubitril/valsartan	Novartis	Heart Failure	Oral	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
PICATO	ingenol mebutate	LEO Pharma	Actinic Keratosis	External	12-2025
OPSUMIT	macitentan	Janssen	Pulmonary Arterial Hypertension	Oral	12-2025
2026 Possible launch date					
BRYHALI	halobetasol	Bausch Health	Plaque Psoriasis	External	2026
ABILIFY MAINTENA	aripiprazole	Otsuka/Lundbeck	Schizophrenia; Bipolar Disorder	Intramuscular	2026
CIMZIA	certolizumab pegol	UCB/Royalty Pharma	Psoriatic Arthritis; Rheumatoid Arthritis; Ankylosing Spondylitis; Crohn's Disease; Plaque Psoriasis; Axial Spondyloarthritis	Subcutaneous	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
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
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
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
NAYZILAM	midazolam	UCB	Epilepsy	Intranasal	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

Trade Name	Generic Name	Brand Company(ies)	Indications	Route of Administration	Anticipated Availability
TRINTELLIX	vortioxetine	Takeda/Lundbeck	Major Depressive Disorder	Oral	06-2026

Extended brand pipeline forecast

Optum Rx brand pipeline forecast

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
2023 Possible launch date									
Zynquista	sotagliflozin	Lexicon Pharmaceuticals	sodium-dependent glucose transporter 1 (SGLT-1) and SGLT-2 inhibitor	Diabetes mellitus	PO	Filed NDA	05/27/2023	No	No
SRP-9001 (RG-6356)	delandistrogene moxeparvovec	Sarepta	gene therapy	Duchenne muscular dystrophy	IV	Filed BLA	05/29/2023	Yes	Yes
ETX-2514 (SUL-DUR)	durlobactam/ sulbactam	Innoviva	broad-spectrum β-lactamase inhibitor/ beta-lactam antimicrobial	Bacterial infections	IV	Filed NDA	05/29/2023	No	No
Botulax	letibotulinumtoxinA	Hugel Pharma	botulinum toxins	Wrinkles	IM	Filed BLA	05/2023	Yes	No
PF-06928316 (RSVpreF)	respiratory syncytial virus vaccine	Pfizer	vaccine	Respiratory syncytial virus	IM	Filed BLA	05/2023	No	No
UCB-4940 (CDP-4940)	bimekizumab	UCB	interleukin-17 receptor inhibitor	Plaque psoriasis	SC	Filed BLA	05/2023	Yes	No
PF-07321332	nirmatrelvir/ ritonavir	Pfizer	protease inhibitor	COVID-19	PO	Filed NDA	05/2023	No	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
AOP-200704	landiolol	Eagle Pharmaceuticals	cardio-selective beta-1 adrenergic blocker	Dysrhythmia	IV	Filed NDA	06/01/2023	No	No
CyclASol	cyclosporine	Novaliq	immunosuppressant	Dry eye disease	OPH	Filed NDA	06/08/2023	No	No
CYT-387	momelotinib	GlaxoSmithKline	janus kinase inhibitor	Myeloproliferative disorders	PO	Filed NDA	06/16/2023	Yes	Yes
F-901318	olorofim	F2G	orotomide antifungal	Aspergillosis	PO/IV	Filed NDA	06/17/2023	No	Yes
efgartigimod SC	efgartigimod-PH20	argenx/ Halozyme	neonatal Fc receptor antibody	Generalized myasthenia gravis	SC	Filed BLA	06/20/2023	Yes	Yes
ADX-2191	methotrexate	Aldeyra Therapeutics	dihydrofolate reductase inhibitor	Vitreoretinal lymphoma	Intravitreal	Filed NDA	06/21/2023	Yes	Yes
obeticholic acid	obeticholic acid	Intercept Pharmaceuticals	farnesoid X receptor agonist	Nonalcoholic steatohepatitis	PO	Filed NDA	06/22/2023	Yes	No
Exxua	gepirone ER	Fabre-Kramer	serotonin single receptor (1A) agonist	Major depressive disorder	PO	Filed NDA	06/23/2023	No	No
DS-100	dehydrated alcohol	Eton	undisclosed	Methanol poisoning	SC	Filed NDA	06/27/2023	No	Yes
Eylea HD	aflibercept	Regeneron	vascular endothelial growth factor inhibitor	Wet age-related macular degeneration/ diabetic macular edema/ diabetic retinopathy	Intravitreal	Filed BLA	06/27/2023	Yes	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
IPX-203	carbidopa/ levodopa	Amneal	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	PO	Filed NDA	06/30/2023	No	No
Roctavian	valoctocogene roxaparvovec	BioMarin	gene therapy	Hemophilia A	IV	Filed BLA	06/30/2023	Yes	Yes
ritlecitinib	ritlecitinib	Pfizer	janus kinase inhibitor	Alopecia areata	PO	Filed NDA	2Q2023	Yes	No
