RxOutlook®

1st Quarter 2023

Optum Rx®

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

Recap of 2022

In 2022, the FDA approved 37 new molecular entities or novel drugs. Of the 37 approvals, 20 drugs were considered first-in-class and 65% used one or more expedited FDA programs (ie, Fast Track, Breakthrough Therapy, Priority Review, or Accelerated Approval). Continuing a trend that started several years ago and for the fourth year out of the last five, the number of novel therapies approved with Orphan Drug status exceeded non-Orphan Drugs (54% were Orphan Drugs). In addition to these 37 drugs, the FDA also approved 3 gene therapies (Zynteglo®, Skysona™, and Hemgenix®), beginning what is expected to be a wave of new gene therapies coming to market over the next several years.

Looking Ahead to 2023

As of February 1, the number of novel drugs approved or with an FDA submission currently under review for 2023 is 59. While some of the drugs currently under review by the FDA may not ultimately be approved, the number of approvals in 2023 is anticipated to be higher than 2022 (which saw was the lowest number of approvals since 2016).

In this edition of RxOutlook, we highlight 9 key pipeline products with an expected approval decision by the end of the second quarter of 2023. This includes two respiratory syncytial virus (RSV) vaccines, one each from GSK and Pfizer, both of which are expected to have approval decisions in May 2023. RSV infection is one of the most common respiratory infections currently without a specific treatment or vaccine in the adult population. Both vaccines could be approved for use in the older adult population (≥ 60 years of age) and available prior to the 2023 RSV season that begins in the Fall and peaks in the Winter.

Two new chronic inflammatory drugs are currently under review by the FDA: mirikizumab, an interleukin-23 (IL-23) antagonist for ulcerative colitis and ritlecitinib, a Janus kinase (JAK) inhibitor for alopecia areata. Mirikizumab would be the second IL-23 targeted therapy for ulcerative colitis and ritlecitinib would be the second JAK inhibitor approved for alopecia areata.

Obeticholic acid, which is currently approved under the brand name Ocaliva® for a rare liver disease, primary biliary cholangitis, could be the first FDA approved treatment for nonalcoholic steatohepatitis (NASH). NASH has generated significant attention in the pipeline over the last several years, due to high prevalence in the U.S. (NASH affects up to 6.5% of the population) and because no drugs are currently approved for the disease.

Perfluorohexyloctane is a novel tear film stabilizer for treatment of dry eye disease (DED) associated with Meibomian gland dysfunction (MGD). While other options are available for DED, this would be the first approved specifically for patients with MGD, a leading cause of DED.

Finally, three Orphan Drugs will be discussed in the report, including tofersen for amyotrophic lateral sclerosis (ALS), concizumab for hemophilia A or B, and delandistrogene moxeparvovec, a gene therapy for Duchenne muscular dystrophy (DMD).

Approval decisions for other key novel therapies are expected in the first half of 2023 but are not reviewed in this report because they were covered in previous editions of RxOutlook. These include: fezolinetant for vasomotor symptoms of menopause; efanesoctocog alfa and valoctocogene roxaparvovec for hemophilia A; zavegepant for acute migraine; beremagene geperpavec for epidermolysis bullosa; and bimekizumab for plaque psoriasis.

Key pipeline drugs with FDA approval decisions expected by end of the 2nd quarter 2023

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Mirikizumab	Eli Lilly	Ulcerative colitis	3/2023
Tofersen	Biogen	Amyotrophic lateral sclerosis*	4/25/2023
Arexvy (respiratory syncytial virus vaccine, recombinant, adjuvanted)	GSK	Respiratory syncytial virus in adults ≥ 60 years	5/3/2023
Abrysvo (respiratory syncytial virus vaccine)	Pfizer	Respiratory syncytial virus in adults ≥ 60 years	5/2023
Delandistrogene moxeparvovec	Sarepta Therapeutics	Duchenne muscular dystrophy*	5/29/2023
Obeticholic acid	Intercept Pharmaceuticals	Nonalcoholic steatohepatitis	6/23/2023
Perfluorohexyloctane	Bausch + Lomb/ Novaliq	Dry eye disease	6/28/2023
Ritlecitinib	Pfizer	Alopecia areata	2Q 2023
Concizumab	Novo Nordisk	Hemophilia A or B*	2Q 2023

* Orphan Drug Designation

Detailed Drug Insights

This section reviews the important characteristics (eg, therapeutic use, clinical profile, competitive environment and regulatory timeline) for key pipeline drugs with potential FDA approvals by the end of the 2nd quarter 2023.

Read more

Extended Generic Pipeline Forecast

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

Read more

Extended Brand Pipeline Forecast

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

Read more

Key Pending Indication Forecast

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

Read more

Past and future reviews

Please note that RxOutlook highlights select near-term approvals. Some drugs may not appear in this issue because they have been reviewed in previous editions of RxOutlook. Drugs of interest that are earlier in development or with expected approvals beyond 2nd quarter 2023 may appear in future reports; however, for those who need an initial look at the larger pipeline, please refer to the Brand Pipeline Forecast Table found later in this report.

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

Mirikizumab (Brand Name: To be determined)

Manufacturer: Eli Lilly
Expected FDA decision: March 2023

Therapeutic use

Mirikizumab is under review for the treatment of moderately to severely active ulcerative colitis (UC).

UC is a chronic inflammatory condition of the large intestine (colon) and the rectum. Patients develop inflammation and ulcers in the lining of the colon, commonly leading to abdominal pain, bloody stools, persistent diarrhea, weight loss, and fatigue. Patients will experience periods of active inflammation or flareups and periods of remission where they are free of symptoms. Treatment usually includes induction therapy (for rapid onset of action) followed by maintenance treatment (for long term disease control).

UC is estimated to affect about 1.8 million people in the U.S.

- Treatment of moderately to severely active UC

Mirikizumab (continued…)

Clinical profile

Mirikizumab, a monoclonal antibody, is an interleukin-23 (IL-23) antagonist. IL-23 is a naturally occurring cytokine that is involved in inflammation and immune responses.

Pivotal trial data:
The efficacy of mirikizumab for induction therapy was evaluated in LUCENT 1, a Phase 3, randomized, double-blind, placebo-controlled study in 1,162 patients with moderately-to-severely active UC who previously failed conventional and/or biologic therapies and/or Janus kinase (JAK) inhibitors. Patients received mirikizumab or placebo intravenously (IV) every 4 weeks on weeks 0, 4, and 8. The primary endpoint was clinical remission at week 12. Clinical remission was achieved in 24.2% of patients with mirikizumab vs. 13.3% with placebo ($p = 0.00006$). Key secondary endpoints (eg, clinical response, symptomatic remission, rapid improvement in symptoms) also favored mirikizumab.

The efficacy of mirikizumab for maintenance therapy was evaluated in LUCENT 2, a Phase 3, randomized, double-blind, placebo-controlled study in 544 patients who completed the 12-week induction study. For LUCENT 2, the primary analysis was based on patients who had responded to mirikizumab induction treatment in LUCENT 1 and were re-randomized to receive mirikizumab subcutaneously (SC) or placebo every 4 weeks, for an additional 40 weeks. The primary endpoint was clinical remission at week 40 of the maintenance period. Clinical remission was achieved in 49.9% and 25.1% of patients with mirikizumab and placebo, respectively ($p < 0.001$). Like LUCENT 1, key secondary endpoints (eg, corticosteroid-free clinical remission, endoscopic remission, maintenance of clinical remission) also favored mirikizumab.

Safety:
In the maintenance study, the most common adverse events with mirikizumab use were nasopharyngitis, arthralgia, exacerbation of UC, injection site reaction, depression, liver enzyme elevation, herpes zoster, and oral candidiasis.

Dosing:
In the pivotal trials, mirikizumab was administered via IV infusion for induction therapy followed by SC injections for maintenance therapy, every 4 weeks.

- IL-23 antagonist
- IV/SC formulations
- Clinical remission at 12 weeks (induction): 24.2% vs. 13.3% with placebo
- Clinical remission at 40 weeks (maintenance): 49.9% vs. 25.1% with placebo
- Common AEs: Nasopharyngitis, arthralgia, exacerbation of UC, injection site reaction, depression, liver enzyme elevation, herpes zoster, oral candidiasis
- Dosing: Every 4 weeks

Mirikizumab (continued…)

Competitive environment

Mirikizumab would provide an additional treatment option for UC and would be the second IL-23 targeted therapy approved for the condition. Janssen's Stelara® (ustekinumab), an IL-12 and IL-23 antagonist, was approved for UC in October 2019.

Mirikizumab will be entering a crowded marketplace, competing not only with Stelara, but other biologics and drugs with different mechanisms of action. Two of these products, Humira® (adalimumab) and Stelara, are expected to have biosimilar versions in 2023. When compared indirectly, the efficacy results for mirikizumab appear similar to Stelara, although cross-trial comparisons are difficult to assess. Direct head-to-head trial data assessing the efficacy and safety of mirikizumab against competitors is lacking.

Mirikizumab is also in development for Crohn's disease, with topline results for a Phase 3 trial expected in 2023. This could potentially expand the target population for mirikizumab, but like UC, there are multiple alternative treatments available and mirikizumab would be a late market entry for this indication.

For reference, the Wholesale Acquisition Cost (WAC) for Stelara SC is approximately $25,500 every 8 weeks or $165,750 per year.

- Advantages: Potentially the first selective IL-23 inhibitor approved for UC, also in development for Crohn's disease
- Disadvantages: Crowded marketplace with biosimilar entrants in 2023 (eg, Humira, Stelara), lack of head-to-head trial data
- Reference WAC (Stelara): $25,500 every 8 weeks ($165,750 per year)

Tofersen (Brand Name: To be determined)

Manufacturer: Biogen
Regulatory designation: Orphan Drug
Expected FDA decision: April 25, 2023 *(FDA Advisory Committee scheduled for March 22, 2023)*

Therapeutic use

Tofersen is under review for the treatment of amyotrophic lateral sclerosis (ALS) associated with a superoxide dismutase 1 (SOD1) mutation.

ALS is a progressive, neurodegenerative disease that results in the loss of motor neurons in the brain and the spinal cord. People with ALS experience muscle weakness and atrophy, causing them to lose independence as they steadily lose the ability to move, speak, eat, and eventually breathe. Average life expectancy for people with ALS is 3 to 5 years from time of symptom onset, with respiratory failure being the main cause of death.

SOD1 gene mutations are implicated in about 2% of all ALS cases. Mutant SOD1 protein is prone to misfolding and may interfere with multiple cellular processes. Biogen estimates 330 people are living with SOD1-ALS in the U.S.

- Treatment of ALS associated with a SOD1 mutation

Tofersen (continued…)

Clinical profile

Tofersen is an antisense oligonucleotide that binds to SOD1 mRNA, allowing for its degradation, and thereby reducing the synthesis of SOD1 protein production.

Pivotal trial data:
The efficacy of tofersen was evaluated in VALOR, a Phase 3, randomized, double-blind, placebo-controlled study in 108 patients with SOD1-ALS. Patients were randomized to tofersen or placebo. The primary endpoint was change from baseline to week 28 in the ALS Functional Rating Scale-Revised (ALSFRS-R) total score in the primary analysis (faster-progressing ALS) population (n = 60). ALSFRS-R assesses 12 domains of physical function and is commonly used to assess disease progression in ALS trials. ALSFRS-R total scores range from 0 to 48, with higher scores indicating better function.

Tofersen did not demonstrate an improvement over placebo for the primary endpoint (ALSFRS-R score). In the faster-progressing population, the change from baseline to week 28 in the ALSFRS-R total score was -8.14 points with placebo vs. -6.98 points with tofersen (difference of 1.2 points, p = 0.97). However, trends favoring tofersen were seen across multiple key secondary endpoints and exploratory measures of biologic activity and clinical function. The first key secondary endpoint was change from baseline in total cerebrospinal fluid (CSF) SOD1 protein. Tofersen was associated with a 38% reduction in SOD1 in the faster-progressing subgroup ($p < 0.0001$ vs. placebo) and 26% reduction in the slower-progressing populations ($p = 0.0007$ vs. placebo). On another key secondary endpoint of change from baseline in plasma neurofilament light chain, a marker of neuronal degeneration, differences were observed between the tofersen vs. placebo groups of 67% and 48% in the faster- ($p < 0.0001$) and slower-progressing populations (no p-value reported), respectively.

In addition to the randomized trial, 95 patients from VALOR were enrolled in an ongoing open-label extension (OLE) trial. At the time of the analysis all participants had an opportunity for at least 12 months of follow-up, with a median exposure to tofersen of approximately 20 months (range: 1, 34). The 12-month data compare early initiation of tofersen (at the start of VALOR) to delayed initiation of tofersen (6 months later, in the OLE). The 12-month integrated data showed that earlier initiation of tofersen led to sustained reductions in neurofilament and slowed decline across multiple efficacy endpoints (eg, ALSFRS-R, respiratory function).

