RxOutlook®

3rd Quarter 2022

Optum Rx®

In this edition of RxOutlook, we highlight 6 key pipeline drugs with an expected FDA decision by the end of the fourth quarter of 2022. Of note, 4 of the 6 drugs discussed have an Orphan Drug designation, including **etranacogene dezaparvovec**, which would potentially be the first gene therapy approved for hemophilia B. Gene therapies for hemophilia represent a potential opportunity to significantly reduce or eliminate the need for chronic prophylactic factor IX replacement therapy in some patients. Other gene therapies are in development for both hemophilia A and B and could be on the market in 2023 or 2024.

Two of the orphan drugs discussed are administered orally and will represent potential high-cost specialty drugs on the pharmacy benefit. **Sparsentan** is a first-in-class dual endothelin angiotensin receptor antagonist for the treatment of IgA nephropathy. Sparsentan would potentially be the first non-steroidal drug approved for the rare kidney disease. **Palovarotene**, a selective retinoic acid gamma receptor agonist, would potentially be the first treatment for fibrodysplasia ossificans progressiva, an ultra-rare disease characterized by abnormal development of bone in areas of the body where bone is not normally present (eg, ligaments, tendons).

Rounding out the list of orphan drugs is Amicus' combination regimen of **miglustat plus cipaglucosidase alfa** for Pompe disease. This regimen would represent an alternative to Sanofi's enzyme replacement therapies for the condition (Lumizyme® and Nexviazyme®).

The remaining two drugs include **omidenepag isopropyl**, a novel eye drop for open-angle glaucoma and ocular hypertension and **poziotinib**, an oral kinase inhibitor for the treatment of non-small cell lung cancer (NSCLC) with HER2 exon 20 insertion mutations. Poziotinib would be the first approved treatment for this specific subset of patients with NSCLC.

Approval decisions for other key novel therapies are expected in the fourth quarter of 2022 but are not reviewed in this report because they were covered in previous editions of RxOutlook. These include: olipudase alfa for acid sphingomyelinase deficiency; teplizumab for delay of type 1 diabetes mellitus; lenacapavir for multi-drug resistant HIV-1 infection; and ublituximab for multiple sclerosis.

Key pipeline drugs with FDA approval decisions expected by end of the 4th quarter 2022

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Miglustat plus cipaglucosidase alfa	Amicus	Pompe disease*	8/29/2022 (miglustat) 10/29/2022 (cipaglucosidase alfa)
Omidenepag isopropyl	Santen Pharmaceutical	Open-angle glaucoma	11/6/2022
Sparsentan	Travere Therapeutics	IgA nephropathy*	11/17/2022
Etranacogene dezaparvovec	CSL Behring/uniQure	Hemophilia B*	11/24/2022

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Poziotinib	Spectrum Pharmaceuticals	Non-small cell lung cancer	11/24/2022
Palovarotene	Ipsen	Fibrodysplasia ossificans progressiva*	12/29/2022

* Orphan Drug Designation

OptumRx closely monitors and evaluates the drug development pipeline to identify noteworthy upcoming drug approvals and reports the essential findings here in RxOutlook. The report is organized in the following manner:

Detailed Drug Insights

This section reviews the important characteristics (eg, therapeutic use, clinical profile, competitive environment and regulatory timeline) for key pipeline drugs with potential FDA approvals by the end of the 4th quarter 2022.

Read more

Extended Generic Pipeline Forecast

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

Read more

Extended Brand Pipeline Forecast

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

Read more

Key Pending Indication Forecast

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

Read more

Past and future reviews

Please note that RxOutlook highlights select near-term approvals. Some drugs may not appear in this issue because they have been reviewed in previous editions of RxOutlook. Drugs of interest that are earlier in development or with expected approvals beyond 4th quarter 2022 may appear in future reports; however, for those who need an initial look at the full pipeline, please refer to the Brand Pipeline Forecast Table found later in this report.

Getting acquainted with pipeline forecast terms

Clinical trial phases

Phase I trials	Researchers test an experimental drug or treatment in a small group of people for the first time to evaluate its safety, determine a safe dosage range, and identify side effects.
Phase II trials	The experimental study drug or treatment is given to a larger group of people to see if it is effective and to further evaluate its safety.
Phase III trials	The experimental study drug or treatment is given to large groups of people to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, and collect information that will allow the experimental drug or treatment to be used safely.
Phase IV trials	Post marketing studies delineate additional information including the drug's risks, benefits, and optimal use.

Pipeline acronyms

ANDA	Abbreviated New Drug Application
BLA	Biologic License Application
CRL	Complete Response Letter
FDA	Food and Drug Administration
MOA	Mechanism of Action
NME	New Molecular Entity
NDA	New Drug Application
sBLA	Supplemental Biologic License Application
sNDA	Supplemental New Drug Application
OTC Drugs	Over-the-Counter Drugs
PDUFA	Prescription Drug User Fee Act
REMS	Risk Evaluation and Mitigation Strategy

Detailed Drug Insights

Cipaglucosidase alfa plus miglustat (Brand Name: To be determined)

Manufacturer: Amicus Therapeutics
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: August 29, 2022 (miglustat), October 29, 2022 (cipaglucosidase alfa)

Therapeutic use

- Treatment of late-onset Pompe disease

Cipaglucosidase alfa plus miglustat is a two-component regimen that is being developed for the treatment of late-onset Pompe disease.

Pompe disease is a rare neuromuscular genetic disorder caused by a defect in the lysosomal acid alpha-glucosidase (GAA) enzyme, which leads to a build-up of glycogen in all tissues, especially skeletal, cardiac, and smooth muscle tissues. The build-up of glycogen can result in tissue damage and ultimately organ dysfunction.

Pompe disease affects approximately 1 in 28,000 newborn infants in the U.S. The severity of the disease and age of onset depends on the degree of enzyme deficiency. Infantile-onset Pompe disease and late-onset Pompe disease are the two clinical subtypes of the condition. Symptoms of infantile-onset Pompe disease begin in the first months of life and most infants will die from cardiac and respiratory complications by the first year of life. Late-onset Pompe disease, can occur later in childhood, adolescence or even adulthood and is characterized by progressive muscle weakness, particularly in the legs and trunk, and muscles that control breathing; respiratory failure as the most common cause of death.

Clinical profile

- Enzyme replacement therapy
- Oral (miglustat) + IV infusion (cipaglucosidase alfa)
- Change in 6MWD: 20.8 meters vs 7.2 meters with Lumizyme (no statistical superiority)
- Common AEs: Falls, headache, nasopharyngitis, myalgia, arthralgia
- Dosing: Miglustat 1 hour before cipaglucosidase alfa every 2 weeks

This two-component regimen includes intravenous cipaglucosidase alfa and oral miglustat. Cipaglucosidase alfa is a novel human recombinant human GAA enzyme replacement; Miglustat binds to and stabilizes cipaglucosidase alfa to prolong its distribution half-life and increase the amount of active therapeutic drug that is delivered to muscles. Cipaglucosidase alfa is different from the other available enzyme replacement therapies (ERT) as the other products are administered as monotherapies.

Pivotal trial data:
The efficacy of cipaglucosidase alfa plus miglustat was evaluated in a 52-week, Phase 3, randomized study in 125 patients with late-onset Pompe disease. Patients were randomized to receive cipaglucosidase alfa plus oral miglustat or another ERT, Lumizyme® (alglucosidase alfa) plus placebo. Patients were stratified by baseline 6-minute walk distance (6MWD) and previous ERT status. The primary endpoint was change from baseline in 6MWD and the key secondary endpoint was change in lung function, measured through forced vital capacity (FVC).

At week 52, mean change from baseline in 6MWD was 20.8 meters in the cipaglucosidase alfa plus miglustat group vs. 7.2 meters in the Lumizyme plus placebo group. The between group difference of 13.6 meters (95% CI: -2.8, 29.9; p = 0.071) did not reach statistical superiority. The mean change from baseline at week 52 in sitting FVC (percent predicted) was -0.9% for the cipaglucosidase alfa plus miglustat group vs. -4.0% in the alglucosidase alfa plus placebo group, with the between group difference of 3.0% (95% CI: 0.7, 5.3; p = 0.023).

Cipaglucosidase alfa plus miglustat (continued…)

Safety:
The safety profile of cipaglucosidase alfa plus miglustat was similar to that of alglucosidase alfa plus placebo. The most frequently reported treatment-emergent adverse events were fall, headache, nasopharyngitis, myalgia, arthralgia, and nausea. The incidence of infusion-associated reactions was similar between the two groups as well.

Dosing:
Each product of this two-component therapy is dosed by body weight. Miglustat is taken orally 1 hour before the IV infusion of cipaglucosidase alfa, and the regimen is administered once every 2 weeks.

Miglustat is currently available generically as a 100 mg capsule for the treatment of Gaucher's disease. In the pivotal trial for Pompe disease, the dosing of miglustat studied was 3 or 4 capsules of miglustat 65 mg depending on body weight.

Competitive environment

If approved, Amicus Therapeutics' cipaglucosidase alfa would provide an additional treatment option for Pompe disease and a potential competitor to Sanofi's Lumizyme and Nexviazyme® (avalglucosidase alfa-ngpt).

Although the regimen did not show statistical superiority over Lumizyme with the primary efficacy endpoint in the overall study population, cipaglucosidase alfa plus miglustat had a numerically higher 6MWD than Lumizyme.

Additionally, since the trial included ERT-experienced patients, cipaglucosidase alfa plus miglustat could be a treatment option for patients previously treated with Lumizyme. Sustained efficacy in improvement of 6MWD and FVC will be analyzed in ongoing long-term studies.