BGB-A317 (BGB-A-317)	tislelizumab	BeiGene	programmed death-1 inhibitor	Esophageal squamous cell carcinoma	IV	Filed BLA	1H2023	Yes	Yes
JS-001	toripalimab	Coherus Biosciences	anti-PD-1 monoclonal antibody	Nasopharyngeal carcinoma	IV	Filed BLA	Mid-2023	Yes	Yes
ARS-1	epinephrine	ARS Pharmaceuticals	non-selective alpha/ beta-adrenergic receptor agonist	Anaphylaxis	Intranasal	Filed NDA	Mid-2023	No	No
RG-6026	glofitamab	Roche	anti-CD20/CD3 T cell monoclonal antibody	Diffuse large B cell lymphoma	IV	Filed BLA	07/01/2023	Yes	No
MEDI-8897	nirsevimab	AstraZeneca/ Sanofi	anti-RSV monoclonal antibody D25	Respiratory syncytial virus	IM	Filed BLA	07/05/2023	No	No
UCB-7665	rozanolixizumab	UCB	neonatal Fc receptor inhibitor	Generalized myasthenia gravis	SC	Filed BLA	07/06/2023	Yes	Yes
VP-102	cantharidin	Verrica	vesicant (blistering agent)	Molluscum	TOP	Filed NDA	07/23/2023	No	No
Risvan	risperidone	Laboratorios Farmacéuticos Rovi	atypical antipsychotic	Schizophrenia	IM	Filed NDA	07/23/2023	Yes	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
quizartinib	quizartinib	Daiichi Sankyo	FLT-3 receptor tyrosine kinase inhibitor	Acute myeloid leukemia	PO	Filed NDA	07/24/2023	Yes	Yes
I/Ontak	denileukin diftitox	Citius	CD25-directed cytotoxin	Cutaneous T-cell lymphoma	IV	Filed BLA	07/28/2023	Yes	Yes
Prochymal	remestemcel-L	Mesoblast	mesenchymal stem cells	Graft vs. host disease	IV	Filed BLA	08/02/2023	Yes	Yes
PDP-716	brimonidine	Visiox Pharma	alpha-2 agonist	Glaucoma	OPH	Filed NDA	08/04/2023	No	No
SAGE-217	zuranolone	Sage Therapeutics/ Biogen	GABA-A receptor allosteric modulator	Major depressive disorder/ postpartum depression	PO	Filed NDA	08/05/2023	No	No
JNJ-64407564	talquetamab	Johnson & Johnson	GPRC5D/CD3 monoclonal antibody	Multiple myeloma	SC	Filed BLA	08/08/2023	Yes	Yes
GC-4419	avasopasem manganese	Galera Therapeutics	dismutase mimetic	Radiotherapy-induced oral mucositis	IV	Filed BLA	08/09/2023	Yes	No
Melblez Kit	melphalan	Delcath	phenylalanine mustard	Hepatocellular cancer/ melanoma	INJ	Filed NDA	08/14/2023	Yes	Yes
R-667 (RG-667)	palovarotene	Ipsen	selective retinoic acid receptor agonist	Fibrodysplasia ossificans progressiva	PO	Filed NDA	08/16/2023	Yes	Yes
Zimura	avacincaptad pegol	IVERIC bio	C5 complement inhibitor	Geographic atrophy	Intravitreal	Filed BLA	08/19/2023	Yes	No
REGN-3918	pozelimab	Regeneron	C5a receptor inhibitor	CHAPLE disorder	IV/SC	Filed BLA	08/20/2023	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
VLA-1553	VLA-1553	Valneva	vaccine	Chikungunya virus	IM	Filed BLA	08/22/2023	No	No
PF-06863135	elranatamab	Pfizer	BCMA CD3-targeted bispecific antibody	Multiple myeloma	SC	Filed BLA	08/22/2023	Yes	Yes
TP-03	lotilaner	Tarsus Pharmaceuticals	antagonist of insect and arachnid GABA-Cl channels	Demodex blepharitis	TOP	Filed NDA	08/25/2023	No	No
SVT-15473	clobetasol	Salvat Laboratories	corticosteroid	Post-ocular surgery	OPH	Filed NDA	08/25/2023	No	No
PF-3084014 (PF-03084014)	nirogacestat	SpringWorks Therapeutics	gamma secretase inhibitor	Desmoid tumors	PO	Filed NDA	08/27/2023	Yes	Yes
ONS-5010	bevacizumab-vikg	Outlook Therapeutics	anti-VEGF antibody	Wet age-related macular degeneration	Intravitreal	Filed BLA	08/29/2023	Yes	No
NurOwn	autologous cultured mesenchymal bone marrow stromal cells secreting neurotrophic factors	BrainStorm Cell Therapeutics	cellular therapy	Amyotrophic lateral sclerosis	IV	Filed BLA	08/2023	Yes	Yes
BL-8040 (BKT-140)	motixafortide	BioLineRx	selective chemokine receptor 4 inverse agonist	Stem cell transplant	SC	Filed NDA	09/09/2023	Yes	Yes
RA-101495	zilucoplan	UCB	complement inhibitor	Generalized myasthenia gravis	SC	Filed NDA	09/14/2023	Yes	Yes
Tecentriq SC	atezolizumab	Roche	programmed death-ligand 1 blocking antibody	Non-small cell lung cancer	SC	Filed BLA	09/15/2023	Yes	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
ATI-1501	metronidazole	Saptalis	nitroimidazole	Fungal infections, anaerobic bacterial infections	PO	Filed NDA	09/23/2023	No	No