Safety:
The most common adverse events with tofersen use were headache, procedural pain, fall, back pain, and pain in extremity.

Dosing:
In the pivotal trial, tofersen was administered via intrathecal injection as three doses once every 2 weeks, followed by maintenance doses once every 4 weeks.

- Antisense oligonucleotide targeting SOD1
- Intrathecal formulation
- Change in ALSFRS-R total score in fast-progressing ALS patients: -6.98 points vs. -8.14 points with placebo (not statistically significant)
- Change in plasma neurofilament: 67% and 48% vs. placebo in the fast- and slow-progressing ALS populations, respectively
- Common AEs: Headache, procedural pain, fall, back pain, pain in extremity
- Dosing: Three doses once every 2 weeks, followed by maintenance doses once every 4 weeks

Tofersen (continued...)

Competitive environment

If approved, tofersen would represent the first biomarker-targeted therapy for ALS, a serious neurodegenerative disease for which there is still a very high unmet need. The only other drugs approved for the broader ALS population are riluzole, which has been shown to prolong survival by an average of 3 to 5 months, and Radicava® (edaravone) and Relyvrio™ (sodium phenylbutyrate/taurursodiol), which have been shown to slow the rate of functional decline in some patients with ALS.

Since tofersen did not demonstrate an improvement in the primary efficacy endpoint of clinical function in the VALOR trial, Biogen is seeking an accelerated approval based on the surrogate biomarker of plasma neurofilament levels. In ALS, higher levels of neurofilaments have been found to predict more rapid decline in clinical function and shortened survival. In addition to the questionable efficacy results, 4 patients treated with tofersen experienced serious neurologic events (eg, myelitis) compared to no similar events in the placebo group in the VALOR study. These events appear to be reversible but additional information is needed.

The target population for tofersen is expected to be very small given SOD1 mutation associated ALS is only 2% of the overall ALS population and all patients with the mutation may not be candidates for therapy.

Finally, tofersen would potentially be the second novel ALS drug approved since Congress passed the Accelerating Access to Critical Therapies for ALS Act (the ACT for ALS). This law requires the FDA and NIH to, among other things, develop an action plan to help foster drug development and facilitate access to drugs for ALS and other neurodegenerative diseases. While tofersen was developed after the law was passed, it will provide some insight for how the FDA will review these types of drugs going forward and the level of evidence required for an approval.

- Advantages: Potentially the first biomarker-targeted ALS treatment, high unmet need, supportive evidence from an OLE trial
- Disadvantages: Failed to meet primary efficacy endpoint in the pivotal trial, serious neurologic adverse events (eg, myelitis), intrathecal administration, small target population

GSK3844766A, Respiratory Syncytial Virus Vaccine, Recombinant, Adjuvanted (Brand Name: Arexvy)

Manufacturer: GSK
Regulatory designation: Fast Track
Expected FDA decision: May 3, 2023 *(FDA Advisory Committee scheduled for February 28 – March 1, 2023)*

Therapeutic use

Arexvy is under review for the prevention of lower respiratory tract disease (LRTD) caused by respiratory syncytial virus (RSV) in adults aged 60 years and older.

RSV is a common respiratory virus that usually causes mild, cold-like symptoms, lasting about 1 to 2 weeks. However, some patients may develop severe RSV infection, including bronchiolitis and pneumonia, which may result in a hospitalization. Infants, young children, and older adults are most at risk for severe RSV infection. In the U.S. and other areas with similar climates, RSV circulation generally starts during the fall and peaks in the winter.

Each year, it is estimated that between 60,000 to 120,000 older adults in the U.S. are hospitalized and 6,000 to 10,000 die due to infection.

- Prevention of RSV LRTD in adults aged 60 years and older

Clinical profile

Arexvy contains a recombinant subunit prefusion RSV F glycoprotein antigen (RSVPreF3) combined with GSK's proprietary $AS01_E$ adjuvant.

Pivotal trial data:
The efficacy of Arexvy was evaluated in AReSVi-006, a Phase 3, randomized, observer-blind, placebo-controlled study in 24,960 adults aged 60 years and older. Patients received a single-dose of the vaccine or placebo. The primary endpoint was prevention of RSV LRTD during the first RSV season. LRTD was defined as ≥ 2 lower respiratory symptoms/signs for ≥ 24 hours including ≥ 1 lower respiratory sign or ≥ 3 lower respiratory symptoms for ≥ 24 hours.

The overall vaccine efficacy was 82.6% (96.95% CI: 57.9, 94.1) against RSV-LRTD, meeting the trial's primary endpoint. Efficacy against severe RSV-LRTD, defined as LRTD with ≥ 2 lower respiratory signs or assessed as severe by the investigator and confirmed by the external adjudication committee, was 94.1% (95% CI: 62.4, 99.9).

Safety:
The most common local adverse events with Arexvy use were erythema, pain and swelling; the most common systemic adverse events were arthralgia, fatigue, fever, headache, and myalgia.

Dosing:
In the pivotal trial, Arexvy was administered as a single dose via intramuscular (IM) injection

- Vaccine
- IM formulation
- Vaccine efficacy: 82.6%
- Common local AEs: Erythema, pain, swelling
- Common systemic AEs: Arthralgia, fatigue, fever, headache, myalgia
- Dosing: Single dose

GSK3844766A, Respiratory Syncytial Virus Vaccine, Recombinant, Adjuvanted (continued...)

Competitive environment

RSV infection is one of the most common infections without a vaccine or treatment in adults. While typically mild in disease course, severe infections are still a significant driver of morbidity and mortality in patients at elevated risk, particularly the elderly population. GSK's vaccine will potentially be the first RSV vaccine approved by the FDA, likely followed shortly thereafter by Pfizer's RSV vaccine.

In the pivotal trial, Arexvy showed significant efficacy against LRTD caused by RSV and appears well tolerated. However, incidence of symptomatic RSV infection was low in the trial, likely due to the atypical RSV season because of the COVID-19 pandemic. The trial was also underpowered to estimate efficacy against more severe RSV outcomes (eg, hospitalization, death). The need for revaccination and the frequency of administration is not yet known.

Following the FDA's approval decisions in May, the CDC's Advisory Committee on Immunization Practices (ACIP) is expected to meet in June to discuss and make recommendations regarding the RSV vaccinations.

- Advantages: No RSV-specific treatments or vaccines currently available in the adult population, high unmet need
- Disadvantages: Overall incidence of symptomatic RSV infection was low in the pivotal study, lack of data for protection against severe RSV outcomes (eg, hospitalization), need for revaccination and frequency are not yet known

PF-06928316, Respiratory Syncytial Virus Vaccine (Brand Name: Abrysvo)

Manufacturer: Pfizer
Regulatory designation: Breakthrough Therapy
Expected FDA decision: May 2023 *(FDA Advisory Committee scheduled for February 28 – March 1, 2023)*

Therapeutic use

Abrysvo is under review for the prevention of lower respiratory tract disease (LRTD) caused by RSV in adults aged 60 years and older.

Clinical profile

Abrysvo contains a bivalent RSV stabilized prefusion F antigen (RSVpreF).

Pivotal trial data:
The efficacy of Abrysvo was evaluated in RENOIR, a Phase 3, randomized, double-blind, placebo-controlled study in 32,614 adults aged 60 years and older. Patients received a single-dose of the vaccine or placebo. The primary endpoint was prevention of RSV lower respiratory tract illness (LRTI) during the first RSV season. LRTI was measured as ≥ 2 or ≥ 3 lower respiratory signs/symptoms lasting more than 1 day.

The overall vaccine efficacy was 66.7% (96.66%: 28.8, 85.8) against RSV LRTI ≥ 2 symptoms and 85.7% (96.66%: 37.9, 98.4) against RSV LRTI ≥ 3 symptoms, meeting the trial's primary endpoints.

Safety:
The most common local adverse events with Abrysvo use were pain at injection site, redness, and swelling; the most common systemic adverse events were fatigue, headache, muscle pain, joint pain, diarrhea, nausea, and fever.

Dosing:
In the pivotal trial, Abrysvo was administered as a single dose via IM injection.

- Prevention of LRTD caused by RSV in adults aged 60 years and older
- Vaccine
- IM formulation
- Vaccine efficacy: 66.7% and 85.7% (protection against 2 or 3 signs/symptoms, respectively)
- Common local AEs: Pain at injection site, redness, swelling
- Common systemic AEs: Fatigue, headache, muscle pain, joint pain, diarrhea, nausea, fever
- Dosing: Single dose

PF-06928316, Respiratory Syncytial Virus Vaccine (continued…)

Competitive environment

As discussed previously, there is a high unmet need for RSV treatments or vaccines, particularly in the elderly population. The FDA approval decision for Pfizer's RSV vaccine in the elderly population is likely to come shortly after the decision for GSK's vaccine. Like the GSK vaccine, Abrysvo showed significant vaccine efficacy against LRTD caused by RSV and it appears well tolerated.

The same trial limitations discussed previously also apply to the Pfizer vaccine (eg, low incidence of symptomatic RSV infection, lack of data for more severe RSV infection outcomes, and unknown if revaccination provides added benefit).

Unlike the GSK vaccine, Pfizer is also studying Abrysvo in pregnant patients to help protect their infants from RSV disease after birth. While this is not included in the initial FDA submission, this could be a potential future use for the vaccine.

Following the FDA's approval decisions in May, the CDC's Advisory Committee on Immunization Practices (ACIP) is expected to meet in June to discuss and make recommendations regarding the RSV vaccinations.

- Advantages: No RSV-specific treatments or vaccines currently available in the adult population, high unmet need, potential future use during pregnancy to protect newborns
- Disadvantages: Overall incidence of symptomatic RSV infection was low in the pivotal study, lack of data for protection against severe RSV outcomes (eg, hospitalization), need for revaccination and the frequency are not yet known

Delandistrogene moxeparvovec (Brand Name: To be determined)

Manufacturer: Sarepta Therapeutics
Regulatory designations: Orphan Drug, Fast Track
Expected FDA decision: May 29, 2023

Therapeutic use

Delandistrogene moxeparvovec is under review for the treatment of ambulatory patients with Duchenne muscular dystrophy (DMD).

DMD is a rare, progressive, neuromuscular disorder. The age of onset is usually between 3 and 5 years. DMD is characterized by weakness and wasting of the muscles of the pelvic area followed by the involvement of the shoulder muscles. As the disease progresses, muscle weakness and atrophy spread to affect additional muscles of the body. By the early teenage years, patients will typically require a wheelchair and serious life-threatening complications may ultimately develop including cardiomyopathy and respiratory difficulties.

DMD is caused by mutations of the dystrophin gene on the X chromosome. The gene regulates the production of the dystrophin protein, which plays an important role in the functioning of muscle cells.

The birth prevalence is estimated to be 1 in every 3,500 live male births.

- Treatment of ambulatory patients with DMD

Delandistrogene moxeparvovec (continued…)

Clinical profile

Delandistrogene moxeparvovec is a gene therapy that delivers to muscle cells a gene that codes for a shortened, functional form of dystrophin (micro dystrophin).

Pivotal trial data:
The FDA submission for accelerated approval for delandistrogene moxeparvovec was based on Study SRP-9001-103 (ENDEAVOR), as well as from Studies SRP-9001-101 and SRP-9001-102, and an integrated analysis across these three early-stage studies comparing functional results to a propensity-score-weighted external control. Of the trials, only ENDEAVOR used the intended commercial manufacturing process material.

ENDEAVOR was an open-label, single-arm study that included 20 ambulatory DMD patients between the ages of 4 to 7. The primary endpoint is the change from baseline in the quantity of micro-dystrophin protein expression at 12 weeks. Key exploratory endpoints were North Star Ambulatory Assessment (NSAA) and certain timed functional tests. The NSAA is a tool that assesses functional and ambulatory status, with the total score ranging from 0 to 34 with higher scores representing better functioning. At 12 weeks, mean micro-dystrophin expression was 54.2% (range: 4.8, 153.9) of normal. At one year, using least squared means, NSAA total scores in the delandistrogene moxeparvovec treated patients improved 3.9 points and participants in the external control improved 0.8 points (difference 3.2 points, $p < 0.0001$).

SRP-9001-102 was a randomized, double-blind, placebo-controlled study in 41 ambulatory patients with DMD between the ages of 4 to 7. In Part 1, results from the treatment and placebo groups were compared through 48 weeks following treatment. In Part 2, the study remained blinded while all participants in the placebo group crossed over to active treatment and all participants were followed for another 48 weeks while safety and efficacy continue to be evaluated for 5 years total after infusion. The co-primary endpoints were micro-dystrophin expression at 12 weeks and change in NSAA total score at 48 weeks compared to placebo in Part 1 of the study. The study met the co-primary endpoint of micro-dystrophin protein expression with patients achieving mean micro- dystrophin expression of 28.1% of normal ($p < 0.0001$). However, the difference for change in NSAA total score at week 48 was not statistically significant ($p = 0.37$).

