Optum Rx®

RxOutlook®

2nd Quarter 2022

In this edition of RxOutlook, we highlight 9 key pipeline drugs with an expected FDA decision in the third quarter of 2022. Two of these drugs are novel therapies for plaque psoriasis. Topical roflumilast would potentially be the first topical phosphodiesterase 4 (PDE4) inhibitor for the condition and would offer an alternative or add-on treatment option to existing generic topicals (corticosteroids and vitamin D analogs). Deucravacitinib is a first-in-class oral tyrosine kinase 2 (TYK2) inhibitor and primarily a competitor to Otezla® (apremilast). The drug pipeline for plaque psoriasis is worth watching as there are four novel therapies, across different routes of administrations and mechanisms, with potential approval by the end of this year.

Bluebird bio is expecting an FDA decision in the third quarter for two novel gene therapies, betibeglogene autotemcel for transfusion-dependent beta thalassemia and elivaldogene autotemcel for cerebral adrenoleukodystrophy (CALD). Both products target ultra-rare orphan conditions with high unmet need, particularly CALD, a condition that has a high mortality rate in the absence of hematopoietic stem cell transplantation. The FDA has scheduled an FDA Advisory Committee to review the data for both gene therapies on June 9 and 10, 2022. The cost for each could exceed over $2 million for a one-time dose.

In addition to the gene therapies and consistent with the recurring pipeline trend, several other notable orphan drugs are expected in the third quarter. This includes olipudase alfa, an enzyme replacement therapy, which would potentially be the first approved treatment for acid sphingomyelinase deficiency and bulevirtide, potentially the first antiviral therapy approved for chronic hepatitis delta virus.

Rounding out the list of drugs included in the report are teclistamab for multiple myeloma, linzagolix for uterine fibroids, and ublituximab for multiple sclerosis. Each of these are entering relatively competitive markets but each product would add to the treatment armamentarium for their respective disease states.

Approval decisions for other key novel therapies are expected in the third quarter of 2022 but are not reviewed in this report because they were covered in previous editions of RxOutlook. These include: vutrisiran for hereditary transthyretin-mediated amyloidosis; narsoplimab for hematopoietic stem cell transplant-associated thrombotic microangiopathy; and teplizumab for delay of type 1 diabetes mellitus.

Key pipeline drugs with FDA approval decisions expected by end of the 3rd quarter 2022

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Olipudase alfa	Sanofi	Acid sphingomyelinase deficiency*	7/3/2022
Bulevirtide	Gilead	Chronic hepatitis delta virus*	3Q 2022
Roflumilast	Arcutis Biotherapeutics	Plaque psoriasis	7/29/2022
Zynteglo (betibeglogene autotemcel)	bluebird bio	Beta thalassemia*	8/19/2022
Teclistamab	J&J/Janssen	Multiple myeloma*	8/29/2022
Deucravacitinib	Bristol Myers Squibb	Plaque psoriasis	9/10/2022

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Linzagolix	ObsEva	Uterine fibroids	9/13/2022
Skysona (elivaldogene autotemcel)	bluebird bio	Cerebral adrenoleukodystrophy*	9/16/2022
Ublituximab	TG Therapeutics	Multiple sclerosis	9/28/2022

* 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 3rd 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 3rd 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 Drug Insights

Olipudase alfa (Brand Name: To be determined)

Manufacturer: Sanofi
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: July 3, 2022

Therapeutic use

Olipudase alfa is in development for treatment of non-central nervous system (CNS) manifestations of acid sphingomyelinase deficiency (ASMD) in pediatric and adult patients.

Acid sphingomyelinase deficiency, also known as Niemann-Pick disease (NPD) type A and type B, is a rare progressive genetic disorder that results from a deficiency of the enzyme acid sphingomyelinase, which is required to metabolize a lipid called sphingomyelin. Due to the deficiency, sphingomyelin and other substances accumulate in various parts of the body and can interfere with organ functions.

NPD type A is the most severe form of the disease with affected patients experiencing loss of early motor skills in the first few months of life and significant loss of neurologic function eventually leading to death by 2 to 3 years of age. NPD type B is a less severe form of the disease with most patients surviving into adulthood. While milder, patients with NPD type B can also experience hepatosplenomegaly, thrombocytopenia, short stature, interstitial lung disease, hyperlipidemia, ocular and abnormalities. Neurologic abnormalities (eg, intellectual disability, psychiatric disorders, and peripheral neuropathy) can also occur.

The exact prevalence of ASMD is unknown but has been estimated at 1 in 250,000 individuals in the general population.

- Treatment of non-CNS manifestations of ASMD in pediatric and adult patients

Olipudase alfa (continued…)

Clinical profile

Olipudase alfa is an enzyme replacement therapy designed to replace deficient or defective acid sphingomyelinase, allowing for the breakdown of sphingomyelin.

Pivotal trial data:
The efficacy of olipudase alfa was evaluated in a Phase 2/3, randomized, double-blinded, placebo-controlled study in 36 adult patients with ASMD. Patients received either placebo or olipudase alfa over 52 weeks. The co-primary endpoints were improvement in lung function and spleen volume. Lung function was measured using the percent predicted diffusing capacity of carbon monoxide (DLco). For spleen volume, the FDA required that the spleen volume endpoint was further combined with a patient-reported outcome (PRO) measurement of symptoms associated with enlarged spleen called Splenomegaly Related Score (SRS). The improvement from baseline in DLco was 22% for olipudase alfa vs. 3% for placebo ($p = 0.0004$). In the olipudase alfa arm, spleen volume was reduced by 39.5% vs. 0.5% increase in the placebo arm ($p < 0.0001$). Compared to baseline, the SRS was reduced by 8.0 points in the olipudase alfa arm and 9.3 points in the placebo arm ($p = 0.70$; statistical significance not met); therefore, the combination endpoint for spleen volume improvement was not met.

Olipudase alfa was also evaluated in a Phase 1/2, single arm, open-label study in 20 pediatric patients with ASMD without acute or rapidly progressive neurological abnormalities. While the primary objective for this study was the safety and tolerability of olipudase alfa, the study also explored secondary clinical endpoints. After 52 weeks, percent predicted DLco increased by a mean of 33% in 9 patients who were able to perform the test at baseline. Spleen volume decreased by 49%.

Safety:
The most common adverse events with olipudase alfa use were headache, nasopharyngitis, upper respiratory tract infection, cough, and arthralgia.

Dosing:
In the pivotal trial, olipudase alfa was administered via intravenous (IV) infusion every two weeks.

- Enzyme replacement therapy
- IV formulation
- Improvement in lung function (percent predicted diffusing capacity of carbon monoxide): 22% with olipudase alfa vs. 3% with placebo
- Spleen volume: Reduced by 39.5% with olipudase alfa vs. 0.5% increase with placebo
- Common AEs: Headache, nasopharyngitis, upper respiratory tract infection, cough, arthralgia
- Dosing: Once every 2 weeks

Olipudase alfa (continued…)

Competitive environment

Olipudase alfa would potentially be the first approved treatment for ASMD, a condition for which there is a high unmet need. The current standard of care is limited to supportive therapy, which includes physical and occupational therapy, nutritional support, treatment for dyslipidemia, and supplemental oxygen. In addition to promising efficacy with improvements in lung function and reductions in spleen volume, olipudase was well tolerated with lower rates of serious adverse events compared to placebo. However, olipudase failed to show improvement in symptoms associated with enlarged spleen despite reductions in spleen volume.

The target population for olipudase alfa is expected to be small given the ultra-rare nature of ASMD and the fact that olipudase only treats the non-CNS related manifestations of the condition. There is a lack of robust data in pediatric patients with the most severe form of ASMD (NPD type A).

- Advantages: Potentially the first approved treatment for ASMD, high unmet need (current standard of care is supportive care), well tolerated
- Disadvantages: Failed to show improvement in symptoms associated with enlarged spleen, small target population, does not address the CNS-related symptoms of ASMD

Bulevirtide (Brand Name: To be determined)

Manufacturer: Gilead
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 3Q 2022

Therapeutic use

Bulevirtide is in development for treatment of chronic hepatitis delta virus (HDV) infection in adults with compensated liver disease.

HDV is known as a "satellite virus," because it can only infect people who are also infected by the hepatitis B virus (HBV). The infection can be acquired either simultaneously with HBV as a coinfection or they could acquire HDV after first being infected with HBV. Chronic HDV is associated with a more accelerated progression of liver disease compared to HBV alone.

The exact prevalence of HDV is the U.S. is unknown, but studies estimate the prevalence ranges from 100,000 to 150,000 individuals affected. Most cases occurring among people who migrate or travel to the U.S. from countries with high HDV endemicity.

- Treatment of chronic HDV infection in adults with compensated liver disease

Clinical profile

Bulevirtide blocks the entry of HBV and HDV into hepatocytes by binding to and inactivating sodium taurocholate cotransporting polypeptide (NTCP), a bile salt liver transporter serving as essential HBV/HDV entry receptor.

- HBV/HDV entry receptor inhibitor
- SC formulation
- Combined virological and biochemical response: 36.7% with bulevirtide 2 mg vs. 28% with bulevirtide 10 mg vs. 0% with placebo
- Common AEs: Increased bile acid salts, injection site reactions
- Dosing: Once daily

Pivotal trial data:
Bulevirtide is being evaluated in an ongoing Phase 3, open-label, randomized study in 150 patients with chronic HDV. Patients were randomized to no antiviral treatment for 48 weeks followed by bulevirtide 10 mg daily for 96 weeks (arm A), treatment with bulevirtide 2 mg daily (arm B), or with bulevirtide 10 mg daily (arm C) for 144 weeks. Primary efficacy data will be assessed at week 48. After week 48, participants in the delayed treatment group of the study will be switched to bulevirtide 10 mg once daily for an additional 96 weeks. The primary endpoint, combined response, was defined as an undetectable HDV RNA or ≥ 2 log10 IU/mL decline from baseline and alanine transaminase (ALT) normalization at week 48.