BBI-4000	sofpironium bromide	Brickell	anticholinergic	Hyperhidrosis	TOP	Filed NDA	09/26/2023	No	No
Nyxol	phentolamine	Ocuphire	Alpha-1 and alpha-2 blocker	Mydriasis reversal	OPH	Filed NDA	09/28/2023	No	No
MILR-1444A	lebrikizumab	Eli Lilly	interleukin-13 inhibitor	Atopic dermatitis	SC	Filed BLA	09/2023	Yes	No
DCR-PHXC	nedosiran	Novo Nordisk	glycolate oxidase antagonist	Primary hyperoxaluria	SC	Filed NDA	3Q2023	Yes	Yes
AT-GAA	cipaglucosidase alfa	Amicus	enzyme therapy	Pompe disease	IV	Filed BLA	3Q2023	Yes	Yes
Xphozah	tenapanor	Ardelyx	sodium/hydrogen exchanger 3 inhibitor	Hyperphosphatemia	PO	Filed NDA	10/17/2023	No	No
IDP-126	IDP-126	Bausch Health	retinoid/ antibiotic	Acne	TOP	Filed NDA	10/20/2023	No	No
CT-P13	infliximab	Celltrion	Tumor necrosis factor blocker	Inflammatory bowel disease	SC	Filed BLA	10/22/2023	No	No
CSF-1	pilocarpine	Orasis Pharmaceuticals	cholinergic muscarinic receptor agonist	Presbyopia	OPH	Filed NDA	10/22/2023	No	No
VBP-15	vamorolone	Santhera Pharmaceuticals	corticosteroid	Duchenne muscular dystrophy	PO	Filed NDA	10/26/2023	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
Entyvio (SC formulation)	vedolizumab	Takeda	integrin receptor antagonist	Ulcerative colitis	SC	Filed BLA	10/28/2023	Yes	No
PF-06886992	meningococcal vaccine [A, B, C, Y, W-135]	Pfizer	vaccine	Meningococcal disease	IM	Filed BLA	10/28/2023	No	No
TAK-755 (SHP-655)	TAK-755	Takeda	ADAMTS13 enzyme	Thrombotic thrombocytopenic purpura	IV	Filed BLA	11/16/2023	Yes	Yes
Neutrolin (CRMD-003, CRMD-004)	citrate/ taurolidine/ heparin	CorMedix	antimicrobial agent/ anticoagulant	Catheter-related infections	IV	Filed NDA	11/16/2023	No	No
NS-2 (ALDX-1E1, ADX-102)	reproxalap	Aldeyra Therapeutics	aldehyde antagonist	Dry eye disease	OPH	Filed NDA	11/23/2023	No	No
LN-144	lifileucel	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Melanoma	IV	Filed BLA	11/24/2023	Yes	Yes
fruquintinib	fruquintinib	Hutchison China MediTech	VEGF-R inhibitor	Colorectal cancer	PO	Filed NDA	11/30/2023	Yes	No
CTX-001 (Exa-cel)	exagamglogene autotemcel	CRISPR Therapeutics/ Vertex	gene editing (CRISPR-Cas9)	Beta-thalassemia; sickle cell anemia	IV	Filed BLA	12/03/2023	Yes	Yes
OX-124	naloxone	Orexo	opioid antagonist	Opioid overdose	Intranasal	Filed NDA	12/03/2023	No	No
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

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
APD-334	etrasimod	Pfizer/ Everest	S1P1 receptor agonist	Ulcerative colitis	PO	Filed NDA	12/21/2023	Yes	No
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
LentiGlobin	lovotibeglogene autotemcel	bluebird bio	gene therapy	Sickle cell disease	IV	Filed BLA	12/24/2023	Yes	Yes
TAK-438	vonoprazan fumarate	Phantom Pharmaceuticals	potassium-competitive acid blocker	Erosive esophagitis	PO	CRL	4Q2023	No	No
niraparib/ abiraterone	niraparib/ abiraterone	Janssen	poly (ADP-ribose) polymerase inhibitor/ CYP17 inhibitor	Prostate cancer	PO	Filed NDA	12/29/2023	Yes	No
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension	INH	Tentative Approval	2023	Yes	No
NVX-CoV2373	coronavirus vaccine	Novavax	vaccine	Novel coronavirus disease 2019 (COVID-19)	IM	InTrial	Late 2023	No	No
Hepcludex	bulevirtide	Gilead	HBV receptor binder	Hepatitis delta virus	SC	CRL	Late 2023	Yes	Yes
LY-3074828	mirikizumab	Eli Lilly	interleukin-23 antagonist	Ulcerative colitis	IV/SC	CRL	Late 2023	Yes	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
Tirzepatide (for weight loss)	tirzepatide	Eli Lilly	glucose-dependent insulinotropic polypeptide receptor and glucagon-like peptide-1 receptor agonist	Chronic weight management	SC	InTrial	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
YN-96D1	rivoceranib (apatinib)	Elevar Therapeutics	vascular endothelial growth factor receptor antagonist	Hepatocellular carcinoma	PO	Filed NDA	01/17/2024	Yes	Yes
SHR-1210	camrelizumab	Elevar Therapeutics	programmed death receptor-1-blocking antibody	Hepatocellular carcinoma	IV	Filed BLA	01/17/2024	Yes	Yes
