OPTUMRx®

RxOutlook®

1st Quarter 2022

In 2021, coronavirus disease 19 (COVID-19) infection and related vaccines and treatments continued to be one of the biggest stories of the year, but it also saw 50 new molecular entity (NME) or novel therapy FDA approvals. Of the 50 NME approvals, 54% were considered first-in-class drugs and 74% used one or more FDA expedited programs or designations (ie, Fast Track, Breakthrough Therapy, Priority Review, and/or Accelerated Approval). Interestingly, for the third year out of the last four, the number of NMEs approved with Orphan Drug status exceeded non-Orphan Drugs (52% were Orphan Drugs). In 2022, we are expecting these macro trends to continue with a high number of drugs approved via expedited FDA programs and/or with Orphan Drug status.

In this edition of RxOutlook, we highlight 7 key pipeline drugs with an expected approval decision by the end of the second quarter 2022. Illustrating the diversity of the drug pipeline, these drugs represent a wide range of therapeutic categories with some being first-in-class options for their indications. Tirzepatide is a novel glucose-dependent insulinotropic polypeptide (GIP) and glucagon-like peptide-1 (GLP-1) receptor agonist that will be competing primarily with other GLP-1 receptor agonists for management of type 2 diabetes. While entering a very competitive marketplace, tirzepatide has demonstrated strong glucose and weight lowering effects. Tapinarof is a novel non-steroidal topical for plaque psoriasis and would offer an alternative or add-on treatment to existing topicals (corticosteroids and vitamin D analogs) used for the condition. The drug pipeline for plaque psoriasis is worth watching as tapinarof is one of four novel therapies with potential approval by the end of this year.

Two new treatments for commonly occurring infections could be approved in the second quarter. Vonoprazan, a first-in-class potassium-competitive acid blocker, used in combination with antibiotics (amoxicillin ± clarithromycin), would offer a novel treatment regimen for *Helicobacter pylori (H. pylori)* infection. Another drug, tebipenem, could be the first oral carbapenem antibiotic approved for the treatment of complicated urinary tract infection (UTI). Carbapenems are highly effective, broad spectrum antibiotics that are currently only available via injection. If approved, tebipenem could reduce the need for hospitalization or reduce the length of stay for certain infections because of its unique oral administration.

The report includes two non-oncology Orphan Drugs – vutrisiran for polyneuropathy of hereditary transthyretin-mediated amyloidosis and AMX-0035 for amyotrophic lateral sclerosis (ALS). Vutrisiran is Alnylam's next generation small interfering RNA therapeutic with similarities to Onpattro® (patisiran). The main differentiator is more convenient dosing (subcutaneous every 3 months vs. intravenous infusion every 3 weeks, respectively). AMX-0035 is a novel combination of sodium phenylbutyrate and taurursodiol and would provide a new option for ALS, a disease with a very high unmet need. The remaining drug in the report is surufatinib, a new targeted therapy for neuroendocrine tumors.

Approval decisions for other key novel therapies are expected in the first half of 2022 but are not reviewed in this report because they were covered in previous editions of RxOutlook. These include: ciltacabtagene autoleucel for multiple myeloma; lenacapavir for HIV-1 infection; vadadustat for chronic kidney disease-anemia; bimekizumab for plaque psoriasis; and mavacamten for obstructive hypertrophic cardiomyopathy. Several of these drugs have experienced regulatory delays in their approval decisions.

Finally, while not discussed in detail in this report, we expect the FDA to announce additional authorizations or full approvals for COVID-19 vaccines and treatments. We recognize the importance of anti-COVID-19 drugs but due to the rapidly changing nature of the pandemic, these drugs are not reviewed in this report.

Key pipeline drugs with FDA approval decisions expected by end of the 2nd quarter 2022

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Vutrisiran	Alnylam	Polyneuropathy of hereditary transthyretin-mediated amyloidosis*	4/14/2022
Surufatinib	Hutchmed	Neuroendocrine tumors*	4/30/2022
Vonoprazan	Phathom Pharmaceuticals	*Helicobacter pylori* infection	5/3/2022
Tapinarof	Dermavant Sciences	Plaque psoriasis	5/26/2022
Tirzepatide	Eli Lilly	Diabetes mellitus type 2	5/2022
Tebipenem	Spero Therapeutics	Complicated urinary tract infection	6/27/2022
Sodium phenylbutyrate/ taurursodiol	Amylyx Pharmaceuticals	Amyotrophic lateral sclerosis*	6/29/2022

* Orphan Drug Designation

OptumRx closely monitors and evaluates the drug development pipeline to identify noteworthy upcoming drug approvals and reports the essential findings here in RxOutlook. 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 2nd quarter 2022.

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.

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

Past and future reviews

Please note that RxOutlook highlights select near-term approvals. Some drugs may not appear in this issue because they have been reviewed in previous editions of RxOutlook. Drugs of interest that are earlier in development or with expected approvals beyond 2nd quarter 2022 may appear in future reports; however, for those who need an initial look at the full pipeline, please refer to the Brand Pipeline Forecast Table found later in this report.

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 insights on key drugs

Vutrisiran (Brand Name: To be determined)

Manufacturer: Alnylam
Regulatory designations: Orphan Drug, Fast Track
Expected FDA decision: April 14, 2022

Therapeutic use

- Treatment of polyneuropathy of hATTR amyloidosis in adults

Vutrisiran is in development for treatment of polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults.

hATTR amyloidosis is a rare inherited condition caused by mutations in the transthyretin (TTR) gene. TTR protein is primarily produced in the liver and is normally a carrier of vitamin A. Mutations in the TTR gene cause abnormal amyloid proteins to accumulate and damage body organs and tissue, such as the peripheral nerves and heart, resulting in intractable peripheral sensory-motor neuropathy, autonomic neuropathy, and/or cardiomyopathy, as well as other disease manifestations.

hATTR amyloidosis is a rare condition, affecting approximately 50,000 people worldwide.

Clinical profile

- siRNA targeting the TTR gene
- SC formulation
- mNIS+7 change: 2.24-point mean decrease (improvement) vs. 14.76-point mean increase (worsening) with placebo
- Common AEs: Diarrhea, pain in extremity, fall, UTI (each of these events occurring at a similar or lower rate as compared with external placebo arm)
- Dosing: Once every 3 months

Vutrisiran is a small interfering RNA (siRNA) therapeutic that blocks the production of abnormal TTR protein before it is made. Administration of vutrisiran may help to reduce deposition and facilitate the clearance of TTR amyloid deposits in tissues.

Pivotal trial data:
The efficacy of vutrisiran was evaluated in HELIOS-A, a Phase 3, randomized, open-label study in 164 patients with hATTR amyloidosis with polyneuropathy. Patients were randomized to receive either vutrisiran via subcutaneous (SC) injection or Onpattro® (patisiran) via intravenous (IV) infusion for 18 months. Onpattro is an existing siRNA therapeutic that targets TTR. The primary endpoint was the change from baseline in modified Neuropathy Impairment Score (mNIS+7) score at 9 months, relative to external placebo data from the APOLLO Phase 3 study of Onpattro. Higher scores of mNIS+7 indicate more neurologic impairment (range: 0 to 304).

Vutrisiran treatment resulted in a 2.24-point mean decrease (improvement) in mNIS+7 score from baseline at 9 months vs. 14.76-point mean increase (worsening) reported for the external placebo group, resulting in a 17.0-point mean difference between vutrisiran relative to placebo ($p = 3.54 \times 10^{-12}$). For reference, Onpattro resulted in a 1.41-point decrease in mNIS+7 score from baseline at 9 months. Vutrisiran achieved a rapid and sustained reduction in serum TTR levels with an 83% mean steady-state serum TTR reduction from baseline.

Safety:
The most common adverse events with vutrisiran use were diarrhea, pain in extremity, fall, and urinary tract infections (UTIs), with each of these events occurring at a similar or lower rate as compared with external placebo arm.

Dosing:
In the pivotal trial, vutrisiran was administered via SC injection once every 3 months.

Vutrisiran (continued...)

Competitive environment

If approved, vutrisiran would provide an additional treatment option for hATTR with the primary differentiator of more convenient dosing vs. its predecessor product (Onpattro). Vutrisiran is expected to be administered by a healthcare provider quarterly via SC injection while Onpattro is dosed every 3 weeks via IV infusion and requires premedications to prevent infusion-related reactions. The only other therapy approved for polyneuropathy of hATTR amyloidosis is Ionis' Tegsedi® (inotersen), which is SC administered but is associated with safety issues such as thrombocytopenia and glomerulonephritis. Ionis has another SC product in development, eplontersen, with data from their Phase 3 trial in patients with polyneuropathy expected in mid-2022.

The initial target population for vutrisiran is expected to be small given the narrow initial indication. For context, Alnylam reported that as of December 31, 2021, there were approximately 2,050 patients on Onpattro globally. Vutrisiran is also being evaluated for treatment of hATTR amyloidosis or wild-type ATTR amyloidosis with cardiomyopathy. While some patients develop both neuropathy and cardiomyopathy, the cardiomyopathy group is a considerably larger patient population (particularly when considering wild-type ATTR) and currently the only drug approved for this use is Pfizer's oral TTR stabilizer, Vyndamax™ (tafamidis). Topline data for vutrisiran's study in cardiomyopathy is expected in early 2024.

For reference, the wholesale acquisition cost (WAC) for Onpattro is approximately $500,000 per year.

- Advantages: Quarterly SC administration, potential future use for cardiomyopathy associated with ATTR amyloidosis
- Disadvantages: Initial use limited to polyneuropathy associated with hATTR amyloidosis, will require administration by a healthcare provider, potential future competition with Ionis' eplontersen
- Reference WAC (Onpattro): ~$500,000 per year

Surufatinib (Brand Name: To be determined)

Manufacturer: Hutchmed
Regulatory designations: Orphan Drug, Fast Track
Expected FDA decision: April 30, 2022

Therapeutic use

Surufatinib is in development for treatment of pancreatic and extra-pancreatic (non-pancreatic) neuroendocrine tumors (NETs).

- Treatment of pancreatic and extra-pancreatic NETs

NETs form in cells that interact with the nervous system or in glands that produce hormones. They are a heterogenous group of tumors that can originate in various parts of the body, most often in the gut or the lungs and can be benign or malignant. NETs are typically classified as pancreatic NET ("pNET") or extra-pancreatic NET ("epNET"). Patients with NETs can have symptoms attributed to hormonal hypersecretion, including, but not limited to: diarrhea in patients with gastrointestinal NETs, bronchospasm in lung NETs, and hypo/hyperglycemia in pNETs.

There are an estimated 19,000 annual new cases of NETs and the overall prevalence may exceed 170,000 in the U.S.

Clinical profile

Surufatinib is an angio-immuno kinase inhibitor that selectively inhibits the tyrosine kinase activity associated with vascular endothelial growth factor receptors (VEGFR) and fibroblast growth factor receptor (FGFR), which both inhibit angiogenesis, and colony stimulating factor-1 receptor (CSF-1R), which regulates tumor-associated macrophages, promoting the body's immune response against tumor cells.

- Angio-immuno kinase inhibitor
- Oral formulation
- Median PFS (pNET study): 10.9 months vs. 3.7 months with placebo
- Median PFS (epNET study): 9.2 months vs. 3.8 months with placebo
- Common AEs: Hypertension, proteinuria, diarrhea, increased blood thyroid stimulating hormone, hypertriglyceridemia, increased blood bilirubin
- Dosing: Once daily

<u>Pivotal trial data:</u>
For pNET, the efficacy of surufatinib was evaluated in a Phase 3, randomized, double-blind, placebo-controlled study in 172 patients with progressive, advanced, well differentiated tumors. Patients were randomized to surufatinib or placebo. The primary endpoint was progression-free survival (PFS). Median PFS was 10.9 months for surufatinib vs. 3.7 months for placebo (hazard ratio [HR] 0.49, 95% CI: 0.32, 0.76; p = 0.0011). The trial met the early stopping criteria at the interim analysis and was terminated on recommendation from the independent data monitoring committee.