After 24 weeks, the proportion of patients achieving the combined response endpoint was 36.7% with bulevirtide 2 mg, 28% in those receiving bulevirtide 10 mg and 0% in those who did not receive antiviral treatment ($p < 0.001$ for both bulevirtide arms vs. no treatment).

Bulevirtide was also evaluated in a Phase 2b, open-label, randomized study in 60 patients with chronic HBV/HDV coinfection. Patients were randomized into four treatment groups: peginterferon alfa, bulevirtide 2 mg plus peginterferon alfa, bulevirtide 5 mg plus peginterferon alfa, or bulevirtide 2 mg, with all treatments administered for 48 weeks. The primary endpoint was undetectable serum HDV RNA at week 72. At 72 weeks, HDV RNA was undetectable in 40% of patients who were in one of the two combination treatment arms vs. no patients who were in either of the monotherapy treatment arms.

Bulevirtide (continued...)

Safety:
The most common adverse events with bulevirtide use were increased bile acid salts and injection site reactions.

Dosing:
In the pivotal trials, bulevirtide was administered via subcutaneous (SC) injection once daily.

Competitive environment

If approved, bulevirtide would be the first FDA approved treatment for HDV infection. There is a high unmet need for treatments for HDV as the current standard of care is generally limited to off-label use of interferon-based therapies (eg, Pegasys®). Interferon-based treatment is effective in approximately 40% of patients achieving undetectable HDV RNA at 24 weeks following completion of treatment; however follow-up data demonstrated that only 12% of treated patients had sustained suppression of HDV 4 years after treatment.

While the data for bulevirtide is promising, questions remain about its long-term efficacy and the optimal duration of use. Bulevirtide does not appear to be curative and very few will reach undetectable levels of HDV. The Phase 2 trial suggests a possible role for combination therapy of bulevirtide plus peginterferon alfa so bulevirtide may not eliminate the need for interferon therapy for treatment of HDV in all patients.

- Advantages: Potentially the first approved treatment for chronic HDV infection, high unmet need (current standard of care is off-label peginterferon therapy), well tolerated
- Disadvantages: Lack of robust long-term data, not curative, may still require concomitant use with peginterferon for maximum efficacy

Roflumilast (Brand Name: To be determined)

Manufacturer: Arcutis Biotherapeutics
Expected FDA decision: July 29, 2022

Therapeutic use

Topical roflumilast is in development for treatment of plaque psoriasis in adults and adolescents.

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 adults and adolescents

Clinical profile

Roflumilast is a selective phosphodiesterase (PDE4) inhibitor. PDE4 is an intracellular enzyme that increases the production of pro-inflammatory mediators and decreases production of anti-inflammatory mediators.

An oral formulation of roflumilast (Daliresp®) is currently FDA approved as a treatment to reduce the risk of chronic obstructive pulmonary disease (COPD) exacerbations in patients with severe COPD associated with chronic bronchitis and a history of exacerbations.

- PDE4 inhibitor
- Topical formulation
- IGA response: 37.5% to 42.4% vs. 6.1% to 6.9% with vehicle
- PASI75 response: 39.0% to 41.6% vs. 5.3% to 7.6% with vehicle
- Limited safety data; reported AEs were similar to vehicle control
- Dosing: Once daily

Pivotal trial data:
Topical roflumilast was evaluated in DERMIS-1 and DERMIS-2, two identical Phase 3, randomized, double-blind, vehicle-controlled studies in 881 patients ages 2 years and older with mild, moderate, or severe plaque psoriasis. Patients received roflumilast 0.3% cream or matching vehicle cream once daily for 8 weeks. The primary endpoint of the studies was Investigator Global Assessment (IGA) success at week 8. IGA success was defined as an IGA score of clear or almost clear and at least a 2-grade improvement from baseline. A key secondary endpoint was the percentage of patients who achieved Psoriasis Area and Severity Index (PASI) 75 response.

In both studies, IGA response was achieved in significantly more patients treated with roflumilast vs. vehicle (DERMIS-1: 42.4% vs. 6.1%; DERMIS-2: 37.5% vs. 6.9%, respectively; $p < 0.001$ for both). Roflumilast was also statistically superior to vehicle for rates of PASI75 response (DERMIS-1: 41.6% vs. 7.6%; DERMIS-2: 39.0% vs. 5.3%; $p < 0.0001$).

Safety:
The safety and tolerability of roflumilast were similar to vehicle control.

Dosing:
In the pivotal trials, roflumilast was administered topically once daily.

Roflumilast (continued...)

Competitive environment

Roflumilast would be the first topical PDE4 inhibitor for the treatment of plaque psoriasis. Plaque psoriasis is a large potential target population and roflumilast was evaluated in a broad patient population (mild, moderate, and severe disease). Roflumilast may also potentially be used as an add-on therapy since systemic side effects are likely to be uncommon due to topical administration.

Roflumilast 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 pharmacotherapies for the condition and there is a lack of head-to-head data comparing roflumilast against other topical treatments. Additionally, roflumilast could also be competing with tapinarof, a first-in-class topical aryl hydrocarbon receptor modulating agent. An FDA approval decision for tapinarof is expected by May 26, 2022.

Roflumilast is being evaluated for atopic dermatitis with data expected in the second half of 2022. If the data is positive, this could significantly expand the potential target population for roflumilast given the high prevalence of atopic dermatitis.

For reference, the wholesale acquisition cost (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: Potentially first topical PDE4 inhibitor approved for psoriasis, large potential target population, could be used as add-on therapy due to topical administration, also being studied for atopic dermatitis
- Disadvantages: Topical marketplace currently dominated by generic corticosteroids and vitamin D analogs, lack of head-to-head trial data, potentially competing with another topical pipeline agent (tapinarof)
- Reference WAC (Wynzora): ~$1,000 per 30 days

Deucravacitinib (Brand Name: To be determined)

Manufacturer: Bristol Myers Squibb
Expected FDA decision: September 10, 2022

Therapeutic use

Deucravacitinib is in development for treatment of adults with moderate-to-severe plaque psoriasis.

Clinical profile

Deucravacitinib is a selective tyrosine kinase 2 (TYK2) inhibitor. By selectively targeting TYK2, deucravacitinib inhibits signaling of interleukin (IL)-23, IL-12 and Type 1 interferon, key cytokines involved in the pathogenesis of immune-mediated diseases.

Pivotal trial data:
The efficacy of deucravacitinib was evaluated in the POETYK PSO-1 and POETYK PSO-2 studies. Both POETYK PSO-1, which included 666 patients, and POETYK PSO-2, which included 1,020 patients, were randomized, double-blind studies that evaluated deucravacitinib vs. placebo vs. Otezla® (apremilast) in patients with moderate-to-severe plaque psoriasis. In both studies, the co-primary endpoints were the percentage of patients who achieved PASI75 response and those who achieved static Physician's Global Assessment (sPGA) score of 0 or 1 at week 16 vs. placebo. Key secondary endpoints of the studies included the percentage of patients who achieved PASI75 and sPGA 0/1 compared to Otezla at week 16.

In POETYK PSO-1, PASI75 response at week 16 was achieved in 58.7%, 35.1%, and 12.7% of patients with deucravacitinib, Otezla, and placebo, respectively ($p < 0.0001$ for deucravacitinib vs. Otezla and placebo). sPGA response was achieved in 53.6%, 32.1%, and 7.2% of patients, respectively ($p < 0.0001$ for deucravacitinib vs. Otezla and placebo).

In POETYK PSO-2, PASI75 response at week 16 was achieved in 53.6%, 40.2%, and 9.4% of patients with deucravacitinib, Otezla, and placebo, respectively ($p < 0.0001$ for deucravacitinib vs. placebo and $p = 0.0003$ vs. Otezla). sPGA response was achieved in 50.3%, 34.3%, and 8.6% of patients, respectively ($p < 0.0001$ for deucravacitinib vs. Otezla and placebo).

Safety:
The most common adverse events with deucravacitinib use were nasopharyngitis, upper respiratory infection.

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

- Treatment of adults with moderate-to-severe plaque psoriasis
- TYK2 inhibitor
- Oral formulation
- PGA response: 50.3% to 53.6% with deucravacitinib vs. 32.1% to 34.3% with Otezla vs. 7.2% to 8.6% with placebo
- PASI75 response: 53.6% to 58.7% with deucravacitinib vs. 35.1% to 40.2% with Otezla vs. 9.4% to 12.7% with placebo
- Common AEs: Nasopharyngitis, upper respiratory infection
- Dosing: Once daily

Deucravacitinib (continued...)

Competitive environment

Deucravacitinib would provide a first-in-class oral treatment for plaque psoriasis. Injectable biologics are commonly used with high response rates in patients with moderate-to-severe plaque psoriasis, but oral options have been limited. Immunosuppressants such as methotrexate and cyclosporine have historically been used but their place in therapy is limited due to safety concerns. Otezla, an oral PDE4 inhibitor, has been available since 2014 and has a safer adverse event profile vs. historical oral treatments but its use has been limited because injectable biologics are more efficacious. While not compared against injectable biologics, deucravacitinib demonstrated superior efficacy vs. Otezla and was associated with numerically lower rates of discontinuations due to adverse events.

Deucravacitinib will be entering a crowded marketplace. Since deucravacitinib was not compared in head-to-head studies against injectable biologics, the likely place in therapy will be similar to Otezla, with its use being limited to patients who require systemic therapy but who either have contraindications or are unwilling to use injectable biologics. Many of the systemic therapies currently available are well established and approved for different immune-mediated diseases whereas the initial use for deucravacitinib will be limited to plaque psoriasis. Deucravacitinib's future place in therapy across different diseases could grow as it's also in development for psoriatic arthritis, ulcerative colitis, Crohn's disease, and lupus.