The proposed initial indication of treatment of late-onset Pompe disease for cipaglucosidase alfa plus miglustat could potentially be limited to adults since only adults were included in the pivotal trial. The standard of care for Pompe disease is Lumizyme, which is approved for patients with either infantile onset or late onset Pompe disease. Sanofi's other ERT, Nexviazyme, is approved in pediatric patients 1 year of age and older with late-onset Pompe disease.

For reference, the Wholesale Acquisition Cost (WAC) for Nexviazyme is approximately $625,000 per year for an adult patient weighting 70 kg. Since these therapies are weight-based, the cost would be lower for pediatric patients.

- Advantages: Another competitor in Pompe disease marketplace, treatment option for ERT-experienced patients
- Disadvantages: primary efficacy endpoint was not statistically significant, initial narrow indication
- Reference WAC (Nexviazyme): ~$625,000 per year (70 kg patient)

Omidenepag isopropyl (Brand Name: To be determined)

Manufacturer: Santen Pharmaceutical
Expected FDA decision: November 6, 2022

Therapeutic use

Omidenepag isopropyl is under review for treatment of open-angle glaucoma (OAG) and ocular hypertension.

OAG is an eye disease where an increase in eye pressure can lead to damage to the eye's optic nerve and that results in vision loss and blindness if untreated. Glaucoma is a leading cause of blindness for people over 60 years old. OAG affects approximately 3 million people in the U.S.

- Treatment of OAG and ocular hypertension

Clinical profile

Omidenepag isopropyl is a non-prostaglandin, prostanoid E receptor 2 agonist. Omidenepag isopropyl reduces eye pressure by increasing aqueous humor drainage (helps increase fluid drain from the eye).

- Prostanoid E receptor 2 agonist
- Ophthalmic formulation
- Noninferiority demonstrated vs. timolol and latanoprost
- Common AEs: Conjunctival hyperemia, corneal thickening, ocular hyperemia
- Dosing: Once daily

Pivotal trial data:
The efficacy of omidenepag was evaluated in a series of randomized, active-controlled studies that compared omidenepag vs. current standards of care in patients with OAG and ocular hypertension. SPECTRUM-4 (N = 409) was a head-to-head study comparing omidenepag vs. timolol, a beta blocker, and then PEONY (N = 370) and AYAME (N = 253) were studies comparing omidenepag vs. latanoprost, a first-line prostaglandin analog. The primary endpoint across the studies was intraocular pressure (IOP). Across all three studies, omidenepag was shown to be noninferior to the active controls for reductions in IOP at month 3.

Safety:
The most common adverse events with omidenepag use were conjunctival hyperemia, corneal thickening, and ocular hyperemia.

Dosing:
In the pivotal trials, omidenepag was administered as one drop in the affected eye(s) once daily.

Omidenepag isopropyl (continued…)

Competitive environment

Omidenepag would provide an additional option for the treatment of OAG with a novel mechanism of action (MOA). Currently marketed first-line prostaglandin analogs act on the prostanoid FP receptors. Due to the difference in mechanism, omidenepag use is not linked to prostaglandin-associated periorbitopathy adverse events that can occur with current prostaglandin analogs. These adverse events are usually cosmetic (eg, increase in the number and length of eyelashes, changes in iris and lash pigmentation) but they may also cause more structural changes (eg, deepening of the upper eyelid sulcus) that can limit future surgical procedures on the eyes. Cosmetic adverse events can be a larger concern when patients are affected with unilateral glaucoma since the cosmetic changes would only impact one eye.

While there may be some safety or tolerability advantages in some patients with glaucoma, there is a lack of evidence suggesting that omidenepag has superior clinical efficacy vs. existing standards of care. Omidenepag will also be entering the market at a time when many alternatives are available across different MOAs, including generic first-line products. Due to this high generic penetration and well-established alternatives, the likely place in therapy for omidenepag is as a second- or third-line product for treatment of OAG.

For reference, the WAC for Rhopressa® (netarsudil), the last novel drug approved for glaucoma, is approximately $3,600 per year.

- Advantages: Novel MOA, lack of prostaglandin-associated periorbitopathy adverse events, once daily administration
- Disadvantages: Alternatives available across different mechanisms of action including generic first-line products, lack of superiority data
- Reference WAC (Rhopressa): ~$3,600 per year

Sparsentan (Brand Name: To be determined)

Manufacturer: Travere Therapeutics
Regulatory designations: Orphan Drug
Expected FDA decision: November 17, 2022

Therapeutic use

- Treatment of IgA nephropathy

Sparsentan is under review for the treatment of immunoglobulin A (IgA) nephropathy.

IgA nephropathy, also known as Berger's disease, is a kidney disease that occurs when IgA deposits build up in the kidneys, causing inflammation that damages kidney tissues. As a result, the kidneys begin to let substances such as blood and protein leak into the urine. Over time, IgA nephropathy can lead to end-stage kidney disease (ESKD) and the need for dialysis. The condition can occur at any age, although the first evidence of kidney disease most frequently appears when people are in their teens to late 30s.

IgA nephropathy is one of the most common kidney diseases, other than those caused by diabetes or high blood pressure. The disease affects over 100,000 people in the U.S. Travere Therapeutics estimates that there are approximately 30,000 to 50,000 patients addressable at launch.

Clinical profile

- Dual endothelin angiotensin receptor antagonist
- Oral formulation
- Mean reduction in proteinuria: 49.8% vs. 15.1% with irbesartan
- Safety data limited
- Dosing: Once daily

Sparsentan is a dual endothelin angiotensin receptor antagonist. Endothelin I and angiotensin II have a role in kidney function decline by contributing to inflammation and fibrosis in the kidney and both endothelin I and angiotensin II are also vasoconstrictive, meaning they cause a narrowing of blood vessels and an increase in pressure in the kidney.

Pivotal trial data:
The efficacy of sparsentan is being evaluated in the ongoing PROTECT study, a randomized, double-blind, active-control trial in 404 adults with IgA nephropathy. Patients with persistent proteinuria despite active angiotensin-converting enzyme (ACE) inhibitor or angiotensin II receptor blocker (ARB) treatment were randomized to receive either sparsentan or an ARB (irbesartan). The study protocol provided for an unblinded interim analysis to evaluate the primary efficacy endpoint – change in proteinuria (urine protein-to-creatinine ratio) from baseline at week 36. Secondary efficacy endpoints include the rate of change in estimated glomerular filtration rate (eGFR) following the initiation of randomized treatment over 58-week and 110-week periods, as well as the rate of change in eGFR over 52-week and 104-week periods following the first six weeks of randomized treatment.

Sparsentan met the prespecified interim primary endpoint with statistical significance, demonstrating a greater than three-fold reduction of proteinuria from baseline after 36 weeks of treatment, compared to irbesartan. After 36 weeks of treatment, patients receiving sparsentan achieved a mean reduction in proteinuria from baseline of 49.8% vs. 15.1% for irbesartan-treated patients ($p < 0.0001$). eGFR data is not yet available.

Sparsentan (continued…)

Safety:
Sparsentan appears to be well tolerated but the safety data is limited.

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

Competitive environment

Sparsentan would potentially be the first non-steroidal drug approved for treatment of IgA nephropathy. The current standard of care includes off-label use of ACE inhibitors and ARBs which can help prevent progression of the disease by reducing proteinuria and lowering blood pressure. Corticosteroids are also used and they work by reducing inflammation. Despite available therapies, there is still a high unmet need. In the pivotal trial, sparsentan demonstrated promising improvements in proteinuria vs. a standard of care treatment option.

Unlike many other orphan conditions, there are established alternative treatments for IgA nephropathy. The pivotal trial only included patients with persistent proteinuria despite active ACE inhibitor or ARB treatment so in practice, sparsentan could be reserved as a second-line therapy after inadequate response to these drugs. There is also limited drug safety information and most importantly, a lack of efficacy data showing improvements in kidney function.

Finally, sparsentan is also in development for focal segmental glomerulosclerosis, another rare kidney disease with a high unmet need.

For reference, the WAC for Tarpeyo® (budesonide), a corticosteroid approved for IgA nephropathy, is approximately $172,000 per year.

- Advantages: Potentially the first non-steroidal drug FDA approved for IgA nephropathy, promising improvements based on large reductions in proteinuria, also in development for focal segmental glomerulosclerosis
- Disadvantages: Existing treatments available including ACE inhibitors/ARBs and corticosteroids, likely reserved for patients who need treatment beyond standard of care, unknown safety and lack of kidney function data
- Reference WAC (Tarpeyo): ~$172,000 per year

Etranacogene dezaparvovec (Brand Name: To be determined)

Manufacturer: CSL Behring/uniQure
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: November 24, 2022

Therapeutic use

Etranacogene dezaparvovec is under review for treatment of adult patients with moderately severe to severe hemophilia B.

- Treatment of patients with moderately severe to severe hemophilia B

Hemophilia B is an inherited bleeding disorder in which the blood does not clot properly. Blood contains many proteins called clotting factors that can help to stop bleeding. People with hemophilia B have low levels of factor IX (FIX). Hemophilia B is classified as mild, moderate, or severe based upon the activity level of FIX. In mild cases, bleeding symptoms may occur only after surgery, injury, or a dental procedure. In some moderate and most severe cases, bleeding symptoms may occur after a minor injury or spontaneously.

Hemophilia B occurs in approximately 1 in 25,000 male births. It is less common than hemophilia A which occurs in approximately 1 in 5,000 male births.