For epNET, the efficacy of surufatinib was evaluated in a Phase 3, randomized, double-blind, placebo-controlled study in 198 patients with unresectable or metastatic, well differentiated tumors. Patients were randomized to surufatinib or placebo. The primary endpoint was median PFS. Median PFS was 9.2 months in the surufatinib group vs. 3.8 months in the placebo group (HR 0.33, 95% CI: 0.22, 0.50; p < 0.0001). Like the pNET study, the trial met the predefined criteria for early discontinuation at the interim analysis.

<u>Safety:</u>
The most common adverse events with surufatinib use were hypertension, proteinuria, diarrhea, increased blood thyroid stimulating hormone, hypertriglyceridemia, and increased blood bilirubin.

<u>Dosing:</u>
In the pivotal trials, surufatinib was administered orally once daily.

Surufatinib (continued...)

Competitive environment

Surufatinib would offer an additional option in the treatment armamentarium for NETs. The current treatment approach for well differentiated NETs includes somatostatin analogs (eg, octreotide), chemotherapy, or targeted drugs such as sunitinib or everolimus.

From a study design perspective, both trials included only patients from China, which may limit the generalizability to a U.S. population. Both studies also did not include an active comparator so there is a lack of head-to-head trial data.

In each of the pivotal studies, there were on-treatment deaths in the surufatinib groups, with a few possibly treatment-related (due to gastrointestinal hemorrhage, disseminated intravascular coagulation and hepatic encephalopathy, and liver injury).

While the initial target population for surufatinib is expected to be small, it's also being studied for other uses (eg, solid tumors, biliary tract cancer).

- Advantages: Additional treatment option for NETs, also being studied for other indications and uses, oral and once daily administration
- Disadvantages: Narrow initial target population, potential safety concerns, study design limitations (all patients were from China, lack of a head-to-head comparator)

Vonoprazan (Brand Name: To be determined)

Manufacturer: Phathom Pharmaceuticals
Expected FDA decision: May 3, 2022

Therapeutic use

Vonoprazan is in development, in combination with amoxicillin and clarithromycin (vonoprazan triple therapy) and vonoprazan in combination with amoxicillin (vonoprazan dual therapy), for the treatment of *Helicobacter pylori (H. pylori)* infection in adults.

H. pylori is a type of bacteria that causes infection in the stomach. As a result of inflammation that can be induced by the infection, patients can develop a range of pathologies including dyspepsia, peptic ulcer disease, gastric cancer, and mucosa-associated lymphoid tissue lymphoma.

The current standard of care for treatment of *H. pylori* infection includes acid-inhibitory drugs (typically proton pump inhibitors [PPIs]) combined with antibiotics such as amoxicillin and clarithromycin.

- In combination with amoxicillin and clarithromycin (vonoprazan triple therapy) or in combination with amoxicillin (vonoprazan dual therapy), for the treatment of *H. pylori* infection in adults

Clinical profile

Vonoprazan is a potassium-competitive acid blocker that blocks acid secretion in the stomach. In preclinical studies, vonoprazan produced sustained inhibition of acid and greater increases in gastric pH (reduced acidity) than a PPI, lansoprazole.

- Potassium-competitive acid blocker
- Oral formulation
- *H. pylori* eradication rate (excluding patients with amoxicillin or clarithromycin resistance): 84.7% with vonoprazan triple therapy vs. 78.5% for vonoprazan dual therapy vs. 78.8% for lansoprazole triple therapy
- Common AEs: Diarrhea, dysgeusia, nausea, headache, vaginal infections
- Dosing: Twice daily for 14 days (in combination with amoxicillin ± clarithromycin)

<u>Pivotal trial data:</u>
The efficacy of vonoprazan was evaluated in PHALCON-HP, a Phase 3, randomized, active-controlled study in 992 patients with a confirmed *H. pylori* infection. Patients were randomized to vonoprazan in combination with amoxicillin and clarithromycin (vonoprazan triple therapy), vonoprazan in combination with amoxicillin (vonoprazan dual therapy), and lansoprazole in combination with amoxicillin and clarithromycin (lansoprazole triple therapy). All three treatment arms were administered for 14 days. The primary endpoint was *H. pylori* eradication rate for the vonoprazan triple and dual therapy compared to lansoprazole triple therapy. Based on FDA feedback, the primary endpoint excluded patients with amoxicillin or clarithromycin resistant strains of *H. pylori*. A secondary superiority analyses was conducted in patients with clarithromycin resistant strains and in all patients.

In the modified intent-to-treat (mITT) population, *H. pylori* eradication rates were 84.7% with vonoprazan triple therapy and 78.5% for vonoprazan dual therapy vs. 78.8% with lansoprazole triple therapy ($p < 0.0001$ and $p = 0.0037$, respectively, for non-inferiority).

The *H. pylori* eradication rate of vonoprazan triple therapy was superior to lansoprazole triple therapy among all patients in the mITT triple population (80.8% vs. 68.5%; $p = 0.0001$) and superior to lansoprazole triple therapy in the subset of patients with H. pylori strains resistant to clarithromycin (65.8% vs. 31.9%; $p < 0.0001$).

The eradication rate of vonoprazan dual therapy was also superior to lansoprazole triple therapy among all patients in the mITT population (77.2% vs. 68.5%; $p = 0.0063$) and superior in the subset of patients with *H. pylori* strains resistant to clarithromycin (69.6% vs. 31.9%; $p < 0.0001$).

Vonoprazan (continued...)

Safety:
The most common adverse events with vonoprazan combination therapy were diarrhea, dysgeusia, nausea, headache, and vaginal infections.

Dosing:
In the pivotal trial, vonoprazan was administered orally twice daily for 14 days (in combination with amoxicillin ± clarithromycin).

Competitive environment

A vonoprazan-based regimen would offer a novel treatment approach for *H. pylori* infections. Currently, many different treatment regimens are available and recommended in the first-line setting with PPIs used as a backbone of therapy. The choice of regimen depends on individual patient allergies and history of antibiotic use (or resistance). Triple therapy with a PPI plus clarithromycin plus amoxicillin has been a first-line treatment option, but eradication rates have declined with failure being driven primarily by underlying clarithromycin resistance.

The data for vonoprazan-based regimens were promising in the head-to-head study vs. lansoprazole triple therapy, including positive data demonstrated in patients with known clarithromycin resistance. In addition to potential efficacy advantages, vonoprazan dual therapy would reduce the pill burden to patients and provide an antibiotic sparing regimen.

However, while vonoprazan-based therapy would offer an alternative to the existing standard of care, PPI-based regimens have been used for decades with many combinations including individual components available generically. While the pivotal study did compare vonoprazan regimens vs. one standard of care option, robust data against other regimens commonly used in patients with existing antibiotic resistance is lacking.

Finally, a single-agent formulation of vonoprazan is also being evaluated for several other gastrointestinal conditions, including gastroesophageal reflux disease (GERD).

- Advantages: Novel treatment regimen for *H. pylori* infection, promising trial results vs. standard of care option, potentially antibiotic sparing (dual therapy regimen), well tolerated
- Disadvantage: PPI-based regimens have been used for decades with individual components available generically, lack of robust head-to-head trial data vs. other standard of care regimens

Tapinarof (Brand Name: To be determined)

Manufacturer: Dermavant Sciences
Expected FDA decision: May 26, 2022

Therapeutic use

Tapinarof is in development for treatment of plaque psoriasis in adult patients.

Psoriasis is a chronic, systemic, inflammatory skin disease characterized by red patches and plaques with silvery scales on the skin. Psoriasis affects 8 million people in the U.S. Plaque psoriasis is the most common form and affects about 80% to 90% of people with psoriasis.

- Treatment of plaque psoriasis in adult patients

Clinical profile

Tapinarof is a first-in-class nonsteroidal, topical aryl hydrocarbon receptor-modulating agent. The aryl hydrocarbon receptor is a ligand-dependent transcription factor with roles in the regulation of cytokine and skin barrier-protein expression and antioxidant activity, which makes it a potential therapeutic target for the treatment of inflammatory skin diseases and potentially other immunologic diseases.

- Aryl hydrocarbon receptor-modulating agent
- Topical formulation
- PGA response: 35.4% to 40.2% vs. 6.0% to 6.3% with vehicle
- Common AEs: Folliculitis, nasopharyngitis, contact dermatitis, headache, URTI, pruritus
- Dosing: Once daily

<u>Pivotal trial data:</u>
The efficacy of tapinarof was evaluated in two identical, Phase 3, randomized, double-blind, vehicle-controlled studies in 1,025 adult patients with mild-to-severe plaque psoriasis. Patients were randomized to use tapinarof 1% cream or vehicle cream once daily for 12 weeks as stand-alone therapy. The primary endpoint of both studies was the proportion of patients who achieved a Physician's Global Assessment (PGA) score of clear (0) or almost clear (1) with a minimum 2-grade improvement from baseline at week 12. The PGA score is on a scale from 0 to 4, with higher scores indicating more severe psoriasis.

A PGA response occurred in 35.4% of the patients in the tapinarof group and in 6.0% of those in the vehicle group in study 1 and in 40.2% and 6.3%, respectively, in study 2 ($p < 0.001$ for both comparisons).

<u>Safety:</u>
The most common adverse events with tapinarof use were folliculitis, nasopharyngitis, contact dermatitis, headache, upper respiratory tract infection (URTI), and pruritus.

<u>Dosing:</u>
In the pivotal trials, tapinarof was administered topically once daily.

Tapinarof (continued...)

Competitive environment

If approved, tapinarof would provide a first-in-class topical treatment for plaque psoriasis. Plaque psoriasis is a large potential target population and tapinarof was evaluated in a broad patient population (mild to severe disease). Tapinarof may also be used as an add-on therapy since systemic side effects are likely to be uncommon due to topical administration.

Tapinarof will be entering a topical marketplace for plaque psoriasis that is currently dominated by corticosteroid and vitamin D analogs, which are available generically. These products are considered first-line agents for the condition and there is a lack of robust head-to-head trials comparing tapinarof against them. Looking ahead, tapinarof will also potentially be competing with Arcutis Biotherapeutics' novel topical formulation of roflumilast, a phosphodiesterase-4 inhibitor. An FDA approval decision for topical roflumilast for the treatment of plaque psoriasis is expected by July 29, 2022.

Tapinarof is also being evaluated for atopic dermatitis with data expected in the first half of 2023. If the data is positive, this could significantly expand the potential target population for tapinarof given the high prevalence of atopic dermatitis.

For reference, the WAC for Wynzora® (calcipotriene/betamethasone dipropionate), a branded combination cream containing a vitamin D analog and corticosteroid, is approximately $1,000 per 30 days.

- Advantages: First-in-class topical treatment, large potential target population (studied in mild-to-severe patients), potential for add-on use due to topical administration, also in development for atopic dermatitis
- Disadvantages: Alternative topicals available generically (corticosteroids and vitamin D analogs), lack of head-to-head trial data vs. other topical therapies, potential future competition (eg, topical roflumilast)
- Reference WAC (Wynzora): ~$1,000 per 30 days

Tirzepatide (Brand Name: To be determined)

Manufacturer: Eli Lilly
Expected FDA decision: 5/2022

Therapeutic use

Tirzepatide is in development as an adjunct to diet and exercise to improve glycemic control in adults with type 2 diabetes mellitus (T2DM).

Approximately 34 million individuals in the U.S. have diabetes with T2DM accounting for an estimated 90% to 95% of all diabetes cases.

- Adjunct to diet and exercise to improve glycemic control in adults with T2DM

Clinical profile

Tirzepatide is a dual glucose-dependent insulinotropic polypeptide (GIP) and glucagon-like peptide-1 (GLP-1) receptor agonist. GIP is a hormone that may complement the effects of GLP-1. GIP is believed to decrease food intake and increase energy expenditure therefore resulting in weight reductions, and when combined with a GLP-1 receptor agonist, may result in greater effects on glucose and body weight.