Finally, due to similarities to the Janus kinase (JAK) inhibitor class, there are some theoretical safety concerns with TYK2 inhibitors, however, were no major signals for adverse events (eg, malignancy, major cardiovascular events, serious infections) in the pivotal studies.

For reference, the WAC for Otezla is approximately $48,000 per year.

- Advantages: Novel mechanism of action, superiority data vs. Otezla, oral and once daily administration, also being studied for other immune-mediated diseases (eg, psoriatic arthritis, ulcerative colitis, Crohn's disease, lupus)
- Disadvantages: Crowded marketplace, similar place in therapy to Otezla due to lack of data vs. injectable biologics, theoretical safety concern due to similarities to JAK inhibitor class
- Reference WAC (Otezla): ~$48,000 per year

Betibeglogene autotemcel (Brand Name: Zynteglo)

Manufacturer: bluebird bio
Regulatory designations: Orphan Drug, Breakthrough Therapy, Fast Track
Expected FDA decision: August 19, 2022 *(FDA Advisory Committee meeting scheduled for June 9-10, 2022)*

Therapeutic use

- Treatment of transfusion-dependent beta-thalassemia

Betibeglogene autotemcel is in development for the treatment of transfusion-dependent beta-thalassemia.

Beta-thalassemia is an inherited blood disorder caused by β-globin gene mutations that reduce or eliminate production of β-globin, affecting hemoglobin development. Patients with severe forms of beta-thalassemia may experience classic signs of anemia including fatigue, weakness, shortness of breath and may even have life-threatening complications if left untreated. These individuals depend on life-long regular red blood cell transfusions and will additionally need iron chelation therapy to combat the excess levels of iron in the body due to the repeated blood transfusions. Allogeneic hematopoietic stem cell transplantation remains the only possible curative therapy.

Bluebird bio estimates that there are an estimated 3,000 patients with beta-thalassemia in the U.S.

Clinical profile

- Gene therapy
- IV formulation
- Transfusion independence: 91% (20 of 22 patients)
- Common AEs: Thrombocytopenia, neutropenia, anemia, stomatitis, leukopenia
- Dosing: One-time dose

Betibeglogene autotemcel is a gene therapy that adds functional copies of beta-globin gene into a patient's own hematopoietic stem cells *ex vivo* via transduction of autologous CD34+ cells with the BB305 lentiviral vector.

Pivotal trial data:
The efficacy of betibeglogene autotemcel was established in NorthStar-2, a Phase 3 single-arm, open-label study in patients aged ≤ 50 years with transfusion dependent beta-thalassemia. At baseline for these patients, the median number of transfusions they received per year was about 16 transfusions per year, with the upper range being 37 transfusions per year. The primary endpoint was transfusion independence at 24 months, defined as a weighted average hemoglobin level of at least 9 g/dL without any red blood cell transfusions for ≥12 months.

Transfusion independence occurred in 20 of 22 patients (91%), including 6 of 7 patients (86%) who were younger than 12 years of age. The 2 patients who did not received transfusion independence had a 67.4% and 22.7% reduction in transfusion volume from 6 months to the last follow-up and overall lower circulating vector copy numbers than those who achieved transfusion independence, possibly suggesting suboptimal uptake of the gene therapy. Average hemoglobin level during transfusion independence was 11.7 g/dL (range: 9.5 to 12.8) with the median duration of transfusion independence being 20.4 months (range: 15.7 to 21.6)..

Betibeglogene autotemcel (continued…)

Safety:
The most common adverse events with administration of betibeglogene autotemcel were thrombocytopenia, neutropenia, anemia, stomatitis, and leukopenia. These were likely a result of busulfan-based myeloablation conditioning therapy.

Dosing:
In the pivotal study, myeloablation with busulfan was administered over a period of 4 days, followed by a one-time IV infusion of betibeglogene autotemcel.

Betibeglogene autotemcel (continued…)

Competitive environment

If approved, betibeglogene autotemcel with only a one-time intravenous infusion, is potentially curative of beta-thalassemia through achievement of transfusion independence and attainment of near normal hemoglobin levels. The only other FDA-approved therapy for treatment of anemia due to transfusion dependent beta-thalassemia is Reblozyl® (luspatercept), which is a subcutaneous injection given chronically every 3 weeks.

With gene therapies like betibeglogene autotemcel, there is the potential risk of developing secondary malignancies. No vector-mediated events, insertional oncogenesis, clonal predominance, and cases of cancer were reported in patients with beta-thalassemia who received betibeglogene autotemcel. However, betibeglogene autotemcel is also being studied for the treatment of sickle cell disease (SCD). Although no cases of cancer were observed in patients with beta-thalassemia, there was one reported case of acute myeloid leukemia (AML) in the ongoing SCD study. The FDA agreed with bluebird bio's assessment that the drug was unlikely to have caused this one case. The FDA Advisory Committee scheduled in June will likely discuss this and more during their assessment of betibeglogene autotemcel.

Betibeglogene autotemcel, as a gene therapy, is complex to prepare and administer. Peripheral-blood hematopoietic stem cells are collected through mobilization and then followed by apheresis. The CD34+ cells must then be isolated, activated, and then transduced *ex vivo* with the BB305 lentiviral vector. Patients will then have to undergo myeloablative conditioning with busulfan over a period of 4 days before betibeglogene autotemcel is infused. The target population for this treatment is also extremely small; bluebird bio estimates that of the ~3,000 patients with beta-thalassemia in the U.S., approximately 1,500 would be eligible for treatment with betibeglogene autotemcel.

Iron burden over time did not decrease for some patients after treatment with the gene therapy. A total of 11 patients restarted iron chelation after infusion of betibeglogene autotemcel at a median of 7.2 months, with only 4 of these patients later discontinuing chelation therapy. Additionally, 7 patients underwent phlebotomy to reduce iron levels.

Lastly and most importantly, due to the short follow-up time of 2 years in the pivotal study, the durability of transfusion independence is unclear. Interim analysis results of the long-term follow-up study, LTF-303, showed that of the 57 patients enrolled, 46 patients (81%) achieved transfusion independence and maintained it through last follow-up. The median post-infusion follow-up of LTF-303 was 41.5 months.

For reference, the cost of Zynteglo was about $1.78 million in Europe.

- Advantages: Potentially curative, one-time dose, no reports of cases of secondary malignancies
- Disadvantages: Unclear durability of transfusion independence, complex therapy for small target population, iron burden did not decrease over time
- Reference WAC (EU price for Zynteglo): $1.78 million for one-time dose

Elivaldogene autotemcel (Brand Name: Skysona)

Manufacturer: bluebird bio
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: September 16, 2022 *(FDA Advisory Committee meeting June 9-10, 2022)*

Therapeutic use

- Treatment of CALD

Elivaldogene autotemcel is in development for the treatment of cerebral adrenoleukodystrophy (CALD).

CALD is the childhood-onset form and most severe manifestation of adrenoleukodystrophy, an X-linked rare neurodegenerative disease caused by mutations in the *ABCD1* gene which encodes the adrenoleukodystrophy (ALD) protein. These mutations result in a build-up of very-long-chain fatty acids (VLCFAs) in predominantly the adrenal and central nervous system. For many boys, adrenal insufficiency is the first detected symptom, but other common initial symptoms are learning disabilities and behavioral problems. As the disease rapid progresses, there is a loss of neurologic function such as seizures, poor coordination, and difficulty swallowing.

In the U.S., 1 in every 21,000 males is thought to be affected by ALD. Specifically, CALD develops in about 35% of affected boys 12 years of age and younger with ALD, and rapid disease progression leads to loss of neurologic function. Most patients with CALD will die within a decade of receiving the diagnosis if they are not treated with hematopoietic stem cell transplantation, and in symptomatic patients with high disease burden at diagnosis, the outcome of transplantation is often unfavorable, as many die from complications from the transplantation.

Clinical profile

- Gene therapy
- IV formulation
- MFD-free survival: 90% (27 of 30 patients)
- Common AEs: Pancytopenia, nausea, vomiting, stomatitis
- Dosing: One-time dose

Elivaldogene autotemcel is a gene therapy that adds functional copies of the *ABCD1* gene into the patient's own hematopoietic stem cells *ex vivo* via transduction of autologous CD34+ cells with lentiviral vector. This will produce the ALD protein that will activate the breakdown of those VLCFAs.

Pivotal trial data:
The efficacy of elivaldogene autotemcel was evaluated in Starbeam (ALD-102), a Phase 2/3, single-arm, open-label study in 32 males aged 17 years or younger with early signs of CALD. The primary endpoint was survival without any of the 6 major functional disabilities (MFDs) at month 24, which include loss of communication, cortical blindness, tube feeding, total incontinence, wheelchair dependence, and complete loss of voluntary movement. CALD is associated with these six major functional disabilities, so they are clinically important to determine the patient's ability to function independently.

Interim analysis results as of October 2020 of this study and the long-term follow-up study (LTF-304) showed that of those who have reached month 24, 27 of 30 patients (90%) are alive and free of MFDs, with no evidence of MFDs through nearly 7 years of follow-up.

Patients who completed the primary clinical study were eligible to enroll in LTF-304, a 13-year long-term follow-up study. So far, 27 patients who completed ALD-102 have enrolled in LTF-304, with 14 patients reaching at least their Year 5 follow-up visit.

Elivaldogene autotemcel (continued…)

Safety:
The most common adverse events with administration of elivaldogene autotemcel were pancytopenia, nausea, vomiting, and stomatitis. These were likely a result of myeloablation conditioning therapy.

Dosing:
In the pivotal study, myeloablation with busulfan and then cyclophosphamide was administered over two consecutive 4-day periods, followed by a one-time IV infusion of elivaldogene autotemcel.