Clinical profile

Etranacogene dezaparvovec is a gene therapy that consists of an AAV5 viral vector carrying a gene cassette with the Padua variant of factor IX (FIX-Padua). FIX-Padua generates FIX proteins that are 5x-8x more active than normal.

- Gene therapy
- IV formulation
- Reduction in all bleeds: 64%; reduction in FIX-treated bleeds: 77%
- Common AEs: Transaminase elevation, infusion-related reactions, headache, influenza-like symptoms
- Dosing: One-time dose

Pivotal trial data:
The efficacy of etranacogene dezaparvovec was evaluated in an open-label, single-arm study in 54 patients with adult hemophilia B patients classified as severe or moderately severe (defined as less than or equal to 2% of normal FIX activity) and requiring prophylactic FIX replacement therapy. The study included a prospective, 6-month observational period during which patients continued to use their current standard of care therapy to establish a baseline annualized bleeding rate (ABR). After the lead-in period, patients received a single administration of etranacogene dezaparvovec. The primary endpoint was 52-week ABR after achievement of stable FIX expression compared with the 6-month lead-in period. For this endpoint, ABR was measured from month 7 to month 18 after infusion, ensuring the observation period represented likely steady-state FIX transgene expression.

After the lead-in period post-infusion, the adjusted ABR for all bleeds was reduced by 64% ($p = 0.0002$) and all FIX-treated bleeds was reduced by 77% ($p < 0.0001$) over months 7 to 18. In addition, 98% of patients treated with a full dose of etranacogene dezaparvovec discontinued use of prophylaxis. Etranacogene dezaparvovec produced mean FIX activity of 39.0 IU/dL at 6 months and 36.9 IU/dL at 18 months post infusion.

Etranacogene dezaparvovec (continued…)

Safety:
The most common adverse events with etranacogene dezaparvovec use were transaminase elevation, infusion-related reactions, headache, and influenza-like symptoms.

Dosing:
In the pivotal trial, etranacogene dezaparvovec was administered intravenously as a one-time dose.

Competitive environment

The current standard of care for patients with severe hemophilia B is chronic FIX prophylactic treatment. If approved, etranacogene dezaparvovec would be the first one-time gene therapy for hemophilia B and it would reduce, and in some cases eliminate, the need for chronic and as-needed FIX replacement therapy. FIX replacement therapy has a high treatment burden and can be very costly particularly in severe patients requiring high doses or prophylactic use of FIX.

Like other gene therapies, particularly for hemophilia, the primary limitation or question is the unknown durability of response. Etranacogene dezaparvovec did significantly reduce the need for FIX replacement therapy but it did not completely eliminate the risk of bleeding events. Over time, FIX activity may decrease post-infusion which could result in reduced efficacy in terms of reductions in bleed events. Sustained efficacy is especially important with gene therapies because of the high projected cost for a one-time dose.

Other gene therapies for hemophilia B are in development and could be available in 2024 to 2025. There may be some patients that are eligible and willing to be treated with gene therapy but who could wait until data is available for these competitors rather than choosing to be early adopters for etranacogene dezaparvovec.

For reference, the WAC for Zolgensma® (onasemnogene abeparvovec-xioi), a gene therapy for spinal muscular atrophy, is approximately $2.1 million for a one-time dose.

- Advantages: Potentially the first gene therapy for hemophilia, eliminates or reduces the need for chronic and as-needed FIX replacement therapy
- Disadvantages: Unclear durability of response, potential future competition
- Reference WAC (Zolgensma): $2.1 million for a one-time dose

Poziotinib (Brand Name: To be determined)

Manufacturer: Spectrum Pharmaceuticals
Regulatory designations: Fast Track
Expected FDA decision: November 24, 2022 *(FDA Advisory Committee scheduled for September 22, 2022)*

Therapeutic use

Poziotinib is under review for treatment of previously treated patients with non-small cell lung cancer (NSCLC) with HER2 exon 20 insertion mutations.

Lung cancer is the second most common cancer in the U.S. (not counting skin cancer). The American Cancer Society estimates about 236,740 new cases of lung cancer in 2022 with NSCLC accounting for 84% of cases. Several oncogenetic driver mutations have been identified in NSCLC. HER2 exon 20 mutations account for approximately 2.7% of cases with Spectrum Pharmaceuticals estimating about 2,300 new cases per year in the U.S.

- Treatment of previously treated patients with NSCLC with HER2 exon 20 insertion mutations

Clinical profile

Poziotinib is an irreversible tyrosine kinase inhibitor targeting EGFR and HER2 with exon 20 insertion mutations, inhibiting proliferation and leading to cancer cell death.

Pivotal trial data:
The efficacy of poziotinib is being evaluated in ZENITH20, a Phase 2, open-label, multi-cohort study in patients with NSCLC with EGFR or HER2 exon 20 insertion mutations. The current FDA submission was based on data from Cohort 2 in 90 patients with previously treated NSCLC with HER2 exon 20 insertions. The primary endpoint was objective response rate (ORR). The intent-to-treat analysis demonstrated an ORR of 27.8% (95% CI: 18.9, 38.2). The median duration of response was 5.1 months, and the median progression free survival (PFS) was 5.5 months.

Safety:
The most common adverse events with poziotinib use were rash, stomatitis, diarrhea, and paronychia.

Dosing:
In the pivotal trial, poziotinib was administered orally once or twice daily.

- EGFR and HER2 kinase inhibitor
- Oral formulation
- ORR: 27.8%
- Median PFS: 5.5 months
- Common AEs: Rash, stomatitis, diarrhea, paronychia
- Dosing: Once or twice daily

Poziotinib (continued…)

Competitive environment

If approved, poziotinib would be the first targeted therapy for NSCLC with HER2 exon 20 insertion mutations. Poziotinib would offer an oral, once daily treatment option in an area of unmet need. Other oral kinase inhibitors are currently approved but for different subsets of NSCLC patients (eg, Exkivity® [mobocertinib] for EGFR exon 20 insertion mutations). While treatment guidelines do not have specific recommendations for patients with HER2 exon 20 mutations, off-label Kadcyla® (ado-trastuzumab emtansine) and Enhertu® (fam-trastuzumab deruxtecan-nxki) are recommended in the broader HER2 NSCLC population based on Phase 2 data.

The FDA submission for poziotinib is based solely on a small, single-arm clinical trial and robust overall survival data are not available. The ORR rate was relatively modest in the Phase 2 study and 12% of patients had to permanently discontinue therapy due to adverse events. Additionally, the target population is expected to be limited given the likely narrow initial indication. A Phase 3 trial was initiated comparing poziotinib vs. chemotherapy (docetaxel) but results are not expected in the near term.

For reference, the WAC for Exkivity is approximately $24,000 per 30 days.

- Advantages: Potentially first targeted therapy approved for HER2 exon 20 insertion mutations, unmet need, oral administration
- Disadvantages: Lack of late-stage trial data and overall survival data, modest response rate, high adverse event rates leading to dose interruptions and discontinuation, narrow initial target population
- Reference WAC (Exkivity): ~$24,000 per 30 days

Palovarotene (Brand Name: To be determined)

Manufacturer: Ipsen
Regulatory designations: Orphan Drug, Breakthrough Therapy, Fast Track
Expected FDA decision: December 29, 2022

Therapeutic use

Palovarotene is under review for the treatment of patients with fibrodysplasia ossificans progressiva (FOP).

FOP is a rare disorder in which muscle tissue and connective tissue such as tendons and ligaments are gradually replaced by bone (ossified). This causes bone formation outside the skeleton that constrains movement. This process generally becomes noticeable in early childhood, starting with the neck and shoulders and proceeding down the body and into the limbs. If the jaw is involved, affected individuals may have trouble eating and/or speaking. Any trauma to the muscles of an individual with FOP (eg, fall or invasive medical procedures) may trigger episodes of muscle swelling and inflammation followed by more rapid ossification in the injured area.

FOP is caused by the mutation of the ACVR1 gene. The ACVR1 protein is found in many tissues of the body including skeletal muscle and cartilage. It helps to control the growth and development of the bones and muscles, including the gradual replacement of cartilage by bone that occurs in normal skeletal maturation from birth to young adulthood. In patients with an ACVR1 mutation, the receptor is turned on when it normally should be silent which causes overgrowth of bone and cartilage.

The exact prevalence of FOP is unknown, but it is estimated to affect approximately 1 in 2 million people worldwide.

- Treatment of patients with FOP

Clinical profile

Palovarotene is an orally administered selective retinoic acid gamma receptor agonist. It is believed to work in FOP by inhibiting the recruitment of inflammatory cells at affected tissue and decreasing systemic inflammation.

Pivotal trial data:
Palovarotene was evaluated in MOVE, a Phase 3, open-label study in patients with FOP. Patients received palovarotene both as a chronic treatment (5 mg once daily) and episodic flare-up treatment (20 mg once daily for 4 weeks, followed by 10 mg once daily for 8 weeks). The primary endpoint was reduction of new heterotopic ossification (HO) volume as assessed by whole body CT compared with untreated patients from Ipsen's global FOP natural history study.

In a preliminary post-hoc analysis, there was a 62% reduction in mean annualized new HO volume in participants treated with palovarotene (8,821 mm^3) (N = 97) vs. untreated (23,318 mm^3) (n = 98) patients (p = 0.0292) over 24 months.

- Selective retinoic acid gamma receptor agonist
- Oral formulation
- Mean annualized new heterotopic ossification volume over 24 months: 8,821 mm^3 vs. 23,318 mm^3 in an untreated natural history study (62% reduction)
- Safety data limited
- Dosing: Once daily

Palovarotene (continued…)

Safety:
The safety data for palovarotene is limited.