- GIP/GLP-1 receptor agonist
- SC formulation
- A1c reduction: 2.01% to 2.30% vs. 1.86% with Ozempic 1 mg
- Mean weight reduction: 7.8 kg to 12.4 kg vs. 6.2 kg with Ozempic 1 mg
- Common AEs: Nausea, diarrhea, vomiting
- Dosing: Once weekly

<u>Pivotal trial data:</u>
The efficacy of tirzepatide was evaluated across five Phase 3, randomized studies (SURPASS 1 to 5) in patients with T2DM. The primary endpoint was the mean change in glycated hemoglobin (HbA1c). A key secondary endpoint was mean weight reduction.

SURPASS-1 (N = 478) compared tirzepatide (5 mg, 10 mg, or 15 mg) vs. placebo. At week 40, tirzepatide reduced HbA1c by 1.87% to 2.07% vs. an increase of 0.04% with placebo ($p < 0.001$ for all tirzepatide doses vs. placebo). Mean weight reduction after 40 weeks was 7.0 kg to 9.5 kg with tirzepatide vs. 0.7 kg with placebo ($p < 0.001$ for all tirzepatide doses vs. placebo).

SURPASS-2 (N = 1,879) compared tirzepatide (5 mg, 10 mg, or 15) vs. the GLP-1 agonist, Ozempic® (semaglutide) 1 mg. At week 40, tirzepatide reduced HbA1c by 2.01% to 2.30% vs. 1.86% with Ozempic ($p = 0.02$ for tirzepatide 5 mg, $p < 0.001$ for tirzepatide 10 mg and 15 mg vs. Ozempic). Mean weight reduction was 7.8 kg to 12.4 kg vs. 6.2 kg with Ozempic ($p < 0.001$ for all tirzepatide doses vs. Ozempic).

SURPASS-3 (N = 1,444) compared tirzepatide (5 mg, 10 mg, or 15 mg) vs. a basal insulin, Tresiba® (insulin degludec). At week 52, tirzepatide reduced HbA1c by 1.93% to 2.37% for tirzepatide vs. 1.34% for Tresiba ($p < 0.0001$ for all tirzepatide doses vs. Tresiba). Tirzepatide reduced bodyweight by 7.5 kg to 12.9 kg vs. an increase of 2.3 kg with Tresiba ($p < 0.0001$ for all tirzepatide doses vs. Tresiba)

SURPASS-4 (N = 2,002) compared tirzepatide (5 mg, 10 mg, or 15 mg) vs. a basal insulin, glargine. At 52 weeks, tirzepatide reduced HbA1c by 2.24% to 2.58% vs. 1.44% with insulin glargine ($p < 0.0001$ for all tirzepatide doses vs. insulin glargine). Tirzepatide reduced bodyweight by 7.1 kg to 11.7 kg vs. an increase of 1.9 kg with insulin glargine ($p < 0.0001$ for all tirzepatide doses vs. insulin glargine).

SURPASS-5 (N = 475) compared tirzepatide (5 mg, 10 mg, and 15 mg) vs. placebo, both as an add-on to titrated insulin glargine with or without metformin. At week 40, tirzepatide reduced HbA1c by 2.23% to 2.59% vs. 0.93% for placebo ($p < 0.001$ for all tirzepatide doses vs. placebo). Tirzepatide reduced bodyweight by 6.2 kg to 10.9 kg vs. an increase of 1.7 kg with placebo ($p < 0.001$ for all tirzepatide doses vs. placebo).

Tirzepatide (continued...)

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

Dosing:
In the pivotal trials, tirzepatide was administered SC once weekly.

Competitive environment

Tirzepatide would provide an additional treatment option for T2DM with a unique dual mechanism of action (MOA). Tirzepatide would primarily be competing with other GLP-1 receptor agonists such as Ozempic. Based on the head-to-head study vs. Ozempic 1 mg, tirzepatide does have favorable A1c reduction and weight loss. However, a higher strength formulation (2 mg) of Ozempic is currently under review by the FDA for T2DM and a higher strength formulation of semaglutide (2.4 mg) is currently approved specifically for weight loss under the brand name Wegovy®.

While A1c reduction is an important consideration in the choice of add-on therapy in management of T2DM, treatment guidelines also recommend choosing a therapy based on comorbid conditions and risk factors (eg, atherosclerotic cardiovascular disease, heart failure, chronic kidney disease). Several drugs used for T2DM have shown benefit beyond glucose control, including proven benefit on cardiovascular outcomes. Eli Lilly's cardiovascular outcomes trial in patients with T2DM has been initiated but results are not expected until 2025.

In addition to the cardiovascular outcomes trial, tirzepatide is also in development for other conditions, including obesity, heart failure, and nonalcoholic steatohepatitis.

For reference, the WAC for Ozempic is approximately $10,000 per year.

- Advantages: Unique dual MOA, promising reductions in A1c and body weight, also in development for other uses (ie, obesity, heart failure, nonalcoholic steatohepatitis)
- Disadvantages: Crowded marketplace, lack of long-term cardiovascular outcomes (data expected in 2025)
- Reference WAC (Ozempic): ~$10,000 per year

Tebipenem (Brand Name: To be determined)

Manufacturer: Spero Therapeutics
Regulatory designations: Fast Track
Expected FDA decision: June 27, 2022 *(FDA Advisory Committee is planned but not yet scheduled)*

Therapeutic use

Tebipenem is in development for treatment in adult patients with complicated urinary tract infections (UTIs), including acute pyelonephritis.

UTIs are common and occur in around 8 million patients annually in the U.S. and of those, about 2.7 million fail or have resistance to first line antibiotics.

Clinical profile

Tebipenem is a carbapenem antibiotic. Carbapenems are highly effective, broad spectrum antibiotics that have been available as injectable drugs and generally reserved for severe or high-risk bacterial infections.

Pivotal trial data:
Tebipenem was evaluated in ADAPT-PO, a Phase 3, randomized, active-control study in 868 hospitalized patients with complicated UTIs or acute pyelonephritis. Patients were randomized to receive oral tebipenem or ertapenem (another carbapenem) via IV infusion for 7 to 10 days (or up to 14 days in patients with bacteremia). The primary endpoint was the overall response (composite clinical cure plus microbiologic eradication) at the test of cure visit (day 19 ± 2). Tebipenem demonstrated non-inferiority vs. ertapenem with an overall response rate of 58.8% vs. 61.6%, respectively (treatment difference -3.3; 95% CI: -9.7, 3.2).

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

Dosing:
In the pivotal trial, tebipenem was administered orally three times daily for 7 to 10 days (or up to 14 days in patients with bacteremia).

- Treatment in adult patients with complicated UTI, including acute pyelonephritis

- Carbapenem antibiotic
- Oral formulation
- Overall response: 58.8% vs. 61.6% with ertapenem (non-inferiority met)
- Common AEs: Diarrhea, headache
- Dosing: Three times daily for 7 to 10 days (or up to 14 days in patients with bacteremia)

Tebipenem (continued...)

Competitive environment

If approved, tebipenem would be the first oral carbapenem and would allow for more outpatient treatment of complicated UTIs. It could also reduce the number of days patients spend in the hospital due to complicated UTIs since patients could be discharged earlier with the availability of a broad spectrum oral carbapenem. There is a growing unmet need for additional broad spectrum oral antibiotics due to increased resistance to drugs historically used for UTIs (eg, fluoroquinolone antibiotics).

Alternative oral drugs from other antibiotic drug classes, such as cephalosporins and fluoroquinolones, are mostly available generically. Given the availability of numerous alternative oral antibiotics and the fact that carbapenems are usually reserved as backline agents to prevent future development of resistance, tebipenem will probably be reserved as a second- or third-line treatment for complicated UTIs.

- Advantages: Potentially the first oral carbapenem, allow for more outpatient treatment of cUTI, growing unmet need due to increased antibiotic resistance (eg, fluoroquinolone resistance)
- Disadvantages: Many alternative oral antibiotics available with different mechanisms (including generics), will be reserved as second- or third-line treatment

AMX-0035 (sodium phenylbutyrate/taurursodiol) (Brand Name: To be determined)

Manufacturer: Amylyx Pharmaceuticals
Regulatory designations: Orphan Drug
Expected FDA decision: June 29, 2022

Therapeutic use

AMX-0035, a fixed-dose combination of sodium phenylbutyrate and taurursodiol, is in development for treatment of amyotrophic lateral sclerosis (ALS).

ALS is a rare, neurodegenerative disorder characterized by the progressive degeneration and eventual death of nerve cells in the brain, brainstem, and spinal cord. ALS affects the muscles needed to move the arms and legs, to speak and swallow, to support the neck and trunk, and to breathe. Most people develop ALS between 40 to 70 years of age and median survival from symptom onset is 2 to 3 years, with respiratory failure being the main cause of death.

Approximately 30,000 people are affected with ALS in the U.S., with an estimated 5,000 new cases diagnosed each year.

- Treatment of ALS

Clinical profile

AMX-0035 is a fixed-dose combination of two small molecules: sodium phenylbutyrate and taurursodiol. AMX-0035 is designed to target the endoplasmic reticulum and mitochondrial-dependent neuronal degeneration pathways thereby reducing neuronal death and dysfunction in patients with ALS.

Sodium phenylbutyrate is currently available as a single-ingredient prescription for urea cycle disorders and taurursodiol is a bile acid that is available as a nutritional supplement.

- Neuroprotective
- Oral formulation
- ALSFRS-R score: -1.24 points per month vs. -1.66 points per month with placebo
- Common AEs: Gastrointestinal (diarrhea, nausea, salivary hypersecretion, abdominal discomfort)
- Dosing: Once daily for 3 weeks followed by twice daily

Pivotal trial data:

The efficacy of AMX-0035 was evaluated in CENTAUR, a Phase 2, randomized, double-blind, placebo-controlled study in 137 people with ALS who had an onset of symptoms within the previous 18 months. Patients were randomized to receive AMX-0035 or placebo. The primary endpoint was the rate of decline in the total score on the Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised (ALSFRS-R; range, 0 to 48, with higher scores indicating better function) through 24 weeks. In a modified intention-to-treat analysis, the mean rate of change in the ALSFRS-R score was -1.24 points per month with AMX-0035 vs. -1.66 points per month with placebo (difference of 0.42 points per month; 95% CI: 0.03, 0.81; p = 0.03). The least squares mean difference at week 24 was 2.32 (95% CI: 0.18, 4.47).

Patients who completed CENTAUR were eligible for enrollment in an open-label extension (OLE) aimed at assessing long-term efficacy of AMX-0035. In the OLE, all patients received AMX-0035 for up to 30 months, regardless of their treatment arm in the original randomized study (AMX-0035 or placebo). An all-cause mortality analysis (35-month maximum follow-up post-randomization) was conducted. Median overall survival (OS) was 25.0 months among participants originally randomized to AXS-0035 vs.18.5 months among those originally randomized to placebo (HR 0.56, 95% CI: 0.34, 0.92; p = 0.023). The estimated probability of survival at 12 months among participants originally randomized to AMX-0035 and placebo was 80.9% (95% CI: 71.1, 87.7) and 72.9% (95% CI: 58.0, 83.3), respectively. At 24 months, the estimates were 51.6% (95% CI: 38.9, 62.9) and 33.9% (95% CI: 19.4, 49.1), respectively.

AMX-0035 (continued...)

Safety:
The most common adverse events with AMX-0035 use were primarily gastrointestinal (diarrhea, nausea, salivary hypersecretion, and abdominal discomfort).

Dosing:
In the pivotal trial, AMX-0035 was administered once daily for 3 weeks and then twice daily thereafter.

Competitive environment

If approved, AMX-0035 would provide a novel oral treatment option for ALS, a condition for which there is still a very high unmet need. The only other drugs approved for ALS are riluzole, which has been shown to prolong survival by an average of 3 to 5 months and Radicava® (edaravone), which has been shown to slow the rate of functional decline in some patients with ALS.