SRP-9001-101 was a proof of concept, open-label, single-arm study in 4 ambulatory patients with DMD between the ages of 4 to 7. The primary endpoint was safety, but secondary endpoints included change in micro-dystrophin expression and NSAA total score. At 12 weeks, mean micro-dystrophin expression was 70.5% (range: 13.5, 182.6) of normal. When compared to a propensity-weighted external control, total NSAA scores for the delandistrogene moxeparvovec treated patients were 9.9 points (unadjusted means) and 9.4 points (least square means) greater after 4 years ($p = 0.0125$).

In an integrated analysis of 1-year functional data from patients who received the target dose of delandistrogene moxeparvovec in the three studies ($n = 52$), NSAA change from baseline one-year after treatment was 2.4 points higher when compared to the propensity-weighted external control group ($p = 0.001$).

- Gene therapy
- IV formulation
- Micro-dystrophin expression at 12 weeks (ENDEAVOR trial): 54.2% of normal
- NSAA improvement after 1-year (integrated analysis of three studies): 2.4-points higher vs. propensity-weighted external control
- Common AEs: Vomiting, decreased appetite, nausea, upper respiratory tract infection, pain in extremity, upper abdominal pain, procedural pain
- Dosing: One-time dose

Delandistrogene moxeparvovec (continued…)

Safety:
The most common adverse events with delandistrogene moxeparvovec use were vomiting, decreased appetite, nausea, upper respiratory tract infection, pain in extremity, upper abdominal pain, and procedural pain.

Dosing:
In the pivotal trials, delandistrogene moxeparvovec was administered via intravenous (IV) infusion as a one-time dose.

Competitive environment

The pharmacologic standard of care for DMD is glucocorticoids which have been shown to improve disease progression (eg, improve motor and pulmonary function, delaying loss of ambulation). Several disease-modifying, exon-skipping therapies have been approved (eg, Exondys 51, Vyondys 53, Amondys 45) but these treatments can only be used in patients with specific mutations and while they provide small improvements in dystrophin expression, clinical benefit has not been established. Despite advancements in supportive care for DMD, there is no cure and patients still suffer from significant morbidity and mortality, especially once they reach their teenage years.

The data for delandistrogene moxeparvovec are promising, with improvements in dystrophin expression and functional data that suggests the gene therapy is altering the trajectory of the disease. While long-term data is limited, the 2- and 4-year results that are available provide some evidence of sustained stabilizing of function.

However, Sarepta's current FDA submission for approval was through the accelerated approval pathway, based on early-stage trial data that relies primarily on a surrogate endpoint of improvement in dystrophin expression. The data is also limited to ambulatory patients between 4 to 7 years of age. A confirmatory Phase 3, randomized, placebo-controlled study in 120 DMD patients is currently ongoing with data expected in 2023. The primary endpoint in this study is change in NSAA total score from baseline to week 52.

For reference, the WAC for other one-time gene therapies ranges from $2.1 million (Zolgensma® for spinal muscular atrophy) to $3.5 million (Hemgenix® for hemophilia B).

- Advantages: Promising dystrophin expression and functional improvement data, high unmet need, one-time treatment
- Disadvantages: Lack of late-stage trial data, study results are limited to ambulatory patients between ages 4 to 7
- Reference WAC: $2.1 million (Zolgensma) to $3.5 million (Hemgenix)

Obeticholic acid (Brand Name: To be determined)

Manufacturer: Intercept Pharmaceuticals
Regulatory designation: Breakthrough Therapy
Expected FDA decision: June 23, 2023

Therapeutic use

Obeticholic acid is under review for the treatment of patients with pre-cirrhotic liver fibrosis due to nonalcoholic steatohepatitis (NASH).

NASH is a progressive liver disease caused by excessive fat accumulation in the liver that leads to inflammation and liver injury. Progressive liver scarring (fibrosis) can lead to cirrhosis, liver failure, cancer, and death.

The prevalence of NASH in the general population is between 1.5% to 6.45%. Of the patients with NASH, about 20% are estimated to have advanced fibrosis (without cirrhosis).

- Treatment of patients with pre-cirrhotic liver fibrosis due to NASH

Clinical profile

Obeticholic acid is a farnesoid X receptor (FXR) agonist. FXR is a key regulator of bile acid, inflammatory, fibrotic, and metabolic pathways.

Obeticholic acid is currently available under the brand name Ocaliva®, for the treatment of primary biliary cholangitis (PBC).

- • FXR agonist
- Oral formulation

Pivotal trial data:
The efficacy of obeticholic acid was evaluated in REGENERATE, an ongoing Phase 3, randomized, double-blind, placebo-controlled study in 2,477 patients with liver fibrosis due to NASH. Patients were randomized to obeticholic acid 10 mg, obeticholic acid 25 mg, or placebo. A pre-specified interim analysis was conducted in 931 patients who had a liver biopsy at baseline and month 18. The co-primary endpoints were achieving at least one stage of fibrosis improvement with no worsening of NASH at month 18 on liver biopsy and resolution of NASH with no worsening of liver fibrosis.

- Fibrosis improvement w/o worsening of NASH: 22.4% with obeticholic acid 25 mg vs. 9.6% with placebo
- NASH resolution w/o worsening of liver fibrosis: 6.1% with obeticholic acid 25 mg vs. 3.5% with placebo (not statistically significant)

Improvement in fibrosis with no worsening of NASH occurred in 22.4% of patients with obeticholic acid 25 mg ($p < 0.0001$ vs. placebo), 14.1% with obeticholic acid 10 mg (not statistically significant vs. placebo), and 9.6% with placebo. NASH resolution with no worsening of liver fibrosis was achieved in 6.5% with obeticholic acid 25 mg, 6.1% with obeticholic acid 10 mg, and 3.5% with placebo (neither result statistically significant vs. placebo).

- Common AE: Pruritus

Safety:
The most common adverse event with obeticholic acid use was pruritus.

- Dosing: Once daily

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

Obeticholic acid (continued...)

Competitive environment

Obeticholic acid would potentially be the first FDA approved treatment for NASH. NASH is a very common chronic condition in the U.S. with millions of patients potentially eligible for treatment. The current first line treatment for NASH is lifestyle modifications – primarily weight loss. A reduction in weight can not only reduce inflammation in the liver but also potentially improve fibrosis. However, only a small subset of patients with NASH can achieve adequate weight loss. Off-label vitamin E, semaglutide, and insulin-sensitizing agents (eg, pioglitazone) can be used but the data for these therapies in NASH is limited.

In the pivotal trial, obeticholic acid showed no significant impact with respect to NASH resolution and only the 25 mg dose demonstrated significant improvements in fibrosis. While the FDA guidance for NASH trials only requires one of these endpoints to be met, the overall results for obeticholic acid were modest. Long-term data (all-cause mortality and liver-related clinical outcomes) will be included in the end-of-study analysis but are not yet available. Additionally, obeticholic acid is associated with an increase in LDL-C which could be a concern in patients with NASH as they often have other comorbidities that puts them at risk for cardiovascular events; however, LDL-C levels do return to near baseline by month 12.

Finally, while obeticholic acid may be the first to market for treatment of NASH, Madrigal Pharmaceuticals has announced positive Phase 3 trial data (both primary endpoints met) for their investigational oral drug, resmetirom. Madrigal is expected to file for accelerated approval for resmetirom in the first half of 2023.

- Advantages: Potentially the first FDA approved therapy for NASH, large potential target population, oral and once daily administration
- Disadvantages: Failed to meet both primary endpoints in the pivotal study, lack of long-term data (eg, all-cause mortality or liver-related complications), potential future competition with Madrigal's resmetirom

Perfluorohexyloctane (Brand Name: To be determined)

Manufacturer: Bausch + Lomb/ Novaliq
Expected FDA decision: June 28, 2023

Therapeutic use

Perfluorohexyloctane is under review for the treatment of signs and symptoms of dry eye disease (DED) associated with Meibomian gland dysfunction (MGD).

- Treatment of signs and symptoms of DED associated with MGD

DED can be classified into two general groups: aqueous deficient DED caused by decreased tear production, or evaporative DED, usually caused by abnormal meibomian gland physiology. The meibomian glands are oil producing glands in the eyes that help prevent the rapid evaporation of tears. Both of these mechanisms (reduced tear production and evaporative DED) are generally present in most patients with DED.

An estimated 6.8% of the U.S. adult population is projected to have diagnosed DED and MGD is believed to be one of those common causes.

Clinical profile

Perfluorohexyloctane is a preservative-free, tear film stabilizer that interacts with the lipophilic part of the tear film, forming a layer at the tear film-air interface that prevents evaporation of the aqueous phase of the tears. In addition, perfluorohexyloctane penetrates meibomian glands, where it has been reported to interact with and dissolve the altered, viscous meibum in the gland.

- Tear film stabilizer
- Eye drops
- tCFS: -2.0 to -2.3 vs. -1.0 to -1.1 with placebo
- Eye dryness VAS: -27.4 to -29.5 vs. -19.0 to -19.7 with placebo
- Common AE: Blurred vision
- Dosing: Four times daily

Pivotal trial data:
The efficacy of perfluorohexyloctane was evaluated in two Phase 3 studies (GOBI and MOJAVE). GOBI was a randomized, double-masked, saline-controlled study in 597 adults with DED associated with MGD. Patients were randomized to perfluorohexyloctane or placebo (saline solution). The co-primary endpoints were the change from baseline in total Corneal Fluorescein Staining (tCFS) (test that measures damage to the cornea) and the eye dryness visual analogue scale (VAS) score at day 57. The change from baseline in tCFS was statistically significant in the perfluorohexyloctane arm vs. the saline arm (-2.0 vs. -1.0; $p < 0.001$). Additionally, eye dryness VAS score was statistically significantly improved in the perfluorohexyloctane arm (-27.4 vs. -19.7; $p < 0.001$).

MOJAVE was a similarly designed study in 620 patients with DED associated with MGD. On day 57, change from baseline in tCFS was statistically significant in the perfluorohexyloctane arm vs. the saline arm (-2.3 vs. -1.1; $p < 0.001$). Additionally, VAS eye dryness score was statistically significantly improved in the perfluorohexyloctane arm (-29.5 vs. -19.0; $p < 0.001$).

Perfluorohexyloctane (continued...)

Safety:
The most common adverse event with perfluorohexyloctane use was blurred vision.

Dosing:
In the pivotal trials, perfluorohexyloctane was instilled into the eye four times daily.

Competitive environment

Perfluorohexyloctane would provide an additional treatment option for DED with a novel mechanism of action (MOA), and it would be the first therapy approved specifically for DED associated with MGD. MGD is one of the primary causes of DED, one of the most common ophthalmic conditions in the U.S. For most patients with MGD, non-pharmacotherapy (eg, warm compresses, lid washing) is sufficient to relieve signs and symptoms of DED. However, patients with a more severe form of the condition or with persistent symptoms may need antibiotics, corticosteroids (limited-duration), or other pharmacotherapies (eg, Restasis® [cyclosporine]).

Current pharmacotherapy options are only modestly effective in some patients with DED and given the high prevalence of the disease, there is a large potential target population. Perfluorohexyloctane met both its primary endpoints (signs and symptoms of DED) but there is a lack of head-to-head trials against existing treatment options for DED. Unlike other prescription eye drops used for DED (eg, cyclosporine and Xiidra® [lifitegrast]), which are administered twice daily, perfluorohexyloctane is administered four times daily.

For reference, the WAC for Xiidra is approximately $7,600 per year.

- Advantages: Novel MOA, potentially the first therapy approved specifically for MGD associated DED
- Disadvantages: Alternative available for broader DED population, lack of head-to-head trial data, requires dosing four times daily
- Reference WAC (Xiidra): ~$7,600 per year

Ritlecitinib (Brand Name: To be determined)

Manufacturer: Pfizer
Regulatory designation: Breakthrough Therapy
Expected FDA decision: 2Q 2023

Therapeutic use

Ritlecitinib is under review for treatment of adults and adolescents 12 years of age and older with alopecia areata.

Alopecia areata is an autoimmune disorder that often appears as patchy baldness. In alopecia areata, the body attacks its own hair follicles, causing hair to fall out, often in clumps. The onset of disease can be at any age, but most people develop alopecia areata during childhood or their teenage years. About half of patients affected will see their hair regrow within 12 months without treatment. However, in patients with more severe forms of alopecia areata, treatment may be necessary to achieve hair regrowth.

The lifetime risk of alopecia areata is estimated to be 2.1% in the U.S. and severe alopecia areata affects more than 300,000 people in the U.S. annually.

- Treatment of adults and adolescents 12 years of age and older with alopecia areata

Clinical profile

Ritlecitinib is a selective Janus kinase 3 (JAK3) inhibitor. JAKs have been a target for a variety of chronic inflammatory and autoimmune conditions.

