OPTUMRx®

RxOutlook®

2nd Quarter 2021

In the second half of 2020, coronavirus disease 2019 (COVID-19) had a significant impact on drug development and led to delays in approvals due to travel restrictions impacting site inspections by the FDA. However, in addition to the pandemic's impact, the FDA provided several high-profile rejections or review extensions for new drug or biologic applications due to issues related to safety and/or efficacy. That trend has continued onward into the first half of 2021 with several key pipeline agents having significant uncertainty around their likelihood of approval (eg, aducanumab for Alzheimer's disease, roxadustat for chronic kidney disease [CKD], abrocitinib for atopic dermatitis). While the overall number of drug approvals for 2021 is expected to be similar to the previous two years, the final number will depend on some of these more contested reviews.

In this edition of RxOutlook, we highlight 10 key pipeline drugs with an expected launch between July to September 2021. Of note, the FDA has raised some questions regarding two of the drugs discussed in the report which could jeopardize their expected approval dates. This includes teplizumab, a novel anti-CD3 monoclonal antibody, for the delay of clinical type 1 diabetes mellitus (T1DM). An FDA Advisory Committee is scheduled to review the data for the drug on May 27. The second is avacopan, a novel compliment C5a receptor antagonist, for the treatment of ANCA-associated vasculitis (an orphan condition characterized by the destruction of small blood vessels). The FDA convened an Advisory Committee on May 6 with panelists voting narrowly in favor of approval by a margin of 10 to 8.

Several orphan drugs are expecting FDA approval decisions in the third quarter for conditions with limited treatment options. This includes odevixibat and maralixibat, which would represent the first approved treatments for rare conditions of the liver: progressive familial intrahepatic cholestasis and Alagille syndrome, respectfully.

In addition to the slate of orphan drugs, a couple key FDA approval decisions are expected in the nephrology space. Finerenone, a mineralocorticoid receptor antagonist similar in mechanism to generically available spironolactone and eplerenone, would potentially be the first drug in the class approved in CKD. Difelikefalin, a selective agonist of kappa opioid receptors, would be the first approved treatment for pruritus associated with hemodialysis and an alternative to existing off-label treatments (eg, antihistamines, corticosteroids, naltrexone, and gabapentin).

Approval decisions for other key novel therapies are expected in the third quarter but are not reviewed here (eg, roxadustat, abrocitinib). These drugs were covered in previous editions of RxOutlook, but as mentioned above, have experienced regulatory delays.

Key pipeline drugs with FDA approval decisions expected between July to September 2021

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Teplizumab	Provention Bio	Delay of type 1 diabetes	7/2/2021
Avacopan	ChemoCentryx	ANCA-associated vasculitis*	7/7/2021
Finerenone	Bayer	Chronic kidney disease	7/9/2021
Odevixibat	Albireo Pharma	Progressive familial intrahepatic cholestasis*	7/20/2021
Retifanlimab	Incyte	Anal cancer*	7/25/2021
Amivantamab	Johnson & Johnson	Non-small cell lung cancer	8/3/2021
Sotorasib	Amgen	Non-small cell lung cancer*	8/16/2021

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Difelikefalin	Cara Therapeutics	Hemodialysis-related pruritus	8/23/2021
Maralixibat	Mirum Pharmaceuticals	Alagille syndrome*	9/29/2021
Atogepant	AbbVie	Migraine prophylaxis	3Q 2021

* Orphan Drug Designation

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

Detailed Drug Insights

This section reviews the important characteristics (eg, therapeutic use, clinical profile, competitive environment and regulatory timeline) for key pipeline drugs with potential FDA approvals by the end of the 3rd Quarter 2021.

Read more

Extended Generic Pipeline Forecast

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

Read more

Extended Brand Pipeline Forecast

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

Read more

Key Pending Indication Forecast

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

Read more

Past and future reviews

Please note that RxOutlook highlights select near-term approvals. Some drugs may not appear in this issue because they have been reviewed in previous editions of RxOutlook. Drugs of interest that are earlier in development or with expected approvals beyond 3rd quarter 2021 may appear in future reports; however, for those who need an initial look at the full pipeline, please refer to the Brand Pipeline Forecast Table found later in this report.

Getting acquainted with pipeline forecast terms

Clinical trial phases

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

Pipeline acronyms

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

Detailed insights on key drugs

Teplizumab (Brand Name: To be determined)

Manufacturer: Provention Bio
Regulatory designations: Breakthrough Therapy
Expected FDA decision: 7/2/2021 ***(FDA Advisory Committee scheduled on 5/27/2021)***

Therapeutic use

- Delay of clinical T1DM in at-risk individuals

Teplizumab is in development for the delay of clinical type 1 diabetes mellitus (T1DM) in at-risk individuals.