Competitive environment

There is a high unmet need for CALD. Allogeneic hematopoietic stem cell transplant remains the only therapeutic intervention for this devastating disease, but only if performed at early stages of the disease. If left untreated, patients will die within a few years. If approved, elivaldogene autotemcel as a single IV infusion may stabilize disease progression and preserve neurological function in patients with early CALD. For patients who do not have a donor for allogenic stem cell transplantation, this therapy provides a treatment option to prevent development of irreversible impairments so that they can continue to function independently.

Similar to betibeglogene autotemcel, elivaldogene autotemcel is extremely complex to prepare and administer. After collecting the patient's hematopoietic stem cells through mobilization and apheresis, the CD34+ cells are isolated, activated, and then transduced *ex vivo* with the elivaldogene autotemcel lentiviral vector. These patients will then have to undergo myeloablative conditioning, which comes with its own set of adverse events, and then re-infused with the transduced CD34+ cells. Bluebird bio believes there is an estimated 40 patients with CALD in the U.S. per year, so the eligible target population is extremely small.

Most importantly, even with the longest follow up of 7 years from the LTF-304 study, the durability of benefits remains unclear.

For reference, the WAC for Zolgensma® (onasemnogene abeparvovec), a one-time gene therapy for another ultra-rare condition, spinal muscular atrophy, is $2.125 million.

- Advantages: One-time IV infusion, high unmet need, possible stabilization of disease progression and neurologic function preservation
- Disadvantages: Unclear durability of disease stabilization and neurologic function preservation, complex therapy for small target population
- Reference WAC (Zolgensma): $2.125 million for one-time dose

Teclistamab (Brand Name: To be determined)

Manufacturer: Johnson & Johnson
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: August 29, 2022

Therapeutic use

Teclistamab is being developed for the treatment of relapsed or refractory (R/R) multiple myeloma.

Multiple myeloma is a 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, leading to severe complications such as renal dysfunction, infections, anemias, and osteoporosis.

The American Cancer Society estimates that in the U.S. during 2022, approximately 34,470 new cases will be diagnosed and about 12,640 deaths are expected to occur from multiple myeloma.

- Treatment of patients with (R/R) multiple myeloma

Clinical profile

Teclistamab is a T-cell redirecting, bispecific antibody targeting both B cell maturation antigen (BCMA) and CD3, a T-cell receptor. BCMA is expressed at high levels on multiple myeloma cells. Teclistamab redirects CD3-positive T-cells to BCMA-expressing myeloma cells to induce killing of tumor cells.

- Humanized T-cell redirecting BCMA x CD3 bi-specific monoclonal antibody
- SC formulation

Pivotal trial data:

The efficacy of teclistamab is being evaluated in MajesTEC-1, an ongoing Phase 1/2, open-label study in patients with R/R multiple myeloma. The first part of this study was to identify the recommend subcutaneous dose of teclistamab, with the second part of this study evaluating the efficacy of teclistamab at that recommended dose. This study included 150 patients with heavily pretreated R/R multiple myeloma defined as having received 3 or more prior lines of treatment that consisted of proteasome inhibitor, immunomodulatory drug, and anti-CD38 antibody.

As of May 2021, the median follow-up was 8 months with the Phase 2 dose. The overall response rate (ORR) was 62% (95% CI: 53.7, 69.9). Of the total patients receiving teclistamab, 59% achieved a very good partial response (VGFR) or better, 29% achieved a complete response (CR) or better, and 21% achieved a stringent complete response (sCR).

- ORR at 8-month median follow up: 62%

Safety:
The most common nonhematologic adverse events with teclistamab use were cytokine release syndrome, injection site erythema, and fatigue. The most common hematologic adverse events were neutropenia, anemia, and thrombocytopenia.

- Common AEs: Cytokine release syndrome, injection site erythema, fatigue, neutropenia, anemia, thrombocytopenia

Dosing:
In the pivotal trial, teclistamab was administered SC once weekly.

- Dosing: Once weekly

Teclistamab (continued…)

Competitive environment

Relapsing is common in patients with multiple myeloma, and several lines of therapy are often needed for these patients. Teclistamab would provide an additional treatment for R/R multiple myeloma and is administered subcutaneously. The other BCMA-targeting therapies that are indicated for treatment of R/R multiple myeloma after at least 4 prior lines of therapy are Blenrep® and two CAR T cell therapies (Carvykti® and Abecma®). Blenrep requires IV infusion and the manufacturing process for CAR T cell therapies is extremely cumbersome and expensive as they have to be individually manufactured for the patient. Additionally, CAR T cell therapies have reported higher rates of cytokine release syndrome and neurotoxicity, and although comparing across trials is difficult, it appears that teclistamab could offer a more tolerable safety profile than these CAR T cell therapies.

The ongoing pivotal trial will also include another cohort of patients previously exposed to another anti-BCMA treatment (eg, CAR T cell therapy), so teclistamab has potential to be an additional treatment option for patients refractory to other BCMA-targeting therapies. Additionally, there are future ongoing studies evaluating teclistamab as a combination therapy, so teclistamab has potential for use at earlier lines of therapy.

However, there is a lack of late-stage trial data for teclistamab and a lack of overall survival data. Additionally, if approved, teclistamab would be entering a very crowded marketplace and compete with other anti-BCMA treatments as well as alkylating-containing therapies or medications like Xpovio®.

For reference, the WAC of Blenrep is approximately $17,000 per 21 days for a patient weighing 70 kg.

- Advantages: Additional treatment option for R/R multiple myeloma, SC administration, more reasonable safety profile than CAR T cell therapies
- Disadvantages: Lack of late-stage trial data, competitive marketplace, lower response rates compared to CAR T cell therapies
- Reference WAC (Blenrep): ~$17,000 per 21 days (70 kg patient)

Linzagolix (Brand Name: Yselty)

Manufacturer: ObsEva SA
Expected FDA decision: September 13, 2022

Therapeutic use

Linzagolix is in development for the management of heavy menstrual bleeding due to uterine fibroids.

Uterine fibroids (or leiomyomas) are noncancerous tumors that grow in and around the wall of the uterus in women of childbearing age. Not all fibroids cause symptoms but women with uterine fibroids who are symptomatic will experience heavy menstrual bleeding, pelvic pressure and pain, enlargement of abdomen, and reproductive dysfunction.

About 20% to 80% of women develop fibroids by the time they reach age 50 and are most common in women in their 40s and early 50s. An estimated 5 million women in the U.S. suffer from symptoms of uterine fibroids.

- Treatment of heavy menstrual bleeding associated with uterine fibroids

Clinical profile

Linzagolix is a gonadotropin-releasing hormone (GnRH) receptor antagonist that competitively binds to pituitary GnRH receptors, thereby reducing the release of luteinizing hormone (LH) and follicle-stimulating hormone (FSH), leading to decreased blood concentrations of ovarian sex hormones estradiol and progesterone and reduced bleeding associated with uterine fibroids.

- GnRH receptor antagonist
- Oral formulation
- Responder rate: 56-77% (lower dose ± ABT) and 71-94% (higher dose ± ABT) vs 29-35% (placebo)
- Common AEs: Hot flushes
- Dosing: Once daily

Pivotal trial data:
The efficacy and safety of linzagolix for the treatment of heavy menstrual bleeding due to uterine fibroids was evaluated in PRIMROSE 1 and 2, which are two Phase 3, randomized, double-blind, placebo-controlled studies. PRIMOSE 1 enrolled 574 women and PRIMROSE 2 enrolled 535 women. Two dosing regimens were studied, both alone and in combination with hormonal add-back therapy (ABT) of estradiol and norethisterone acetate. The primary efficacy endpoint was reduced menstrual blood loss (MBL) at 24 weeks, with reduction defined as MBL ≤ 80 mL and ≥ 50% reduction from baseline.

All active treatment groups had reductions in MBL at 24 weeks compared to placebo ($p \leq 0.003$). In PRIMROSE 1, the responder rates for MBL at 24 weeks were 56% (lower dose), 67% (lower dose + ABT), 71% (higher dose), and 75% (higher dose + ABT) compared to 35% in the placebo group. In PRIMROSE 2, the responder rates at 24 weeks were 57% (lower dose), 77% (lower dose + ABT), 78% (higher dose), and 94% (higher dose + ABT) compared to 29% in the placebo group.

Safety:
The most common adverse event with linzagolix use was hot flushes.

Dosing:
In the pivotal trials, linzagolix was administered orally once daily for both the higher linzagolix dosing regimen and lower linzagolix dosing regimen.

Linzagolix (continued...)

Competitive environment

If approved, linzagolix would be the third oral GnRH antagonist for the treatment of uterine fibroids. Oriahnn® (elagolix/estradiol/norethindrone acetate) and Myfembree® (relugolix/estradiol/norethindrone) are the other two oral GnRH antagonists with this indication. Linzagolix however, would be the only GnRH antagonist that provides flexible dosing options. The other oral GnRH antagonists are fixed dose combinations with hormonal ABT. Patients may also not need or want the additional hormone therapy and would have the option to just take only linzagolix as monotherapy.

Additionally, although comparing across trials is difficult, the responder rates for reduction in MBL for both Oriahnn and Myfembree at week 24 were 68% to 76% and 71% to 72% respectively, compared to the responder rates of 71% to 94% for the higher linzagolix dosing regimen group.

Linzagolix would be competing with the other two oral GnRH antagonists, as well as other treatment modalities for uterine fibroids such as hormonal contraceptives, progestin-releasing intrauterine devices (IUDs), tranexamic acid, and injectable GnRH agonists. Surgical options are also available for patients.

Additionally, linzagolix would have a limited initial indication if approved for the treatment of uterine fibroids. It is currently in development for the treatment of endometriosis and would provide an additional use to linzagolix if approved but the other oral GnRH antagonists have other established indications. Relugolix, which is in Myfembree, is marketed as Orgovyx® for the treatment of advanced prostate cancer and elagolix, which is in Oriahnn, is marketed as Orilissa® for the treatment of endometriosis.