STS-101	dihydroergotamine	Satsuma Pharmaceuticals	ergotamine	Migraine	Intranasal	Filed NDA	01/2024	No	No
MIN-101	roluperidone	Minerva Neurosciences	sigma-2 and 5HT-2A receptor antagonist	Schizophrenia	PO	Filed NDA	02/26/2024	Yes	No
APP-13007	clobetasol propionate	Formosa Pharmaceuticals	corticosteroid	Eye inflammation/ pain	OPH	Filed NDA	03/04/2024	No	No
NVK-002	atropine	Vyluma	anticholinergic	Myopia	OPH	InTrial	1Q2024	No	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
OTL-200	atidarsagene autotemcel	Orchard Therapeutics	gene therapy	Leukodystrophy	IV	InTrial	1Q2024	Yes	Yes
ADP-A2M4 (MAGE-A4)	afamitresgene autoleucel	Adaptimmune	SPEAR T-cell therapy	Sarcoma	IV	InTrial	1Q2024	Yes	Yes
K-127	pyridostigmine	Amneal	cholinesterase inhibitor	Myasthenia gravis	PO	InTrial	1Q2024	No	No
EB-101	EB-101	Abeona Therapeutics	gene therapy	Epidermolysis Bullosa	TOP	InTrial	1Q2024	Yes	Yes
RP-L201	RP-L201	Rocket Pharmaceuticals	gene therapy	Leukocyte adhesion deficiency-I	IV	InTrial	1Q2024	Yes	Yes
LTX-03	hydrocodone bitartrate/ acetaminophen	Acura Pharmaceuticals	opioid analgesic	Pain	PO	Filed NDA	1Q2024	No	No
GSK-2140944	gepotidacin	GlaxoSmithKline	bacterial Type II topoisomerase inhibitor	Bacterial infections	PO/IV	InTrial	2Q2024	No	No
glatiramer acetate depot	glatiramer acetate long-acting	Viatris	immunomodulator	Multiple sclerosis	IM	InTrial	2Q2024	Yes	No
RPL-554	ensifentrine	Verona Pharma	phosphodiesterase-3 and phosphodiesterase-4 inhibitor	Chronic obstructive pulmonary disease	INH	InTrial	2Q2024	Yes	No
ALPHA-1062	galantamine prodrug	Alpha Cognition	acetylcholinesterase inhibitor	Alzheimer's disease	PO	InTrial	2Q2024	No	No
LAI-287	insulin icodec	Novo Nordisk	ultra-long-acting basal insulin	Diabetes mellitus	SC	InTrial	1H2024	No	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
MK-7264	gefapixant	Merck	P2X3 antagonist	Chronic cough	PO	CRL	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
LY-686017	tradipitant	Vanda Pharmaceuticals	neurokinin 1 receptor antagonist	Gastroparesis	PO	InTrial	1H2024	No	No
VNRX-5133	cefepime/ taniborbactam	VenatoRx Pharmaceuticals	cephalosporin/ beta-lactamase inhibitor	Bacterial infections	IV	InTrial	1H2024	Yes	No
PTC-AADC	eladocagene exuparvovec	PTC Therapeutics	gene therapy	Aromatic L-amino acid decarboxylase deficiency	Intracerebral	InTrial	1H2024	Yes	Yes
arimoclomol	arimoclomol	Orphazyme	cytoprotectives	Niemann-Pick disease	PO	CRL	1H2024	Yes	Yes
PF-06838435 (SPK-9001)	fidanacogene elaparvovec	Pfizer/ Spark Therapeutics	gene therapy	Hemophilia B	IV	InTrial	1H2024	Yes	Yes
LNP-023	iptacopan	Novartis	factor B inhibitor	Paroxysmal nocturnal hemoglobinuria	PO	InTrial	1H2024	Yes	Yes
MGL-3196	resmetirom	Madrigal Pharmaceuticals	beta-selective thyroid hormone receptor agonist	Nonalcoholic steatohepatitis	PO	InTrial	1H2024	Yes	No
GRN-163L	imetelstat	Geron	telomerase inhibitor	Myelofibrosis/ myelodysplastic	IV	InTrial	1H2024	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
				syndrome/ acute myelogenous leukemia					
LY-3002813	donanemab	Eli Lilly	beta-amyloid monoclonal antibody	Alzheimer's disease	IV	CRL	1H2024	Yes	No
DAY-101	DAY-101	Day One Biopharmaceuticals	pan-Raf kinase inhibitor	Brain cancer	PO	InTrial	1H2024	Yes	Yes
ACE-011	sotatercept	Merck	activin receptor type IIA-Fc fusion protein	Pulmonary arterial hypertension	SC	InTrial	1H2024	Yes	Yes
D-PLEX100	doxycycline	PolyPid	tetracycline	Surgical site infections	IMPLANT	InTrial	1H2024	No	No
mRNA-1345	mRNA-1345	Moderna	vaccine	Respiratory syncytial virus	IM	InTrial	1H2024	No	No
PAX-101	suramin	PaxMedica	unknown	trypanosomiasis	IV	InTrial	Mid-2024	No	No
RTT-01	tiratricol	Egetis Therapeutics	thyroid-stimulating hormone receptor	Monocarboxylate transporter 8 deficiency	PO	InTrial	Mid-2024	Yes	Yes
PB-2452	bentracimab	SFJ Pharmaceuticals	antiplatelet monoclonal antibody	Antiplatelet drug toxicity	IV	InTrial	Mid-2024	No	No