T1DM is caused by the autoimmune destruction of insulin-producing beta cells in the pancreas. In genetically susceptible patients, T1DM typically progresses over several months or years and goes from asymptomatic stages to overt hyperglycemia. Progression to T1DM is characterized by the appearance of autoantibodies and then dysglycemia. Once patients develop overt hyperglycemia, insulin treatment is necessary.

Only about 5 to 10% of patients with diabetes have T1DM. Based on a Center for Disease Control and Prevention (CDC) report, the estimated number of new cases of T1DM in children and adolescents younger than age 20 years was 18,291. While most common in children and adolescents, T1DM can also develop in adulthood.

Provention Bio estimates that there are 30,000 individuals who are at-risk for T1DM (two or more diabetes-related autoantibodies, dysglycemia, and a familial direct relative with T1DM).

Clinical profile

- Anti-CD3 monoclonal antibody
- IV formulation
- Median time to diagnosis of T1DM: 59.6 months vs. 27.1 months with placebo
- Diabetes-free at end of follow-up: 50% vs. 22% with placebo
- Common AEs: Rash, transient lymphopenia
- Dosing: Once daily for a 14-day treatment course

Teplizumab is an anti-CD3 monoclonal antibody that works by modifying CD8+ T lymphocytes, which are thought to be important cells that kill beta cells in patients that develop T1DM.

Pivotal trial data:
The efficacy of teplizumab was evaluated in one Phase 2, randomized, placebo-controlled, double-blind study in 76 patients who did not yet have diabetes but were at high risk for development of clinical disease. All patients were required to have a familial direct relative with T1DM. Patients were randomized to a single 14-day course of teplizumab or placebo. The primary endpoint was the elapsed time from randomization to the clinical diagnosis of diabetes which was evaluated using oral glucose-tolerance tests at 6-month intervals.

In the extended follow-up (923-day median), the median time to diagnosis was 59.6 for teplizumab and 27.1 months for placebo-treated patients (hazard ratio [HR] 0.457, p = 0.01). Overall, 50% of teplizumab-treated patients vs. 22% of the placebo-treated remained diabetes-free at the end of the follow-up period.

Safety:
The most common adverse events with teplizumab use were rash and transient lymphopenia.

Dosing:
In the pivotal trial, teplizumab was administered intravenously (IV) daily for a 14-day treatment course.

Teplizumab (continued...)

Competitive environment

If approved, teplizumab would be the first therapy to delay the onset of T1DM. Delaying progression to clinical T1DM would reduce or eliminate the need for insulin early in childhood and adolescence. While adherence to insulin therapy and strict glucose monitoring is effective in managing T1DM, it can be challenging for many patients to maintain and can have a significant impact on quality of life.

The trial results for teplizumab were promising, with a single 14-day treatment course providing a significant delay in onset to T1DM vs. placebo. However, teplizumab is not a cure for T1DM and patients receiving the drug remain at risk for eventually developing the disease after a single treatment course.

The likely eligible patient population for teplizumab will be limited given T1DM represents a small subset of the overall diabetes population and use will be further narrowed since teplizumab was only evaluated in patients at high risk for developing T1DM (eg, patients with familial direct relatives with T1DM).

Finally, there is some regulatory uncertainty around the difference between the formulation of teplizumab evaluated in the trials vs. the formulation that will be commercialized.

- Advantages: Potentially the first therapy for delay of T1DM, promising trial results (eg, delaying insulin dependence, beta cell function) vs. placebo, short initial treatment course
- Disadvantages: Evaluated in a small single trial (76 patients), unknown whether repeat doses will be required, narrow target population (patients at high risk for developing T1DM), regulatory uncertainty, IV administration

Avacopan (Brand Name: To be determined)

Manufacturer: ChemoCentryx
Regulatory designations: Orphan Drug
Expected FDA decision: 7/7/2021 ***(FDA Advisory Committee met on 5/6/2021; panelists voted 10 to 8 in favor of approval)***

Therapeutic use

Avacopan is in development for treatment of antineutrophil cytoplasmic antibody (ANCA)-associated vasculitis.

ANCA-associated vasculitis is a group of diseases (granulomatosis with polyangiitis, eosinophilic granulomatosis with polyangiitis and microscopic polyangiitis), characterized by destruction and inflammation of small blood vessels. The condition can affect different organs in the body, including the kidney (most commonly affected), stomach, intestine, and lung. Skin lesions also occur as blood from small vessels leak under the skin. ANCA-associated vasculitis occurs when neutrophils attack small and medium vessels of the body. The exact pathophysiology is unclear, but overactivation of the alternative complement pathway is believed to be involved.