Linzagolix may also have a limitation to its use due to the risk of continued bone loss. Oriahnn and Myfembree have recommendations to limit their use to only 24 months due to the risk of bone loss.

For reference, the WAC for Myfembree is approximately $1,000 per 28 days.

- Advantages: Flexible dosing options, large target population, promising efficacy
- Disadvantages: Alternative treatments available, limited initial indication, potential limitation to its use due to risk of continued bone loss
- Reference WAC (Myfembree): ~$1,000 per 28 days

Ublituximab (Brand Name: To be determined)

Manufacturer: TG Therapeutics
Expected FDA decision: September 28, 2022

Therapeutic use

- Treatment of relapsing forms of MS

Ublituximab is in development of the treatment of relapsing forms of multiple sclerosis (MS).

MS is a chronic, autoimmune disease of the central nervous system that attacks the protective layer of nerve fibers, which disrupts signaling to and from the brain.

Symptoms of MS are unpredictable and will vary in type and severity from one person to another as well as over time in the same individual. Patients with relapsing forms of MS have new or worsening neurologic symptoms (relapses) that may partially or fully recover. Some of these symptoms may continue and become permanent.

According to the National Multiple Sclerosis Society, 913,925 people over 18 years old are living with MS in the U.S. and 85% of people with MS will initially present with relapsing MS.

Clinical profile

- Anti-CD20 monoclonal antibody
- IV formulation
- ARR: 0.076 to 0.091 vs. 0.178 to 0.188 for Aubagio
- Common AEs: Infusion-related reactions
- Dosing: IV infusion on day 1, IV infusion on day 15, then every 24 weeks thereafter

Ublituximab is a glycoengineered anti-CD20 monoclonal antibody. When ublituximab binds to CD20-expressing B-cells, it leads to destruction of the cell through antibody-dependent cellular cytotoxicity (ADCC) and complement dependent cytotoxicity (CDC).

Pivotal trial data:
The efficacy of ublituximab was evaluated in two Phase 3, randomized, double-blinded, active-controlled studies (ULTIMATE I, ULTIMATE II) in 1,094 patients aged 18 to 55 with relapsed forms of MS. Patients were randomized to receive ublituximab IV infusions or oral Aubagio® (teriflunomide). The primary endpoint was the annualized relapse rate (ARR) at week 96.

In ULTIMATE I, the ARR for ublituximab vs. Aubagio was 0.076 and 0.188 respectively (relative reduction in ARR of 59.4%; $p < 0.0001$). In ULTIMATE II, the ARRs were 0.091 for ublituximab and 0.178 for Aubagio (relative reduction in ARR of 49.1%; $p = 0.0022$).

Safety:
The most common adverse events were infusion-related reactions, which included pyrexia, chills, headache, and influenza-like illness.

Dosing:
In the pivotal trials, the dosing was an IV infusion on day 1 over 4 hours, IV infusion over 1 hour on day 15, then every 24 weeks thereafter.

Ublituximab (continued...)

Competitive environment

If approved, ublituximab would be the third anti-CD20 monoclonal antibody approved for MS joining Ocrevus® (ocrelizumab) and Kesimpta® (ofatumumab). Ublituximab is uniquely designed to lack sugar molecules that are normally expressed on the antibody. This glycoengineering process has potential for enhanced potency of ublituximab, especially the ADCC activity.

While comparing across trials is difficult, the annualized relapse rates seen in the pivotal trials for ublituximab appear similar to the rates seen in both Ocrevus and Kesimpta trials. However, it's important to note that there is a lack of head-to-head data comparing ublituximab with the other anti-CD20 monoclonal antibodies. Instead, ublituximab was actively compared to Aubagio, which is generally considered one of the lower efficacy MS drugs compared to other disease modifying therapies.

Ublituximab would be a late-market entry into a very competitive marketplace. Ocrevus was first-in-class anti-CD20 therapy for MS and has been established in the market since March of 2017. Kesimpta, recently approved in August of 2020, is the only anti-CD20 therapy that is given via SC administration. Ublituximab, like Ocrevus, would be given via IV infusion. Besides these monoclonal antibodies that target CD20, there are alternative treatments for relapsing forms of MS, including other oral and injectable disease-modifying therapies.

For reference, the WAC of Ocrevus is approximately $68,000 per year and Kesimpta is approximately $83,000 per year.

- Advantages: Potential for enhanced potency, additional treatment for relapsing forms of MS
- Disadvantages: Competitive marketplace, IV infusion (vs. Kesimpta SC administration)
- Reference WAC (Ocrevus): $68,000 per year

Extended generic pipline forecast

Optum Rx generic pipeline forecast

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
2022 Possible launch date					
TOVIAZ	fesoterodine	Pfizer	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
NEUPRO	rotigotine	UCB	External	All	2022
JEVTANA KIT	cabazitaxel	Sanofi	Intravenous	All	2022
BALCOLTRA	levonorgestrel/ethinyl estradiol/ferrous bisglycinate	Avion	Oral	All	2022
IXEMPRA KIT	ixabepilone	R-Pharm	Intravenous	All	1H-2022
OXAYDO	oxycodone	Egalet	Oral	All	1H-2022
PRADAXA	dabigatran etexilate mesylate	Boehringer Ingelheim	Oral	All	2Q-2022
DAYTRANA	methylphenidate	Noven Therapeutics	External	All	2Q-2022
ALIMTA	pemetrexed disodium	Eli Lilly	Intravenous	All	05-2022
CAPRELSA	vandetanib	Genzyme/Sanofi	Oral	All	06-2022
VIIBRYD	vilazodone	Abbvie	Oral	All	06-2022
LUCENTIS	ranibizumab	Roche	Intravitreal	All	06-2022
FLOVENT HFA	fluticasone propionate	GlaxoSmithKline	Inhalation	All	2H-2022
XYREM	sodium oxybate	Jazz	Oral	All	2H-2022
IRESSA	gefitinib	AstraZeneca	Oral	All	07-2022
EVAMIST	estradiol	Perrigo/Elan	External	All	07-2022
IMPOYZ	clobetasol propionate	Encore Dermatology/Dr. Reddy's	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
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

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
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
KOMBIGLYZE XR	saxagliptin/metformin	Bristol-Myers Squibb/Astra Zeneca	Oral	All	2023
ONGLYZA	saxagliptin	Bristol-Myers Squibb/Astra Zeneca	Oral	All	1H-2023
NOXAFIL	posaconazole	Merck	Intravenous	All	01-2023
HUMIRA	adalimumab	AbbVie	Subcutaneous	All	01-2023
CAMBIA	diclofenac potassium	Assertio	Oral	All	01-2023
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
FIRVANQ KIT	vancomycin	Azurity	Oral	All	01-2023
SPIRIVA HANDIHALER	tiotropium	Boehringer Ingelheim	Inhalation	All	01-2023
FORTEO	teriparatide	Eli Lilly	Injection	All	01-2023
LEXISCAN	regadenoson	Astellas	Intravenous	All	01-2023
LATUDA	lurasidone	Sunovion	Oral	All	02-2023
AGGRASTAT	tirofiban	Medicure	Intravenous	All	03-2023
AUBAGIO	teriflunomide	Sanofi/Genzyme	Oral	All	03-2023
GATTEX	teduglutide recombinant	Takeda	Subcutaneous	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
NEULASTA ONPRO	pegfilgrastim	Amgen/Insulet	Subcutaneous	All	2H-2023
XURIDEN	uridine	Wellstat Therapeutics	Oral	All	07-2023
TOLAK	fluorouracil	Pierre Fabre	External	All	07-2023

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
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
EGRIFTA	tesamorelin	Theratechnologies	Subcutaneous	All	08-2023
STELARA	ustekinumab	Janssen	Subcutaneous; intravenous	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
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
AMZEEQ	minocycline	Journey Medical	External	All	12-2023
2024 Possible launch date					
EYLEA	aflibercept	Regeneron	Intravitreal	All	2024
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
ACTEMRA	tocilizumab	Roche/Chugai	Intravenous; subcutaneous	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
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

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
SAXENDA	liraglutide	Novo Nordisk	Subcutaneous	All	05-2024
ARANESP	darbepoetin alfa	Amgen/Kirin	Intravenous; subcutaneous	All	05-2024
NYMALIZE	nimodipine	Arbor	Oral	All	05-2024
VICTOZA	liraglutide recombinant	Novo Nordisk	Subcutaneous	All	06-2024
HAEGARDA	C1 esterase inhibitor	CSL Behring	Subcutaneous	All	06-2024
SLYND	drospirenone	Exeltis	Oral	All	08-2024
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
LAZANDA	fentanyl citrate	Depomed	Intranasal	All	10-2024
RYDAPT	midostaurin	Novartis	Oral	All	10-2024
VUITY	pilocarpine	AbbVie	Ophthalmic	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

Optum Rx 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									
VP-102	cantharidin	Verrica	vesicant (blistering agent)	Molluscum	TOP	Filed NDA	05/24/2022	No	No
GSK-2894512 (WBI-1001)	tapinarof	Dermavant Sciences	therapeutic aryl hydrocarbon receptor modulating agent (TAMA)	Plaque psoriasis	TOP	Filed NDA	05/26/2022	Yes	No
Tyvaso DPI	treprostinil	United Therapeutics/ MannKind	prostacyclin mimetic	Pulmonary arterial hypertension/ pulmonary hypertension	INH	Filed NDA	05/28/2022	Yes	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