- JAK3 inhibitor
- Oral formulation

Pivotal trial data:
The efficacy of ritlecitinib was evaluated in ALLEGRO, a Phase 2b/3, randomized, placebo-controlled, double-blind study in 718 patients 12 years of age and older with alopecia areata. Patients included in the study had 50% or more scalp hair loss, as measured by the Severity of Alopecia Tool (SALT), including patients with alopecia totalis (complete scalp hair loss) and alopecia universalis (complete scalp, face, and body hair loss), and were experiencing a current episode of alopecia areata that had lasted at least 6 months. Patients were randomized to receive once daily ritlecitinib 30 mg or 50 mg (with or without one month of initial treatment with once daily ritlecitinib 200 mg), ritlecitinib 10 mg or placebo. The primary endpoint was the proportion of patients with scalp hair regrowth in response to ritlecitinib treatment, based on an absolute SALT Score ≤ 20% at week 24.

In this study, statistically significantly higher proportions of patients treated with ritlecitinib 30 mg and 50 mg (with or without the loading dose) achieved the primary endpoint after 6 months of treatment vs. placebo. Scalp hair regrowth was achieved in 23.39% to 30.65% of patients with ritlecitinib 50 mg (without or with one month loading dose, respectively) and 14.29% to 22.31% with ritlecitinib 30 mg (without or with one month loading dose, respectively) vs. 1.54% with placebo.

- Scalp hair regrowth: 23.4% to 30.7% with ritlecitinib 50 mg vs. 14.3% to 22.3% with ritlecitinib 30 mg vs. 1.5% with placebo
- Common AEs: Headache, nasopharyngitis, upper respiratory tract infections
- Dosing: Once daily

Ritlecitinib (continued...)

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

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

Competitive environment

If approved, ritlecitinib would add another JAK inhibitor to the treatment armamentarium for alopecia areata. Historically, pharmacotherapy for alopecia areata included injectable or topical corticosteroids, oral immunosuppressants, minoxidil, and anthralin. In June 2022, the FDA approved the first treatment for alopecia areata, Eli Lilly's JAK inhibitor, Olumiant® (baricitinib).

Compared indirectly with Olumiant, ritlecitinib appears similarly effective for treatment of alopecia areata. The selectivity for JAK3 inhibition may confer a theoretical safety benefit for ritlecitinib but it is likely that class-wide JAK boxed warnings (ie, serious infections, mortality, malignancy, major adverse cardiovascular events, and thrombosis) will apply to ritlecitinib as well.

In addition to alopecia areata, Pfizer is evaluating ritlecitinib for rheumatoid arthritis, ulcerative colitis, Crohn's disease, and vitiligo.

For reference, the WAC for Olumiant is approximately $31,500 (2 mg dose) to $63,000 (4 mg dose) per year.

- Advantages: Selective JAK3 inhibition, potential future use for other conditions (eg, rheumatoid arthritis, ulcerative colitis)
- Disadvantages: Alternative treatments available including Olumiant, lack of head-to-head trial data, potential future competition with a third JAK inhibitor (deuruxolitinib)
- Reference WAC (Olumiant): $31,500 (2 mg dose) to $63,000 (4 mg dose) per year

Concizumab (Brand Name: To be determined)

Manufacturer: Novo Nordisk
Regulatory designation: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 2Q 2023

Therapeutic use

Concizumab is under review for prophylaxis to prevent or reduce the frequency of bleeding episodes in patients 12 years and older with hemophilia A or B with inhibitors.

Hemophilia is a blood clotting disorder that can cause spontaneous bleeding as well as bleeding following injuries or surgery. Hemophilia A is caused by low levels of factor VIII (FVIII) and hemophilia B is caused by low levels of factor IX (FIX). Hemophilia occurs in about 1 of every 5,000 male births. There are an estimated 33,000 males in the U.S. living with the disorder. Hemophilia A is about four times as common as hemophilia B.

The standard of care for patients with hemophilia are factor replacement therapies. Patients with hemophilia can develop an antibody (also known as an inhibitor) that binds to clotting factors making them less effective at forming blood clots needed to stop bleeding. Treatment of bleeding episodes becomes extremely difficult because more clotting factor or a different type of clotting factor is needed. It is estimated that 30% of people living with hemophilia A and 1% to 3% of people living with hemophilia B have inhibitors.

- Prophylaxis to prevent or reduce the frequency of bleeding episodes in patients 12 years and older with hemophilia A or B with inhibitors

Clinical profile

Concizumab is an anti-tissue factor pathway inhibitor (TFPI). By blocking TFPI, concizumab increases the production of thrombin, a blood clotting protein, which helps to clot the blood and prevent bleeding.

Pivotal trial data:
The efficacy of concizumab was evaluated in Explorer 7, a Phase 3, randomized, open-label study in 133 males aged 12 years and older with hemophilia A or B and inhibitors. Patients were randomized to no prophylaxis (arm 1) or concizumab prophylaxis (arm 2) or assigned to concizumab prophylaxis (arms 3 and 4). The primary analysis compared the annualized bleeding rate (ABR) between arms 1 and 2.

The estimated ABR was 1.7 for concizumab vs. 11.8 for no prophylaxis (ABR ratio 0.14, 95% CI: 0.07, 0.29; $p < 0.001$). The overall median ABR of concizumab was zero, compared to 9.8 for no prophylaxis.

Safety:
Concizumab appears to be well tolerated but limited safety data is available.

Dosing:
In the pivotal trial, concizumab was administered SC once daily.

- Anti-TFPI monoclonal antibody
- SC formulation
- Mean ABR: 1.7 vs. 11.8 for no prophylaxis
- Median ABR: 0 vs. 9.8 for no prophylaxis
- Dosing: Once daily

Concizumab (continued…)

Competitive environment

If approved, concizumab would offer a novel MOA for the treatment of both hemophilia A and B. The current standard of care for hemophilia includes factor replacement therapies. Additionally, Hemlibra® (emicizumab), a bispecific FIXa- and FX-directed antibody, is commonly used for treatment of hemophilia A. Compared to factor replacement therapies, the main advantages for concizumab are that it allows for self-administered SC administration, and that it could provide a benefit to patients who have developed inhibitors to factor replacement therapy.

Hemlibra is a self-administered SC injection approved for hemophilia A, with or without FVIII inhibitors. Unlike concizumab which must be dosed daily, Hemlibra is dosed once every week to once every four weeks. Therefore, the dosing convenience advantage for concizumab is primarily for hemophilia B. Overall, concizumab appears to be well-tolerated but there may be lingering concern related to three non-fatal thrombotic events that generated an FDA clinical hold back in 2020. The hold was lifted after discussions with the FDA.

Finally, the initial indication for concizumab is expected to be limited to patients with inhibitors. While relatively common for hemophilia A, inhibitors are only present in up to 3% of patients with hemophilia B. A Phase 3 trial (Explorer 8) is evaluating concizumab in patients with hemophilia without inhibitors. The trial met its primary endpoint, confirming superiority of concizumab vs. no prophylaxis in reducing the ABR. However, the secondary endpoint, confirming non-inferiority of concizumab prophylaxis to previous prophylaxis factor replacement therapy was not met.

For reference, the WAC for Hemlibra is approximately $425,000 per year.

- Advantages: Novel MOA, self-administered SC formulation
- Disadvantages: Alternatives available – including Hemlibra for hemophilia A, initial indication limited to patients with inhibitors, once daily administration
- Reference WAC (Hemlibra): ~$425,000 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
2023 Possible launch date					
ALPHAGAN P	brimonidine	Allergan	Ophthalmic	All	2023
PREZISTA	darunavir	Janssen	Oral	All	2023
FORTEO	teriparatide	Eli Lilly	Injection	All	2023
DYLOJECT	diclofenac	Hospira/Pfizer/Javelin	Intravenous	All	2023
DULERA	formoterol fumarate/mometasone furoate	Organon	Inhalation	All	2023
NASCOBAL	cyanocobalamin	Par/Endo	Intranasal	All	2023
TEFLARO	ceftaroline fosamil	Allergan	Intravenous	All	2023
ONEXTON	clindamycin/benzoyl peroxide	Bausch Health	External	All	2023
ONGLYZA	saxagliptin	Bristol-Myers Squibb/Astra Zeneca	Oral	All	1H-2023
KOMBIGLYZE XR	saxagliptin/metformin	Bristol-Myers Squibb/Astra Zeneca	Oral	All	1H-2023
NOXAFIL	posaconazole	Merck	Intravenous	All	1Q-2023
LEXISCAN	regadenoson	Astellas	Intravenous	All	1Q-2023
NEUPRO	rotigotine	UCB	External	All	1Q-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
ACTEMRA	tocilizumab	Roche/Chugai	Intravenous; subcutaneous	All	2Q-2023
PROVAYBLUE	methylene blue	Provepharm/American Regent	Intravenous	All	04-2023
CLINDESSE	clindamycin phosphate	Perrigo	Vaginal	All	04-2023
TYSABRI	natalizumab	Biogen	Intravenous	All	05-2023
LIVALO	pitavastatin	Eli Lilly/Kowa Pharmaceuticals	Oral	All	05-2023
XURIDEN	uridine	Wellstat Therapeutics	Oral	All	07-2023
TOLAK	fluorouracil	Pierre Fabre	External	All	07-2023
MOZOBIL	plerixafor	Sanofi/Genzyme	Subcutaneous	All	07-2023
EGRIFTA	tesamorelin	Theratechnologies	Subcutaneous	All	08-2023
CYSTADROPS	cysteamine	Recordati	Ophthalmic	All	08-2023

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
KATERZIA	amlodipine	Azurity	Oral	All	08-2023
VYVANSE	lisdexamfetamine	Shire/Takeda	Oral	All	08-2023
STELARA	ustekinumab	Janssen	Subcutaneous; intravenous	All	09-2023
CAROSPIR	spironolactone	CMP Pharma	Oral	All	09-2023
VIBATIV	telavancin	Theravance	Intravenous	All	09-2023
LEXETTE	halobetasol	Mayne	External	All	09-2023
PROLENSA	bromfenac	Bausch Health	Ophthalmic	All	4Q-2023
NEULASTA ONPRO	pegfilgrastim	Amgen/Insulet	Subcutaneous	All	10-2023
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
2024 Possible launch date					
EYLEA	aflibercept	Regeneron	Intravitreal	All	2024
VESICARE LS	solifenacin	Astellas	Oral	All	1H-2024
FIRVANQ KIT	vancomycin	Azurity	Oral	All	1H-2024
GIAZO	balsalazide disodium	Bausch Health	Oral	All	01-2024
MYRBETRIQ	mirabegron	Astellas	Oral	All	01-2024
GRALISE	gabapentin	Assertio Therapeutics	Oral	All	01-2024
SPIRIVA HANDIHALER	tiotropium	Boehringer Ingelheim	Inhalation	All	01-2024
BALCOLTRA	levonorgestrel/ethinyl estradiol/ferrous bisglycinate	Avion/Albion	Oral	All	01-2024
TASIGNA	nilotinib	Novartis	Oral	All	01-2024
SIMPONI	golimumab	Janssen	Subcutaneous	All	02-2024
SIMPONI ARIA	golimumab	Janssen	Intravenous	All	02-2024
NATESTO	testosterone	Acerus	Nasal	All	02-2024
EMFLAZA	deflazacort	PTC Therapeutics	Oral	All	02-2024
CIMZIA	certolizumab pegol	UCB/Royalty Pharma	Subcutaneous	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

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
DUAVEE	conjugated estrogens/bazedoxifene acetate	Pfizer/Ligand Pharmaceuticals	Oral	All	05-2024
SAXENDA	liraglutide	Novo Nordisk	Subcutaneous	All	05-2024
ARANESP	darbepoetin alfa	Amgen/Kirin	Intravenous; subcutaneous	All	05-2024
NYMALIZE	nimodipine	Arbor	Oral	All	05-2024
HAEGARDA	C1 esterase inhibitor	CSL Behring	Subcutaneous	All	06-2024
VICTOZA	liraglutide	Novo Nordisk	Subcutaneous	All	06-2024
TWYNEO	tretinoin/benzoyl peroxide	Galderma	External	All	07-2024
SLYND	drospirenone	Exeltis/Insud	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
2025 Possible launch date					
BOSULIF	bosutinib	Pfizer	Oral	All	2025
DALVANCE	dalbavancin	AbbVie	Intravenous	All	2025
COMPLERA	emtricitabine/rilpivirine/tenofovir disoproxil fumarate	Gilead/Janssen	Oral	All	2025
NAMZARIC	memantine/donepezil	Allergan/Adamas	Oral	All	01-2025
TRACLEER	bosentan	Actelion/Janssen	Oral	All	01-2025
RISPERDAL CONSTA	risperidone	Janssen	Injection	All	01-2025
HALAVEN	eribulin	Eisai	Intravenous	All	01-2025
MYDAYIS	amphetamine/dextroamphetamine mixture	Takeda	Oral	All	01-2025
CORLANOR	ivabradine	Amgen	Oral	All	01-2025
PHOSLYRA	calcium acetate	Fresenius	Oral	All	01-2025
FINACEA Foam	azelaic acid	LEO Pharma	External	All	01-2025
SANCUSO	granisetron	Kyowa Hakko Kirin/ProStrakan	External	All	01-2025
PROLIA	denosumab	Amgen	Subcutnaeous	All	02-2025
XGEVA	denosumab	Amgen	Subcutaneous	All	02-2025
SOLIRIS	eculizumab	Alexion	Intravenous	All	03-2025