iMAB-362	zolbetuximab	Astellas	GC182 monoclonal antibody	Gastric adenocarcinoma	IV	InTrial	Mid-2024	Yes	Yes
Zeftera	ceftobiprole	Basilea	cephalosporin antibiotic	Bacterial infections	IV	InTrial	Mid-2024	No	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
X4P-001 (X-4P-001, X4-136, X4P-001-RD)	mavorixafor	X4 Pharma	CXC receptor type 4 inhibitor	WHIM syndrome	PO	InTrial	Mid-2024	Yes	Yes
RP-L102 (RPL-102)	RP-L102	Rocket Pharmaceuticals	gene therapy	Fanconi anemia	IV	InTrial	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
Cx-601	darvadstrocel	Takeda	allogeneic stem cell therapy	Crohn's disease	IV	InTrial	Mid-2024	Yes	Yes
MSP-2017	etripamil	Milestone	calcium channel blocker	Arrhythmia	Intranasal	InTrial	Mid-2024	TBD	No
RG-6058	tiragolumab	Roche	TIGIT monoclonal antibody	Non-small cell lung cancer/ esophageal cancer	IV	InTrial	Mid-2024	Yes	No
SNDX-5613	SNDX-5613	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
Obe-cel	obecabtagene autoleucel	Autolus Therapeutics	autologous chimeric antigen receptor T-cells	Acute lymphoblastic leukemia	IV	InTrial	Mid-2024	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
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
MDMA	midomafetamine	MAPS Public Benefit Corporation	psychoactive drug	Post-traumatic stress disorder	PO	InTrial	3Q2024	Yes	No
KarXT	xanomeline/ trospium	Karuna Therapeutics	muscarinic acetylcholine receptor agonist/ muscarinic receptor antagonist	Schizophrenia	PO	InTrial	3Q2024	No	No
TAVT-45	abiraterone acetate	Tavanta Therapeutics	CYP17 inhibitor	Prostate cancer	PO	InTrial	3Q2024	Yes	No
nemolizumab	nemolizumab	Galderma	interleukin-31 receptor antagonist	Atopic dermatitis	SC	InTrial	2H2024	Yes	No
FCX-007 (GM-HDF-COL7, INXN-3002)	dabocemagene autoficel	Castle Creek Pharmaceutical	gene-modified autologous fibroblast	Epidermolysis bullosa	Intradermal	InTrial	2H2024	Yes	Yes
AT-007	govorestat	Applied Therapeutics	aldose reductase inhibitor	Galactosemia	PO	InTrial	2H2024	Yes	Yes
REGN-1979	odronextamab	Regeneron	CD20/CD3 monoclonal antibody	Follicular lymphoma/ diffuse large b-cell lymphoma	IV	InTrial	2H2024	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
AVB-S6-500	batiraxcept	Aravive Biologics	GAS6/AXL inhibitor	Ovarian cancer	IV	InTrial	2H2024	Yes	No
EBV-CTL (ATA-129)	tabelecleucel	Atara Biotherapeutics	cell therapy	Lymphoproliferative disorder	IV	InTrial	2H2024	Yes	Yes
REGN-5458	REGN-5458	Regeneron	BCMA and CD3 bispecific antibody inhibitor	Multiple myeloma	IV	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	InTrial	2H2024	Yes	Yes
CTP-543	deuruxolitinib	Sun Pharma	janus kinase inhibitor	Alopecia areata	PO	InTrial	2H2024	Yes	No
CSL-312	garadacimab	CSL Limited	anti-factor XIIa monoclonal antibody	Hereditary angioedema	SC	InTrial	2H2024	Yes	Yes
ALXN-2040	danicopan	AstraZeneca	complement factor D inhibitor	Paroxysmal nocturnal hemoglobinuria	PO	InTrial	2H2024	Yes	Yes
IONIS-APOCIII-LRx (ISIS-678354)	olezarsen	Ionis	antisense drug	Familial chylomicronemia syndrome	SC	InTrial	2H2024	Yes	No
MVA-BN RSV	MVA-BN RSV	Bavarian Nordic	vaccine	Respiratory syncytial virus	IM	InTrial	2H2024	No	No
XMT-1536	upifitamab rilsodotin	Mersana Therapeutics	antibody-drug conjugate	Ovarian cancer	IV	InTrial	2H2024	Yes	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
CT-053 (Zevor-cel)	CT-053	CARsgen Therapeutics	B-cell maturation antigen-directed genetically modified autologous T cell immunotherapy	Multiple myeloma	IV	InTrial	2H2024	Yes	Yes
BBP-305	encaleret	BridgeBio	Ca sensing receptor antagonist	Autosomal dominant hypocalcemia type 1	PO	InTrial	2H2024	Yes	Yes
PTC-923	sepiapterin	PTC Therapeutics	phenylalanine hydroxylase activator	Phenylketonuria	PO	InTrial	2H2024	Yes	Yes
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
TPX-0005	repotrectinib	Bristol Myers Squibb	tyrosine kinase inhibitor	Non-small cell lung cancer	PO	InTrial	2024	Yes	Yes
pIL-12 (DNA IL-12)	tavokinogene telsaplasmid	OncoSec Medical	gene therapy	Melanoma	Intratumoral	InTrial	2024	Yes	Yes