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
AXS-05	dextromethorphan/ bupropion	Axsome	N-methyl-D-aspartate antagonist/ antidepressant	Major depressive disorder	PO	Filed NDA	2Q2022	No	No
Zydena	udenafil	Mezzion Pharma	phosphodiesterase type 5 (PDE5) inhibitor	Congenital single ventricle heart disease	PO	Filed NDA	2Q2022	No	Yes
FT-218	sodium oxybate extended-release	Avadel	dopamine receptor agonist	Narcolepsy	PO	Filed NDA	2Q2022	Yes	Yes
F-627	benegrastim	Evive Biotech	granulocyte colony-stimulating factor (G-CSF)	Chemotherapy-induced neutropenia	SC	Filed BLA	2Q2022	Yes	No
Libervant	diazepam	Aquestive Therapeutics	benzodiazepine	Seizures	PO	Filed NDA	Mid-2022	No	Yes
Neutrolin (CRMD-003, CRMD-004)	citrate/ taurolidine/ heparin	CorMedix	antimicrobial agent/ anticoagulant	Catheter-related infections	IV	Filed NDA	Mid-2022	No	No
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	Yes
REGEN-COV	casirivimab/imdevimab	Regeneron/Roche	monoclonal antibody	COVID-19	IV/IM/SC	Filed BLA	07/13/2022	No	No
ALN-TTRsc02	vutrisiran	Alnylam	small interfering RNA therapeutic	Hereditary transthyretin-mediated amyloidosis	SC	Filed BLA	07/14/2022	Yes	Yes
Hepcludex	bulevirtide	Gilead	HBV receptor binder	Hepatitis delta virus	SC	Filed BLA	07/19/2022	No	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ARQ-151	roflumilast	Arcutis Biotherapeutics	phosphodiesterase-4 (PDE-4) inhibitor	Plaque psoriasis	TOP	Filed NDA	07/29/2022	No	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
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
PRV-031	teplizumab	Provention Bio	CD3 antigen inhibitor	Diabetes mellitus	IV	Filed BLA	08/17/2022	Yes	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	SC	Filed BLA	08/29/2022	Yes	No
RT-002 (Daxi)	daxibotulinumtoxinA	Revance Therapeutics	botulinum toxins	Glabellar lines (frown lines)	IM	Filed BLA	09/08/2022	Yes	No
SPI-2012	eflapegrastim	Spectrum	granulocyte colony-stimulating factor (GCSF)	Chemotherapy-induced neutropenia	SC	Filed BLA	09/09/2022	Yes	No
BMS-986165	deucravacitinib	Bristol Myers Squibb	tyrosine kinase 2 inhibitor	Plaque psoriasis	PO	Filed NDA	09/10/2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
OBE-2109 (KLH-2109)	linzagolix	ObsEva	gonadotropin-releasing hormone (GnRH) antagonist	Uterine fibroids	PO	Filed NDA	09/13/2022	No	No
Lenti-D	elivaldogene autotemcel	Bluebird Bio	gene therapy	Cerebral adrenoleukodystrophy	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
Pedmark (STS)	sodium thiosulfate	Fennec	reducing agent	Ototoxicity	IV	Filed NDA	09/23/2022	Yes	Yes
ublituximab	ublituximab	TG Therapeutics	anti-CD-20 monoclonal antibody	Multiple sclerosis	IV	Filed BLA	09/28/2022	Yes	No
TAS-120	futibatinib	Otsuka/ Taiho	fibroblast growth factor receptor inhibitor	Cholangiocarcinoma	PO	Filed NDA	09/30/2022	Yes	Yes
Furoscix	furosemide	scPharmaceuticals	diuretic	Heart failure	SC	Filed NDA	10/08/2022	Yes	No
SPN-830	apomorphine	Supernus Pharmaceuticals	non-ergoline dopamine agonist	Parkinson's disease	SC infusion	Filed NDA	10/08/2022	Yes	No
Ovastat	treosulfan	Medexus Pharmaceuticals	alkylating agent	Hematopoietic stem cell transplantation	IV	Filed BLA	10/22/2022	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
tremelimumab	tremelimumab	AstraZeneca	cytotoxic T lymphocyte-associated antigen 4 (CTLA4) inhibitor	Hepatocellular carcinoma	IV	Filed BLA	10/25/2022	Yes	Yes
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension	INH	Tentative Approval	10/27/2022	Yes	No
AT-GAA	cipaglucosidase alfa	Amicus	enzyme therapy	Pompe disease	IV	Filed BLA	10/29/2022	Yes	Yes
PS-433540 (RE-021; DARA)	sparsentan	Travere Therapeutics	dual-acting angiotensin/endothelin receptor antagonist (DARA)	IgA nephropathy	PO	Filed NDA	11/17/2022	No	Yes
HM781-36B	poziotinib	Spectrum Pharmaceuticals	pan-HER inhibitor	Non-small cell lung cancer	PO	Filed NDA	11/24/2022	Yes	No
IMGN-853 (M-9346A-sulfo-SPDB-DM4)	mirvetuximab soravtansine	ImmunoGen	folate receptor-1 antagonist	Ovarian cancer	IV	Filed BLA	11/29/2022	Yes	Yes
131I-8H9	omburtamab	Y-mAbs Therapeutics	B7-H3 antagonist	Brain cancer	Intrathecal	Filed BLA	11/30/2022	Yes	Yes
RTA-408	omaveloxolone	Reata Pharmaceuticals	Nrf2 activator	Friedreich's ataxia	PO	Filed NDA	11/30/2022	Yes	Yes
omecamtiv mecarbil	omecamtiv mecarbil	Cytokinetics	mysoin activator	Heart failure	PO	Filed NDA	11/30/2022	No	No
AEB-1102	pegzilarginase	Aeglea BioTherapeutics	enzyme replacement/ arginase-I stimulator	Arginase 1 deficiency	IV	Filed BLA	12/12/2022	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
MRTX-849	adagrasib	Mirati Therapeutics	KRAS inhibitor	Non-small cell lung cancer	PO	Filed NDA	12/14/2022	Yes	No
RBX-2660	RBX-2660	Rebiotix	microbiota suspension	Clostridium difficile infection	Rectal	Filed NDA	4Q2022	No	Yes
GC-5107	human immunoglobulin	GC Pharma	human immunoglobulin	Primary immunodeficiencies	IV	CRL	4Q2022	Yes	No
UCB-4940 (CDP-4940)	bimekizumab	UCB	interleukin-17 (IL-17) receptor inhibitor	Plaque psoriasis	SC	CRL	2H2022	Yes	No
Lucassin	terlipressin	Mallinckrodt	V-1 (vasopressin) agonist	Hepato-renal syndrome	IV	CRL	2H2022	Yes	Yes
ET-104	zonisamide	Eton	anticonvulsant	Seizures	PO	CRL	2H2022	No	No
NVX-CoV2373	coronavirus vaccine	Novavax	vaccine	Novel coronavirus disease 2019 (COVID-19)	IM	InTrial	2H2022	No	No
GS-CA1 (GS-6207)	lenacapavir	Gilead	HIV capsid inhibitor	HIV-1	SC	CRL	2H2022	Yes	No
Adstiladrin	nadofaragene firadenovec	FerGene	gene therapy	Bladder cancer	Intravesical	CRL	2H2022	Yes	No
CAM-2038	buprenorphine	Braeburn	opioid receptor agonist (partial)	Opioid use disorder	SC	CRL	2H2022	Yes	No
Doria	risperidone	Laboratorios Farmacéuticos Rovi	atypical antipsychotic	Schizophrenia	IM	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
Roctavian	valoctocogene roxaparvovec	BioMarin	gene therapy	Hemophilia A	IV	CRL	Late 2022	Yes	Yes
2023 Possible launch date									
BAN-2401	lecanemab	Eisai/Biogen	beta-amyloid monoclonal antibody	Alzheimer's disease	IV	Filed BLA	01/09/2023	Yes	No
ONS-5010	bevacizumab-vikg	Outlook Therapeutics	anti-VEGF antibody	Wet age-related macular degeneration	Intravitreal	InTrial	01/31/2023	Yes	No
GSK-1278863	daprodustat	GlaxoSmithKline	hypoxia-inducible factor-prolyl hydroxylase (HIF-PH) inhibitor	Anemia	PO	Filed NDA	02/01/2023	Yes	No
NX-1207 (NYM-4805, REC 0482)	fexapotide triflutate	Nymox	pro-apoptotic	Benign prostatic hyperplasia	Intratumoral	Filed NDA	03/03/2023	Yes	No
TAK-438	vonoprazan fumarate	Phantom Pharmaceuticals	potassium-competitive acid blocker	Erosive esophagitis	PO	Filed NDA	03/14/2023	No	No
OPNT-003	nalmefene	Opiant	opioid receptor antagonist	Opioid overdose	Intranasal	InTrial	1Q2023	No	No
JS-001	toripalimab	Coherus Biosciences	anti-PD-1 monoclonal antibody	Nasopharyngeal carcinoma	IV	CRL	1Q2023	Yes	Yes
PRO-140	leronlimab	CytoDyn	C-C chemokine receptor 5 (CCR5) antagonist	HIV	SC	InTrial	1Q2023	Yes	No
pegcetacoplan (intravitreal)	pegcetacoplan	Apellis	compliment C3 inhibitor	Geographic atrophy	Intravitreal	InTrial	1Q2023	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
RG-7828	mosunetuzumab	Roche	anti-CD20/CD3 monoclonal antibody	Follicular lymphoma	IV/SC	InTrial	1Q2023	Yes	Yes
PTC-AADC	eladocagene exuparvovec	PTC Therapeutics	gene therapy	Aromatic L-amino acid decarboxylase deficiency	Intracerebral	InTrial	1Q2023	Yes	Yes
DCR-PHXC	nedosiran	Novo Nordisk	glycolate oxidase antagonist	hyperoxaluria	SC	InTrial	1Q2023	Yes	Yes
NuThrax	anthrax vaccine adsorbed/ CPG-7909	Emergent Biosolutions	vaccine/ oligodeoxynucleotide	Anthrax	IM	Filed BLA	03/31/2023	No	No
VBP-15	vamorolone	Santhera	corticosteroid	Duchenne muscular dystrophy	PO	InTrial	1Q2023	Yes	Yes
KB-103	beremagene geperpavec	Krystal Biotech	gene therapy	Epidermolysis bullosa	Topical	InTrial	1Q2023	Yes	Yes
NiCord	omidubicel	Gamida	cellular therapy	Hematological cancers	IV	InTrial	1Q2023	Yes	Yes
NNZ-2566	trofinetide	Neuren	insulin-like growth factor 1 derivative	Rett syndrome	PO	InTrial	1Q2023	Yes	Yes
LY-3527727	pirtobrutinib	Eli Lilly	Bruton's tyrosine kinase inhibitor	Mantle cell lymphoma	PO	InTrial	1Q2023	Yes	No
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
LY-3074828	mirikizumab	Eli Lilly	IL-23 inhibitor	Ulcerative colitis	SC	Filed BLA	04/28/2023	Yes	No
ABBV-951	foscarbidopa/ foslevodopa	AbbVie	aromatic amino acid decarboxylation inhibitor/ aromatic amino acid	Parkinson's disease	SC	Filed NDA	05/20/2023	Yes	No
Zynquista	sotagliflozin	Lexicon	sodium-dependent glucose transporter 1 (SGLT-1) and SGLT-2 inhibitor	Diabetes mellitus	PO	CRL	2Q2023	No	No
NOV-03	perfluorohexyloctane	Bausch/ Novaliq	tear film stabilizer	Dry eye disease	OPH	InTrial	2Q2023	No	No
CYT-387	momelotinib	Sierra Oncology	janus associated kinase (JAK) 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
BHV-3500	vazegepant	Biohaven	calcitonin gene-related peptide receptor antagonist	Migraine	Intranasal	InTrial	1H2023	No	No
pivmecillinam	pivmecillinam	Utility Therapeutics	amidinopenicillin	Urinary tract infections	PO	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
SYD-985	[vic-] trastuzumab duocarmazine	Synthon	HER2-targeting antibody-drug conjugate	Breast cancer	IV	InTrial	1H2023	Yes	No
ritlecitinib	ritlecitinib	Pfizer	janus kinase inhibitor	Alopecia areata	PO	InTrial	1H2023	Yes	No
TAK-003	Dengue fever vaccine	Takeda	vaccine	Dengue fever	SC	InTrial	1H2023	Yes	No
obeticholic acid	obeticholic acid	Intercept Pharmaceuticals	farnesoid X receptor agonist	Nonalcoholic steatohepatitis	PO	CRL	1H2023	Yes	No
PRX-102	pegunigalsidase alfa	Protalix	enzyme replacement	Fabry disease	IV	CRL	1H2023	Yes	No
RAD-1901	elacestrant	Radius Health	selective estrogen receptor degrader	Breast cancer	PO	InTrial	1H2023	Yes	No
LN-144	lifileucel	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Melanoma	IV	InTrial	1H2023	Yes	Yes
CDZ-173	leniolisib	Pharming/ Novartis	phosphatidylinositol-3-4-5-trisphosphate inhibitor	Primary immunodeficiencies	PO	InTrial	1H2023	Yes	Yes
LN-145	LN-145	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Cervical Cancer	IV	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