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
BENLYSTA	belimumab	GSK	Intravenous; subcutaneous	All	03-2025
AURYXIA	ferric citrate	Keryx/Akebia Therapeutics	Oral	All	03-2025
YERVOY	ipilimumab	Bristol-Myers Squibb	Intravenous	All	03-2025
HORIZANT	gabapentin enacarbil	Arbor	Oral	All	04-2025
JYNARQUE	tolvaptan	Otsuka	Oral	All	04-2025
BRILINTA	ticagrelor	AstraZeneca	Oral	All	05-2025
TRADJENTA	linagliptin	Eli Lilly/Boehringer Ingelheim	Oral	All	05-2025
JENTADUETO XR	linagliptin/metformin	Boehringer Ingelheim/Eli Lilly	Oral	All	05-2025
JENTADUETO	linagliptin/metformin	Boehringer Ingelheim/Eli Lilly	Oral	All	05-2025
APTIOM	eslicarbazepine	Sunovion/Bial	Oral	All	05-2025
TIROSINT-SOL	levothyroxine	IBSA Institut Biochemique	Oral	All	05-2025
PERJETA	pertuzumab	Genentech	Intravenous	All	06-2025
NULOJIX	belatacept	Bristol-Myers Squibb	Intravenous	All	06-2025
NUCYNTA	tapentadol	Collegium	Oral	All	06-2025
NUCYNTA ER	tapentadol	Collegium	Oral	All	06-2025
THIOLA EC	tiopronin	Mission Pharmacal/Travere/Retrophin	Oral	All	3Q-2025
RAVICTI	glycerol phenylbutyrate	Horizon	Oral	All	07-2025
RYANODEX	dantrolene	Eagle Pharmaceuticals	Intravenous	All	07-2025
SOLIQUA	insulin glargine/lixisenatide	Sanofi	Subcutaneous	All	07-2025
ENTRESTO	sacubitril/valsartan	Novartis	Oral	All	07-2025
RYTARY	carbidopa/levodopa	Impax/Amneal	Oral	All	07-2025
ADZENYS XR-ODT	amphetamine polistirex	Neos Therapeutics	Oral	All	09-2025
FYCOMPA	perampanel	Eisai	Oral	All	09-2025
OFEV	nintedanib	Boehringer Ingelheim	Oral	All	10-2025
XIGDUO XR	dapagliflozin/metformin	AstraZeneca	Oral	All	10-2025
FARXIGA	dapagliflozin	AstraZeneca	Oral	All	10-2025
QTERN	dapagliflozin/saxagliptin	AstraZeneca	Oral	All	10-2025
FUROSCIX	furosemide	scPharmaceuticals	Subcutaneous	All	10-2025
ELELYSO	taliglucerase alfa	Pfizer	Intravenous	All	10-2025
EDURANT	rilpivirine	Janssen	Oral	All	10-2025
PICATO	ingenol mebutate	LEO Pharma	External	All	12-2025
OPSUMIT	macitentan	Janssen	Oral	All	12-2025
1H 2026					
BRYHALI	halobetasol	Bausch Health	External	All	2026

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
ABILIFY MAINTENA	aripiprazole	Otsuka/Lundbeck	Intramuscular	All	2026
POMALYST	pomalidomide	Celgene	Oral	All	1Q-2026
MOTEGRITY	prucalopride	Takeda	Oral	All	01-2026
YONSA	abiraterone	Sun	Oral	All	01-2026
VELPHORO	sucroferric oxyhydroxide	Vifor Fresenius Medical Care Renal Pharma (VFMCRP)	Oral	All	01-2026
BYVALSON	nebivolol/valsartan	AbbVie	Oral	All	01-2026
LUCEMYRA	lofexidine	US Worldmeds	Oral	All	01-2026
EDARBI	azilsartan kamedoxomil	Arbor	Oral	All	01-2026
SERNIVO	betamethasone dipropionate	Encore Dermatology	External	All	01-2026
ELLA	ulipristal	Afaxys/Perrigo	Oral	All	01-2026
TYVASO	treprostinil	United Therapeutics	Inhalation	All	01-2026
BROMSITE	bromfenac	Sun	Ophthalmic	All	01-2026
PROMACTA	eltrombopag	Novartis	Oral	All	01-2026
CYRAMZA	ramucirumab	Eli Lilly	Intravenous	All	01-2026
BRIVIACT	brivaracetam	UCB	Oral; intravenous	All	02-2026
XELJANZ XR	tofacitinib	Pfizer	Oral	All	2Q-2026
XELJANZ	tofacitinib	Pfizer	Oral	All	2Q-2026
JANUVIA	sitagliptan	Merck	Oral	All	05-2026
JANUMET	sitagliptan/metformin	Merck	Oral	All	05-2026
NAYZILAM	midazolam	UCB	Intranasal	All	05-2026
EVOMELA	melphalan	Acrotech/Aurobindo	Intravenous	All	06-2026
CERDELGA	eliglustat	Sanofi/Genzyme	Oral	All	06-2026
SUPPRELIN LA	histrelin	Endo	Subcutaneous	All	06-2026
TRINTELLIX	vortioxetine	Takeda/Lundbeck	Oral	All	06-2026