When compared indirectly, AMX-0035 slowed ALS disease progression similarly to Radicava, although it should be noted that patients in the AMX-0035 study could be on concomitant therapy with riluzole and/or Radicava. The long-term extension trial provided promising survival data and overall, AMX-0035 appears to have an acceptable safety profile.

Secondary endpoints (eg, muscle strength, lung function) did not differ statistically between AMX-0035 and placebo in the pivotal study, although the study was not powered to detect differences for those outcomes.

- Advantages: Novel treatment for ALS, high unmet need, promising survival data in a long-term extension study, acceptable safety profile, oral administration
- Disadvantages: Secondary outcomes (eg, muscle strength, lung function) did not differ vs. placebo in the pivotal study

1st Quarter 2022

Extended generic pipeline forecast

OptumRx generic pipeline forecast

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
2022 Possible launch date					
DALIRESP	roflumilast	AstraZeneca	Oral	All	2022
TOVIAZ	fesoterodine	Pfizer	Oral	All	2022
SYNDROS	dronabinol	Insys Therapeutics	Oral	All	2022
DULERA	formoterol fumarate/mometasone furoate	Organon	Inhalation	All	2022
THALOMID	thalidomide	Celgene	Oral	All	2022
ONEXTON	clindamycin/benzoyl peroxide	Bausch Health	External	All	2022
IXEMPRA Kit	ixabepilone	R-Pharm	Intravenous	All	1H-2022
OXAYDO	oxycodone	Egalet	Oral	All	01-2022
NEUPRO	rotigotine	UCB	External	All	01-2022
BALCOLTRA	levonorgestrel/ethinyl estradiol/ferrous bisglycinate	Avion	Oral	All	01-2022
SELZENTRY	maraviroc	ViiV Healthcare	Oral	All	02-2022
VIMPAT	lacosamide	UCB	Oral; intravenous	All	03-2022
ZIPSOR	diclofenac potassium	Depomed	Oral	All	03-2022
CHOLBAM	cholic acid	Retrophin	Oral	All	03-2022
ABRAXANE	paclitaxel	Celgene/Abraxis	Injection	All	03-2022
REVLIMID	lenalidomide	Bristol-Myers Squibb/Celgene	Oral	All	03-2022
ARESTIN	minocycline hydrochloride	Bausch Health	Subgingival	All	03-2022
PRADAXA	dabigatran etexilate mesylate	Boehringer Ingelheim	Oral	All	2Q-2022
ZOLADEX	goserelin	TerSera Therapeutics	Subcutaneous	All	04-2022
ALIMTA	pemetrexed disodium	Eli Lilly	Intravenous	All	05-2022
VELCADE	bortezomib	Takeda	Intravenous	All	05-2022
TARGINIQ ER	oxycodone/naloxone	Purdue	Oral	All	05-2022
CAPRELSA	vandetanib	Genzyme/Sanofi	Oral	All	06-2022
VIIBRYD	vilazodone	Forest/Allergan	Oral	All	06-2022
ELESTRIN	estradiol	Mylan	External	All	06-2022
LUCENTIS	ranibizumab	Roche	Intravitreal	All	06-2022

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
FLOVENT HFA	fluticasone propionate	GlaxoSmithKline	Inhalation	All	2H-2022
IRESSA	gefitinib	AstraZeneca	Oral	All	07-2022
EVAMIST	estradiol	Perrigo/Elan	External	All	07-2022
KEVEYIS	dichlorphenamide	Strongbridge Biopharma	Oral	All	08-2022
ORAVIG	miconazole	Galt Pharmaceuticals	Oral	All	09-2022
SUPREP BOWEL PREP KIT	magnesium sulfate anhydrous/potassium sulfate/sodium sulfate	Braintree	Oral	All	09-2022
AMZEEQ	minocycline	Foamix	External	All	10-2022
XERESE	acyclovir/hydrocortisone	Bausch Health	External	All	11-2022
FOLOTYN	pralatrexate	Acrotech/Aurobindo	Intravenous	All	11-2022
RAYOS	prednisone	Horizon	Oral	All	12-2022
TREANDA	bendamustine	Cephalon/Teva	Intravenous	All	12-2022
ZIOPTAN	tafluprost	Akorn	Ophthalmic	All	12-2022
ARAZLO	tazarotene	Ortho Dermatologics	External	All	12-2022
LEVEMIR	insulin detemir recombinant	Novo Nordisk	Subcutaneous	All	12-2022
TRESIBA FLEXTOUCH	insulin degludec	Novo Nordisk	Subcutaneous	All	12-2022
2023 Possible launch date					
PREZISTA	darunavir	Janssen	Oral	All	2023
PROLENSA	bromfenac	Bausch Health	Ophthalmic	All	2023
ALPHAGAN P	brimonidine	Allergan	Ophthalmic	All	2023
MYRBETRIQ	mirabegron	Astellas	Oral	All	2023
EYLEA	aflibercept	Regeneron	Intravitreal	All	2023
NEULASTA ONPRO	pegfilgrastim	Amgen/Insulet	Subcutaneous	All	2023
KOMBIGLYZE XR	saxagliptin/metformin	Bristol-Myers Squibb/Astra Zeneca	Oral	All	1H-2023
ONGLYZA	saxagliptin	Bristol-Myers Squibb/Astra Zeneca	Oral	All	1H-2023
ACTEMRA	tocilizumab	Roche/Chugai	Intravenous and subcutaneous	All	1H-2023
NOXAFIL	posaconazole	Merck	Intravenous	All	01-2023
HUMIRA	adalimumab	AbbVie	Subcutaneous	All	01-2023
XYREM	sodium oxybate	Jazz	Oral	All	01-2023
CAMBIA	diclofenac potassium	Assertio	Oral	All	01-2023

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
TROKENDI XR	topiramate	Supernus	Oral	All	01-2023
DUOBRII	halobetasol propionate/tazarotene	Bausch Health	External	All	01-2023
NASCOBAL	cyanocobalamin	Par/Endo	Intranasal	All	01-2023
DYLOJECT	diclofenac	Hospira/Pfizer/Javelin	Intravenous	All	01-2023
TEFLARO	ceftaroline fosamil	Allergan	Intravenous	All	01-2023
GLOPERBA	colchicine	Avion Pharmaceuticals	Oral	All	01-2023
FIRVANQ KIT	vancomycin	Azurity	Oral	All	01-2023
ESBRIET	pirfenidone	InterMune/Genentech/Roche	Oral	All	01-2023
SPIRIVA HANDIHALER	tiotropium	Boehringer Ingelheim	Inhalation	All	01-2023
FORTEO	teriparatide	Eli Lilly	Injection	All	01-2023
BROMSITE	bromfenac	Sun	Ophthalmic	All	01-2023
LEXISCAN	regadenoson	Astellas	Intravenous	All	01-2023
BYETTA	exenatide	AstraZeneca	Subcutaneous	All	01-2023
LATUDA	lurasidone	Sunovion	Oral	All	02-2023
GATTEX	teduglutide recombinant	Takeda	Subcutaneous	All	03-2023
AGGRASTAT	tirofiban	Medicure	Intravenous	All	03-2023
AUBAGIO	teriflunomide	Sanofi/Genzyme	Oral	All	03-2023
PROVAYBLUE	methylene blue	Provepharm/American Regent	Intravenous	All	04-2023
CLINDESSE	clindamycin phosphate	Perrigo	Vaginal	All	04-2023
LIVALO	pitavastatin	Eli Lilly/Kowa Pharmaceuticals	Oral	All	05-2023
KYNMOBI	apomorphine	Sunovion	Sublingual	All	05-2023
XURIDEN	uridine	Wellstat Therapeutics	Oral	All	07-2023
TOLAK	fluorouracil	Pierre Fabre	External	All	07-2023
MOZOBIL	plerixafor	Sanofi/Genzyme	Subcutaneous	All	07-2023
CYSTADROPS	cysteamine	Recordati	Ophthalmic	All	08-2023
VYVANSE	lisdexamfetamine	Shire/Takeda	Oral	All	08-2023
KATERZIA	amlodipine	Azurity	Oral	All	08-2023
STELARA	ustekinumab	Janssen	Subcutaneous	All	09-2023
VIBATIV	telavancin	Theravance	Intravenous	All	09-2023
LEXETTE	halobetasol	Mayne	External	All	09-2023
VOTRIENT	pazopanib	Novartis	Oral	All	10-2023
OZURDEX	dexamethasone	Allergan	Ophthalmic	All	11-2023

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
AMTURNIDE	aliskiren/amlodipine/hydrochlorothiazide	Novartis	Oral	All	11-2023
KOGENATE FS	octocog alpha	Bayer	Intravenous	All	11-2023
HELIXATE FS	antihemophilic factor VIII	CSL Behring/Bayer	Intravenous	All	11-2023
KALBITOR	ecallantide	Dyax	Subcutaneous	All	12-2023
2024 Possible launch date					
VESICARE LS	solifenacin	Astellas	Oral	All	1H-2024
GIAZO	balsalazide disodium	Bausch Health	Oral	All	01-2024
GILENYA	fingolimod	Novartis	Oral	0.5 mg	01-2024
GRALISE	gabapentin	Assertio Therapeutics	Oral	All	01-2024
TASIGNA	nilotinib	Novartis	Oral	All	01-2024
SIMPONI ARIA	golimumab	Janssen	Intravenous	All	02-2024
SIMPONI	golimumab	Janssen	Subcutaneous	All	02-2024
NATESTO	testosterone	Acerus	Nasal	All	02-2024
CIMZIA	certolizumab pegol	UCB/Royalty Pharma	Subcutaneous	All	02-2024
SYMPAZAN	clobazam	Aquestive	Oral	All	02-2024
ISENTRESS	raltegravir	Merck	Oral	All	04-2024
DUTREBIS	lamivudine/raltegravir	Merck	Oral	All	04-2024
EVEKEO ODT	amphetamine	Arbor	Oral	All	04-2024
PROBUPHINE	buprenorphine	Titan Pharmaceuticals/Braeburn Pharmaceuticals	Subdermal	All	04-2024
RADICAVA	edaravone	Mitsubishi Tanabe	Intravenous	All	05-2024
DUAVEE	conjugated estrogens/bazedoxifene acetate	Pfizer/Ligand Pharmaceuticals	Oral	All	05-2024
SAXENDA	liraglutide	Novo Nordisk	Subcutaneous	All	05-2024
ARANESP	darbepoetin alfa	Amgen/Kirin	Intravenous and subcutaneous	All	05-2024
ULESFIA	benzyl alcohol	Concordia/Shionogi	External	All	05-2024
VICTOZA	liraglutide recombinant	Novo Nordisk	Subcutaneous	All	06-2024
HAEGARDA	C1 esterase inhibitor	CSL Behring	Subcutaneous	All	06-2024
BRILINTA	ticagrelor	AstraZeneca	Oral	All	2H-2024
INVEGA HAFYERA	paliperidone	Janssen	Intramuscular	All	09-2024
SPRYCEL	dasatinib	Bristol-Myers Squibb	Oral	All	09-2024
SUSTOL	granisetron	Heron Therapeutics	Subcutaneous	All	09-2024
PRIALT	ziconotide acetate	TerSera Therapeutics	Intrathecal	All	10-2024

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
LAZANDA	fentanyl citrate	Depomed	Intranasal	All	10-2024
RYDAPT	midostaurin	Novartis	Oral	All	10-2024
STENDRA	avanafil	Metuchen Pharmaceuticals	Oral	All	10-2024
QSYMIA	phentermine/topiramate	Vivus	Oral	All	12-2024
SIKLOS	hydroxyurea	Addmedica/Medunik	Oral	All	12-2024