The exact prevalence of the condition is unknown, but a 2017 population-based study found an annual incidence in the U.S. of 3.3/100,000, with a prevalence of 42/100,000. ChemoCentryx estimates that there are 40,000 to 100,000 patients in U.S. with ~8,000 diagnosed annually.

- Treatment of ANCA-associated vasculitis

Avacopan (continued...)

Clinical profile

Avacopan is an orally administered compliment C5a receptor antagonist that blocks neutrophil chemoattraction and activation.

Pivotal trial data:
The efficacy of avacopan was evaluated in the ADVOCATE trial, a Phase 3, randomized, double-blind, active-controlled study in 331 patients with ANCA-associated vasculitis. Patients were randomized to receive avacopan plus either rituximab or cyclophosphamide (followed by azathioprine/mycophenolate) or prednisone plus either rituximab or cyclophosphamide (followed by azathioprine/mycophenolate). The first primary endpoint was remission, defined as a Birmingham Vasculitis Activity Score (BVAS) of 0 (on a scale from 0 to 63, with higher scores indicating greater disease activity) at week 26 and no glucocorticoid use in the previous 4 weeks. The second primary endpoint was sustained remission, defined as remission at both weeks 26 and 52. Both end points were tested for noninferiority (by a margin of 20 percentage points) and for superiority.

Remission at week 26 was observed in 72.3% receiving avacopan vs. 70.1% receiving prednisone ($p < 0.001$ for noninferiority; $p = 0.24$ for superiority). Sustained remission at week 52 was observed in 65.7% receiving avacopan vs. 54.9% receiving prednisone ($p < 0.001$ for noninferiority; $p = 0.007$ for superiority).

Safety:
Serious adverse events (excluding worsening vasculitis) occurred in 37.3% of the patients receiving avacopan and in 39.0% of those receiving prednisone.

Dosing:
In the pivotal trial, avacopan was administered orally twice daily.

- C5a receptor antagonist
- Oral formulation
- Remission at week 26: 72.3% vs. 70.1% with prednisone (noninferiority met; superiority not met vs. prednisone)
- Sustained remission at week 52: 65.7% vs. 54.9% with prednisone (noninferiority and superiority met vs. prednisone)
- Safety: Limited data available
- Dosing: Twice daily

Avacopan (continued...)

Competitive environment

If approved, avacopan would offer a first-in-class treatment for ANCA-associated vasculitis. The current standard of care for the treatment of ANCA-associated vasculitis includes induction therapy with high dose corticosteroids plus cyclophosphamide or rituximab; maintenance immunosuppressive therapy, usually with azathioprine, rituximab, or methotrexate, continues for at least 12 to 18 months. However, these regimens are associated with poor tolerability because of the need for long-term use of high dose corticosteroids.

The primary advantage with avacopan use is that it can reduce or eliminate the need for corticosteroids, thereby improving the tolerability of the treatment regimen. There may be some modest efficacy benefit vs. prednisone. In the pivotal trial, avacopan was noninferior to prednisone taper for inducing remission at week 26 but was superior to prednisone taper in terms of achieving sustained remission at week 52. Additionally, avacopan provided modest improvement in renal function vs. prednisone (as measured by the estimated glomerular filtration rate [eGFR]).

If approved, avacopan would compete with a standard of care that is inexpensive and has been used for many years. In addition to corticosteroids plus immunosuppressants for the broader ANCA-associated vasculitis population, Nucala® (mepolizumab) is also approved for eosinophilic granulomatosis with polyangiitis, one subtype of ANCA-associated vasculitis.

Finally, avacopan must be used in combination with immunosuppressants, so it does not completely change the treatment paradigm in terms of the overall regimen for ANCA-associated vasculitis.

- Advantages: First-in-class drug for ANCA-associated vasculitis, reduces/ eliminates the need for high dose corticosteroids which are associated with poor tolerability, may improve sustained remission rates, oral administration
- Disadvantages: Corticosteroids are available generically and have been part of the standard of care for years, avacopan did not demonstrate superiority vs. corticosteroids at week 26, does not eliminate the need for concomitant treatment with immunosuppressants

Finerenone (Brand Name: To be determined)

Manufacturer: Bayer
Expected FDA decision: 7/9/2021

Therapeutic use

- Treatment of patients with CKD and T2DM

Finerenone is in development for treatment of patients with chronic kidney disease (CKD) and type 2 diabetes mellitus (T2DM).

CKD is a condition in which the kidneys are damaged and cannot filter blood as well as they should. If left untreated, CKD can progress to kidney failure requiring dialysis or kidney transplant. CKD is also associated with increased risk of cardiovascular events (eg, stroke and myocardial infarction). Risk factors for CKD include diabetes, high blood pressure, heart disease, family history of CKD, and obesity.