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
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
NS-2 (ALDX-1E1, ALDX-1E2, ADX-102)	reproxalap	Aldeyra Therapeutics	aldehyde antagonist	Dry eye disease	OP	InTrial	Mid-2023	No	No
MIN-101	roluperidone	Minerva Neurosciences	sigma-2 and 5HT-2A receptor antagonist	Schizophrenia	PO	InTrial	Mid-2023	Yes	No
PT-027	budesonide/albuterol	AstraZeneca	Glucocorticoid/beta agonist	Asthma	Inh	InTrial	Mid-2023	No	No
quizartinib	quizartinib	Daiichi Sankyo	FLT-3 receptor tyrosine kinase inhibitor	Acute myeloid leukemia	PO	CRL	Mid-2023	Yes	Yes
pIL-12 (DNA IL-12)	tavokinogene telsaplasmid	OncoSec Medical	gene therapy	Melanoma	Intratumoral	InTrial	Mid-2023	Yes	Yes
LY-686017	tradipitant	Vanda Pharmaceuticals	neurokinin 1 receptor (NK-1R) antagonist	Motion sickness/ gastroparesis	PO	InTrial	Mid-2023	No	No
RA-101495	zilucoplan	UCB	complement inhibitor	Myasthenia gravis	SC	InTrial	Mid-2023	Yes	Yes
ERY-ASP (ERY-001)	L-asparaginase (eryaspase)	Erytech	L-asparaginase	Acute lymphoblastic leukemia	IV	InTrial	Mid-2023	Yes	Yes
SAR-439859	amcenestrant	Sanofi	selective estrogen receptor degrader	Breast cancer	PO	InTrial	Mid-2023	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ATI-1501	metronidazole	Saptalis	nitroimidazole	Fungal infections, anaerobic bacterial infections	PO	InTrial	Mid-2023	No	No
MEDI-8897 (RSV MAbs)	nirsevimab	AstraZeneca/ Sanofi	anti-RSV monoclonal antibody D25	Respiratory syncytial virus	IM	InTrial	Mid-2023	Yes	No
EBV-CTL (ATA-129)	tabelecleucel	Atara Biotherapeutics	cell therapy	Lymphoproliferative disorder	IV	InTrial	Mid-2023	Yes	Yes
UCB-7665	rozanolixizumab	UCB	neonatal Fc receptor inhibitor	Myasthenia gravis	SC/IV	InTrial	Mid-2023	Yes	Yes
SER-109	SER-109	Seres Therapeutics	ecobiotic agent	Clostridium difficile infection	PO	InTrial	Mid-2023	No	Yes
RG-7433 (ABT-263)	navitoclax	AbbVie	Bcl-2 inhibitor	Myelofibrosis	PO	InTrial	Mid-2023	Yes	Yes
MT-1621	deoxythymidine/ deoxycytidine	Zogenix	deoxynucleoside	Thymidine kinase 2 deficiency	PO	InTrial	Mid-2023	Yes	Yes
Iomab-B	iodine I 131 monoclonal antibody BC8	Actinium	anti-CD45 monoclonal antibody	Acute myeloid leukemia/ Myelodysplastic syndrome	IV	InTrial	Mid-2023	Yes	Yes
LY-3002813	donanemab	Eli Lilly	beta-amyloid monoclonal antibody	Alzheimer's disease	IV	InTrial	Mid-2023	Yes	No

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	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
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
Melblez Kit	melphalan	Delcath	phenylalanine mustard	Hepatocellular cancer (liver)/ Biliary tract cancer/ Melanoma	INJ	InTrial	Mid-2023	Yes	Yes
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

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
TransCon PTH	palopegteriparatide	Ascendis Pharma	parathyroid hormone	Hypoparathyroidism	SC	InTrial	3Q2023	Yes	Yes
arimoclomol	arimoclomol	Orphazyme	cytoprotectives	Niemann-Pick disease	PO	CRL	3Q2023	Yes	Yes
CyclASol	cyclosporine	Novaliq	immunosuppressant	Dry eye disease	OPH	InTrial	3Q2023	No	No
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
K-127	pyridostigmine	Amneal	cholinesterase inhibitor	Myasthenia gravis	PO	InTrial	4Q2023	No	No
MILR-1444A	lebrikizumab	Eli Lilly	interleukin-13 inhibitor	Atopic dermatitis	SC	InTrial	4Q2023	Yes	Yes
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 (CXCR4) 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
PB-2452	bentracimab	PhaseBio	antiplatelet monoclonal antibody	Antiplatelet drug toxicity	IV	InTrial	2H2023	No	No
IDP-120	tretinoin/ benzoyl peroxide	Bausch	retinoid	Acne	TOP	InTrial	2H2023	No	No
resminostat	resminostat	4SC AG	pan histone deacetylase inhibitor	Mycosis fungoides/ Sézary syndrome	PO	InTrial	2H2023	Yes	No
dronabinol XL AdVersa	dronabinol controlled-release	Tetra Bio-Pharma	cannabinoid receptor agonist	Nausea and vomiting	Buccal	InTrial	2H2023	No	No
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	CRL	2H2023	Yes	Yes
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
MT-7117	MT-7117	Mitsubishi Tanabe Pharma	Undisclosed	Erythropoietic protoporphyria	PO	InTrial	2H2023	Yes	No
GEN-3013	epcoritamab	AbbVie	CD3/CD20 monoclonal antibody	Diffuse large B-cell lymphoma	SC	InTrial	2H2023	Yes	No
SB-206	SB-206	Novan Therapeutics	nitric oxide-releasing compound	Molluscum contagiosum	TOP	InTrial	2H2023	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ADCT-301	camidanlumab tesirine	ADC Therapeutics/ Genmab	antibody drug conjugate	Hodgkin's Lymphoma	IV	InTrial	2H2023	Yes	No
ISO-901	modufolin	Isofol Medical	reduced folate	Colorectal cancer	IV	InTrial	2H2023	Yes	No
RG-6107	crovalimab	Roche	C5 inhibitor	Paroxysmal nocturnal hemoglobinuria	IV/SC	InTrial	2H2023	Yes	Yes
KN-046	KN-046	Alphamab Oncology	PD-L1/CTLA-4 bispecific monoclonal antibody	Thymic cancer	IV	InTrial	2H2023	Yes	Yes
efgartigimod SC	efgartigimod-PH20	argenx/ Halozyme	neonatal Fc receptor antibody	Generalized myasthenia gravis	SC	InTrial	2H2023	Yes	Yes
GSK-3844766A	GSK-3844766A	GlaxoSmithKline	vaccine	Respiratory syncytial virus	IM	InTrial	2H2023	No	No
RG-1450	gantenerumab	Roche	beta-amyloid monoclonal antibody	Alzheimer's disease	SC	InTrial	2H2023	Yes	No
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
ADP-A2M4 (MAGE-A4)	afamitresgene autoleucel	Adaptimmune	SPEAR T-cell therapy	Sarcoma	IV	InTrial	2H2023	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
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
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	zuranolone	Sage Therapeutics/ Biogen	GABA-A receptor allosteric modulator	Major depressive disorder	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