Dosing:
In the pivotal study, palovarotene was administered orally once daily.

Competitive environment

Palovarotene would potentially be the first approved drug for FOP. There is currently no definitive treatment for FOP. The only option for these patients is a short course of high-dose steroids during flare-ups which may help reduce the inflammation and tissue swelling seen in the early stages of FOP. Based on the post-hoc analysis of the pivotal trial, palovarotene reduced new heterotopic ossification volume, providing some evidence of potential impact on disease pathophysiology.

However, there are some concerns related to the drug and the trial data. First, early growth plate closure was observed in patients less than 14 years of age. Since palovarotene potentially reduces new heterotopic ossifications, the drug would have greater benefit in skeletally immature patients, but the potential for early growth plate closure may limit its use in young patients.

Second, as it relates to the efficacy, the pivotal study had a pause back in 2020 based on results of a pre-specified interim futility analysis which found that the study was unlikely to meet its primary endpoint. A post-hoc analysis using a different statistical method was conducted and is the basis of the FDA submission.

Finally, the target population for palovarotene is expected to be very small even relative to other orphan drugs, given the ultra-rare nature of FOP and because it may be restricted to patients who meet a certain age criterion.

- Advantages: Potentially the first approved therapy for FOP, high unmet need (current standard of care is limited to high-dose corticosteroids for treatment of flare-ups)
- Disadvantages: Early growth plate closure observed in patients < 14 years, questionable efficacy results, narrow target population

Extended generic pipline forecast

Optum Rx generic pipeline forecast

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
2022 Possible launch date					
ONEXTON	clindamycin/benzoyl peroxide	Bausch Health	External	All	2022
NEUPRO	rotigotine	UCB	External	All	2022
XYREM	sodium oxybate	Jazz	Oral	All	2H-2022
OXAYDO	oxycodone	Egalet	Oral	All	2H-2022
IXEMPRA Kit	ixabepilone	R-Pharm	Intravenous	All	2H-2022
IRESSA	gefitinib	AstraZeneca	Oral	All	3Q-2022
KEVEYIS	dichlorphenamide	Strongbridge Biopharma	Oral	All	08-2022
ORAVIG	miconazole	Galt Pharmaceuticals	Oral	All	09-2022
XERESE	acyclovir/hydrocortisone	Bausch Health	External	All	11-2022
FOLOTYN	pralatrexate	Acrotech/Aurobindo	Intravenous	All	11-2022
RAYOS	prednisone	Horizon	Oral	All	12-2022
TREANDA	bendamustine	Cephalon/Teva	Intravenous	All	12-2022
ZIOPTAN	tafluprost	Akorn	Ophthalmic	All	12-2022
2023 Possible launch date					
PREZISTA	darunavir	Janssen	Oral	All	2023
PROLENSA	bromfenac	Bausch Health	Ophthalmic	All	2023
ALPHAGAN P	brimonidine	Allergan	Ophthalmic	All	2023
MYRBETRIQ	mirabegron	Astellas	Oral	All	2023
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	01-2023
HUMIRA	adalimumab	AbbVie	Subcutaneous	All	01-2023
CAMBIA	diclofenac potassium	Assertio	Oral	All	01-2023
TROKENDI XR	topiramate	Supernus	Oral	All	01-2023
DUOBRII	halobetasol propionate/tazarotene	Bausch Health	External	All	01-2023
NASCOBAL	cyanocobalamin	Par/Endo	Intranasal	All	01-2023
DYLOJECT	diclofenac	Hospira/Pfizer/Javelin	Intravenous	All	01-2023
TEFLARO	ceftaroline fosamil	Allergan	Intravenous	All	01-2023

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
FIRVANQ KIT	vancomycin	Azurity	Oral	All	01-2023
SPIRIVA HANDIHALER	tiotropium	Boehringer Ingelheim	Inhalation	All	01-2023
LEXISCAN	regadenoson	Astellas	Intravenous	All	01-2023
FORTEO	teriparatide	Eli Lilly	Injection	All	01-2023
DULERA	formoterol fumarate/mometasone furoate	Organon	Inhalation	All	01-2023
BALCOLTRA	levonorgestrel/ethinyl estradiol/ferrous bisglycinate	Avion	Oral	All	01-2023
LATUDA	lurasidone	Sunovion	Oral	All	02-2023
AGGRASTAT	tirofiban	Medicure	Intravenous	All	03-2023
AUBAGIO	teriflunomide	Sanofi/Genzyme	Oral	All	03-2023
GATTEX	teduglutide recombinant	Takeda	Subcutaneous	All	03-2023
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
LIVALO	pitavastatin	Eli Lilly/Kowa Pharmaceuticals	Oral	All	05-2023
TYSABRI	natalizumab	Biogen	Intravenous	All	05-2023
NEULASTA ONPRO	pegfilgrastim	Amgen/Insulet	Subcutaneous	All	2H-2023
XURIDEN	uridine	Wellstat Therapeutics	Oral	All	07-2023
TOLAK	fluorouracil	Pierre Fabre	External	All	07-2023
MOZOBIL	plerixafor	Sanofi/Genzyme	Subcutaneous	All	07-2023
EGRIFTA	tesamorelin	Theratechnologies	Subcutaneous	All	08-2023
CYSTADROPS	cysteamine	Recordati	Ophthalmic	All	08-2023
VYVANSE	lisdexamfetamine	Shire/Takeda	Oral	All	08-2023
KATERZIA	amlodipine	Azurity	Oral	All	08-2023
CAROSPIR	spironolactone	CMP Pharma	Oral	All	09-2023
STELARA	ustekinumab	Janssen	Subcutaneous; intravenous	All	09-2023
VIBATIV	telavancin	Theravance	Intravenous	All	09-2023
LEXETTE	halobetasol	Mayne	External	All	09-2023
VOTRIENT	pazopanib	Novartis	Oral	All	10-2023
OZURDEX	dexamethasone	Allergan	Ophthalmic	All	11-2023
AMTURNIDE	aliskiren/amlodipine/hydrochlorothiazide	Novartis	Oral	All	11-2023
KOGENATE FS	octocog alpha	Bayer	Intravenous	All	11-2023
HELIXATE FS	antihemophilic factor VIII	CSL Behring/Bayer	Intravenous	All	11-2023

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
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
GIAZO	balsalazide disodium	Bausch Health	Oral	All	01-2024
GILENYA	fingolimod	Novartis	Oral	0.5 mg	01-2024
GRALISE	gabapentin	Assertio Therapeutics	Oral	All	01-2024
TASIGNA	nilotinib	Novartis	Oral	All	01-2024
SIMPONI ARIA	golimumab	Janssen	Intravenous	All	02-2024
SIMPONI	golimumab	Janssen	Subcutaneous	All	02-2024
NATESTO	testosterone	Acerus	Nasal	All	02-2024
CIMZIA	certolizumab pegol	UCB/Royalty Pharma	Subcutaneous	All	02-2024
SYMPAZAN	clobazam	Aquestive	Oral	All	02-2024
ISENTRESS	raltegravir	Merck	Oral	All	04-2024
DUTREBIS	lamivudine/raltegravir	Merck	Oral	All	04-2024
PROBUPHINE	buprenorphine	Titan Pharmaceuticals/Braeburn Pharmaceuticals	Subdermal	All	04-2024
RADICAVA	edaravone	Mitsubishi Tanabe	Intravenous	All	05-2024
DUAVEE	conjugated estrogens/bazedoxifene acetate	Pfizer/Ligand Pharmaceuticals	Oral	All	05-2024
SAXENDA	liraglutide	Novo Nordisk	Subcutaneous	All	05-2024
ARANESP	darbepoetin alfa	Amgen/Kirin	Intravenous; subcutaneous	All	05-2024
NYMALIZE	nimodipine	Arbor	Oral	All	05-2024
VICTOZA	liraglutide recombinant	Novo Nordisk	Subcutaneous	All	06-2024
HAEGARDA	C1 esterase inhibitor	CSL Behring	Subcutaneous	All	06-2024
SLYND	drospirenone	Exeltis	Oral	All	08-2024
SPRYCEL	dasatinib	Bristol-Myers Squibb	Oral	All	09-2024
SUSTOL	granisetron	Heron Therapeutics	Subcutaneous	All	09-2024
PRIALT	ziconotide acetate	TerSera Therapeutics	Intrathecal	All	10-2024
LAZANDA	fentanyl citrate	Depomed	Intranasal	All	10-2024
RYDAPT	midostaurin	Novartis	Oral	All	10-2024
VUITY	pilocarpine	AbbVie	Ophthalmic	All	10-2024
STENDRA	avanafil	Metuchen Pharmaceuticals	Oral	All	10-2024
QSYMIA	phentermine/topiramate	Vivus	Oral	All	12-2024
SIKLOS	hydroxyurea	Addmedica/Medunik	Oral	All	12-2024

Trade Name	Generic Name	Brand Company(ies)	Route of Administration	Strength(s)	Anticipated Generic Availability
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
SOLIRIS	eculizumab	Alexion	Intravenous	All	03-2025
BENLYSTA	belimumab	GSK	Intravenous; subcutaneous	All	03-2025
ABILIFY MAINTENA	aripiprazole	Otsuka/Lundbeck	Intramuscular	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
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