Diabetes is one of the leading causes of CKD. There are 26.8 million people diagnosed with diabetes in the U.S. and about 1 in 3 people with diabetes develop some degree of CKD.

Clinical profile

- Mineralocorticoid receptor antagonist
- Oral formulation
- Composite renal event: 17.8% vs. 21.1% with placebo
- Common AE: Hyperkalemia
- Dosing: Once daily

Finerenone is a non-steroidal, selective mineralocorticoid receptor antagonist. Mineralocorticoid receptor overactivation is a driver of kidney and cardiovascular damage through inflammatory and fibrotic processes.

<u>Pivotal trial data:</u>
The efficacy of finerenone was evaluated in FIDELIO-DKD, a Phase 3, randomized, double-blind, placebo-controlled study in 5,734 patients with CKD and T2DM. Patients received finerenone or placebo. All the patients were treated with renin–angiotensin system blockade that had been adjusted before randomization to the maximum tolerated dose on the manufacturer's label. The primary outcome, assessed in a time-to-event analysis, was a composite of kidney failure, a sustained decrease of at least 40% in the estimated glomerular filtration rate (eGFR) from baseline, or death from renal causes. The key secondary composite outcome, also assessed in a time-to-event analysis, was death from cardiovascular causes, nonfatal myocardial infarction, nonfatal stroke, or hospitalization for heart failure.

During a median follow-up of 2.6 years, a primary outcome event occurred in 17.8% of patients treated with finerenone vs. 21.1% of patients treated with placebo (HR 0.82, 95% CI: 0.73, 0.93; p = 0.001). A key secondary outcome event occurred in 13.0% and 14.8% of patients treated with finerenone and placebo, respectively (HR 0.86, 95% CI: 0.75, 0.99; p = 0.03).

Finerenone (continued...)

Safety:
In the pivotal trial, hyperkalemia-related adverse events were twice as frequent with finerenone as with placebo (18.3% and 9.0%, respectively), and the frequency of hyperkalemia leading to discontinuation of the trial regimen was also higher with finerenone (2.3% and 0.9% in the respective groups). Overall, adverse events were otherwise similar in the finerenone and placebo groups.

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

Competitive environment

Finerenone would potentially be the first mineralocorticoid receptor antagonist approved for CKD.

Mineralocorticoid receptor antagonists (eg, spironolactone and eplerenone) have been available for decades and have shown some benefit in treating hypertension and reducing albuminuria in patients with CKD. However, the adoption of this class for CKD has been limited by their tendency to cause hyperkalemia, which is particularly problematic in CKD patients who are already at increased risk for hyperkalemia due to their underlying disease. Compared to the other drugs in the class, finerenone is the first to be studied in a large, randomized controlled trial in the CKD population and it may reduce some of the risk of hyperkalemia compared to the older drugs in the class. However, it does not eliminate the risk of hyperkalemia entirely.

Historically, pharmacological treatment of CKD in T2DM patients was limited to angiotensin-converting enzyme (ACE) inhibitors and angiotensin receptor blockers (ARBs). These drugs provide renal protection and lower blood pressure. More recently, data is emerging that sodium-glucose cotransporter 2 (SGLT2) inhibitors reduce the risk of kidney disease progression and cardiovascular events in patients with CKD. Most notably, Farxiga® (dapagliflozin), has shown benefit in CKD patients with or without T2DM and recently received FDA approval for this indication. Although different in mechanism, finerenone and SGLT2 inhibitors could compete in this space.

For reference, the wholesale acquisition cost (WAC) for Farxiga is approximately $6,500 per year.

- Advantages: Potentially first drug in the class approved for CKD, may reduce risk of hyperkalemia vs. other drugs in class (ie, spironolactone, eplerenone), oral and once daily administration
- Disadvantages: Other generically available drugs in class used off-label for CKD, indication limited to patients with T2DM, potentially competing with SGLT2 inhibitors in CKD and data is less robust, lack of head-to-head trial data
- Reference WAC (Farxiga): ~$6,500 per year

Retifanlimab (Brand Name: To be determined)

Manufacturer: Incyte
Regulatory designations: Orphan Drug
Expected FDA decision: 7/25/2021

Therapeutic use

Retifanlimab is in development for treatment of adult patients with locally advanced or metastatic squamous cell carcinoma of the anal canal (SCAC) who have progressed on, or who are intolerant of, platinum-based chemotherapy.

Anal cancer is relatively rare, with the American Cancer Society estimating about 9,090 new cases (6,070 in women and 3,020 in men) and about 1,430 deaths in 2021. The majority of anal cancer cases (nearly 9 out of 10) are squamous cell carcinomas.