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
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
APD-334	etrasimod	Pfizer/ Arena Pharmaceuticals	S1P1 receptor agonist	Ulcerative colitis	PO	InTrial	2H2023	Yes	No
iMAB-362	zolbetuximab	Astellas	GC182 monoclonal antibody	Gastric adenocarcinoma	IV	InTrial	2023	Yes	Yes
P-2B001 (P2-B001, P2B-001, P2B001)	pramipexole/ rasagiline	Pharma Two B	dopamine agonist/ monoamine oxidase B (MAO-B) inhibitor	Parkinson's disease	PO	InTrial	2023	No	No
DE-117	omidenepag isopropyl	Santen Pharmaceutical/ Ube Industries	Prostaglandin E Receptor 2 (PTGER2) agonist	Glaucoma	OPH	CRL	2023	No	No
AAI-101	cefepime/ enmetazobactam	Advanz/ Allecra	beta-lactam/b-lactamase inhibitor	Urinary tract infection	IV	InTrial	2023	No	No
GLPG-0634	filgotinib	Gilead/ Galapagos	janus kinase 1 inhibitor	Ulcerative colitis	PO	CRL	2023	Yes	No
VNRX-5133	cefepime/ taniborbactam	VenatoRx Pharmaceuticals	cephalosporin/ beta-lactamase inhibitor	Bacterial infections	IV	InTrial	2023	Yes	No
magrolimab	magrolimab	Gilead	CD47 monoclonal antibody	Myelodysplastic syndrome	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
MOR-202	felzartamab	MorphoSys/ I-Mab Biopharma	anti-CD38 monoclonal antibody	Multiple myeloma	IV	InTrial	2023	Yes	No
Qtrypta	zolmitriptan	Zosano	triptans	Acute migraines	TOP	CRL	2023	No	No
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
ALXN-1840 (WTX-101)	bis-choline tetrathiomolybdate	AstraZeneca	chelating agent	Wilson's disease	PO	InTrial	2023	Yes	Yes
RG-6171	giredestrant	Roche	selective estrogen receptor degrader	Breast cancer	PO	InTrial	2023	Yes	No
VLA-1553	VLA-1553	Valneva	vaccine	Chikungunya virus	IM	InTrial	2023	No	No
FT-2102	olutasidenib	Forma Therapeutics	dehydrogenase 1 inhibitor	Acute myeloid leukemia	PO	InTrial	2023	Yes	Yes
SDP-037, SDN-037	difluprednate	Sun Pharma Advanced Research Company (SPARC)	corticosteroid	Ocular inflammation/pain	OP	InTrial	2023	No	No
R-1658 (RG-1658, JTT-705, RO-4607381)	dalcetrapib	DalCor	cholesteryl ester transfer protein inhibitor	Acute coronary syndrome	PO	InTrial	2023	Yes	No
AGEN-1884	zalifrelimab	Agenus	immune checkpoint modulator antibody	Cervical cancer	IV	InTrial	2023	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Entyvio (SC formulation)	vedolizumab	Takeda	integrin receptor antagonist	Ulcerative colitis	SC	CRL	Late 2023	Yes	No
TAK-755 (SHP-655)	TAK-755	Takeda	ADAMTS13 enzyme	Thrombotic thrombocytopenic purpura	IV	InTrial	Late 2023	Yes	Yes
PSD-502	lidocaine/ prilocaine	Plethora/ Recordati	sodium channel blocker	Premature ejaculation	TOP	InTrial	Late 2023	No	No
SAR-408701	SAR-408701	Sanofi	antibody-drug conjugate	Non-small cell lung cancer	IV	InTrial	Late 2023	Yes	No
MGL-3196 (VIA-3196)	resmetirom	Madrigal	beta-selective thyroid hormone receptor agonist	Nonalcoholic steatohepatitis	PO	InTrial	Late 2023	Yes	No
SB-525	Giroctocogene fitelparvovec	Pfizer/ Sangamo Therapeutics	gene therapy	Hemophilia A	IV	InTrial	Late 2023	Yes	Yes
CPN-301	clobetasol propionate	Formosa Pharmaceuticals/ AimMax Therapeutics	corticosteroid	Eye inflammation/ pain	OPH	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 (apoA-I)	Myocardial infarction	IV	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
AG-10 (AG10)	acoramidis	BridgeBio	Tetrameric transthyretin (TTR) stabilizer	Transthyretin amyloid cardiomyopathy	PO	InTrial	Late 2023	Yes	No
KSI-301	KSI-301	Kodiak Sciences	vascular endothelial growth factor (VEGF) inhibitor	Wet age-related macular degeneration; retinal vein occlusion; diabetic macular edema	Intravitreal	InTrial	Late 2023	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	Drug class	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Opdivo	nivolumab	Bristol Myers Squibb	PD-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 (ESCC)	IV	05/28/2022
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 (IL-4/IL-13) inhibitor	Atopic dermatitis (> 6 months)	Treatment of atopic dermatitis (patients 6 months to 5 years old)	SC	06/09/2022
Beovu	brolucizumab	Novartis	anti-VEGF antibody	Diabetic macular edema	Treatment of diabetic macular edema	Intravitreal	06/13/2022
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 (BBS) or Alström syndrome	SC	06/16/2022
Breyanzi	lisocabtagene maraleucel	Bristol Myers Squibb	CD19-directed CAR T cell therapy	Large B-cell lymphoma	Treatment of adults with relapsed or refractory large B-cell lymphoma after failure of first-line therapy	IV	06/24/2022

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
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	06/27/2022
Olumiant	baricitinib	Eli Lilly	janus kinase 1/2 inhibitor	Alopecia areata	Treatment of adults with severe alopecia areata	PO	06/30/2022
Vaxneuvance	pneumococcal 15-valent conjugate	Merck	vaccine	Pneumococcal disease	Prevention of invasive pneumococcal disease in children 6 weeks through 17 years of age	IM	07/01/2022
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	07/07/2022
Opzelura	ruxolitinib	Incyte	janus kinase inhibitor	Vitiligo	Treatment of adolescent and adult patients with vitiligo (age ≥12 years)	TOP	07/18/2022
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	07/25/2022
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 inhibitor	Eosinophilic esophagitis	Treatment of adult and pediatric patients aged 12 years and older with eosinophilic esophagitis	SC	08/03/2022
Actemra	tocilizumab	Genentech	interleukin-6 receptor antagonist	COVID-19	Treatment of COVID-19 in hospitalized adults who are receiving systemic corticosteroids and require supplemental oxygen, non-invasive or invasive mechanical ventilation, or extracorporeal membrane oxygenation	IV	08/03/2022

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Nuplazid	pimavanserin	Acadia	5-HT-2A receptor agonist	Alzheimer's disease psychosis	Treatment of hallucinations and delusions associated with Alzheimer's disease psychosis	PO	08/04/2022
Myfembree	relugolix/ estradiol/ norethindrone acetate	Myovant	gonadotropin-releasing hormone (GnRH) receptor antagonist/ estrogen/ progestin	Endometriosis	Management of moderate to severe pain associated with endometriosis	PO	08/06/2022
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	08/08/2022
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	09/19/2022
Enhertu	fam-trastuzumab deruxtecan-nxki	AstraZeneca/ Daiichi Sankyo	HER2-directed antibody and topoisomerase inhibitor conjugate	Non-small cell lung cancer	Treatment of adult patients in the U.S. with unresectable or metastatic non-small cell lung cancer whose tumors have a HER2 mutation and who have received a prior systemic therapy	IV	09/30/2022
Tibsovo	ivosidenib	Servier	isocitrate dehydrogenase-1 inhibitor	Acute myeloid leukemia	Treatment for patients with previously untreated IDH1-mutated acute myeloid leukemia	PO	09/30/2022
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	10/06/2022
Skyrizi	risankizumab-rzaa	AbbVie	interleukin-23 (IL-23) antagonist	Crohn's disease	Treatment of patients 16 years and older with moderate to severe Crohn's disease	SC	10/20/2022

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Brukinsa	zanubrutinib	BeiGene	kinase inhibitor	chronic lymphocytic leukemia/ small lymphocytic lymphoma	Treatment of adult patients with chronic lymphocytic leukemia or small lymphocytic lymphoma	PO	10/22/2022
Nubeqa	darolutamide	Bayer	androgen receptor inhibitor	Hormone-sensitive prostate cancer	In combination with docetaxel in patients with metastatic hormone-sensitive prostate cancer	PO	11/03/2022
Imfinzi	durvalumab	AstraZeneca	programmed death-ligand 1 blocking antibody	Biliary tract cancer	In combination with standard-of-care chemotherapy, for patients with locally advanced or metastatic biliary tract cancer	IV	11/05/2022
Rinvoq	upadacitinib	AbbVie	janus associated kinase (JAK) inhibitor	Non-radiographic axial spondyloarthritis	Treatment of non-radiographic axial spondyloarthritis	PO	11/07/2022
Vraylar	cariprazine	AbbVie	dopamine D3-preferring D3/D2 receptor partial agonist	Major depressive disorder	Adjunctive treatment of patients with major depressive disorder	PO	12/22/2022
Imbruvica	ibrutinib	AbbVie	kinase inhibitor	Chronic graft versus host disease	Treatment of pediatric and adolescent patients one year and older with chronic graft versus host disease after failure of one or more lines of systemic therapy	PO	12/28/2022
Tymlos	abaloparatide	Radius Health	human parathyroid hormone related peptide analog	Osteoporosis (men)	Treatment of men with osteoporosis at high risk for fracture	SC	01/01/2023

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