Extended brand pipeline forecast

Optum Rx brand pipeline forecast

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
2022 Possible launch date									
Zynteglo	betibeglogene autotemcel	bluebird Bio	gene therapy	Beta thalassemia	IV	Filed BLA	08/19/2022	Yes	Yes
JNJ-64007957	teclistamab	Janssen	BCMA and CD3 bispecific antibody	Multiple myeloma	SC	Filed BLA	08/29/2022	Yes	Yes
Libervant	diazepam	Aquestive Therapeutics	benzodiazepine	Seizures	PO	Filed NDA	08/2022	No	Yes
AXS-05	dextromethorphan/ bupropion	Axsome	N-methyl-D-aspartate antagonist/ antidepressant	Major depressive disorder	PO	Filed NDA	08/2022	No	No
RT-002 (Daxi)	daxibotulinumtoxinA	Revance Therapeutics	botulinum toxins	Glabellar lines (frown lines)	IM	Filed BLA	09/08/2022	Yes	No
SPI-2012	eflapegrastim	Spectrum	granulocyte colony-stimulating factor (GCSF)	Chemotherapy-induced neutropenia	SC	Filed BLA	09/09/2022	Yes	No
BMS-986165	deucravacitinib	Bristol Myers Squibb	tyrosine kinase 2 inhibitor	Plaque psoriasis	PO	Filed NDA	09/10/2022	Yes	No
OBE-2109 (KLH-2109)	linzagolix	ObsEva	gonadotropin-releasing hormone antagonist	Uterine fibroids	PO	Filed NDA	09/13/2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Lenti-D	elivaldogene autotemcel	bluebird Bio	gene therapy	Cerebral adrenoleukodystrophy	IV	Filed BLA	09/16/2022	Yes	Yes
HTX-019	aprepitant	Heron Therapeutics	substance P/neurokinin-1 receptor antagonist	Postoperative nausea and vomiting	IV	Filed NDA	09/17/2022	No	No
Dasynoc	dasatinib	Xspray Pharma	kinase inhibitor	Chronic myeloid leukemia	PO	Filed NDA	09/18/2022	No	No
Pedmark (STS)	sodium thiosulfate	Fennec	reducing agent	Ototoxicity	IV	Filed NDA	09/23/2022	Yes	Yes
AMX-0035	sodium phenylbutyrate/ taurursodiol	Amylyx Pharmaceuticals	neuroprotective	Amyotrophic lateral sclerosis	PO	Filed NDA	09/29/2022	Yes	Yes
TAS-120	futibatinib	Otsuka/ Taiho	fibroblast growth factor receptor inhibitor	Cholangiocarcinoma	PO	Filed NDA	09/30/2022	Yes	Yes
BI-655130	spesolimab	Boehringer Ingelheim	IL-36 receptor antibody	Generalized pustular psoriasis	IV	Filed BLA	3Q2022	Yes	Yes
GZ-402665	olipudase alfa	Sanofi	enzyme replacement therapy	Acid sphingomyelinase deficiency	IV	Filed BLA	10/03/2022	Yes	Yes
Furoscix	furosemide	scPharmaceuticals	diuretic	Heart failure	SC	Filed NDA	10/08/2022	Yes	No
SPN-830	apomorphine	Supernus Pharmaceuticals	non-ergoline dopamine agonist	Parkinson's disease	SC infusion	Filed NDA	10/08/2022	Yes	No
Ovastat	treosulfan	Medexus Pharmaceuticals	alkylating agent	Hematopoietic stem cell transplantation	IV	Filed BLA	10/22/2022	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
tremelimumab	tremelimumab	AstraZeneca	cytotoxic T lymphocyte-associated antigen 4 inhibitor	Hepatocellular carcinoma	IV	Filed BLA	10/25/2022	Yes	Yes
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension	INH	Tentative Approval	10/27/2022	Yes	No
AT-GAA	cipaglucosidase alfa	Amicus	enzyme therapy	Pompe disease	IV	Filed BLA	10/29/2022	Yes	Yes
DE-117	omidenepag isopropyl	Santen Pharmaceutical/ Ube Industries	prostaglandin E receptor 2 agonist	Glaucoma	OPH	Filed NDA	11/06/2022	No	No
PRV-031	teplizumab	Provention Bio	CD3 antigen inhibitor	Diabetes mellitus	IV	Filed BLA	11/17/2022	Yes	No
PS-433540 (RE-021; DARA)	sparsentan	Travere Therapeutics	dual endothelin angiotensin receptor antagonist	IgA nephropathy	PO	Filed NDA	11/17/2022	Yes	Yes
Hepcludex	bulevirtide	Gilead	HBV receptor binder	Hepatitis delta virus	SC	Filed BLA	11/19/2022	Yes	Yes
AMT-061	etranacogene dezaparvovec	CSL Behring/ uniQure	gene therapy	Hemophilia B	IV	Filed BLA	11/24/2022	Yes	Yes
HM781-36B	poziotinib	Spectrum Pharmaceuticals	pan-HER inhibitor	Non-small cell lung cancer	PO	Filed NDA	11/24/2022	Yes	No
pegcetacoplan (intravitreal)	pegcetacoplan	Apellis	compliment C3 inhibitor	Geographic atrophy	Intravitreal	Filed BLA	11/26/2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
IMGN-853	mirvetuximab soravtansine	ImmunoGen	folate receptor-1 antagonist	Ovarian cancer	IV	Filed BLA	11/28/2022	Yes	Yes
131I-8H9	omburtamab	Y-mAbs Therapeutics	B7-H3 antagonist	Brain cancer	Intrathecal	Filed BLA	11/30/2022	Yes	Yes
Lucassin	terlipressin	Mallinckrodt	V-1 (vasopressin) agonist	Hepato-renal syndrome	IV	Filed NDA	12/13/2022	Yes	Yes
MRTX-849	adagrasib	Mirati Therapeutics	KRAS inhibitor	Non-small cell lung cancer	PO	Filed NDA	12/14/2022	Yes	No
JS-001	toripalimab	Coherus Biosciences	anti-PD-1 monoclonal antibody	Nasopharyngeal carcinoma	IV	Filed BLA	12/23/2022	Yes	Yes
GS-CA1 (GS-6207)	lenacapavir	Gilead	HIV capsid inhibitor	HIV-1	SC	Filed NDA	12/27/2022	Yes	No
ublituximab	ublituximab	TG Therapeutics	anti-CD-20 monoclonal antibody	Multiple sclerosis	IV	Filed BLA	12/28/2022	Yes	No
R-667 (RG-667)	palovarotene	Ipsen	selective retinoic acid receptor agonist	Fibrodysplasia ossificans progressiva	PO	Filed NDA	12/29/2022	Yes	Yes
RG-7828	mosunetuzumab	Genentech	anti-CD20/CD3 monoclonal antibody	Follicular lymphoma	IV/SC	Filed BLA	12/29/2022	Yes	Yes
RBX-2660	RBX-2660	Rebiotix	microbiota suspension	Clostridium difficile infection	Rectal	Filed NDA	4Q2022	No	Yes
BGB-A317 (BGB-A-317)	tislelizumab	BeiGene	programmed death-1 inhibitor	Esophageal squamous cell carcinoma	IV	Filed BLA	2H2022	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
2023 Possible launch date									
NexoBrid	bromelain	MediWound	peptide hydrolase replacement agent	Burns/ Skin injury	TOP	Filed BLA	01/01/2023	No	Yes
BAN-2401	lecanemab	Eisai/Biogen	beta-amyloid monoclonal antibody	Alzheimer's disease	IV	Filed BLA	01/06/2023	Yes	No
TAK-438	vonoprazan fumarate	Phantom Pharmaceuticals	potassium-competitive acid blocker	Erosive esophagitis	PO	Filed NDA	01/11/2023	No	No
ACER-001	sodium phenylbutyrate	Acer Therapeutics	nitrogen-binding agent	Urea cycle disorders	PO	Filed NDA	01/15/2023	Yes	No
BIIB-067 (ISIS-333611)	tofersen	Biogen/ Ionis	antisense drug	Amyotrophic lateral sclerosis	Intrathecal	Filed NDA	01/25/2023	Yes	Yes
NiCord	omidubicel	Gamida	cellular therapy	Hematological cancers	IV	Filed BLA	01/30/2023	Yes	Yes
PT-027	budesonide/albuterol	AstraZeneca/ Avillion	glucocorticoid/short acting beta agonist	Asthma	INH	Filed NDA	01/31/2023	No	No
GSK-1278863	daprodustat	GlaxoSmithKline	hypoxia-inducible factor-prolyl hydroxylase (HIF-PH) inhibitor	Anemia	PO	Filed NDA	02/01/2023	Yes	No
LY-3527727	pirtobrutinib	Eli Lilly	Bruton's tyrosine kinase inhibitor	Mantle cell lymphoma	PO	Filed NDA	02/04/2023	Yes	No
LY-3002813	donanemab	Eli Lilly	beta-amyloid monoclonal antibody	Alzheimer's disease	IV	Filed BLA	02/04/2023	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
FT-2102	olutasidenib	Forma Therapeutics	dehydrogenase 1 inhibitor	Acute myeloid leukemia	PO	Filed NDA	02/15/2023	Yes	Yes
RAD-1901	elacestrant	Radius Health	selective estrogen receptor degrader	Breast cancer	PO	Filed NDA	02/17/2023	Yes	No
CYT-387	momelotinib	Sierra Oncology	janus kinase inhibitor	Myeloproliferative disorders	PO	Filed NDA	02/17/2023	Yes	Yes
KB-103	beremagene geperpavec	Krystal Biotech	gene therapy	Epidermolysis bullosa	Topical	Filed BLA	02/22/2023	Yes	Yes
PF-07321332	nirmatrelvir/ ritonavir	Pfizer	protease inhibitor	COVID-19	PO	Filed NDA	02/25/2023	No	No