Clinical profile

Retifanlimab is a monoclonal antibody that inhibits programmed cell death protein 1 (PD-1). Upregulation of PD-1 ligands occurs in some tumors and signaling through this pathway can contribute to inhibition of active T-cell immune surveillance of tumors. Blocking PD-1 activity can result in decreased tumor growth.

Pivotal trial data:
The efficacy of retifanlimab was evaluated in POD1UM-202, a Phase 2, open-label, single-arm study in 94 patients with SCAC who have progressed on, or who are intolerant of, platinum-based chemotherapy. The primary endpoint was objective response rate (ORR) as determined by independent central review using RECIST v1.1. Secondary endpoints include additional measures of clinical benefit - duration of response (DOR), disease control rate (DCR), progression-free survival (PFS) and overall survival (OS).

The ORR was 13.8%. The median DOR was 9.5 months (95% CI: 5.6, not estimable [NE]). Median PFS and OS were 2.3 (95% CI: 1.9, 3.6) and 10.1 (95% CI: 7.9, NE) months, respectively.

Safety:
The most common adverse events with retifanlimab use were fatigue and diarrhea.

Dosing:
In the pivotal trial, retifanlimab was administered IV every 4 weeks.

- Treatment of adult patients with locally advanced or metastatic SCAC who have progressed on, or who are intolerant of, platinum-based chemotherapy
- Monoclonal antibody targeting PD-1
- IV formulation
- ORR: 13.8%
- Median PFS: 2.3 months
- Median OS: 10.1 months
- Common AEs: Fatigue, diarrhea
- Dosing: Once every 4 weeks

Retifanlimab (continued...)

Competitive environment

Retifanlimab would potentially be the first approved therapy for second-line treatment of SCAC, a cancer for which there are limited treatment options aside from chemotherapy. Like other drugs in the class, retifanlimab is in development for other cancers and earlier settings of anal cancer.

However, it will be competing with other established PD-1 inhibitors (eg, Keytruda® [pembrolizumab], Opdivo® [nivolumab]) that are FDA approved for many other cancers (ie, Keytruda is approved for 18 different indications) and Keytruda and Opdivo are actually recommended in treatment guidelines off-label for anal cancer based on some limited trial data.

The data available for retifanlimab is based on one early stage trial; a larger phase 3 study in treatment naïve patients was initiated in late 2020 but the study is not expected to complete until 2024.

For reference, the WAC for Opdivo is approximately $13,500 per 4 weeks.

- Advantages: Potentially first approved therapy for second-line treatment of SCAC, limited alternative treatment options, also in development for other cancers and earlier settings of anal cancer
- Disadvantages: Competing with other established PD-1 inhibitors (eg, Keytruda, Opdivo) that are approved for many other cancers and recommended off-label for anal cancer, lack of late-stage trial data
- Reference WAC (Opdivo): ~$13,500 per 4 weeks

Odevixibat (Brand Name: To be determined)

Manufacturer: Albireo Pharma
Regulatory designations: Orphan Drug, Fast Track
Expected FDA decision: 7/20/2021

Therapeutic use

Odevixibat is in development for the treatment of pruritus in patients with progressive familial intrahepatic cholestasis (PFIC).

PFIC is a heterogeneous group of genetic disorders, characterized by impaired bile flow, or cholestasis. Signs and symptoms of PFIC typically begin in infancy. Affected individuals experience severe itching, jaundice, failure to gain weight and grow at the expected rate, portal hypertension, and an enlarged liver and spleen. Patients will often progress to liver failure and require partial external biliary diversion (PEBD) or liver transplantation.

There are three known types of PFIC: PFIC1, PFIC2, and PFIC3, with each type being associated with a different genetic cause. PFIC affects approximately 1 in 50,000 to 100,000 people worldwide. Albireo Pharma estimates a U.S. addressable population of about 600 people.

- Treatment of pruritus in patients with PFIC

Clinical profile

Odevixibat is an ileal bile acid transport (IBAT) inhibitor. In a healthy individual, most of the bile acid released from the liver for digestion and absorption of nutrients is recirculated back to the liver via IBAT. But in people with cholestatic liver diseases, bile flow is interrupted, which results in elevated levels of toxic bile acids accumulating in the body and liver. By inhibiting IBAT, odevixibat reduces bile acids returning to the liver and in turn reduces serum bile acid.

- IBAT inhibitor
- Oral formulation
- Pruritus response: 53.5% vs. 28.7% with placebo
- Serum bile acid response: 33.3% vs. no patients with placebo
- Mean change in serum bile acid: -114.3 µmol/L vs. 13.1 µmol/L with placebo
- Common AEs: Diarrhea, frequent bowel movements
- Dosing: Once daily

Odevixibat (continued...)