Extended brand pipeline forecast

Optum Rx brand pipeline forecast

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
2023 Possible launch date									
BIVV-001	efanesoctocog alfa	Sanofi	recombinant Factor VIII	Hemophilia A	IV	Filed BLA	02/28/2023	Yes	Yes
RTA-408	omaveloxolone	Reata Pharmaceuticals	Nrf2 activator	Friedreich's ataxia	PO	Filed NDA	02/28/2023	Yes	Yes
omecamtiv mecarbil	omecamtiv mecarbil	Cytokinetics	cardiac myosin activator	Heart failure	PO	Filed NDA	02/28/2023	No	No
NNZ-2566	trofinetide	Acadia Pharmaceuticals	insulin-like growth factor 1 derivative	Rett syndrome	PO	Filed NDA	03/12/2023	Yes	Yes
CD-101	rezafungin	Cidara Therapeutics	echinocandin	Fungal infections	IV	Filed NDA	03/22/2023	No	Yes
BHV-3500	zavegepant	Biohaven	calcitonin gene-related peptide receptor antagonist	Migraine	Intranasal	Filed NDA	03/24/2023	No	No
CDZ-173	leniolisib	Pharming/ Novartis	phosphatidylinositol-3-4-5-trisphosphate inhibitor	Primary immunodeficiencies	PO	Filed NDA	03/29/2023	Yes	Yes
LY-3074828	mirikizumab	Eli Lilly	IL-23 inhibitor	Ulcerative colitis	IV/SC	Filed BLA	03/30/2023	Yes	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
Ovastat	treosulfan	Medexus Pharmaceuticals	alkylating agent	Hematopoietic stem cell transplantation	IV	Filed BLA	1Q2023	Yes	Yes
AMP-012	naloxone	Amphastar	opioid antagonist	Opioid overdose	Intranasal	Filed NDA	1Q2023	No	No
Roctavian	valoctocogene roxaparvovec	BioMarin	gene therapy	Hemophilia A	IV	Filed BLA	03/31/2023	Yes	Yes
Botulax	letibotulinumtoxinA	Hugel Pharma	botulinum toxins	Wrinkles	IM	Filed BLA	04/06/2023	Yes	No
Rizaport (VersaFilm)	rizatriptan	IntelGenx	triptans	Acute migraines	PO	Filed NDA	04/17/2023	No	No
quizartinib	quizartinib	Daiichi Sankyo	FLT-3 receptor tyrosine kinase inhibitor	Acute myeloid leukemia	PO	Filed NDA	04/24/2023	Yes	Yes
BIIB-067 (ISIS-333611)	tofersen	Biogen/ Ionis	antisense oligonucleotide targeting SOD1	Amyotrophic lateral sclerosis	Intrathecal	Filed NDA	04/25/2023	Yes	Yes
SER-109	SER-109	Seres Therapeutics	microbiome therapeutic	Clostridium difficile infection	PO	Filed BLA	04/26/2023	No	Yes
Aripiprazole 2-month	aripiprazole	Lundbeck/ Otsuka Pharmaceutical	atypical antipsychotic	Schizophrenia/ bipolar disorder	IM	Filed NDA	04/27/2023	No	No
TransCon PTH	palopegteriparatide	Ascendis Pharma	parathyroid hormone	Hypoparathyroidism	SC	Filed BLA	04/30/2023	Yes	Yes
AV-7909 (CPG 7909)	anthrax vaccine adsorbed	Emergent Biosolutions	vaccine/ oligodeoxynucleotide	Anthrax	IM	Filed BLA	04/30/2023	No	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
NiCord	omidubicel	Gamida	stem cell therapy	Hematological cancers	IV	Filed BLA	05/01/2023	Yes	Yes
TV-46000	risperidone	Teva Pharmaceuticals/ MedinCell	atypical antipsychotic	Schizophrenia	SC	Filed NDA	05/03/2023	No	No
GSK-3844766A	respiratory syncytial virus vaccine, recombinant, adjuvanted	GlaxoSmithKline	vaccine	Respiratory syncytial virus	IM	Filed BLA	05/03/2023	No	No
PRX-102	pegunigalsidase alfa	Protalix	enzyme replacement	Fabry disease	IV	Filed BLA	05/09/2023	Yes	No
SYD-985	[vic-] trastuzumab duocarmazine	Byondis	HER2-targeting antibody-drug conjugate	Breast cancer	IV	Filed BLA	05/12/2023	Yes	No
KB-103	beremagene geperpavec	Krystal Biotech	gene therapy	Epidermolysis bullosa	Intradermal	Filed BLA	05/19/2023	Yes	Yes
ABBV-951	foscarbidopa/ foslevodopa	AbbVie	aromatic amino acid decarboxylation inhibitor/ aromatic amino acid	Parkinson's disease	SC	Filed NDA	05/20/2023	Yes	No
GEN-3013	epcoritamab	AbbVie	CD3/CD20 monoclonal antibody	Large B-cell lymphoma	SC	Filed BLA	05/21/2023	Yes	No
OPNT-003	nalmefene	Opiant Pharmaceuticals	opioid receptor antagonist	Opioid overdose	Intranasal	Filed NDA	05/22/2023	No	No
ESN-364	fezolinetant	Astellas	NK3 receptor antagonist	Menopause	PO	Filed NDA	05/22/2023	No	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
UCB-4940 (CDP-4940)	bimekizumab	UCB	interleukin-17 receptor inhibitor	Plaque psoriasis	SC	Filed BLA	05/22/2023	Yes	No
TAK-003	Dengue fever vaccine	Takeda	vaccine	Dengue fever	SC	Filed BLA	05/23/2023	Yes	No
ALT-803	nogapendekin alfa inbakicept	ImmunityBio	interleukin-15 (IL-15) super agonist/ IL-15R alpha-Fc fusion complex	Bladder cancer	Intravesical	Filed BLA	05/23/2023	Yes	No
CAM-2038	buprenorphine	Braeburn	opioid receptor agonist (partial)	Opioid use disorder	SC	Filed NDA	05/23/2023	Yes	No
PF-07321332	nirmatrelvir/ ritonavir	Pfizer	protease inhibitor	COVID-19	PO	Filed NDA	05/25/2023	No	No
SRP-9001 (RG-6356)	delandistrogene moxeparvovec	Sarepta	gene therapy	Duchenne muscular dystrophy	IV	Filed BLA	05/29/2023	Yes	Yes
ETX-2514 (SUL-DUR)	durlobactam/ sulbactam	Innoviva	broad-spectrum β-lactamase inhibitor/ beta-lactam antimicrobial	Bacterial infections	IV	Filed NDA	05/29/2023	No	No
Zynquista	sotagliflozin	Lexicon Pharmaceuticals	sodium-dependent glucose transporter 1 (SGLT-1) and SGLT-2 inhibitor	Diabetes mellitus	PO	Filed NDA	05/2023	No	No
PF-06928316 (RSVpreF)	respiratory syncytial virus vaccine	Pfizer	vaccine	Respiratory syncytial virus	IM	Filed BLA	05/2023	No	No
AOP-200704	landiolol	Eagle Pharmaceuticals	cardio-selective beta-1 adrenergic blocker	Dysrhythmia	IV	Filed NDA	06/01/2023	No	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
CyclASol	cyclosporine	Novaliq	immunosuppressant	Dry eye disease	OPH	Filed NDA	06/08/2023	No	No
CYT-387	momelotinib	GlaxoSmithKline	janus kinase inhibitor	Myeloproliferative disorders	PO	Filed NDA	06/16/2023	Yes	Yes
FT-218	sodium oxybate extended-release	Avadel	dopamine receptor agonist	Narcolepsy	PO	Tentative Approval	06/17/2023	Yes	Yes
F-901318	olorofim	F2G	orotomide antifungal	Aspergillosis	PO/IV	Filed NDA	06/17/2023	No	Yes
efgartigimod SC	efgartigimod-PH20	argenx/ Halozyme	neonatal Fc receptor antibody	Generalized myasthenia gravis	SC	Filed BLA	06/20/2023	Yes	Yes
obeticholic acid	obeticholic acid	Intercept Pharmaceuticals	farnesoid X receptor agonist	Nonalcoholic steatohepatitis	PO	Filed NDA	06/22/2023	Yes	No
Travivo	gepirone ER	Fabre-Kramer	5-HT-1A receptor agonist	Major depressive disorder	PO	Filed NDA	06/23/2023	No	No
DS-100	dehydrated alcohol	Eton	undisclosed	Methanol poisoning	SC	Filed NDA	06/27/2023	No	Yes
NOV-03	perfluorohexyloctane	Bausch/ Novaliq	tear film stabilizer	Dry eye disease	OPH	Filed NDA	06/28/2023	No	No
ritlecitinib	ritlecitinib	Pfizer	janus kinase inhibitor	Alopecia areata	PO	Filed NDA	2Q2023	Yes	No
NN-7415	concizumab	Novo Nordisk	anti-tissue factor pathway inhibitor	Hemophilia A and hemophilia B	SC	Filed BLA	2Q2023	Yes	Yes
JS-001	toripalimab	Coherus Biosciences	anti-PD-1 monoclonal antibody	Nasopharyngeal carcinoma	IV	Filed BLA	1H2023	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
IPX-203	carbidopa/ levodopa	Amneal	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	PO	Filed NDA	06/30/2023	No	No
AT-GAA	cipaglucosidase alfa	Amicus	enzyme therapy	Pompe disease	IV	Filed BLA	1H2023	Yes	Yes
BGB-A317 (BGB-A-317)	tislelizumab	BeiGene	programmed death-1 inhibitor	Esophageal squamous cell carcinoma	IV	Filed BLA	1H2023	Yes	Yes
RG-6026	glofitamab	Roche	anti-CD20/CD3 T cell monoclonal antibody	Diffuse large B cell lymphoma	IV	Filed BLA	07/01/2023	Yes	No
ARS-1	epinephrine	ARS Pharmaceuticals	non-selective alpha/ beta-adrenergic receptor agonist	Anaphylaxis	Intranasal	Filed NDA	Mid-2023	No	No
MEDI-8897	nirsevimab	AstraZeneca/ Sanofi	anti-RSV monoclonal antibody D25	Respiratory syncytial virus	IM	Filed BLA	07/05/2023	No	No
UCB-7665	rozanolixizumab	UCB	neonatal Fc receptor inhibitor	Generalized myasthenia gravis	SC	Filed BLA	07/06/2023	Yes	Yes
Risvan	risperidone	Laboratorios Farmacéuticos Rovi	atypical antipsychotic	Schizophrenia	IM	Filed NDA	07/23/2023	Yes	No
VP-102	cantharidin	Verrica	vesicant (blistering agent)	Molluscum	TOP	Filed NDA	07/24/2023	No	No
Prochymal	remestemcel-L	Mesoblast	mesenchymal stem cells	Graft vs. host disease	IV	Filed BLA	07/31/2023	Yes	Yes
PDP-716	brimonidine	Visiox Pharma	alpha-2 agonist	Glaucoma	OPH	Filed NDA	08/04/2023	No	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
SAGE-217	zuranolone	Sage Therapeutics/ Biogen	GABA-A receptor allosteric modulator	Major depressive disorder/ postpartum depression	PO	Filed NDA	08/05/2023	No	No
JNJ-64407564	talquetamab	Johnson & Johnson	GPRC5D/CD3 monoclonal antibody	Multiple myeloma	SC	Filed BLA	08/08/2023	Yes	Yes
GC-4419	avasopasem manganese	Galera Therapeutics	dismutase mimetic	Radiotherapy-induced oral mucositis	IV	Filed BLA	08/09/2023	Yes	No
Melblez Kit	melphalan	Delcath	phenylalanine mustard	Hepatocellular cancer/ melanoma	INJ	Filed NDA	08/14/2023	Yes	Yes
Zimura	avacincaptad pegol	IVERIC bio	C5 complement inhibitor	Geographic atrophy	Intravitreal	Filed BLA	08/19/2023	Yes	No
VLA-1553	VLA-1553	Valneva	vaccine	Chikungunya virus	IM	Filed BLA	08/22/2023	No	No
TP-03	lotilaner	Tarsus Pharmaceuticals	antagonist of insect and arachnid GABA-Cl channels	Demodex blepharitis	TOP	Filed NDA	08/25/2023	No	No
PF-3084014 (PF-03084014)	nirogacestat	SpringWorks Therapeutics	gamma secretase inhibitor	Desmoid tumors	PO	Filed NDA	08/27/2023	Yes	Yes
ONS-5010	bevacizumab-vikg	Outlook Therapeutics	anti-VEGF antibody	Wet age-related macular degeneration	Intravitreal	Filed BLA	08/29/2023	Yes	No
REGN-3918	pozelimab	Regeneron	C5a receptor inhibitor	CHAPLE disorder	IV/SC	Filed BLA	08/31/2023	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
AKCEA-TTR-LRx	eplontersen	AstraZeneca/ Ionis	antisense oligonucleotide	Hereditary transthyretin-mediated amyloid polyneuropathy	SC	Filed BLA	08/2023	Yes	Yes
BL-8040 (BKT-140)	motixafortide	BioLineRx	selective chemokine receptor 4 inverse agonist	Stem cell transplant	SC	Filed NDA	09/09/2023	Yes	Yes
RA-101495	zilucoplan	UCB	complement inhibitor	Generalized myasthenia gravis	SC	Filed NDA	09/14/2023	Yes	Yes
ATI-1501	metronidazole	Saptalis	nitroimidazole	Fungal infections, anaerobic bacterial infections	PO	Filed NDA	09/23/2023	No	No
BBI-4000	sofpironium bromide	Brickell	anticholinergic	Hyperhidrosis	TOP	Filed NDA	09/26/2023	No	No
I/Ontak	denileukin diftitox	Citius	CD25-directed cytotoxin	Cutaneous T-cell lymphoma	IV	Filed BLA	09/28/2023	Yes	Yes
Nyxol	phentolamine	Ocuphire	Alpha-1 and alpha-2 blocker	Mydriasis reversal	OPH	Filed NDA	09/28/2023	No	No
R-667 (RG-667)	palovarotene	Ipsen	selective retinoic acid receptor agonist	Fibrodysplasia ossificans progressiva	PO	CRL	3Q2023	Yes	Yes
DCR-PHXC	nedosiran	Novo Nordisk	glycolate oxidase antagonist	hyperoxaluria	SC	Filed NDA	3Q2023	Yes	Yes
MILR-1444A	lebrikizumab	Eli Lilly	interleukin-13 inhibitor	Atopic dermatitis	SC	Filed BLA	3Q2023	Yes	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
Neutrolin (CRMD-003, CRMD-004)	citrate/ taurolidine/ heparin	CorMedix	antimicrobial agent/ anticoagulant	Catheter-related infections	IV	CRL	3Q2023	No	No
ADX-2191	methotrexate	Aldeyra Therapeutics	dihydrofolate reductase inhibitor	Vitreoretinal lymphoma	Intravitreal	Filed NDA	10/21/2023	Yes	Yes
CT-P13	infliximab	Celltrion	Tumor necrosis factor blocker	Inflammatory bowel disease	SC	Filed BLA	10/22/2023	Yes	No
VBP-15	vamorolone	Santhera Pharmaceuticals	corticosteroid	Duchenne muscular dystrophy	PO	Filed NDA	10/26/2023	Yes	Yes
PF-06886992	meningococcal vaccine [A, B, C, Y, W-135]	Pfizer	vaccine	Meningococcal disease	IM	Filed BLA	10/28/2023	No	No
CSF-1	pilocarpine	Orasis Pharmaceuticals	cholinergic muscarinic receptor agonist	Presbyopia	OPH	Filed NDA	11/03/2023	No	No
NS-2 (ALDX-1E1, ADX-102)	reproxalap	Aldeyra Therapeutics	aldehyde antagonist	Dry eye disease	OP	Filed NDA	11/23/2023	No	No
OX-124	naloxone	Orexo	opioid antagonist	Opioid overdose	Intranasal	Filed NDA	12/03/2023	No	No
ACT-132577	aprocitentan	Idorsia	endothelin receptor antagonist	Hypertension	PO	Filed NDA	12/20/2023	No	No
APD-334	etrasimod	Pfizer/ Everest	S1P1 receptor agonist	Ulcerative colitis	PO	Filed NDA	12/21/2023	Yes	No
OTL-200	atidarsagene autotemcel	Orchard Therapeutics	gene therapy	Leukodystrophy	IV	InTrial	4Q2023	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	CRL	4Q2023	Yes	Yes
CTX-001 (Exa-cel)	exagamglogene autotemcel	CRISPR Therapeutics/ Vertex	gene editing (CRISPR)	Beta-thalassemia; sickle cell anemia	IV	InTrial	4Q2023	Yes	Yes
IDP-126	IDP-126	Bausch Health	retinoid/ antibiotic	Acne	TOP	InTrial	4Q2023	No	No
MGL-3196 (VIA-3196)	resmetirom	Madrigal	beta-selective thyroid hormone receptor agonist	Nonalcoholic steatohepatitis	PO	InTrial	4Q2023	Yes	No
LN-144	lifileucel	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Melanoma	IV	InTrial	4Q2023	Yes	Yes
LentiGlobin	lovotibeglogene autotemcel	bluebird bio	gene therapy	Sickle cell disease	IV	InTrial	4Q2023	Yes	Yes
Hepcludex	bulevirtide	Gilead	HBV receptor binder	Hepatitis delta virus	SC	CRL	2H2023	Yes	Yes
arimoclomol	arimoclomol	Orphazyme	cytoprotectives	Niemann-Pick disease	PO	CRL	2H2023	Yes	Yes
SPN-830	apomorphine	Supernus Pharmaceuticals	non-ergoline dopamine agonist	Parkinson's disease	SC infusion	CRL	2H2023	Yes	No
CUTX-101	copper histidinate	Fortress Biotech	copper replacement	Menkes Disease	SC	InTrial	2H2023	Yes	Yes
ALXN-1840 (WTX-101)	bis-choline tetrathiomolybdate	AstraZeneca	chelating agent	Wilson's disease	PO	InTrial	2H2023	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
GC-5107	human immunoglobulin	GC Biopharma	human immunoglobulin	Primary immunodeficiencies	IV	CRL	2023	Yes	No
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension	INH	Tentative Approval	2023	Yes	No
TAK-438	vonoprazan fumarate	Phantom Pharmaceuticals	potassium-competitive acid blocker	Erosive esophagitis	PO	CRL	2023	No	No
R-1646 (RO-4926219, AF-219, MK-7264)	gefapixant	Merck/ Roche	P2X3 antagonist	Chronic cough	PO	CRL	Late 2023	No	No
P-2B001 (P2-B001, P2B-001, P2B001)	pramipexole/ rasagiline	Pharma Two B	dopamine agonist/ monoamine oxidase B inhibitor	Parkinson's disease	PO	InTrial	Late 2023	No	No
TAK-755 (SHP-655)	TAK-755	Takeda	ADAMTS13 enzyme	Thrombotic thrombocytopenic purpura	IV	InTrial	Late 2023	Yes	Yes
NVX-CoV2373	coronavirus vaccine	Novavax	vaccine	Novel coronavirus disease 2019 (COVID-19)	IM	InTrial	Late 2023	No	No
PTC-AADC	eladocagene exuparvovec	PTC Therapeutics	gene therapy	Aromatic L-amino acid decarboxylase deficiency	Intracerebral	InTrial	Late 2023	Yes	Yes
Humacyl	human acellular vessel	Humacyte	cellular therapy	End-stage renal disease	Implant	InTrial	Late 2023	Yes	No
fruquintinib	fruquintinib	Hutchison China MediTech	VEGFR inhibitor	Colorectal cancer	PO	InTrial	Late 2023	Yes	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
iDose travoprost	travoprost	Glaukos	prostaglandin analog	Glaucoma/ Ocular hypertension	Intraocular	InTrial	Late 2023	No	No
YN-96D1	rivoceranib (apatinib)	Elevar Therapeutics	vascular endothelial growth factor receptor antagonist	Gastric cancer	PO	InTrial	Late 2023	Yes	Yes
Iomab-B	iodine I 131 monoclonal antibody BC8	Actinium	anti-CD45 monoclonal antibody	Acute myeloid leukemia	IV	InTrial	Late 2023	Yes	Yes
CPN-301	clobetasol propionate	Formosa Pharmaceuticals/ AimMax Therapeutics	corticosteroid	Eye inflammation/ pain	OPH	InTrial	Late 2023	No	No
Tirzepatide (for weight loss)	tirzepatide	Eli Lilly	glucose-dependent insulinotropic polypeptide receptor and glucagon-like peptide-1 receptor agonist	Chronic weight management	SC	InTrial	Late 2023	No	No
ACE-011	sotatercept	Merck	activin receptor type IIA-Fc fusion protein	Pulmonary arterial hypertension	SC	InTrial	Late 2023	Yes	Yes
ATRS-1902	hydrocortisone	Halozyme Therapeutics/ Antares Pharma	glucocorticoid	Acute adrenal insufficiency	IM/IV	InTrial	Late 2023	No	No
2024 Possible launch date									
CK-301	cosibelimab	Checkpoint Therapeutic	anti programmed cell death ligand 1	Cutaneous squamous cell carcinoma	IV	Filed BLA	01/04/2024	Yes	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
SB-206	SB-206	Novan Therapeutics	nitric oxide-releasing compound	Molluscum contagiosum	TOP	Filed NDA	01/06/2024	No	No
TC-002	latanoprost	TearClear	prostaglandin analog	Glaucoma	OPH	InTrial	1Q2024	No	No
NVK-002	atropine	Vyluma	anticholinergic	Myopia	OPH	InTrial	1Q2024	No	No
AXS-07	meloxicam/rizatriptan	Axsome Therapeutics	non-steroidal anti-inflammatory drug/triptan	Migraine	PO	CRL	1Q2024	No	No
Zeftera	ceftobiprole	Basilea	cephalosporin antibiotic	Bacterial infections	IV	InTrial	1Q2024	No	No
LY-686017	tradipitant	Vanda Pharmaceuticals	neurokinin 1 receptor antagonist	Gastroparesis	PO	InTrial	1Q2024	No	No
SDN-037	difluprednate	Visiox	corticosteroid	Ocular inflammation/pain	OPH	InTrial	1Q2024	No	No
ADP-A2M4 (MAGE-A4)	afamitresgene autoleucel	Adaptimmune	SPEAR T-cell therapy	Sarcoma	IV	InTrial	1Q2024	Yes	Yes
K-127	pyridostigmine	Amneal	cholinesterase inhibitor	Myasthenia gravis	PO	InTrial	1Q2024	No	No
STS-101	dihydroergotamine	Satsuma Pharmaceuticals	ergotamine	Migraine	Intranasal	InTrial	1Q2024	No	No
glatiramer acetate depot	glatiramer acetate long-acting	Viatris	immunomodulator	Multiple sclerosis	IM	InTrial	1Q2024	Yes	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
EB-101	EB-101	Abeona Therapeutics	gene therapy	Epidermolysis Bullosa	TOP	InTrial	1Q2024	Yes	Yes
ARQ-154	roflumilast	Arcutis Biotherapeutics	phosphodiesterase-4 inhibitor	Seborrheic dermatitis	TOP	InTrial	1Q2024	No	No
RP-L201	RP-L201	Rocket Pharmaceuticals	gene therapy	Leukocyte adhesion deficiency-I	IV	InTrial	1Q2024	Yes	Yes
ALPHA-1062	galantamine prodrug	Alpha Cognition	acetylcholinesterase inhibitor	Alzheimer's disease	PO	InTrial	2Q2024	No	No
LAI-287	insulin icodec	Novo Nordisk	ultra-long-acting basal insulin	Diabetes mellitus	SC	InTrial	1H2024	No	No
PB-2452	bentracimab	SFJ Pharmaceuticals	antiplatelet monoclonal antibody	Antiplatelet drug toxicity	IV	InTrial	1H2024	No	No
VNRX-5133	cefepime/ taniborbactam	VenatoRx Pharmaceuticals	cephalosporin/ beta-lactamase inhibitor	Bacterial infections	IV	InTrial	1H2024	Yes	No
PF-06838435 (SPK-9001)	fidanacogene elaparvovec	Pfizer/ Spark Therapeutics	gene therapy	Hemophilia B	IV	InTrial	1H2024	Yes	Yes
RPL-554	ensifentrine	Verona Pharma	phosphodiesterase-3 and phosphodiesterase-4 inhibitor	Chronic obstructive pulmonary disease	INH	InTrial	1H2024	Yes	No
GRN-163L	imetelstat	Geron	telomerase inhibitor	Myelofibrosis/ myelodysplastic syndrome/ acute myelogenous leukemia	IV	InTrial	1H2024	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
LY-3002813	donanemab	Eli Lilly	beta-amyloid monoclonal antibody	Alzheimer's disease	IV	CRL	1H2024	Yes	No
DAY-101	DAY-101	Day One Biopharmaceuticals	pan-Raf kinase inhibitor	Brain cancer	PO	InTrial	1H2024	Yes	Yes
D-PLEX100	doxycycline	PolyPid	tetracycline	Surgical site infections	IMPLANT	InTrial	1H2024	No	No
mRNA-1345	mRNA-1345	Moderna	vaccine	Respiratory syncytial virus	IM	InTrial	1H2024	No	No
CTP-543	deuruxolitinib	Sun Pharma/ Concert	janus kinase inhibitor	Alopecia areata	PO	InTrial	1H2024	Yes	No
PAX-101	suramin	PaxMedica	unknown	trypanosomiasis	IV	InTrial	Mid-2024	No	No
RTT-01	tiratricol	Egetis Therapeutics	thyroid-stimulating hormone receptor	Monocarboxylate transporter 8 deficiency	PO	InTrial	Mid-2024	Yes	Yes
X4P-001 (X-4P-001, X4-136, X4P-001-RD)	mavorixafor	X4 Pharma	CXC receptor type 4 inhibitor	WHIM syndrome	PO	InTrial	Mid-2024	Yes	Yes
RP-L102 (RPL-102)	RP-L102	Rocket Pharmaceuticals	gene therapy	Fanconi anemia	IV	InTrial	Mid-2024	Yes	Yes
SPI-014	lanthanum dioxycarbonate	Unicycive	phosphate binder	Hyperphosphatemia	PO	InTrial	Mid-2024	No	No
PF-06939926	fordadistrogene movaparvovec	Pfizer	gene therapy	Duchenne muscular dystrophy	IV	InTrial	Mid-2024	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
ADCT-301	camidanlumab tesirine	ADC Therapeutics/ Genmab	antibody drug conjugate	Hodgkin's Lymphoma	IV	InTrial	Mid-2024	Yes	No
Cx-601	darvadstrocel	Takeda	allogeneic stem cell therapy	Crohn's disease	IV	InTrial	Mid-2024	Yes	Yes
MSP-2017	etripamil	Milestone	calcium channel blocker	Arrhythmia	Intranasal	InTrial	Mid-2024	Yes	No
SNDX-5613	SNDX-5613	Syndax	Menin-mixed lineage leukemia 1 inhibitor	Acute myelogenous leukemia	PO	InTrial	Mid-2024	Yes	Yes
KarXT	xanomeline/ trospium	Karuna Therapeutics	muscarinic acetylcholine receptor agonist/ muscarinic receptor antagonist	Schizophrenia	PO	InTrial	Mid-2024	No	No
Obe-cel	obecabtagene autoleucel	Autolus Therapeutics	autologous chimeric antigen receptor T-cells	Acute lymphoblastic leukemia	IV	InTrial	Mid-2024	Yes	Yes
TAVT-45	abiraterone acetate	Tavanta Therapeutics	CYP17 inhibitor	Prostate cancer	PO	InTrial	3Q2024	Yes	No
FCX-007 (GM-HDF-COL7, INXN-3002)	dabocemagene autoficel	Castle Creek Pharmaceutical	gene-modified autologous fibroblast	Epidermolysis bullosa	Intradermal	InTrial	2H2024	Yes	Yes
CORT-125134	relacorilant	Corcept Therapeutics	glucocorticoid receptor II antagonist	Cushing's syndrome	PO	InTrial	2H2024	Yes	Yes
REGN-1979	odronextamab	Regeneron	CD20/CD3 monoclonal antibody	Follicular lymphoma/ diffuse large b-cell lymphoma	IV	InTrial	2H2024	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
AVB-S6-500	batiraxcept	Aravive Biologics	GAS6/AXL inhibitor	Ovarian cancer	IV	InTrial	2H2024	Yes	No
EBV-CTL (ATA-129)	tabelecleucel	Atara Biotherapeutics	cell therapy	Lymphoproliferative disorder	IV	InTrial	2H2024	Yes	Yes
REGN-5458	REGN-5458	Regeneron	BCMA and CD3 bispecific antibody inhibitor	Multiple myeloma	IV	InTrial	2H2024	Yes	No
Dasynoc	dasatinib	Xspray Pharma	kinase inhibitor	Chronic myeloid leukemia	PO	InTrial	2H2024	Yes	Yes
SEL-212	SVP-rapamycin/ pegsiticase	Selecta Biosciences/ 3SBio	synthetic vaccine particle (SVP)/ enzyme combination	Gout	IV	InTrial	2H2024	Yes	No
ALXN-2040	danicopan	AstraZeneca	complement factor D inhibitor	Paroxysmal nocturnal hemoglobinuria	PO	InTrial	2H2024	Yes	Yes
VAC-18193 (JNJ-64400141)	VAC-18193	Johnson & Johnson	vaccine	Respiratory syncytial virus	IM	InTrial	2H2024	No	No
XMT-1536	upifitamab rilsodotin	Mersana Therapeutics	antibody-drug conjugate	Ovarian cancer	IV	InTrial	2H2024	Yes	No
CT-053 (Zevor-cel)	CT-053	CARsgen Therapeutics	B-cell maturation antigen-directed genetically modified autologous T cell immunotherapy	Multiple myeloma	IV	InTrial	2H2024	Yes	Yes
BBP-305	encaleret	BridgeBio	Ca sensing receptor antagonist	Autosomal dominant hypocalcemia type 1	PO	InTrial	2H2024	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
ZW-25	zanidatamab	Zymeworks	HER2 monoclonal antibody	Biliary tract cancer	IV	InTrial	2H2024	Yes	Yes
Entyvio (SC formulation)	vedolizumab	Takeda	integrin receptor antagonist	Ulcerative colitis	SC	CRL	2024	Yes	No
Multikine	Leukocyte Interleukin (CS-001P3)	CEL-SCI	immunomodulator	Head and Neck cancer	SC	InTrial	2024	Yes	Yes
ND-0612H	levodopa/ carbidopa	NeuroDerm	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	SC	InTrial	2024	Yes	No
iMAB-362	zolbetuximab	Astellas	GC182 monoclonal antibody	Gastric adenocarcinoma	IV	InTrial	2024	Yes	Yes
pIL-12 (DNA IL-12)	tavokinogene telsaplasmid	OncoSec Medical	gene therapy	Melanoma	Intratumoral	InTrial	2024	Yes	Yes
NurOwn	autologous cultured mesenchymal bone marrow stromal cells secreting neurotrophic factors (MSC-NTF)	BrainStorm Cell Therapeutics	cellular therapy	Amyotrophic lateral sclerosis	IV	InTrial	2024	Yes	Yes
GSK-2140944	gepotidacin	GlaxoSmithKline	bacterial Type II topoisomerase inhibitor	Bacterial infections	PO/IV	InTrial	2024	No	No
AAI-101	cefepime/ enmetazobactam	Advanz/ Allecra	beta-lactam/b-lactamase inhibitor	Urinary tract infection	IV	InTrial	2024	No	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
NRX-101 (Cyclurad)	d-cycloserine/ lurasidone	NeuroRx	N-methyl-D-aspartate receptor modulator/ 5-HT2A receptor antagonist	Bipolar disorder	PO	InTrial	2024	No	No
OMS-721	narsoplimab	Omeros	anti-MASP-2 monoclonal antibody	Hematopoietic stem cell transplant-associated thrombotic microangiopathy	IV	CRL	2024	Yes	Yes
LNP-023	iptacopan	Novartis	factor B inhibitor	IgA nephropathy; paroxysmal nocturnal hemoglobinuria	PO	InTrial	2024	Yes	Yes
MT-7117	dersimelagon	Mitsubishi Tanabe Pharma	Undisclosed	Erythropoietic protoporphyria	PO	InTrial	2024	Yes	No
MOR-202	felzartamab	I-Mab	anti-CD38 monoclonal antibody	Multiple myeloma	IV	InTrial	2024	Yes	No
SAR-408701	SAR-408701	Sanofi	antibody-drug conjugate	Non-small cell lung cancer	IV	InTrial	2024	Yes	No
CF-101	piclidenoson	Can-Fite BioPharma	A3 adenosine receptor agonist	Plaque psoriasis	PO	InTrial	2024	Yes	No
AXS-14	S-reboxetine	Axsome Therapeutics	selective noradrenaline reuptake inhibitor	Fibromyalgia	PO	InTrial	2024	No	No
Mino-Lok	minocycline-EDTA-ETOH	Citrus	tetracyclines	Bacterial infection	Intracatheter	InTrial	2024	No	No
RG-6107	crovalimab	Roche	C5 inhibitor	Paroxysmal nocturnal hemoglobinuria	IV/SC	InTrial	2024	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
SGX-301	synthetic hypericin	Soligenix	synthetic hypericin	Cutaneous T-cell lymphoma	TOP	InTrial	2024	Yes	Yes
QGE-031	ligelizumab	Novartis	anti-IgE antibody	Chronic spontaneous urticaria	SC	InTrial	2024	Yes	No
ND-0612L	levodopa/ carbidopa	NeuroDerm	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	SC	InTrial	2024	Yes	No
RG-7433 (ABT-263)	navitoclax	AbbVie	Bcl-2 inhibitor	Myelofibrosis	PO	InTrial	2024	Yes	Yes
MT-1621	deoxythymidine/ deoxycytidine	UCB	deoxynucleoside	Thymidine kinase 2 deficiency	PO	InTrial	2024	Yes	Yes
Sativex	nabiximols	GW Pharmaceuticals/ Otsuka	cannabinoid product	Spasticity	PO	InTrial	2024	No	No
RG-6058	tiragolumab	Roche	TIGIT monoclonal antibody	Non-small cell lung cancer/ esophageal cancer	IV	InTrial	2024	Yes	No
TAK-935	soticlestat	Takeda	cholesterol 24-hydroxylase inhibitor	Lennox-Gastaut syndrome/ Dravet syndrome	PO	InTrial	2024	Yes	Yes
IONIS-APOCIII-LRx (ISIS-678354)	olezarsen	Ionis	antisense drug	Familial chylomicronemia syndrome	SC	InTrial	2024	Yes	No