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	03/18/2023	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
LY-3074828	mirikizumab	Eli Lilly	IL-23 inhibitor	Ulcerative colitis	SC	Filed BLA	04/28/2023	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
AV-7909 (CPG 7909)	anthrax vaccine adsorbed	Emergent Biosolutions	vaccine/ oligodeoxynucleotide	Anthrax	IM	Filed BLA	04/30/2023	No	No
SYD-985	[vic-] trastuzumab duocarmazine	Byondis	HER2-targeting antibody-drug conjugate	Breast cancer	IV	Filed BLA	05/12/2023	Yes	No
ABBV-951	foscarbidopa/ foslevodopa	AbbVie	aromatic amino acid decarboxylation inhibitor/ aromatic amino acid	Parkinson's disease	SC	Filed NDA	05/20/2023	Yes	No
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
Zynquista	sotagliflozin	Lexicon	sodium-dependent glucose transporter 1 (SGLT-1) and SGLT-2 inhibitor	Diabetes mellitus	PO	Filed NDA	05/31/2023	No	No
AOP-200704	landiolol	Eagle Pharmaceuticals	cardio-selective beta-1 adrenergic blocker	Dysrhythmia	IV	Filed NDA	06/01/2023	No	No
CyclASol	cyclosporine	Novaliq	immunosuppressant	Dry eye disease	OPH	InTrial	06/09/2023	No	No
FT-218	sodium oxybate extended-release	Avadel	dopamine receptor agonist	Narcolepsy	PO	Tentative Approval	06/17/2023	Yes	Yes
ESN-364	fezolinetant	Astellas	NK3 receptor antagonist	Menopause	PO	Filed NDA	06/23/2023	No	No
PTC-AADC	eladocagene exuparvovec	PTC Therapeutics	gene therapy	Aromatic L-amino acid decarboxylase deficiency	Intracerebral	InTrial	2Q2023	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
OPNT-003	nalmefene	Opiant Pharmaceuticals	opioid receptor antagonist	Opioid overdose	Intranasal	InTrial	1H2023	No	No
UCB-4940 (CDP-4940)	bimekizumab	UCB	interleukin-17 receptor inhibitor	Plaque psoriasis	SC	CRL	1H2023	Yes	No
ritlecitinib	ritlecitinib	Pfizer	janus kinase inhibitor	Alopecia areata	PO	Filed NDA	06/30/2023	Yes	No
PRX-102	pegunigalsidase alfa	Protalix	enzyme replacement	Fabry disease	IV	CRL	1H2023	Yes	No
CUTX-101	copper histidinate	Fortress Biotech	copper replacement	Menkes Disease	SC	InTrial	1H2023	Yes	Yes
SER-109	SER-109	Seres Therapeutics	ecobiotic agent	Clostridium difficile infection	PO	InTrial	1H2023	No	Yes
Roctavian	valoctocogene roxaparvovec	BioMarin	gene therapy	Hemophilia A	IV	CRL	1H2023	Yes	Yes
NOV-03	perfluorohexyloctane	Bausch/ Novaliq	tear film stabilizer	Dry eye disease	OPH	Filed NDA	06/30/2023	No	No
BL-8040 (BKT-140)	motixafortide	BioLineRx	selective chemokine receptor 4 inverse agonist	Stem cell transplant	SC	InTrial	Mid-2023	Yes	Yes
TV-46000	risperidone	Teva Pharmaceuticals/ MedinCell	atypical antipsychotic	Schizophrenia	SC	CRL	MId-2023	No	No
Aripiprazole 2-month	aripiprazole	Lundbeck/ Otsuka Pharmaceutical	atypical antipsychotic	Schizophrenia/ bipolar disorder	IM	InTrial	Mid-2023	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
RA-101495	zilucoplan	UCB	complement inhibitor	Myasthenia gravis	SC	InTrial	Mid-2023	Yes	Yes
ERY-ASP (ERY-001)	L-asparaginase (eryaspase)	Erytech	L-asparaginase	Acute lymphoblastic leukemia	IV	InTrial	Mid-2023	Yes	Yes
TAK-003	Dengue fever vaccine	Takeda	vaccine	Dengue fever	SC	InTrial	Mid-2023	Yes	No
ATI-1501	metronidazole	Saptalis	nitroimidazole	Fungal infections, anaerobic bacterial infections	PO	InTrial	Mid-2023	No	No
ONS-5010	bevacizumab-vikg	Outlook Therapeutics	anti-VEGF antibody	Wet age-related macular degeneration	Intravitreal	InTrial	Mid-2023	Yes	No
obeticholic acid	obeticholic acid	Intercept Pharmaceuticals	farnesoid X receptor agonist	Nonalcoholic steatohepatitis	PO	CRL	Mid-2023	Yes	No
MEDI-8897 (RSV MAbs)	nirsevimab	AstraZeneca/ Sanofi	anti-RSV monoclonal antibody D25	Respiratory syncytial virus	IM	InTrial	Mid-2023	No	No
DCR-PHXC	nedosiran	Novo Nordisk	glycolate oxidase antagonist	hyperoxaluria	SC	InTrial	Mid-2023	Yes	Yes
Botulax	letibotulinumtoxinA	Hugel Pharma	botulinum toxins	Wrinkles	IM	CRL	Mid-2023	Yes	No
VBP-15	vamorolone	Santhera	corticosteroid	Duchenne muscular dystrophy	PO	InTrial	Mid-2023	Yes	Yes
LN-144	lifileucel	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Melanoma	IV	InTrial	Mid-2023	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
UCB-7665	rozanolixizumab	UCB	neonatal Fc receptor inhibitor	Generalized myasthenia gravis	SC	InTrial	Mid-2023	Yes	Yes
Neutrolin (CRMD-003, CRMD-004)	citrate/ taurolidine/ heparin	CorMedix	antimicrobial agent/ anticoagulant	Catheter-related infections	IV	CRL	Mid-2023	No	No
SGX-301	synthetic hypericin	Soligenix	synthetic hypericin	Cutaneous T-cell lymphoma	TOP	InTrial	Mid-2023	Yes	Yes
AKCEA-TTR-LRx	eplontersen	AstraZeneca/ Ionis	antisense oligonucleotide	Hereditary transthyretin-mediated amyloid polyneuropathy	SC	InTrial	Mid-2023	Yes	Yes
RG-7433 (ABT-263)	navitoclax	AbbVie	Bcl-2 inhibitor	Myelofibrosis	PO	InTrial	Mid-2023	Yes	Yes
MT-1621	deoxythymidine/ deoxycytidine	Zogenix	deoxynucleoside	Thymidine kinase 2 deficiency	PO	InTrial	Mid-2023	Yes	Yes
RG-7440 (GDC-0068)	ipatasertib	Roche	pan-Akt inhibitor	Prostate cancer	PO	InTrial	Mid-2023	Yes	No
RG-6026	glofitamab	Roche	anti-CD20/CD3 T cell monoclonal antibody	Diffuse large B cell lymphoma	IV	InTrial	Mid-2023	Yes	No
IPX-203	carbidopa/ levodopa	Amneal	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	PO	InTrial	Mid-2023	No	No
BBI-4000	sofpironium bromide	Brickell	anticholinergic	Hyperhidrosis	TOP	InTrial	Mid-2023	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Melblez Kit	melphalan	Delcath	phenylalanine mustard	Hepatocellular cancer (liver)/ Biliary tract cancer/ Melanoma	INJ	InTrial	Mid-2023	Yes	Yes
SRP-9001 (RG-6356)	SRP-9001	Sarepta/ Roche	gene therapy	Duchenne muscular dystrophy	IV	InTrial	3Q2023	Yes	No
NS-2 (ALDX-1E1, ALDX-1E2, ADX-102)	reproxalap	Aldeyra Therapeutics	aldehyde antagonist	Dry eye disease	OP	InTrial	3Q2023	No	No
OTL-200 (GSK-2696274)	OTL-200 (GSK-2696274)	Orchard Therapeutics	gene therapy	Leukodystrophy	IV	InTrial	3Q2023	Yes	Yes
TransCon PTH	palopegteriparatide	Ascendis Pharma	parathyroid hormone	Hypoparathyroidism	SC	InTrial	3Q2023	Yes	Yes
arimoclomol	arimoclomol	Orphazyme	cytoprotectives	Niemann-Pick disease	PO	CRL	3Q2023	Yes	Yes
ETX-2514 (SUL-DUR)	durlobactam/ sulbactam	Entasis Therapeutics	broad-spectrum β-lactamase inhibitor/ beta-lactam antimicrobial	Bacterial infections	IV	InTrial	3Q2023	No	No
GC-4419	avasopasem manganese	Galera Therapeutics	dismutase mimetic	Radiotherapy-induced oral mucositis	IV	InTrial	3Q2023	Yes	No
ARS-1	epinephrine	ARS Pharmaceuticals	non-selective alpha/ beta-adrenergic receptor agonist	Anaphylaxis	Intranasal	InTrial	3Q2023	No	No
RG-1450	gantenerumab	Roche	beta-amyloid monoclonal antibody	Alzheimer's disease	SC	InTrial	4Q2023	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
TRC-101	veverimer	Tricida	carrier protein modulator	Chronic kidney disease	PO	CRL	4Q2023	Yes	No
OX-124	naloxone	Orexo	opioid antagonist	Opioid overdose	Intranasal	InTrial	4Q2023	No	No
MBG-453	sabatolimab	Novartis	anti-TIM-3	Myelodysplastic syndrome	IV	InTrial	4Q2023	Yes	No
IDP-126	IDP-126	Bausch Health	retinoid/ antibiotic	Acne	TOP	InTrial	4Q2023	No	No
MILR-1444A	lebrikizumab	Eli Lilly	interleukin-13 inhibitor	Atopic dermatitis	SC	InTrial	4Q2023	Yes	No