Pivotal trial data:
The efficacy of odevixibat was evaluated in PEDFIC 1 and PEDFIC 2. PEDFIC 1 was a Phase 3, randomized, double-blind, placebo-controlled study in 62 children with PFIC1 or PFIC2 and a history of significant pruritus. Patients received odevixibat 40 µg/kg/day, odevixibat 120 µg/kg/day, or placebo. The primary endpoints were change in pruritus based on a patient's proportion of positive pruritus assessments (PPAs; defined as a scratching score of ≤ 1 or a ≥ 1-point drop from baseline on Albireo observer-reported instrument) over 24 weeks and the proportion of patients with serum bile acids (sBA) response (defined as a ≥ 70% reduction from baseline or sBAs ≤ 70 µmol/L) at week 24.

The proportion of PPAs was 53.5% in the odevixibat arms vs. 28.7% in the placebo arm ($p = 0.004$). Additionally, 33.3% of patients in the odevixibat arms achieved sBA response vs. no patients in the placebo arm ($p = 0.003$). As a secondary endpoint, mean reduction of bile acids was 114.3 µmol/L in the odevixibat arms compared to an increase of 13.1 µmol/L in the placebo arm ($p = 0.002$). Both doses of odevixibat were statistically significant for each of the endpoints.

PEDFIC 2 was an extension study that included patients treated with odevixibat (patient group P1O), as well as patients treated with placebo (patient group P1P) in PEDFIC 1. Patients with 48 weeks of cumulative odevixibat exposure (P10 group) achieved a mean reduction in sBAs from 251.8 µmol/L to 85.1 µmol//L ($p < 0.0001$) and a mean monthly improvement in the pruritus score, defined as a drop from baseline of 1.0 point or more on the 0 to 4 point scale, from 3.0 to 1.4 ($p < 0.0001$). In patients exposed to odevixibat for 48 weeks, mean height Z scores also improved from -1.6 to -0.5 ($p = 0.02$) from baseline to PEDFIC 2 week 24, and mean weight Z scores normalized over 48 weeks (-0.9 to 0.2; $p = 0.03$).

Safety:
The most common adverse events with odevixibat use were diarrhea and frequent bowel movements.

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

Odevixibat (continued...)

Competitive environment

Odevixibat would potentially be the first approved treatment for PFIC. There is a high unmet need as the only effective treatments for the condition are surgical interventions and transplantation. The disease is typically unresponsive to conventional treatments that are used off-label, like ursodeoxycholic acid, antihistamines, or rifampicin.

The trial results were promising with odevixibat meeting both primary endpoints: reduction in pruritus and serum bile acid. Odevixibat is also being evaluated for other rare liver conditions such as Alagille syndrome which would expand the eligible population for this drug.

Although odevixibat demonstrated improvements in quality of life and bile acid levels, data on more long-term clinical outcomes, such as prevention of liver failure or reducing the need for liver transplantation, is lacking. The initial target population is expected to be very small, due to the rarity of this condition and that odevixibat would be used in patients with more moderate-to-severe disease.

Odevixibat could eventually compete with another pipeline drug that shares a similar mechanism of action: maralixibat (see below). Both drugs address bile duct dysfunction but in different ways. Their initial indications are for different uses, but they could eventually have mirroring indications.

- Advantages: Potentially first approved treatment for PFIC, high unmet need, promising trial results, also in development for other liver conditions (ie, biliary atresia and Alagille syndrome)
- Disadvantages: Lack of long-term data showing benefit for liver protection, small initial target population, potential future competition with maralixibat

Maralixibat (Brand Name: To be determined)

Manufacturer: Mirum Pharmaceuticals
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 9/29/2021

Therapeutic use

- Treatment of ALGS in patients 1 year and older

Maralixibat is in development for the treatment of Alagille Syndrome (ALGS) in patients 1 year and older.

ALGS is a rare genetic disorder in which bile ducts are decreased in number and have structural abnormalities, leading to increased bile acid levels and progressive liver disease. Common symptoms include jaundice, pruritus and xanthomas, with 60% to 70% of ALGS patients requiring liver transplantation before adulthood. Children with ALGS experience decreased quality of life from the intense pruritus, skin lesions and sleep/mood disruptions. The pruritus associated with ALGS is present in most affected children by the second year of life.

While the exact number of ALGS cases is unknown, it is estimated to affect 1 in every 30,000 individuals in the U.S.