Extended brand pipeline forecast

OptumRx Brand Pipeline Forecast

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
2022 Possible launch date									
AG-348	mitapivat	Agios	pyruvate kinase-R activator	Pyruvate kinase deficiency	PO	Filed NDA	02/17/2022	Yes	Yes
dextroamphetamine transdermal	dextroamphetamine	Noven Pharmaceuticals	CNS stimulant	Attention deficit hyperactivity disorder	TOP	Filed NDA	02/22/2022	No	No
RTA-402	bardoxolone methyl	Reata Pharmaceuticals	Nrf2 activator	Alport syndrome	PO	Filed NDA	02/25/2022	Yes	Yes
GC-5107	human immunoglobulin	GC Pharma	human immunoglobulin	Primary immunodeficiencies	IV	Filed BLA	02/25/2022	Yes	No
Tyvaso DPI	treprostinil	United Therapeutics	prostacyclin mimetic	Pulmonary arterial hypertension/ pulmonary hypertension	INH	Filed NDA	02/28/2022	Yes	No
Filsuvez (AP-101)	episalvan	Amryt Pharma	triterpene	Epidermolysis bullosa	TOP	Filed NDA	02/28/2022	No	Yes
JNJ-4528 (LCAR-B38M)	ciltacabtagene autoleucel	Legend Biotech/ Janssen	CAR T cell therapy	Multiple myeloma	IV	Filed BLA	02/28/2022	Yes	Yes
pacritinib	pacritinib	CTI BioPharma	janus kinase-2 inhibitor	Myelofibrosis	PO	Filed NDA	02/28/2022	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
GS-CA1 (GS-6207)	lenacapavir	Gilead	HIV capsid inhibitor	HIV-1	SC	Filed NDA	02/28/2022	Yes	No
Corplex donepezil	donepezil	Corium International	acetylcholinesterase inhibitor	Alzheimer's disease	TOP	Filed NDA	03/11/2022	No	No
BMS-986213	relatlimab/ nivolumab	Bristol Myers Squibb	lymphocyte-activation gene 3 blocking antibody/PD-1 immune checkpoint inhibitor	Melanoma	IV	Filed BLA	03/19/2022	Yes	No
CCD-1042	ganaxolone	Marinus Pharmaceuticals	allosteric modulator of GABA(a) receptors	CDKL5 deficiency disorder epilepsy	PO	Filed NDA	03/20/2022	No	Yes
TG-1303	ublituximab	TG Therapeutics	anti-CD-20 monoclonal antibody	Chronic lymphocytic leukemia	IV	Filed NDA	03/25/2022	Yes	Yes
Zydena	udenafil	Mezzion Pharma	phosphodiesterase type 5 inhibitor	Congenital single ventricle heart disease	PO	Filed NDA	03/26/2022	No	Yes
Tlando	testosterone	Antares Pharma	androgen	Hypogonadism	PO	Filed NDA	03/28/2022	No	No
Ozempic Forte	semaglutide	Novo Nordisk	glucagon-like peptide 1 agonist	Type 2 diabetes mellitus	SC	Filed NDA	03/28/2022	No	No
AKB-6548	vadadustat	Otsuka Pharmaceutical	hypoxia-inducible factor-prolyl hydroxylase inhibitor	Chronic kidney disease-related anemia	PO	Filed NDA	03/29/2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
IBI-308	sintilimab	Eli Lilly	programmed death-1 receptor inhibitor	Non-small cell lung cancer	IV	Filed BLA	03/2022	Yes	No
F-627	benegrastim	Evive Biotech	granulocyte colony-stimulating factor	Chemotherapy-induced neutropenia	SC	Filed BLA	03/31/2022	Yes	No
UCB-4940 (CDP-4940)	bimekizumab	UCB	interleukin-17 receptor inhibitor	Plaque psoriasis	SC	Filed BLA	1Q2022	Yes	No
Kyzatrex	testosterone undecanoate	Marius Pharmaceuticals	testosterone replacement therapy	Hypogonadism	PO	Filed NDA	1Q2022	No	No
S5G4T-1 (DER-45-EV)	benzoyl peroxide	Sol-Gel Technologies	benzoyl peroxide	Rosacea	TOP	Filed NDA	1Q2022	No	No
FT-218	sodium oxybate extended-release	Avadel	dopamine receptor agonist	Narcolepsy	PO	Filed NDA	1Q2022	Yes	Yes
Libervant	diazepam	Aquestive Therapeutics	benzodiazepine	Seizures	PO	Filed NDA	1Q2022	No	Yes
Botulax	letibotulinumtoxinA	Hugel Pharma	botulinum toxins	Wrinkles	IM	Filed BLA	03/31/2022	Yes	No
BXCL-501	dexmedetomidine	BioXcel Therapeutics	selective alpha 2a receptor agonist	Schizophrenia and bipolar disorder	PO	Filed NDA	04/05/2022	No	No
REGEN-COV	casirivimab/imdevimab	Regeneron/Roche	Monoclonal antibody	COVID-19	IV/IM/SC	Filed BLA	04/13/2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ALN-TTRsc02	vutrisiran	Alnylam	small interfering RNA therapeutic	Hereditary transthyretin-mediated amyloidosis	SC	Filed BLA	04/14/2022	Yes	Yes
MYK-461 (SAR-439152)	mavacamten	Bristol Myers Squibb	cardiac myosin allosteric modulator	Obstructive hypertrophic cardiomyopathy	PO	Filed NDA	04/28/2022	Yes	Yes
TV-46000	risperidone	Teva Pharmaceuticals/ MedinCell	atypical antipsychotic	Schizophrenia	SC	Filed NDA	04/30/2022	No	No
AXS-07	meloxicam/rizatriptan	Axsome Therapeutics	non-steroidal anti-inflammatory drug/triptan	Migraine	PO	Filed NDA	04/30/2022	No	No
HMPL-012	surufatinib	Hutchison China MediTech	angio-immunokinase inhibitor	Neuroendocrine tumors	PO	Filed NDA	04/30/2022	Yes	Yes
JS-001	toripalimab	Shanghai Junshi Biosciences/ Coherus Biosciences	anti-PD-1 monoclonal antibody	Nasopharyngeal carcinoma	IV	Filed BLA	05/01/2022	Yes	Yes
Takecab	vonoprazan fumarate	Phathom Pharmaceuticals	potassium-competitive acid blocker	H. pylori infection	PO	Filed NDA	05/08/2022	No	No
MT-1186	edaravone	Mitsubishi Tanabe Pharma	free radical scavenger	Amyotrophic lateral sclerosis	PO	Filed NDA	05/12/2022	Yes	Yes
AK-105	penpulimab	Akeso	anti-PD-1 monoclonal antibody	Nasopharyngeal carcinoma	IV	Filed BLA	05/24/2022	Yes	Yes
VP-102	cantharidin	Verrica	vesicant (blistering agent)	Molluscum	TOP	Filed NDA	05/24/2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
GSK-2894512 (WBI-1001)	tapinarof	Dermavant Sciences	therapeutic aryl hydrocarbon receptor modulating agent	Plaque psoriasis	TOP	Filed NDA	05/26/2022	Yes	No
LY-3298176	tirzepatide	Eli Lilly	glucose-dependent insulinotropic polypeptide/glucagon-like peptide-1 receptor agonist	Type 2 diabetes mellitus	SC	Filed NDA	05/30/2022	No	No
ACER-001	sodium phenylbutyrate	Acer Therapeutics	nitrogen-binding agent	Urea cycle disorders	PO	Filed NDA	06/05/2022	No	No
BI-655130	spesolimab	Boehringer Ingelheim	IL-36 receptor antibody	Generalized pustular psoriasis	IV	Filed BLA	06/15/2022	Yes	Yes
SPR-994	tebipenem	Spero Therapeutics	carbapenem	Complicated urinary tract infections	PO	Filed NDA	06/27/2022	No	No
AMX-0035	sodium phenylbutyrate/ taurursodiol	Amylyx Pharmaceuticals	neuroprotective	Amyotrophic lateral sclerosis	PO	Filed NDA	06/29/2022	Yes	Yes
177Lu-PSMA-617	Lutetium	Novartis	Radiopharmaceutical	Prostate cancer	IV	Filed BLA	1H2022	Yes	No
Cuprior	trientine tetrahydrochloride	Orphalan	chelating agent	Wilson's disease	PO	Filed NDA	06/2022 - 07/2022	Yes	Yes
GZ-402665	olipudase alfa	Sanofi	enzyme replacement therapy	Acid sphingomyelinase deficiency	IV	Filed BLA	07/03/2022	Yes	Yes
BGB-A317 (BGB-A-317)	tislelizumab	BeiGene	programmed death-1 inhibitor	Esophageal squamous cell carcinoma	IV	Filed BLA	07/12/2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Hepcludex	bulevirtide	Gilead	HBV receptor binder	Hepatitis delta virus	SC	Filed BLA	07/19/2022	No	Yes
ARQ-151	roflumilast	Arcutis Biotherapeutics	phosphodiesterase-4 inhibitor	Plaque psoriasis	TOP	Filed NDA	07/29/2022	Yes	No
OMS-721	narsoplimab	Omeros	anti-MASP-2 monoclonal antibody	Hematopoietic stem cell transplant-associated thrombotic microangiopathy	IV	Filed BLA	07/29/2022	Yes	Yes
AT-GAA	cipaglucosidase alfa	Amicus	enzyme therapy	Pompe disease	IV	Filed BLA	07/29/2022	Yes	Yes
Ultomiris SC	ravulizumab-cwvz	AstraZeneca/ Alexion	C5 complement inhibitor	Paroxysmal nocturnal hemoglobinuria; Hemolytic uremic syndrome	SC	Filed BLA	07/2022	Yes	Yes
Priorix	measles/mumps/rubella	GlaxoSmithKline	Vaccine	measles/mumps/rubella vaccine	SC	Filed BLA	08/02/2022	No	No
Zynteglo (LentiGlobin)	betibeglogene autotemcel	Bluebird Bio	gene therapy	Beta thalassemia	IV	Filed BLA	08/19/2022	Yes	Yes
JNJ-64007957	teclistamab	Janssen	BCMA and CD3 bispecific antibody	Multiple myeloma	IV	Filed BLA	08/29/2022	Yes	No
BMS-986165	deucravacitinib	Bristol-Myers Squibb	tyrosine kinase 2 inhibitor	Plaque psoriasis	PO	Filed NDA	09/10/2022	Yes	No
OBE-2109 (KLH-2109)	linzagolix	ObsEva	gonadotropin-releasing hormone antagonist	Uterine fibroids	PO	Filed NDA	09/13/2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Lenti-D	elivaldogene tavalentivec	Bluebird Bio	gene therapy	Adrenomyeloneuropathy	IV	Filed BLA	09/16/2022	Yes	Yes
HTX-019	aprepitant	Heron Therapeutics	substance P/neurokinin-1 receptor antagonist	Postoperative nausea and vomiting	IV	Filed NDA	09/17/2022	No	No
Dasynoc	dasatinib	Xspray Pharma	kinase inhibitor	Chronic myeloid leukemia	PO	Filed NDA	09/18/2022	No	No
ublituximab	ublituximab	TG Therapeutics	anti-CD-20 monoclonal antibody	Multiple sclerosis	IV	Filed BLA	09/28/2022	Yes	No
PRV-031	teplizumab	Provention Bio/ MacroGenics	CD3 antigen inhibitor	Diabetes mellitus	IV	CRL	3Q2022	Yes	No
Furoscix	furosemide	scPharmaceuticals	diuretic	Heart failure	SC	CRL	3Q2022	Yes	No
SPN-830	apomorphine	Supernus Pharmaceuticals	non-ergoline dopamine agonist	Parkinson's disease	SC infusion	Filed NDA	10/08/2022	Yes	No
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension	INH	Tentative Approval	10/27/2022	Yes	No
HM781-36B	poziotinib	Spectrum Pharmaceuticals	pan-HER inhibitor	Non-small cell lung cancer	PO	Filed NDA	11/24/2022	Yes	No
omecamtiv mecarbil	omecamtiv mecarbil	Amgen	mysoin activator	Heart failure	PO	Filed NDA	11/30/2022	No	No
MRTX-849	adagrasib	Mirati Therapeutics	KRAS inhibitor	Non-small cell lung cancer	PO	Filed NDA	12/14/2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
dovitinib	dovitinib	Allarity Therapeutics	fibroblast growth factor receptor 3 inhibitor	Renal cell carcinoma	PO	Filed NDA	12/21/2022	Yes	No
Zynquista	sotagliflozin	Lexicon	sodium-dependent glucose transporter 1 (SGLT-1) and SGLT-2 inhibitor	Diabetes mellitus	PO	Filed NDA	12/30/2022	No	No
RG-7828	mosunetuzumab	Roche	anti-CD20/CD3 monoclonal antibody	Follicular lymphoma	IV/SC	InTrial	4Q2022	Yes	Yes
VBP-15	vamorolone	Santhera	corticosteroid	Duchenne muscular dystrophy	PO	InTrial	4Q2022	Yes	Yes
Roctavian	valoctocogene roxaparvovec	BioMarin	gene therapy	Hemophilia A	IV	CRL	4Q2022	Yes	Yes
CDZ-173	leniolisib	Pharming/ Novartis	phosphatidylinositol-3-4-5-trisphosphate inhibitor	Primary immunodeficiencies	PO	InTrial	4Q2022	Yes	Yes
IMGN-853 (M-9346A-sulfo-SPDB-DM4)	mirvetuximab soravtansine	ImmunoGen	folate receptor-1 antagonist	Ovarian cancer	IV	InTrial	4Q2022	Yes	Yes