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
SNDX-6352	axatilimab	Syndax Pharmaceuticals	colony stimulating factor 1 receptor monoclonal antibody	Graft vs. host disease	IV	InTrial	2024	Yes	Yes
Dihydroergotamine autoinjector	dihydroergotamine	Amneal Pharmaceuticals	ergot derivative	Migraine	SC	InTrial	2024	No	No
HP-5000	diclofenac	Hisamitsu Pharmaceutical	non-steroidal anti-inflammatory drug	Osteoarthritis	Transdermal	InTrial	2024	No	No
LY-03010	paliperidone	Luye Pharma	atypical antipsychotic	Schizophrenia	IM	InTrial	2024	No	No
SEP-363856 (SEP-856)	ulotaront	Sumitomo Dainippon Pharma	trace amine-associated receptor 1 agonist	Schizophrenia	PO	InTrial	Late 2024	No	No
ALN-APC (ALN-AT3)	fitusiran	Sanofi/ Alnylam	RNAi therapeutic	Hemophilia A and B	SC	InTrial	Late 2024	Yes	Yes
PRN-1008	rilzabrutinib	Sanofi	BTK inhibitor	Immune thrombocytopenia	PO	InTrial	Late 2024	No	Yes
MAT-2203	amphotericin B	Matinas BioPharma	fungicidal agent	Cryptococcal meningitis	PO	InTrial	Late 2024	No	Yes
RG-1594	ocrelizumab	Genentech	CD20-directed cytolytic antibody	Multiple sclerosis	SC	InTrial	Late 2024	Yes	No
CAM-2029	octreotide	Camurus	somatostatin analogue	Acromegaly	SC	InTrial	Late 2024	Yes	Yes
NBI-74788	crinecerfont	Neurocrine Biosciences	CRF receptor antagonist	Congenital adrenal hyperplasia	PO	InTrial	Late 2024	Yes	Yes