Clinical profile

- ASBT inhibitor
- Oral formulation
- Mean change in serum bile acid levels: -17 µmol/L with maralixibat vs. 94 µmol/L with placebo
- Mean change in Itch Reported Outcome score: 0.3 with maralixibat vs. 1.7 with placebo
- Common AEs: Diarrhea, abdominal pain
- Dosing: Once daily

Maralixibat is a novel apical sodium-dependent bile acid transporter (ASBT) inhibitor. The ASBT is present in the small intestine and is responsible for the uptake of bile acids and recycling them back to the liver. ASBT inhibition increases the excretion of bile acids in the feces, which lowers serum bile acid (sBA) levels and improves ALGS symptoms.

Pivotal trial data:
The efficacy of maralixibat was evaluated in one Phase 2b, randomized, double-blind, placebo-controlled study (ICONIC) in 30 patients aged 12 months to 18 years old with ALGS. Patients were treated with maralixibat during a run-in period until there was a response (defined as a reduction of sBA ≥ 50% from baseline) and then were randomized to maralixibat daily or placebo for 4 weeks. After the 4 weeks, all patients were treated with maralixibat for the remainder of the 48 weeks. The primary endpoint was the mean change of fasting sBA levels. A key secondary endpoint was the average morning Itch Reported Outcome (ItchRO) score.

The mean change in sBA levels was -87 µmol/L ($p < 0.001$) with maralixibat during the run-in period. In the randomized period, the mean change in sBA levels was -17 µmol/L with maralixibat and 94 µmol/L with placebo ($p = 0.038$). For the overall group, the change was -101 µmol/L ($p = 0.003$) at week 48.

The mean reduction in weekly average ItchRO score was -1.7 during the run-in period. ItchRO increased by 0.3 with maralixibat and 1.7 with placebo during the randomization period ($p = 0.008$) and there was a -1.6-point reduction for the overall group at week 48.

Safety:
The most common adverse events with maralixibat were diarrhea and abdominal pain.

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

Maralixibat (continued...)

Competitive environment

If approved, maralixibat would be the first FDA approved treatment for Alagille Syndrome. Current management focuses on supportive care of subsequent liver disease, pruritus and malnutrition. Cholestyramine, colesevelam, ursodiol and naltrexone have been used off-label to treat pruritus in ALGS, with variable success. Maralixibat is also being evaluated for other rare liver conditions such as PFIC and biliary atresia, which would expand the eligible population for this drug.

Although trial results were promising and demonstrated improvements in serum bile acid levels and pruritus, they did fail to assess the effect of maralixibat on long-term liver benefit or if treatment reduces the likelihood of a liver failure or need for liver transplant.

In addition, as mentioned above, Albireo Pharma's odevixibat is an IBAT inhibitor that works similarly to maralixibat. While the initial indication for these drugs are for different uses, they are both in development for the same indications and would eventually have mirroring indications. Moreover, there are tolerability concerns with maralixibat, as 42% of patients experienced diarrhea and 71% experienced any gastrointestinal (GI) related adverse events during clinical trials. However, odevixibat was relatively well tolerated in clinical trials and when compared indirectly, did not have the same rates of GI related adverse events. Odevixibat could represent a more well-tolerated option for ALGS and a direct competitor to maralixibat in the future.

- Advantages: Potentially first approved treatment for ALGS, high unmet need, oral and once daily administration, also in development for other liver conditions (ie, PFIC and biliary atresia)
- Disadvantages: Patients experienced high rates of GI side effects in clinical trials, lack of long-term data showing benefits for liver protection, potential future competition with odevixibat

Amivantamab (Brand Name: To be determined)

Manufacturer: Johnson & Johnson
Regulatory Designations: Breakthrough Therapy
Expected FDA decision: 8/3/2021

Therapeutic use

Amivantamab is in development for the treatment of adults with metastatic non-small cell lung cancer (NSCLC) with epidermal growth factor receptor (EGFR) exon 20 insertion mutations whose disease has progressed on or after platinum-based chemotherapy.

- Treatment of metastatic NSCLC in adult patients with EGFR exon 20 insertion mutations whose disease has progressed on or after platinum-based chemotherapy

Lung cancer is the most common cancer in the world, with approximately 541,000 Americans diagnosed and 230,000 new cases annually in the U.S. NSCLC makes up 80 to 85% of all lung cancers, with most patients (66%) having advanced or metastatic disease at initial diagnosis. The most frequent sites of distant metastasis are the liver, adrenal glands, bones and brain.

The most common driver mutations in NSCLC are alterations in EGFR, which is a tyrosine kinase receptor that helps cells grow and divide. The frequency of EGFR exon 20 insertion mutations in NSCLC ranges from 1 to 10%, with 4% most commonly reported. NSCLC driven by these mutations are generally insensitive to current standard of care tyrosine kinase inhibitor (TKI) treatment and are associated with a worse prognosis compared to other EGFR mutations. Patients with an EFGR exon 20 insertion mutation have a median survival of less than 7 months.