PRO-140	leronlimab	CytoDyn	C-C chemokine receptor 5 antagonist	HIV	SC	InTrial	4Q2022	Yes	No
pivmecillinam	pivmecillinam	Utility Therapeutics	amidinopenicillin	Urinary tract infections	PO	InTrial	4Q2022	No	No
SYD-985	[vic-] trastuzumab duocarmazine	Synthon	HER2-targeting antibody-drug conjugate	Breast cancer	IV	InTrial	4Q2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
MOR-202	felzartamab	MorphoSys/ I-Mab Biopharma	anti-CD38 monoclonal antibody	Multiple myeloma	IV	InTrial	4Q2022	Yes	No
TAK-609	idursulfase-IT	Takeda	enzyme replacement	Hunter syndrome	Intrathecal	InTrial	4Q2022	Yes	Yes
131I-8H9	omburtamab	Y-mAbs Therapeutics	B7-H3 antagonist	Brain cancer	Intrathecal	InTrial	4Q2022	Yes	Yes
RTA-408	omaveloxolone	Reata Pharmaceuticals	Nrf2 activator	Friedreich's ataxia	PO	InTrial	4Q2022	Yes	Yes
Ovastat	treosulfan	Medexus Pharmaceuticals	alkylating agent	Hematopoietic stem cell transplantation	IV	CRL	4Q2022	Yes	Yes
NVX-CoV2373	coronavirus vaccine	Novavax	vaccine	Novel coronavirus disease 2019 (COVID-19)	IM	InTrial	2H2022	No	No
Doria	risperidone	Laboratorios Farmacéuticos Rovi	atypical antipsychotic	Schizophrenia	IM	CRL	2H2022	Yes	No
Adstiladrin	nadofaragene firadenovec	FerGene	gene therapy	Bladder cancer	Intravesical	CRL	2H2022	Yes	No
ET-105	lamotrigine	Eton	anticonvulsant	Epilepsy	PO	CRL	2H2022	No	No
CS-1001	sugemalimab	EQRx/ CStone Pharmaceuticals	anti-PD-L1 antibody	Non-small cell lung cancer	IV	InTrial	2H2022	Yes	No
DCR-PHXC	nedosiran	Dicerna/ Alnylam	glycolate oxidase antagonist	hyperoxaluria	SC	InTrial	2H2022	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Qtrypta	zolmitriptan	Zosano	triptans	Acute migraines	TOP	CRL	2H2022	No	No
CAM-2038	buprenorphine	Braeburn	opioid receptor agonist (partial)	Opioid use disorder	SC	CRL	2H2022	Yes	No
SPI-2012	eflapegrastim	Spectrum	granulocyte colony-stimulating factor	Chemotherapy-induced neutropenia	SC	CRL	2H2022	Yes	No
Pedmark (STS)	sodium thiosulfate	Fennec	reducing agent	Ototoxicity	IV	CRL	2H2022	Yes	Yes
ET-104	zonisamide	Eton	anticonvulsant	Seizures	PO	CRL	2022	No	No
RT-002 (Daxi)	daxibotulinumtoxinA	Revance Therapeutics	botulinum toxins	Glabellar lines (frown lines)	IM	CRL	2022	Yes	No
AXS-05	dextromethorphan/ bupropion	Axsome	N-methyl-D-aspartate antagonist/ antidepressant	Major depressive disorder	PO	Filed NDA	2022	No	No
OPNT-003	nalmefene	Opiant	opioid receptor antagonist	Opioid overdose	Intranasal	InTrial	Late 2022	No	No
ERY-ASP (ERY-001)	L-asparaginase (eryaspase)	Erytech	L-asparaginase	Acute lymphoblastic leukemia	IV	InTrial	Late 2022	Yes	Yes
TAK-003	Dengue fever vaccine	Takeda	vaccine	Dengue fever	SC	InTrial	Late 2022	Yes	No
obeticholic acid	obeticholic acid	Intercept Pharmaceuticals	farnesoid X receptor agonist	Nonalcoholic steatohepatitis	PO	CRL	Late 2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
NuThrax	anthrax vaccine adsorbed/ CPG-7909	Emergent Biosolutions	vaccine/ oligodeoxynucleotide	Anthrax	IM	InTrial	Late 2022	Yes	No
PS-433540 (RE-021; DARA)	sparsentan	Travere Therapeutics	dual-acting angiotensin/endothelin receptor antagonist	IgA nephropathy; focal segmental glomerulosclerosis	PO	InTrial	Late 2022	No	Yes
PRX-102	pegunigalsidase alfa	Protalix	enzyme replacement	Fabry disease	IV	CRL	Late 2022	Yes	No
KB-103	beremagene geperpavec	Krystal Biotech	gene therapy	Epidermolysis bullosa	Topical	InTrial	Late 2022	Yes	Yes
PTX-022	rapamycin	Palvella Therapeutics	mTOR kinase inhibitor	Pachyonychia congenita	TOP	InTrial	Late 2022	No	Yes
LN-145	LN-145	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Cervical Cancer	IV	InTrial	Late 2022	Yes	No
2023 Possible launch dates									
pegcetacoplan (intravitreal)	pegcetacoplan	Apellis	C3 inhibitor	Geographic atrophy	Intravitreal	InTrial	1Q2023	Yes	No
BAN-2401	lecanemab	Eisai/ BioArctic	beta-amyloid monoclonal antibody	Alzheimer's disease	IV	InTrial	1Q2023	Yes	No
PTC-AADC	eladocagene exuparvovec	PTC Therapeutics	gene therapy	Aromatic L-amino acid decarboxylase deficiency	Intracerebral	InTrial	1Q2023	Yes	Yes
EBV-CTL (ATA-129)	tabelecleucel	Atara Biotherapeutics	cell therapy	Lymphoproliferative disorder	IV	InTrial	1Q2023	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
NiCord	omidubicel	Gamida	cellular therapy	Hematological cancers	IV	InTrial	1Q2023	Yes	Yes
BHV-3500	vazegepant	Biohaven	calcitonin gene-related peptide receptor antagonist	Migraine	Intranasal	InTrial	1Q2023	No	No
pIL-12 (DNA IL-12)	tavokinogene telsaplasmid	OncoSec Medical	gene therapy	Melanoma	Intratumoral	InTrial	1Q2023	Yes	Yes
LY-686017	tradipitant	Vanda Pharmaceuticals	neurokinin 1 receptor antagonist	Motion sickness/ gastroparesis	PO	InTrial	1Q2023	No	No
GS-010	GS-010	GenSight Biologics	gene therapy	Optic neuropathy	Intraocular	InTrial	1Q2023	Yes	Yes
ONS-5010	bevacizumab-vikg	Outlook Therapeutics	anti-VEGF antibody	Wet age-related macular degeneration	Intravitreal	InTrial	1Q2023	Yes	No
NNZ-2566	trofinetide	Neuren	insulin-like growth factor 1 derivative	Rett syndrome	IV/PO	InTrial	1Q2023	Yes	Yes
AEB-1102	pegzilarginase	Aeglea BioTherapeutics	enzyme replacement/ arginase-I stimulator	Arginase 1 deficiency	IV	InTrial	1Q2023	Yes	Yes
LY-3527727	pirtobrutinib	Eli Lilly	Bruton's tyrosine kinase inhibitor	Mantle cell lymphoma	PO	InTrial	1Q2023	Yes	No
LN-144	lifileucel	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Melanoma	IV	InTrial	1Q2023	Yes	Yes
SGX-301	synthetic hypericin	Soligenix	synthetic hypericin	Cutaneous T-cell lymphoma	TOP	InTrial	1Q2023	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
CUTX-101	copper histidinate	Fortress Biotech	copper replacement	Menkes Disease	SC	InTrial	1Q2023	Yes	Yes
BIVV-001	efanesoctocog alfa	Sanofi	recombinant Factor VIII	Hemophilia A	IV	InTrial	1Q2023	Yes	Yes
ADAIR	dextroamphetamine	Vallon Pharmaceuticals	CNS stimulant	Attention deficit hyperactivity disorder	PO	InTrial	2Q2023	No	No
CYT-387	momelotinib	Sierra Oncology	janus kinase inhibitor	Myeloproliferative disorders	PO	InTrial	2Q2023	Yes	Yes
R-667 (RG-667)	palovarotene	Ipsen	selective retinoic acid receptor agonist	Fibrodysplasia ossificans progressiva	PO	InTrial	1H2023	Yes	Yes
RG-6171	giredestrant	Roche	selective estrogen receptor degrader	Breast cancer	PO	InTrial	1H2023	Yes	No
arimoclomol	arimoclomol	Orphazyme	cytoprotectives	Niemann-Pick disease	PO	CRL	1H2023	Yes	Yes
RAD-1901	elacestrant	Radius Health	selective estrogen receptor down-regulator	Breast cancer	PO	InTrial	1H2023	Yes	No
GSK-1278863	daprodustat	GlaxoSmithKline	hypoxia-inducible factor-prolyl hydroxylase inhibitor	Anemia	PO	InTrial	1H2023	Yes	No
ABBV-951	levodopa/carbidopa	AbbVie	aromatic amino acid/aromatic amino acid decarboxylation inhibitor	Parkinson's disease	SC	InTrial	1H2023	Yes	No
NOV-03	perfluorohexyloctane	Bausch/ Novaliq	tear film stabilizer	Dry eye disease	OPH	InTrial	1H2023	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
MIN-101	roluperidone	Minerva Neurosciences	sigma-2 and 5HT-2A receptor antagonist	Schizophrenia	PO	InTrial	1H2023	Yes	No
BL-8040 (BKT-140)	motixafortide	BioLineRx	selective chemokine receptor 4 inverse agonist	Stem cell transplant	SC	InTrial	1H2023	Yes	Yes
Aripiprazole 2-month	aripiprazole	Lundbeck/ Otsuka Pharmaceutical	atypical antipsychotic	Schizophrenia/ bipolar disorder	IM	InTrial	1H2023	No	No
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	CRL	1H2023	Yes	Yes
LY-3074828	mirikizumab	Eli Lilly	IL-23 inhibitor	Ulcerative colitis	SC	InTrial	1H2023	Yes	No
MEDI-8897 (RSV MAbs)	nirsevimab	Sanofi	anti-RSV monoclonal antibody D25	Respiratory synctial virus	Undisclosed	InTrial	1H2023	Yes	No
CK-301	cosibelimab	Checkpoint Therapeutic	anti programmed cell death ligand 1	Cutaneous squamous cell carcinoma	IV	InTrial	1H2023	Yes	No
AMT-061	etranacogene dezaparvovec	CSL Behring/ uniQure	gene therapy	Hemophilia B	IV	InTrial	1H2023	Yes	Yes
ISO-901	modufolin	Isofol Medical	reduced folate	Colorectal cancer	IV	InTrial	1H2023	Yes	No
RG-7433 (ABT-263)	navitoclax	AbbVie	Bcl-2 inhibitor	Myelofibrosis	PO	InTrial	1H2023	Yes	Yes
Iomab-B	iodine I 131 monoclonal antibody BC8	Actinium	anti-CD45 monoclonal antibody	Acute myeloid leukemia/ Myelodysplastic syndrome	IV	InTrial	1H2023	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
RG-7440 (GDC-0068)	ipatasertib	Roche	pan-Akt inhibitor	Prostate cancer	PO	InTrial	1H2023	Yes	No
ADCT-301	camidanlumab tesirine	ADC Therapeutics/ Genmab	antibody drug conjugate	Hodgkin's Lymphoma	IV	InTrial	1H2023	Yes	No
NS-2 (ALDX-1E1, ALDX-1E2, ADX-102)	reproxalap	Aldeyra Therapeutics	aldehyde antagonist	Dry eyes	OP	InTrial	Mid-2023	No	No
RA-101495	zilucoplan	UCB	complement inhibitor	Myasthenia gravis	SC	InTrial	Mid-2023	Yes	Yes
SAR-439859	amcenestrant	Sanofi	selective estrogen receptor degrader	Breast cancer	PO	InTrial	Mid-2023	Yes	No
ATI-1501	metronidazole	Saptalis	nitroimidazole	Fungal infections, anaerobic bacterial infections	PO	InTrial	Mid-2023	No	No
CyclASol	cyclosporine	Novaliq	immunosuppressant	Dry eyes	OPH	InTrial	Mid-2023	No	No
UCB-7665	rozanolixizumab	UCB	neonatal Fc receptor inhibitor	Myasthenia gravis	SC/IV	InTrial	Mid-2023	Yes	No
SER-109	SER-109	Seres Therapeutics	ecobiotic agent	Clostridium difficile infection	PO	InTrial	Mid-2023	No	Yes
MT-1621	deoxythymidine/ deoxycytidine	Zogenix	deoxynucleoside	Thymidine kinase 2 deficiency	PO	InTrial	Mid-2023	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
LY-3002813	donanemab	Eli Lilly	beta-amyloid monoclonal antibody	Alzheimer's disease	IV	InTrial	Mid-2023	Yes	No
RG-6026	glofitamab	Roche	anti-CD20/CD3 T cell monoclonal antibody	Diffuse large B cell lymphoma	IV	InTrial	Mid-2023	Yes	No
PB-2452	bentracimab	PhaseBio	antiplatelet monoclonal antibody	Antiplatelet drug toxicity	IV	InTrial	Mid-2023	No	No
PT-027	budesonide/albuterol	AstraZeneca	Glucocorticoid/beta agonist	Asthma	Inh	InTrial	Mid-2023	No	No