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	CRL	2024	Yes	Yes
SDN-037	difluprednate	Visiox	corticosteroid	Ocular inflammation/pain	OPH	InTrial	2024	No	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
SYD-985	[vic-] trastuzumab duocarmazine	Byondis	HER2-targeting antibody-drug conjugate	Breast cancer	IV	CRL	2024	Yes	No
AAI-101	cefepime/enmetazobactam	Advanz/ Allecra	beta-lactam/b-lactamase inhibitor	Urinary tract infection	IV	InTrial	2024	No	No
TransCon PTH	palopegteriparatide	Ascendis Pharma	parathyroid hormone	Hypoparathyroidism	SC	CRL	2024	Yes	Yes
NRX-101 (Cyclurad)	d-cycloserine/ lurasidone	NeuroRx	N-methyl-D-aspartate receptor modulator/ 5-HT2A receptor antagonist	Bipolar disorder	PO	InTrial	2024	No	No
OMS-721	narsoplimab	Omeros	anti-MASP-2 monoclonal antibody	Hematopoietic stem cell transplant-associated thrombotic microangiopathy	IV	CRL	2024	Yes	Yes
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
GC-5107	human immunoglobulin	GC Biopharma	human immunoglobulin	Primary immunodeficiencies	IV	CRL	2024	Yes	No
SAR-408701	SAR-408701	Sanofi	antibody-drug conjugate	Non-small cell lung cancer	IV	InTrial	2024	Yes	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
CF-101	piclidenoson	Can-Fite BioPharma	A3 adenosine receptor agonist	Plaque psoriasis	PO	InTrial	2024	Yes	No
AXS-14	S-reboxetine	Axsome Therapeutics	selective noradrenaline reuptake inhibitor	Fibromyalgia	PO	InTrial	2024	No	No
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
SPN-830	apomorphine	Supernus Pharmaceuticals	non-ergoline dopamine agonist	Parkinson's disease	SC infusion	CRL	2024	Yes	No
ALT-803	nogapendekin alfa inbakicept	ImmunityBio	interleukin-15 (IL-15) super agonist/ IL-15R alpha-Fc fusion complex	Bladder cancer	Intravesical	CRL	2024	Yes	No
RG-6107	crovalimab	Roche	C5 inhibitor	Paroxysmal nocturnal hemoglobinuria	IV/SC	InTrial	2024	Yes	Yes
CUTX-101	copper histidinate	Fortress Biotech	copper replacement	Menkes Disease	SC	InTrial	2024	Yes	Yes
ND-0612L	levodopa/ carbidopa	NeuroDerm	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	SC	InTrial	2024	Yes	No
RG-7433 (ABT-263)	navitoclax	AbbVie	Bcl-2 inhibitor	Myelofibrosis	PO	InTrial	2024	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
Iomab-B	iodine I 131 monoclonal antibody BC8	Actinium	anti-CD45 monoclonal antibody	Acute myeloid leukemia	IV	InTrial	2024	Yes	Yes
Sativex	nabiximols	GW Pharmaceuticals/ Otsuka	cannabinoid product	Spasticity	PO	InTrial	2024	No	No
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
HP-5000	diclofenac	Hisamitsu Pharmaceutical	non-steroidal anti-inflammatory drug	Osteoarthritis	Transdermal	InTrial	2024	No	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
SEP-363856 (SEP-856)	ulotaront	Sumitomo Dainippon Pharma	trace amine-associated receptor 1 agonist	Schizophrenia	PO	InTrial	Late 2024	No	No
APN-311	dinutuximab beta	Recordati	anti-GD2 antigen	Neuroblastoma	IV	InTrial	Late 2024	Yes	Yes
CORT-125134	relacorilant	Corcept Therapeutics	glucocorticoid receptor II antagonist	Cushing's syndrome	PO	InTrial	Late 2024	Yes	Yes
PRN-1008	rilzabrutinib	Sanofi	BTK inhibitor	Immune thrombocytopenia	PO	InTrial	Late 2024	No	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
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
RG-1594	ocrelizumab	Genentech	CD20-directed cytolytic antibody	Multiple sclerosis	SC	InTrial	Late 2024	Yes	No
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

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	Disease State	New Proposed Indication Use	Route	Estimated Approval Date
Prevymis	letermovir	Merck	CMV DNA terminase complex inhibitor	Cytomegalovirus	Prevention of cytomegalovirus disease in adults who receive kidney transplants	PO/IV	06/05/2023
Linzess	linaclotide	AbbVie	guanylate cyclase-C agonist	Functional constipation	Treatment of children and adolescents 6 to 17 years of age with functional constipation	PO	06/14/2023