Drug name	Generic name	Company	Mechanism of Action	Disease State	Route	FDA Status	Estimated release date	Specialty drug	Orphan drug
ABBV-399	telisotuzumab	AbbVie	antibody (anti-c-Met)-drug conjugate	Non-small cell lung cancer	IV	InTrial	Late 2024	Yes	No

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

Key pending indication forecast

Optum Rx key pending indication forecast

Brand name	Generic name	Company	Mechanism of Action	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Kevzara	sarilumab	Regeneron/ Sanofi	interleukin-6 receptor monoclonal antibody	Polymyalgia rheumatica	Treatment of polymyalgia rheumatica	SC	02/28/2023
Lynparza	olaparib	AstraZeneca/ Merck	poly (ADP-ribose) polymerase inhibitor	Prostate cancer	In combination with abiraterone and prednisone or prednisolone, for treatment of adult patients with metastatic castration-resistant prostate cancer	PO	03/16/2023
Evkeeza	evinacumab	Regeneron	angiopoietin-like 3 inhibitor	Homozygous familial hypercholesterolemia (pediatric patients)	As an adjunct to other lipid-lowering therapies to treat children aged 5 to 11 years with homozygous familial hypercholesterolemia	IV	03/30/2023
Tafinlar	dabrafenib	Novartis	kinase inhibitor	Brain tumors	In combination with Mekinist (trametinib), for the treatment of low-grade glioma and high-grade glioma in pediatric patients	PO	1Q2023
Injectafer	ferric carboxymaltose	Daiichi Sankyo	iron replacement product	Chronic heart failure - anemia	Treatment of heart failure and iron deficiency, either with or without anemia	IV	1Q2023
Polivy	polatuzumab vedotin-piiq	Genentech	CD79b-directed antibody-drug conjugate	Diffuse large B-cell lymphoma	In combination with Rituxan (rituximab) plus cyclophosphamide, doxorubicin and prednisone (R-CHP) for the treatment of people with previously untreated diffuse large B-cell lymphoma	IV	04/02/2023

Brand name	Generic name	Company	Mechanism of Action	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Qulipta	atogepant	AbbVie	calcitonin gene-related peptide receptor antagonist	Chronic migraine prophylaxis	Preventive treatment of chronic migraine in adults	PO	04/21/2023
Padcev	enfortumab vedotin-ejfv	Seagen	nectin-4-directed antibody and microtubule inhibitor conjugate	Urothelial cancer (with Keytruda)	In combination with Keytruda (pembrolizumab), for treatment of patients with locally advanced or metastatic urothelial cancer who are not eligible to receive cisplatin-containing chemotherapy	IV	04/21/2023
Sogroya	somapacitan-beco	Novo Nordisk	growth hormone analog	Pediatric growth hormone deficiency	Treatment of pediatric growth hormone deficiency	SC	04/28/2023
Trikafta	elexacaftor/tezacaftor/ivacaftor; ivacaftor	Vertex	cystic fibrosis transmembrane conductance regulator modulators	Cystic fibrosis (pediatric)	Treatment of cystic fibrosis in patients aged 2 years and older who have at least one F508del mutation in the CFTR gene or a mutation in the CFTR gene that is responsive based on in vitro data	PO	04/28/2023
Kalydeco	ivacaftor	Vertex	cystic fibrosis transmembrane conductance regulator potentiator	Cystic fibrosis (pediatric expansion)	Treatment of cystic fibrosis in patients age 1 month and older who have one mutation in the CFTR gene that is responsive to ivacaftor based on clinical and/or in vitro assay data	PO	05/03/2023
Rexulti	brexpiprazole	Otsuka/ Lundbeck	serotonin-dopamine activity modulator	Alzheimer's disease	Treatment for agitation in patients with Alzheimer's disease	PO	05/10/2023
Ayvakit	avapritinib	Blueprint Medicines	selective KIT and PDGFRa inhibitor	Indolent systemic mastocytosis	Treatment of adults with indolent systemic mastocytosis	PO	05/22/2023
Rinvoq	upadacitinib	AbbVie	Janus associated kinase inhibitor	Crohn's disease	Treatment of Crohn's disease	PO	05/26/2023

Brand name	Generic name	Company	Mechanism of Action	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Prevymis	letermovir	Merck	CMV DNA terminase complex inhibitor	Cytomegalovirus	Prevention of cytomegalovirus disease in adults who receive kidney transplants	PO/IV	06/05/2023
Linzess	linaclotide	AbbVie	guanylate cyclase-C agonist	Functional constipation	Treatment of children and adolescents 6 to 17 years of age with functional constipation	PO	06/14/2023
Bylvay	odevixibat	Albireo	ileal bile acid transporter inhibitor	Alagille syndrome	Treatment of Alagille syndrome	PO	06/15/2023
Camzyos	mavacamten	Bristol Myers Squibb	cardiac myosin inhibitor	Reduce septal reduction therapy	To reduce the need for septal reduction therapy in adults with symptomatic New York Heart Association (NYHA) class II-III obstructive hypertrophic cardiomyopathy	PO	06/16/2023
Adbry	tralokinumab-ldrm	Leo Pharma	interleukin-13 antagonist	Atopic dermatitis (adolescents)	Treatment of moderate-to-severe atopic dermatitis in adolescents patients whose disease is not adequately controlled with topical prescription therapies or when those therapies are not advisable	SC	2Q2023
Farxiga	dapagliflozin	AstraZeneca	sodium glucose cotransporter-2 inhibitor	Heart failure with preserved ejection fraction	Treatment of patients with heart failure with preserved ejection fraction	PO	2Q2023
Ultomiris	ravulizumab-cwvz	AstraZeneca	C5 complement inhibitor	Neuromyelitis optica	Treatment of neuromyelitis optica	IV	Mid-2023
Talzenna	talazoparib	Pfizer	poly (ADP-ribose) polymerase inhibitor	Prostate cancer	In combination with Xtandi (enzalutamide), for the treatment of metastatic castration-resistant prostate cancer	PO	Mid-2023

Brand name	Generic name	Company	Mechanism of Action	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Voxzogo	vosoritide	BioMarin	C type natriuretic peptide analog	Achondroplasia (< 5 years)	To increase linear growth in pediatric patients with achondroplasia who are under 5 years of age	SC	07/03/2023
Eylea	aflibercept	Regeneron	vascular endothelial growth factor-A (VEGF-A) inhibitor/ placental growth factor (PlGF) inhibitor	Macular degeneration/ diabetic macular edema (high-dose)	High-dose Eylea for wet age-related macular degeneration and diabetic macular edema	Intravitreal	07/09/2023
Rubraca	rucaparib	Clovis Oncology	poly (ADP-ribose) polymerase inhibitor	Ovarian cancer	First-line maintenance treatment for women with advanced ovarian cancer regardless of biomarker status who have responded to first-line platinum-based chemotherapy	PO	07/13/2023
Livmarli	maralixibat	Mirum Pharmaceuticals	ileal bile acid transporter inhibitor	Progressive familial intrahepatic cholestasis	Treatment of pruritus in patients with progressive familial intrahepatic cholestasis	PO	08/14/2023
Daxxify	daxibotulinumtoxinA-lanm	Revance Therapeutics	acetylcholine release inhibitor/ neuromuscular blocking agent	Cervical dystonia	Treatment of cervical dystonia	IM	08/19/2023
Ingrezza	valbenazine	Neurocrine Biosciences	vesicular monoamine transporter 2 inhibitor	Huntington's disease	Treatment of Huntington's disease chorea	PO	08/20/2023
Cosentyx	secukinumab	Novartis	interleukin-17 receptor antagonist	Hidradenitis suppurativa	Treatment of hidradenitis suppurativa	SC	08/31/2023
Prevnar 20	pneumococcal 20-valent conjugate	Pfizer	vaccine	Pneumococcal disease (pediatric)	Active immunization for the prevention of pneumonia and invasive disease in pediatric patients	IM	08/31/2023

Brand name	Generic name	Company	Mechanism of Action	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Jardiance	empagliflozin	Boehringer Ingelheim/ Eli Lilly	sodium-dependent glucose transporter 2 inhibitor	Chronic kidney disease	To reduce kidney disease progression and cardiovascular mortality risk in patients with chronic kidney disease	PO	09/20/2023
Leqembi	lecanemab-irmb	Eisai/ Biogen	amyloid beta-directed antibody	Alzheimer's disease (full approval)	Treatment of Alzheimer’s disease (accelerated approval to full approval)	IV	3Q2023
Onpattro	patisiran	Alnylam	RNAi therapeutic	Transthyretin amyloidosis with cardiomyopathy	Treatment of transthyretin amyloidosis patients with cardiomyopathy	IV	10/08/2023
Zoryve	roflumilast	Arcutis Biotherapeutics	phosphodiesterase-4 inhibitor	Plaque psoriasis (children ages 2 to 11)	Treatment of plaque psoriasis in children ages 2 to 11	TOP	10/19/2023
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 inhibitor	Chronic spontaneous urticaria	Treatment of chronic spontaneous urticaria in biologic-naïve patients	SC	10/2023

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