IPX-203	carbidopa/ levodopa	Amneal	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	PO	InTrial	Mid-2023	No	No
OTL-200 (GSK-2696274)	OTL-200 (GSK-2696274)	Orchard Therapeutics	gene therapy	Leukodystrophy	IV	InTrial	Mid-2023	Yes	Yes
OTL-103 (GSK-2696275)	OTL-103 (GSK-2696275)	Orchard Therapeutics	gene therapy	Wiskott-Aldrich syndrome	IV	InTrial	Mid-2023	Yes	Yes
CD-101	rezafungin	Cidara Therapeutics	echinocandin	Fungal infections	IV	InTrial	Mid-2023	No	Yes
BBI-4000	sofpironium bromide	Brickell	anticholinergic	Hyperhidrosis	TOP	InTrial	Mid-2023	No	No
SB-206	SB-206	Novan Therapeutics	nitric oxide-releasing compound	Molluscum contagiosum	TOP	InTrial	Mid-2023	No	No
Melblez Kit	melphalan	Delcath	phenylalanine mustard	Hepatocellular cancer (liver)/ Biliary tract cancer/ Melanoma	INJ	InTrial	Mid-2023	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ETX-2514 (SOL-DUR)	ETX-2514	Entasis Therapeutics	broad-spectrum β-lactamase inhibitor with beta-lactam antimicrobial	Bacterial infections	IV	InTrial	Mid-2023	Yes	No
ALXN-1840 (WTX-101)	bis-choline tetrathiomolybdate (TTM)	AstraZeneca/ Alexion	chelating agent	Wilson's disease	PO	InTrial	Mid-2023	Yes	Yes
TransCon PTH	palopegteriparatide	Ascendis Pharma	parathyroid hormone	Hypoparathyroidism	SC	InTrial	3Q2023	Yes	Yes
ALPHA-1062	galantamine prodrug	Alpha Cognition	acetylcholinesterase inhibitor	Alzheimer's disease	PO	InTrial	3Q2023	No	No
TRC-101	veverimer	Tricida	carrier protein modulator	Chronic kidney disease	PO	CRL	4Q2023	Yes	No
ESN-364	fezolinetant	Astellas	NK3 receptor antagonist	Menopause	PO	InTrial	4Q2023	No	No
X4P-001 (X-4P-001, X4-136, X4P-001-RD)	mavorixafor	X4 Pharma	CXC receptor type 4 inhibitor	WHIM syndrome	PO	InTrial	4Q2023	Yes	Yes
FMXIN-001	naloxone	Nasus Pharma	opioid antagonist	Opioid overdose	Intranasal	InTrial	4Q2023	No	No
STS-101	dihydroergotamine	Satsuma Pharmaceuticals	ergotamine	Migraine	Intranasal	InTrial	4Q2023	No	No
SPI-014	lanthanum dioxycarbonate	Unicycive	Phosphate binder	Hyperphosphatemia	PO	InTrial	4Q2023	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
APD-334	etrasimod	Pfizer/ Arena Pharmaceuticals	S1P1 receptor agonist	Ulcerative colitis	PO	InTrial	2H2023	Yes	No
Entyvio (SC formulation)	vedolizumab	Takeda	integrin receptor antagonist	Ulcerative colitis	SC	CRL	2H2023	Yes	No
GSK-2140944	gepotidacin	GlaxoSmithKline	bacterial Type II topoisomerase inhibitor	Bacterial infections	PO/IV	InTrial	2H2023	No	No
CTX-001	autologous CRISPR-Cas9 modified CD34+ human hematopoietic stem and progenitor cells	CRISPR Therapeutics/ Vertex	gene therapy	Beta-thalassemia; sickle cell anemia	IV	InTrial	2H2023	Yes	Yes
K-127	pyridostigmine	Amneal	Cholinesterase inhibitor	Myasthenia gravis	PO	InTrial	2H2023	No	No
MT-7117	MT-7117	Mitsubishi Tanabe Pharma	Undisclosed	Erythropoietic protoporphyria	PO	InTrial	2H2023	Yes	No
REGN-5458	REGN-5458	Regeneron	BCMA and CD3 bispecific antibody inhibitor	Multiple myeloma	IV	InTrial	2H2023	No	No
GEN-3013	epcoritamab	AbbVie	CD3/CD20 monoclonal antibody	Diffuse large B-cell lymphoma	SC	InTrial	2H2023	Yes	No
REGN-3918	pozelimab	Regeneron	C5a receptor inhibitor	Paroxymal nocturnal hemoglobinuria	IV, SC	InTrial	2H2023	Yes	Yes
RG-1450	gantenerumab	Roche	beta-amyloid monoclonal antibody	Alzheimer's disease	SC	InTrial	2H2023	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
TP-03	lotilaner	Tarsus Pharmaceuticals	antagonist of insect and arachnid GABA-Cl channels	Demodex blepharitis	TOP	InTrial	2H2023	No	No
ADV-7103	tripotassium citrate monohydrate/ potassium hydrogen carbonate	Advicenne	potassium	Distal rental tubular acidosis	PO	InTrial	2H2023	Yes	No
Tonmya	cyclobenzaprine	Tonix	muscle relaxant	Fibromyalgia	PO	InTrial	2H2023	No	No
SMT-19969	ridinilazole	Summit Therapeutics	bibenzimidazole analog	Clostridium difficile infection	PO	InTrial	2H2023	No	No
ADP-A2M4 (MAGE-A4)	ADP-A2M4 (MAGE-A4)	Adaptimmune	SPEAR T-cell therapy	Sarcoma	IV	InTrial	2H2023	Yes	Yes
OX-124	naloxone	Orexo	opioid antagonist	Opioid overdose	Intranasal	InTrial	2H2023	No	No
IMGN-632	IMGN-632	ImmunoGen	anti-CD123 antibody-drug conjugate	Blastic plasmacytoid dendritic cell neoplasm	IV	InTrial	2H2023	Yes	No
RP-L102 (RPL-102)	RP-L102	Rocket Pharmaceuticals	gene therapy	Fanconi anemia	IV	InTrial	2H2023	Yes	Yes
MBG-453	sabatolimab	Novartis	anti-TIM-3	Myelodysplastic syndrome	IV	InTrial	2H2023	Yes	No
IDP-126	IDP-126	Bausch Health	retinoid/ antibiotic	Acne	TOP	InTrial	2H2023	No	No
glatiramer acetate depot	glatiramer acetate long-acting	Mylan/ Mapi Pharma	immunosuppressant	Multiple sclerosis	IM	InTrial	2H2023	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
SAR-408701	SAR-408701	Sanofi	antibody-drug conjugate	Non-small cell lung cancer	IV	InTrial	2H2023	Yes	No
CNM-Au8	CNM-Au8	Clene	gold nanocrystal	Amyotrophic lateral sclerosis	PO	InTrial	2H2023	Yes	Yes
Mino-Lok	minocycline-EDTA-ETOH	Citrus	tetracyclines	Bacterial infection	Intracatheter	InTrial	2H2023	No	No
SAGE-217	allopregnanolone	SAGE	gamma aminobutyric acid-A receptor allosteric modulator	Major depressive disorder/ Postpartum depression	PO	InTrial	2H2023	No	No
AKCEA-TTR-LRx	eplontersen	AstraZeneca/ Ionis	antisense oligonucleotide	Hereditary transthyretin-mediated amyloid polyneuropathy	SC	InTrial	2H2023	Yes	No
QGE-031	ligelizumab	Novartis	anti-IgE antibody	Chronic spontaneous urticaria	SC	InTrial	2H2023	Yes	No
iDose travoprost	travoprost	Glaukos Corporation	prostaglandin analog	Glaucoma/ Ocular hypertension	Intraocular	InTrial	2H2023	No	No
I/Ontak	denileukin diftitox	Citius	CD25-directed cytotoxin	Cutaneous T-cell lymphoma	IV	InTrial	2H2023	Yes	Yes
RG-6058	tiragolumab	Roche	TIGIT monoclonal antibody	Small cell lung cancer	IV	InTrial	2H2023	Yes	No
MILR-1444A	lebrikizumab	Eli Lilly	interleukin-13 inhibitor	Atopic dermatitis	SC	InTrial	2H2023	Yes	Yes
iMAB-362	zolbetuximab	Astellas	GC182 monoclonal antibody	Gastric adenocarcinoma	IV	InTrial	2023	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
AAI-101	cefepime/ enmetazobactam	Advanz/ Allecra	beta-lactam/b-lactamase inhibitor	Urinary tract infection	IV	InTrial	2023	No	No
P-2B001 (P2-B001, P2B-001, P2B001)	pramipexole/ rasagiline	Pharma Two B	dopamine agonist/ monoamine oxidase B inhibitor	Parkinson's disease	PO	InTrial	2023	No	No
DE-117	omidenepag isopropyl	Santen Pharmaceutical/ Ube Industries	prostaglandin E receptor 2 agonist	Glaucoma	OPH	CRL	2023	No	No
GLPG-0634	filgotinib	Gilead/ Galapagos	janus kinase-1 inhibitor	Ulcerative colitis	PO	CRL	2023	Yes	No
magrolimab	magrolimab	Gilead	CD47 monoclonal antibody	Myelodysplastic syndrome	IV	InTrial	2023	Yes	Yes
ALT-803	nogapendekin alfa inbakicept	ImmunityBio	interleukin-15 (IL-15) super agonist/ IL-15R alpha-Fc fusion complex	Bladder cancer	Intravesical	InTrial	2023	Yes	No
KN-046	KN-046	Alphamab Oncology	PD-L1/CTLA-4 bispecific monoclonal antibody	Thymic cancer	IV	InTrial	2023	Yes	Yes
RG-6171	giredestrant	Roche	steroidal selective estrogen receptor degrader	Breast cancer	PO	InTrial	2023	Yes	No
VLA-1553	VLA-1553	Valneva	vaccine	Chikungunya virus	IM	InTrial	2023	No	No
PAX-101	suramin	PaxMedica	unknown	trypanosomiasis	IV	InTrial	2023	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
FT-2102	olutasidenib	Forma Therapeutics	dehydrogenase 1 inhibitor	Acute myeloid leukemia	PO	InTrial	2023	Yes	Yes
IDP-120	tretinoin/ benzoyl peroxide	Bausch	retinoid	Acne	TOP	InTrial	2023	No	No
resminostat	resminostat	4SC AG	pan histone deacetylase inhibitor	Mycosis fungoides/ Sézary syndrome	PO	InTrial	2023	Yes	No
dronabinol XL AdVersa	dronabinol controlled-release	Tetra Bio-Pharma	cannabinoid receptor agonist	Nausea and vomiting	Buccal	InTrial	2023	No	No
SDP-037, SDN-037	difluprednate	Sun Pharma Advanced Research Company (SPARC)	Corticosteroid	Ocular inflammation/pain	OP	InTrial	2023	No	No
REGN-475 (SAR-164877)	fasinumab	Regeneron	selective anti-nerve growth factor monoclonal antibody	Osteoarthritis	SC	InTrial	2023	Yes	No
R-1658 (RG-1658, JTT-705, RO-4607381)	dalcetrapib	DalCor	cholesteryl ester transfer protein inhibitor	Acute coronary syndrome	PO	InTrial	2023	Yes	No
NX-1207 (NYM-4805, REC 0482)	fexapotide triflutate	Nymox	pro-apoptotic	Benign prostatic hyperplasia	Intratumoral	InTrial	2023	Yes	No
scCeftriaxone	ceftriaxone	scPharmaceuticals	Penicillin binding protein inhibitor	Bacterial infections	SC	InTrial	2023	No	No
Betalutin	177 lu-dota-tetulomab (177 lu-tetraxetan-tetulomab, 177 lu-	Nordic Nanovector	anti-CD37 antibody radionuclide	Non-Hodgkin lymphoma	IV	InTrial	2023	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
	lilotomab satetraxetan, 177 lu-DOTA-HH1)								
AGEN-1884	zalifrelimab	Agenus	immune checkpoint modulator antibody	Cervical cancer	IV	InTrial	2023	Yes	No
SB-525	girocotocogene fitelparvovec	Pfizer/ Sangamo Therapeutics	gene therapy	Hemophilia A	IV	InTrial	Late 2023	Yes	Yes
PSD-502	lidocaine/ prilocaine	Plethora/ Recordati	sodium channel blocker	Premature ejaculation	TOP	InTrial	Late 2023	No	No
PPP-001	delta-9-tetrahydrocannabinol/ cannabidiol	Tetra Bio-Pharma	cannabinoid product	Pain	INH	InTrial	Late 2023	Yes	Yes
CSL-112 (reconstituted HDL, rHDL)	CSL-112 (reconstituted HDL, rHDL)	CSL Limited	plasma-derived apolipoprotein A-I	Myocardial infarction	IV	InTrial	Late 2023	Yes	No
PF-06838435 (SPK-9001)	fidanacogene elaparvovec	Pfizer/ Spark Therapeutics	gene therapy	Hemophilia B	IV	InTrial	Late 2023	Yes	Yes
AG-10 (AG10)	acoramidis	BridgeBio	tetrameric transthyretin stabilizer	Transthyretin amyloid cardiomyopathy	PO	InTrial	Late 2023	Yes	No
RG-6107	crovalimab	Roche	C5 inhibitor	Paroxysmal nocturnal hemoglobinuria	IV/SC	InTrial	Late 2023	Yes	Yes
KSI-301	KSI-301	Kodiak Sciences	vascular endothelial growth factor inhibitor	Wet age-related macular degeneration; retinal vein	Intravitreal	InTrial	Late 2023	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
				occlusion; diabetic macular edema					