SAGE-217	zuranolone	Sage Therapeutics/ Biogen	GABA-A receptor allosteric modulator	Major depressive disorder	PO	InTrial	4Q2023	No	No
LentiGlobin	lovotibeglogene autotemcel	bluebird bio	gene therapy	Sickle cell disease	IV	InTrial	4Q2023	Yes	Yes
X4P-001 (X-4P-001, X4-136, X4P-001-RD)	mavorixafor	X4 Pharma	CXC receptor type 4 (CXCR4) inhibitor	WHIM syndrome	PO	InTrial	4Q2023	Yes	Yes
FMXIN-001	naloxone	Nasus Pharma	opioid antagonist	Opioid overdose	Intranasal	InTrial	4Q2023	No	No
STS-101	dihydroergotamine	Satsuma Pharmaceuticals	ergotamine	Migraine	Intranasal	InTrial	4Q2023	No	No
SPI-014	lanthanum dioxycarbonate	Unicycive	phosphate binder	Hyperphosphatemia	PO	InTrial	4Q2023	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
MIN-101	roluperidone	Minerva Neurosciences	sigma-2 and 5HT-2A receptor antagonist	Schizophrenia	PO	InTrial	2H2023	Yes	No
PB-2452	bentracimab	PhaseBio	antiplatelet monoclonal antibody	Antiplatelet drug toxicity	IV	InTrial	2H2023	No	No
PRO-140	leronlimab	CytoDyn	C-C chemokine receptor 5 antagonist	HIV	SC	InTrial	2H2023	Yes	No
Zeftera	ceftobiprole	Basilea	cephalosporin antibiotic	Bacterial infections	IV	InTrial	2H2023	No	No
quizartinib	quizartinib	Daiichi Sankyo	FLT-3 receptor tyrosine kinase inhibitor	Acute myeloid leukemia	PO	CRL	2H2023	Yes	Yes
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	CRL	2H2023	Yes	Yes
CTX-001	exagamglogene autotemcel	CRISPR Therapeutics/ Vertex	gene therapy	Beta-thalassemia; sickle cell anemia	IV	InTrial	2H2023	Yes	Yes
VNRX-5133	cefepime/ taniborbactam	VenatoRx Pharmaceuticals	cephalosporin/ beta-lactamase inhibitor	Bacterial infections	IV	InTrial	2H2023	Yes	No
OTL-103 (GSK-2696275)	OTL-103 (GSK-2696275)	Orchard Therapeutics	gene therapy	Wiskott-Aldrich syndrome	IV	InTrial	2H2023	Yes	Yes
EBV-CTL (ATA-129)	tabelecleucel	Atara Biotherapeutics	cell therapy	Lymphoproliferative disorder	IV	InTrial	2H2023	Yes	Yes
MT-7117	MT-7117	Mitsubishi Tanabe Pharma	Undisclosed	Erythropoietic protoporphyria	PO	InTrial	2H2023	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
GEN-3013	epcoritamab	AbbVie	CD3/CD20 monoclonal antibody	Diffuse large B-cell lymphoma	SC	InTrial	2H2023	Yes	No
SB-206	SB-206	Novan Therapeutics	nitric oxide-releasing compound	Molluscum contagiosum	TOP	InTrial	2H2023	No	No
REGN-3918	pozelimab	Regeneron	C5a receptor inhibitor	CHAPLE disorder	IV/SC	InTrial	2H2023	Yes	Yes
CK-301	cosibelimab	Checkpoint Therapeutic	anti programmed cell death ligand 1	Cutaneous squamous cell carcinoma	IV	InTrial	2H2023	Yes	No
RG-6107	crovalimab	Roche	C5 inhibitor	Paroxysmal nocturnal hemoglobinuria	IV/SC	InTrial	2H2023	Yes	Yes
ALXN-1840 (WTX-101)	bis-choline tetrathiomolybdate	AstraZeneca	chelating agent	Wilson's disease	PO	InTrial	2H2023	Yes	Yes
CDZ-173	leniolisib	Pharming/ Novartis	phosphatidylinositol-3-4-5-trisphosphate inhibitor	Primary immunodeficiencies	PO	InTrial	2H2023	Yes	Yes
LN-145	LN-145	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Cervical Cancer	IV	InTrial	2H2023	Yes	No
efgartigimod SC	efgartigimod-PH20	argenx/ Halozyme	neonatal Fc receptor antibody	Generalized myasthenia gravis	SC	InTrial	2H2023	Yes	Yes
NN-7415	concizumab	Novo Nordisk	anti-tissue factor pathway inhibitor	Hemophilia A and hemophilia B	SC	InTrial	2H2023	Yes	Yes
GSK-3844766A	GSK-3844766A	GlaxoSmithKline	vaccine	Respiratory syncytial virus	IM	InTrial	2H2023	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
F-901318	olorofim	F2G	orotomide antifungal	Aspergillosis	PO/IV	InTrial	2H2023	No	Yes
TP-03	lotilaner	Tarsus Pharmaceuticals	antagonist of insect and arachnid GABA-Cl channels	Demodex blepharitis	TOP	InTrial	2H2023	No	No
ADP-A2M4 (MAGE-A4)	afamitresgene autoleucel	Adaptimmune	SPEAR T-cell therapy	Sarcoma	IV	InTrial	2H2023	Yes	Yes
CNM-Au8	CNM-Au8	Clene	gold nanocrystal	Amyotrophic lateral sclerosis	PO	InTrial	2H2023	Yes	Yes
iDose travoprost	travoprost	Glaukos Corporation	prostaglandin analog	Glaucoma/ Ocular hypertension	Intraocular	InTrial	2H2023	No	No
I/Ontak	denileukin diftitox	Citius	CD25-directed cytotoxin	Cutaneous T-cell lymphoma	IV	InTrial	2H2023	Yes	Yes
APD-334	etrasimod	Pfizer/ Arena Pharmaceuticals	S1P1 receptor agonist	Ulcerative colitis	PO	InTrial	2H2023	Yes	No
pivmecillinam	pivmecillinam	Utility Therapeutics	amidinopenicillin	Urinary tract infections	PO	InTrial	2023	No	No
VP-102	cantharidin	Verrica	vesicant (blistering agent)	Molluscum	TOP	CRL	2023	No	No
NVX-CoV2373	coronavirus vaccine	Novavax	vaccine	Novel coronavirus disease 2019 (COVID-19)	IM	InTrial	2023	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
OMS-721	narsoplimab	Omeros	anti-MASP-2 monoclonal antibody	Hematopoietic stem cell transplant-associated thrombotic microangiopathy	IV	CRL	2023	Yes	Yes
GC-5107	human immunoglobulin	GC Pharma	human immunoglobulin	Primary immunodeficiencies	IV	CRL	2023	Yes	No
Qtrypta	zolmitriptan	Zosano	triptans	Acute migraines	TOP	CRL	2023	No	No
Doria	risperidone	Laboratorios Farmacéuticos Rovi	atypical antipsychotic	Schizophrenia	IM	CRL	2023	Yes	No
CAM-2038	buprenorphine	Braeburn	opioid receptor agonist (partial)	Opioid use disorder	SC	CRL	2023	Yes	No
Adstiladrin	nadofaragene firadenovec	FerGene	gene therapy	Bladder cancer	Intravesical	CRL	2023	Yes	No
Entyvio (SC formulation)	vedolizumab	Takeda	integrin receptor antagonist	Ulcerative colitis	SC	CRL	Late 2023	Yes	No
R-1646 (RO-4926219, AF-219, MK-7264)	gefapixant	Merck/ Roche	P2X3 antagonist	Chronic cough	PO	CRL	Late 2023	No	No
ADV-7103	tripotassium citrate monohydrate/ potassium hydrogen carbonate	Advicenne	potassium	Distal rental tubular acidosis	PO	InTrial	Late 2023	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
TAK-755 (SHP-655)	TAK-755	Takeda	ADAMTS13 enzyme	Thrombotic thrombocytopenic purpura	IV	InTrial	Late 2023	Yes	Yes
PSD-502	lidocaine/ prilocaine	Plethora/ Recordati	sodium channel blocker	Premature ejaculation	TOP	InTrial	Late 2023	No	No
LY-686017	tradipitant	Vanda Pharmaceuticals	neurokinin 1 receptor (NK-1R) antagonist	Motion sickness/ gastroparesis	PO	InTrial	Late 2023	No	No
GSK-2140944	gepotidacin	GlaxoSmithKline	bacterial Type II topoisomerase inhibitor	Bacterial infections	PO/IV	InTrial	Late 2023	No	No
MOR-202	felzartamab	I-Mab	anti-CD38 monoclonal antibody	Multiple myeloma	IV	InTrial	Late 2023	Yes	No
SAR-408701	SAR-408701	Sanofi	antibody-drug conjugate	Non-small cell lung cancer	IV	InTrial	Late 2023	Yes	No
MGL-3196 (VIA-3196)	resmetirom	Madrigal	beta-selective thyroid hormone receptor agonist	Nonalcoholic steatohepatitis	PO	InTrial	Late 2023	Yes	No
RG-6171	giredestrant	Roche	selective estrogen receptor degrader	Breast cancer	PO	InTrial	Late 2023	Yes	No
Iomab-B	iodine I 131 monoclonal antibody BC8	Actinium	anti-CD45 monoclonal antibody	Acute myeloid leukemia/ Myelodysplastic syndrome	IV	InTrial	Late 2023	Yes	Yes
CPN-301	clobetasol propionate	Formosa Pharmaceuticals/	corticosteroid	Eye inflammation/ pain	OPH	InTrial	Late 2023	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
		AimMax Therapeutics							
PPP-001	delta-9-tetrahydrocannabinol/ cannabidiol	Tetra Bio-Pharma	cannabinoid product	Pain	INH	InTrial	Late 2023	Yes	Yes