Bylvay	odevixibat	Albireo	ileal bile acid transporter inhibitor	Alagille syndrome	Treatment of Alagille syndrome	PO	06/15/2023
Camzyos	mavacamten	Bristol Myers Squibb	cardiac myosin inhibitor	Reduce septal reduction therapy	To reduce the need for septal reduction therapy in adults with symptomatic New York Heart Association (NYHA) class II-III obstructive hypertrophic cardiomyopathy	PO	06/16/2023
Adbry	tralokinumab-ldrm	Leo Pharma	interleukin-13 antagonist	Atopic dermatitis (adolescents)	Treatment of moderate-to-severe atopic dermatitis in adolescents patients whose disease is not adequately controlled with topical prescription therapies or when those therapies are not advisable	SC	2Q2023
Lynparza	olaparib	AstraZeneca/ Merck	poly (ADP-ribose) polymerase inhibitor	Prostate cancer	In combination with abiraterone and prednisone or prednisolone, for treatment of adult patients with metastatic castration-resistant prostate cancer	PO	2Q2023

Brand name	Generic name	Company	Mechanism of Action	Disease State	New Proposed Indication Use	Route	Estimated Approval Date
Injectafer	ferric carboxymaltose	Daiichi Sankyo	iron replacement product	Chronic heart failure - anemia	Treatment of heart failure and iron deficiency, either with or without anemia	IV	2Q2023
Ultomiris	ravulizumab-cwvz	AstraZeneca	C5 complement inhibitor	Neuromyelitis optica	Treatment of neuromyelitis optica	IV	1H2023
Talzenna	talazoparib	Pfizer	poly (ADP-ribose) polymerase inhibitor	Prostate cancer	In combination with Xtandi (enzalutamide), for the treatment of metastatic castration-resistant prostate cancer	PO	Mid-2023
Leqembi	lecanemab-irmb	Eisai/ Biogen	amyloid beta-directed antibody	Alzheimer's disease (full approval)	Treatment of Alzheimer's disease (accelerated approval to full approval)	IV	07/06/2023
Rubraca	rucaparib	Clovis Oncology	poly (ADP-ribose) polymerase inhibitor	Ovarian cancer	First-line maintenance treatment for women with advanced ovarian cancer regardless of biomarker status who have responded to first-line platinum-based chemotherapy	PO	07/13/2023
Lonsurf	trifluridine/ tipiracil	Taiho Pharmaceutical	mnucleoside metabolic inhibitor/ thymidine phosphorylase inhibitor	Colorectal cancer	Monotherapy or in combination with bevacizumab for the treatment of adult patients with metastatic colorectal cancer who have been previously treated with fluoropyrimidine-, oxaliplatin-, and irinotecan-based chemotherapy, an anti-VEGF biological therapy, and if RAS wild-type, an anti-EGFR therapy	PO	08/13/2023
Livmarli	maralixibat	Mirum Pharmaceuticals	ileal bile acid transporter inhibitor	Progressive familial intrahepatic cholestasis	Treatment of pruritus in patients with progressive familial intrahepatic cholestasis	PO	08/14/2023

Brand name	Generic name	Company	Mechanism of Action	Disease State	New Proposed Indication Use	Route	Estimated Approval Date
Daxxify	daxibotulinumtoxinA-lanm	Revance Therapeutics	acetylcholine release inhibitor/ neuromuscular blocking agent	Cervical dystonia	Treatment of cervical dystonia	IM	08/19/2023
Ingrezza	valbenazine	Neurocrine Biosciences	vesicular monoamine transporter 2 inhibitor	Huntington's disease	Treatment of Huntington's disease chorea	PO	08/20/2023
Wilate	von Willebrand factor/coagulation factor VIII complex	Octapharma	von Willebrand Factor	von Willebrand disease (prophylaxis)	Routine prophylaxis to reduce the frequency of bleeding episodes in children and adults with any type of von Willebrand disease	IV	08/23/2023
Reblozyl	luspatercept-aamt	Bristol Myers Squibb	erythroid maturation agent	Myelodysplastic syndrome	Treatment of anemia without previous use of erythropoiesis-stimulating agents in adult patients with very low- to intermediate-risk myelodysplastic syndromes who may require red blood cell transfusions	SC	08/28/2023
Cosentyx	secukinumab	Novartis	interleukin-17 receptor antagonist	Hidradenitis suppurativa	Treatment of hidradenitis suppurativa	SC	08/31/2023