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

Key pending indication forecast

OptumRx Key Pending Indication Forecast

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Rinvoq	upadacitinib	AbbVie	janus kinase inhibitor	Ankylosing spondylitis	Treatment of adult patients with active ankylosing spondylitis	PO	1Q 2022
Rinvoq	upadacitinib	AbbVie	janus kinase inhibitor	Ulcerative colitis	Treatment of adults with moderately to severely active ulcerative colitis	PO	3/16/2022
Imcivree	setmelanotide	Rhythm Pharmaceuticals	MC4R agonist	Bardet-Biedl syndrome/ Alström syndrome	Treatment of obesity and control of hunger in adult and pediatric patients 6 years of age and older with Bardet-Biedl syndrome or Alström syndrome	SC	3/16/2022
Fintepla	fenfluramine	Zogenix	serotonin receptor agonist	Lennox Gastaut Syndrome	Adjunctive treatment for seizures in adults and children with Lennox Gastaut Syndrome	PO	3/25/2022
Ukoniq	umbralisib	TG Therapeutics	phosphoinositide-3 kinase delta inhibitor	Chronic lymphocytic leukemia and small lymphocytic lymphoma	Treatment for patients with chronic lymphocytic leukemia and small lymphocytic lymphoma	IV	3/26/2022
Reblozyl	luspatercept-aamt	Bristol Myers Squibb	erythroid maturation agent	Non-transfusion dependent beta thalassemia	Treatment of anemia in adults with non-transfusion dependent beta thalassemia	SC	3/27/2022

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Keytruda	pembrolizumab	Merck	programmed death receptor-1-blocking antibody	Endometrial carcinoma	Treatment of patients with advanced endometrial carcinoma that is microsatellite instability-high or mismatch repair deficient, who have disease progression following prior systemic therapy in any setting and are not candidates for curative surgery or radiation	IV	3/28/2022
Jardiance	empagliflozin	Boehringer Ingelheim/ Eli Lilly	sodium-dependent glucose transporter 2 inhibitor	Heart failure	Treatment of people with chronic heart failure in patients with preserved ejection fraction	PO	3/30/2022
Lynparza	olaparib	AstraZeneca	poly (ADP-ribose) polymerase inhibitor	Breast cancer	Adjuvant treatment of patients with BRCA-mutated, human epidermal growth factor receptor 2 (HER2)-negative high-risk early breast cancer who have already been treated with chemotherapy either before or after surgery.	PO	3/31/2022
Yescarta	axicabtagene ciloleucel	Kite/ Gilead	CAR T cell therapy	Large B-cell lymphoma	Treatment of adults with relapsed or refractory large B-cell lymphoma in the second-line setting	IV	4/1/2022
Vaxneuvance	pneumococcal 15-valent conjugate	Merck	vaccine	Pneumococcal disease	Prevention of invasive pneumococcal disease in children 6 weeks through 17 years of age	IM	4/1/2022
Opzelura	ruxolitinib	Incyte	janus kinase inhibitor	Vitiligo	Treatment of adolescent and adult patients with vitiligo (age ≥12 years)	TOP	4/18/2022
Aliqopa	copanlisib	Bayer	kinase inhibitor	B-cell non-Hodgkin's Lymphoma	In combination with rituximab, for treatment of patients with relapsed indolent B-cell non-Hodgkin's Lymphoma	IV	4/21/2022
Kymriah	tisagenlecleucel	Novartis	CAR T cell therapy	Follicular lymphoma	Treatment of adult patients with relapsed or refractory follicular lymphoma after two prior lines of treatment	IV	4/27/2022

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Qelbree	viloxazine	Supernus	selective norepinephrine reuptake inhibitor	Attention deficit hyperactivity disorder (adults)	Treatment of adults with attention deficit hyperactivity disorder	PO	4/29/2022
Fasenra	benralizumab	AstraZeneca	interleukin-5 receptor alpha inhibitor	Nasal polyposis	Treatment of nasal polyposis	SC	4/30/2022
Myfembree	relugolix/ estradiol/ norethindrone acetate	Myovant	gonadotropin-releasing hormone receptor antagonist/ estrogen/ progestin	Endometriosis	Management of moderate to severe pain associated with endometriosis	PO	5/7/2022
Olumiant	baricitinib	Eli Lilly	janus associated kinase 1/2 inhibitor	COVID-19	Treatment of certain hospitalized patients with COVID-19	PO	5/13/2022
Enhertu	trastuzumab deruxtecan	AstraZeneca/ Daiichi Sankyo	HER2-directed antibody and topoisomerase inhibitor conjugate	Breast cancer	Treatment of adult patients with unresectable or metastatic HER2-positive breast cancer who have received a prior anti-HER2-based regimen	IV	5/17/2022
Opdivo	nivolumab	Bristol Myers Squibb	programmed death receptor-1-blocking antibody	Esophageal squamous cell carcinoma	In combination with Yervoy (ipilimumab) and Opdivo in combination with fluoropyrimidine- and platinum-containing chemotherapy, for first-line treatments for adult patients with unresectable advanced, recurrent or metastatic esophageal squamous cell carcinoma	IV	5/28/2022
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 inhibitor	Atopic dermatitis (> 6 months)	Treatment of atopic dermatitis (patients 6 months to 5 years old)	SC	6/9/2022
Beovu	brolucizumab	Novartis	anti-VEGF antibody	Diabetic macular edema	Treatment of diabetic macular edema	Intravitreal	6/13/2022

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Oxlumo	lumasiran	Alnylam	HAO1-directed small interfering ribonucleic acid	Advanced primary hyperoxaluria type 1	For the reduction of plasma oxalate in the treatment of patients with advanced primary hyperoxaluria type 1	SC	6/14/2022
Ultomiris	ravulizumab-cwvz	AstraZeneca	C5 compliment inhibitor	Generalized myasthenia gravis	Treatment of adults with generalized myasthenia gravis	IV	6/29/2022
Skyrizi	risankizumab-rzaa	AbbVie	interleukin-23 antagonist	Crohn's disease	Treatment of patients 16 years and older with moderate to severe Crohn's disease	SC	7/20/2022
Evrysdi	risdiplam	Genentech	survival of motor neuron 2 splicing modifier	Spinal muscular atrophy	Treatment of pre-symptomatic pediatric patients under two months of age with spinal muscular atrophy	PO	7/25/2022
Stelara	ustekinumab	Janssen	human interleukin-12 and -23 antagonist	Juvenile psoriatic arthritis (5 years and older)	Treatment of pediatric patients ages 5 years and older with juvenile psoriatic arthritis	SC/IV	8/8/2022
Libtayo	cemiplimab-rwlc	Regeneron Pharmaceuticals	programmed death receptor-1 blocking antibody	Non-small cell lung cancer	In combination with chemotherapy as first-line treatment in advanced non-small cell lung cancer	IV	9/19/2022
Rinvoq	upadacitinib	AbbVie	janus kinase inhibitor	Non-radiographic axial spondyloarthritis	Treatment of non-radiographic axial spondyloarthritis	PO	11/7/2022
Krystexxa	pegloticase	Horizon Therapeutics	PEGylated uric acid specific enzyme	Gout (in combination with methotrexate)	In combination with methotrexate, for the treatment of chronic gout in adult patients	IV	11/10/2022

IM = intramuscular, INH = inhaled, IV = intravenous, OPH = ophthalmic, PO = oral, SC = subcutaneous, TOP = topical

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