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

Key pending indication forecast

Optum Rx key pending indication forecast

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Libtayo	cemiplimab-rwlc	Regeneron Pharmaceuticals	programmed death receptor-1 blocking antibody	Non-small cell lung cancer	In combination with chemotherapy as first-line treatment in advanced non-small cell lung cancer	IV	9/19/2022
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 inhibitor	Prurigo nodularis	Treatment of prurigo nodularis	SC	9/30/2022
Oxlumo	lumasiran	Alnylam	HAO1-directed small interfering ribonucleic acid	Advanced primary hyperoxaluria type 1	For the reduction of plasma oxalate in the treatment of patients with advanced primary hyperoxaluria type 1	SC	10/6/2022
Imfinzi	durvalumab	AstraZeneca	programmed death-ligand 1 blocking antibody	Biliary tract cancer	In combination with standard-of-care chemotherapy, for patients with locally advanced or metastatic biliary tract cancer	IV	11/5/2022
Rinvoq	upadacitinib	AbbVie	janus associated kinase inhibitor	Non-radiographic axial spondyloarthritis	Treatment of non-radiographic axial spondyloarthritis	PO	11/7/2022
Brexafemme	ibrexafungerp	Scynexis	triterpenoid antifungal	Recurrent vulvovaginal candidiasis (prevention)	Prevention of recurrent vulvovaginal candidiasis	PO	11/30/2022
Vraylar	cariprazine	AbbVie	dopamine D3-preferring D3/D2 receptor partial agonist	Major depressive disorder	Adjunctive treatment of patients with major depressive disorder	PO	12/22/2022

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed New Indication	Route of administration	Estimated approval date
Imbruvica	ibrutinib	AbbVie	kinase inhibitor	Chronic graft versus host disease	Treatment of pediatric and adolescent patients one year and older with chronic graft versus host disease after failure of one or more lines of systemic therapy	PO	12/28/2022
Tymlos	abaloparatide	Radius Health	human parathyroid hormone related peptide analog	Osteoporosis (men)	Treatment of men with osteoporosis at high risk for fracture	SC	1/1/2023
Brukinsa	zanubrutinib	BeiGene	kinase inhibitor	Chronic lymphocytic leukemia/ small lymphocytic lymphoma	Treatment of adult patients with chronic lymphocytic leukemia or small lymphocytic lymphoma	PO	1/20/2023
Keytruda	pembrolizumab	Merck	programmed death receptor-1-blocking antibody	Non-small cell lung cancer	Adjuvant treatment of patients with stage IB (≥4 centimeters), II or IIIA non-small cell lung cancer following complete surgical resection	IV	1/29/2023
Orkambi	ivacaftor/ lumacaftor	Vertex	cystic fibrosis transmembrane conductance regulator potentiator	Cystic fibrosis	Treatment of cystic fibrosis in patients age 12 months to less than 24 months of age	PO	1/31/2023
Qulipta	atogepant	AbbVie	calcitonin gene-related peptide receptor antagonist	Chronic migraine prophylaxis	Preventive treatment of chronic migraine in adults	PO	4/21/2023
Rinvoq	upadacitinib	AbbVie	janus associated kinase inhibitor	Crohn's disease	Treatment of Crohn's disease	PO	5/26/2023

References:

Aihara M, Lu F, Kawata H, Iwata A, Odani-Kawabata N, Shams NK. Omidenepag isopropyl versus latanoprost in primary open-angle glaucoma and ocular hypertension: The Phase 3 AYAME study. Am J Ophthalmol. 2020 Dec;220:53-63.

Amicus Therapeutics Press Release. Amicus Therapeutics Website. Amicus Therapeutics receives notification of PDUFA date extensions for AT-GAA. https://ir.amicusrx.com/news-releases/news-release-details/amicus-therapeutics-receives-notification-pdufa-date-extensions. May 10, 2022. Accessed August 12, 2022.

BioMedTracker Drug Intelligence Platform. BioMedTracker Web site. http://www.biomedtracker.com

CSL Behring Press Release. CSL Behring Web site. Final analysis of pivotal HOPE-B study demonstrates durable and sustained therapeutic effect of etranacogene dezaparvovec gene therapy in hemophilia B – data presented at EAHAD 2022. https://www.cslbehring.com/newsroom/2022/hope-b-data-presented-at-eahad2022. February 4, 2022. Accessed June 2, 2022.

Kemper, AR. The condition review workgroup. Evidence report: newborn screening for Pompe disease. Updated June 3, 2013. Accessed April 12, 2022. https://akastage-www.hrsa.gov/sites/default/files/hrsa/advisory-committees/heritable-disorders/rusp/previous-nominations/pompe-external-evidence-review-report-2013.pdf.

Centers for Disease Control and Prevention (CDC). Hemophilia. CDC Web site. https://www.cdc.gov/ncbddd/hemophilia/index.html. Accessed June 1, 2022.

Ipsen Press Release. Ipsen Web site. Ipsen announces U.S. FDA priority review for palovarotene New Drug Application in patients with fibrodysplasia ossificans progressiva following resubmission. https://www.ipsen.com/press-releases/ipsen-announces-u-s-fda-priority-review-for-palovarotene-new-drug-application-in-patients-with-fibrodysplasia-ossificans-progressiva-following-resubmission. June 29, 2022. Accessed July 6, 2022.

Medline Plus. Fibrodysplasia ossificans progressiva. Medline Plus Web site. https://medlineplus.gov/genetics/condition/fibrodysplasia-ossificans-progressiva. Accessed July 6, 2022.

National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) IgA Nephropathy. NIDDK Web site. https://www.niddk.nih.gov/health-information/kidney-disease/iga-nephropathy. Accessed June 1, 2022.

National Organization for Rare Disorders (NORD). Fibrodysplasia ossificans progressiva. NORD Web site. https://rarediseases.org/rare-diseases/fibrodysplasia-ossificans-progressiva. Accessed July 6, 2022.

National Organization for Rare Disorders (NORD). Hemophilia B. NORD Web site. https://rarediseases.org/rare-diseases/hemophilia-b. Accessed June 1, 2022.

National Organization for Rare Disorders (NORD). IgA nephropathy. NORD Web site. https://rarediseases.org/rare-diseases/iga-nephropathy. Accessed June 1, 2022.

Santen Pharmaceutical Press Release. Santen Pharmaceutical Web site. Santen and UBE announces U.S. Food and Drug Administration accepts resubmission of NDA for STN1011700 / DE-117 (omidenepag isopropyl) for treating glaucoma and ocular hypertension. https://www.santen.com/en/news/20220610.pdf. June 10, 2022. Accessed August 12, 2022.

Schoser B, Roberts M, Byrne BJ, et al. Safety and efficacy of cipaglucosidase alfa plus miglustat versus alglucosidase alfa plus placebo in late-onset Pompe disease (PROPEL): an international, randomised, double-blind, parallel-group, phase 3 trial. Lancet Neurol. 2021;20(12):1027-1037.

Spectrum Pharmaceuticals Press Release. Spectrum Pharmaceuticals Web site. Spectrum Pharmaceuticals announces acceptance of New Drug Application filing for poziotinib. https://investor.sppirx.com/news-releases/news-release-details/spectrum-pharmaceuticals-announces-acceptance-new-drug. February 11, 2022. Accessed July 5, 2022.

Spectrum Pharmaceuticals Corporate Presentation. Spectrum Pharmaceuticals Web site. https://investor.sppirx.com/static-files/2977ecaa-f5d9-42e3-91af-ed6738a39993. May 24, 2022. Accessed July 5, 2022.

Travere Therapeutics Press Release. Travere Therapeutics Web site. Travere Therapeutics announces FDA acceptance and priority review of New Drug Application for sparsentan for the treatment of IgA nephropathy. https://ir.travere.com/news-releases/news-release-details/travere-therapeutics-announces-fda-acceptance-and-priority. May 16, 2022. Accessed June 1, 2022.

Travere Therapeutics Press Release. Travere Therapeutics Web site. Travere Therapeutics announces positive topline interim results from the ongoing Phase 3 PROTECT study of sparsentan in IgA nephropathy. https://ir.travere.com/news-releases/news-release-details/travere-therapeutics-announces-positive-topline-interim-results. August 16, 2021. Accessed June 1, 2022.

Travere Therapeutics Corporate Overview (May 2022). Travere Therapeutics Web site. https://ir.travere.com/static-files/4bc604c8-9800-4b10-bff2-3dcedb218255. May 23, 2022. Accessed June 1, 2022.

uniQure Press Release. uniQure Web site. uniQure announces FDA acceptance of Biologics License Application for etranacogene dezaparvovec under priority review. https://tools.eurolandir.com/tools/Pressreleases/GetPressRelease/?ID=4109235&lang=en-GB&companycode=nl-qure&v=. May 24, 2022. Accessed June 1, 2022.

UpToDate Database. https://www.uptodate.com.