Jardiance	empagliflozin	Boehringer Ingelheim/ Eli Lilly	sodium-dependent glucose transporter 2 inhibitor	Chronic kidney disease	To reduce kidney disease progression and cardiovascular mortality risk in patients with chronic kidney disease	PO	09/20/2023
Onpattro	patisiran	Alnylam	RNAi therapeutic	Transthyretin amyloidosis with cardiomyopathy	Treatment of transthyretin amyloidosis patients with cardiomyopathy	IV	10/08/2023
Opdivo	nivolumab	Bristol Myers Squibb	programmed death receptor-1-blocking antibody	Melanoma	Monotherapy in the adjuvant setting for the treatment of patients with completely resected stage IIB or IIC melanoma	IV	10/13/2023

Brand name	Generic name	Company	Mechanism of Action	Disease State	New Proposed Indication Use	Route	Estimated Approval Date
Keytruda	pembrolizumab	Merck	programmed death receptor-1-blocking antibody	Non-small cell lung cancer	Treatment of patients with resectable stage II, IIIA, or IIIB non-small cell lung cancer in combination with platinum containing chemotherapy as neoadjuvant treatment, and then continued as a single agent as adjuvant treatment	IV	10/16/2023
Zoryve	roflumilast	Arcutis Biotherapeutics	phosphodiesterase-4 inhibitor	Plaque psoriasis (children ages 2 to 11)	Treatment of plaque psoriasis in children ages 2 to 11	TOP	10/19/2023
Voxzogo	vosoritide	BioMarin	C type natriuretic peptide analog	Achondroplasia (< 5 years)	To increase linear growth in pediatric patients with achondroplasia who are under 5 years of age	SC	10/21/2023
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 inhibitor	Chronic spontaneous urticaria	Treat adults and adolescents aged 12 years and older with chronic spontaneous urticaria that is not adequately controlled with the current standard of care, H1 antihistamine treatment	SC	10/22/2023
Jardiance	empagliflozin	Boehringer Ingelheim/ Eli Lilly	sodium-glucose co-transporter 2 inhibitor	Type 2 diabetes (pediatric)	As an adjunct to diet and exercise to improve glycemic control in children 10 years and older with type 2 diabetes	PO	11/08/2023
Exparel	bupivacaine (liposomal suspension)	Pacira	local anesthetic	Analgesia	For sciatic nerve block in the popliteal fossa as well as femoral nerve block in the adductor canal	INJ	11/13/2023
Xhance	fluticasone	Optinose	corticosteroid	Chronic sinusitis	Treatment of chronic sinusitis	Intranasal	12/16/2023
Abecma	idecabtagene vicleucel	Bristol Myers Squibb	B-cell maturation antigen-directed genetically	Multiple myeloma	Treatment of adult patients with relapsed and refractory multiple myeloma who have received an immunomodulatory agent, a	IV	12/16/2023

Brand name	Generic name	Company	Mechanism of Action	Disease State	New Proposed Indication Use	Route	Estimated Approval Date
			modified autologous T cell immunotherapy		proteasome inhibitor, and an anti-CD38 monoclonal antibody		
Keytruda	pembrolizumab	Merck	programmed death receptor-1-blocking antibody	Gastric cancer	In combination with fluoropyrimidine- and platinum-containing chemotherapy, for the first-line treatment of patients with locally advanced unresectable or metastatic gastric or gastroesophageal junction adenocarcinoma	IV	12/16/2023
Braftovi	encorafenib	Pfizer	kinase inhibitor	Non-small cell lung cancer	In combination with Mektovi (binimetinib), for patients with metastatic non-small cell lung cancer with a BRAF V600E mutation, as detected by an FDA-approved test	PO	4Q2023
Mektovi	binimetinib	Pfizer	kinase inhibitor	Non-small cell lung cancer	In combination with Braftovi (encorafenib), for patients with metastatic non-small cell lung cancer with a BRAF V600E mutation, as detected by an FDA-approved test	PO	4Q2023
Vabysmo	faricimab	Roche/ Genentech	vascular endothelial growth factor and angiopoietin-2 inhibitor	Retinal vein occlusion	Treatment of macular edema following retinal vein occlusion	Intravitreal	01/09/2024

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