Clinical profile

Amivantamab is a monoclonal bispecific antibody targeting EGFR and mesenchymal epithelial transition factor (MET). Both EGFR and MET are tyrosine kinase receptors responsible for uncontrolled cellular division and metastasis when overexpressed.

- EGFR and MET monoclonal antibody
- IV formulation
- ORR response: 40%
- Median PFS: 8.3 months
- Median OS: 22.8 months
- Common AEs: Rash, infusion related reactions, paronychia, stomatitis, pruritis
- Dosing: Once weekly for cycle 1 (28-day cycle) and every other week thereafter

<u>Pivotal trial data:</u>
The efficacy of amivantamab was evaluated in the CHRYSALIS trial, a Phase 1, single-arm, open-label study in 81 patients 18 years or older with metastatic or unresectable NSCLC and EGFR exon 20 insertion mutations whose disease progressed on or after platinum-based chemotherapy. The primary endpoint was ORR. Secondary endpoints included PFS and OS.

The ORR was 40% (95% CI: 29, 51). For patients who had received two prior treatment regimens, the ORR was 29%, whereas patients who had received three or more treatment regimens had an ORR of 58%. Median PFS was 8.3 months (95% CI: 6.5, 10.9) and median OS was 22.8 months (95% CI: 14.6, not reached).

<u>Safety:</u>
The most common adverse events with amivantamab use were rash, infusion related reactions, paronychia, stomatitis, and pruritis.

<u>Dosing:</u>
In the pivotal trial, amivantamab was administered intravenously every week for cycle 1 (28-day cycle) and every other week thereafter (cycle 2 onwards).

Amivantamab (continued...)

Competitive environment

If approved, amivantamab would offer the first treatment approved by the FDA for NSCLC with EGFR exon 20 insertion mutations. Clinical guidelines do not provide specific recommendations for EGFR exon 20 insertion mutations and patients have limited options following chemotherapy.

Amivantamab was only studied in one small, single-arm clinical trial and is targeting a narrow patient population, which may limit its early use. In addition, there are other products in the pipeline, such as Takeda's oral TKI mobocertinib, which is in development for a similar population as amivantamab. Takeda filed a New Drug Application (NDA) with the FDA and a decision is expected by October 26, 2021. Compared indirectly, amivantamab does appear to be better tolerated as mobocertinib was associated with high rates of GI adverse events (90% of patients reported diarrhea).

For reference, the WAC for Zepzelca™ (lurbinectedin), a recently approved alkylating agent used for the treatment of metastatic small cell lung cancer, is approximately $13,300 per cycle (21 days).

- Advantages: Potentially first approved therapy for NSCLC with EGFR exon 20 insertion mutation, well tolerated, unmet need
- Disadvantages: Narrow target population, evaluated in a small single trial (81 patients), IV administration, potential future competition with Takeda's mobocertinib
- Reference WAC (Zepzelca): ~$13,300 per cycle (21 days)

Sotorasib (Brand Name: Lumakras™)

Manufacturer: Amgen
Regulatory Designations: Orphan Drug, Breakthrough Therapy, Fast Track
Expected FDA decision: 8/16/2021

Therapeutic use

Sotorasib is in development for the treatment of metastatic NSCLC in adults with KRAS G12C who have previously tried systemic anticancer therapy.

KRAS G12C is another common driver mutation in NSCLC. About 13% of patients with NSCLC have the KRAS G12C mutation in the U.S. and each year approximately 25,000 new patients in the U.S. are diagnosed with KRAS G12C mutated NSCLC. Poor outcomes have been associated in the second-line treatment of KRAS G12C driven NSCLC.

- Treatment of metastatic NSCLC in adult patients with KRAS G12C mutations who have previously tried systemic anticancer therapy

Clinical profile

Sotorasib is a selective KRAS p.G12C inhibitor. The KRAS gene is responsible for cellular division/growth and a mutation can cause abnormal cell growth.

- Selective KRAS G12C inhibitor
- Oral formulation
- ORR: 37.1%
- Median PFS: 6.8 months
- Common AEs: Diarrhea, nausea, increased ALT and AST
- Dosing: Once daily

<u>Pivotal trial data:</u>
The efficacy of sotorasib was evaluated in CodeBreak 100, a Phase 1/2, randomized, open-label, single-arm trial in 126 patients 18 years or older with advanced NSCLC with KRAS G12C who have previously received systemic anticancer therapy. The primary endpoint was ORR. A key secondary endpoint was median PFS.

The ORR was 37.1% (95% CI: 28.6, 46.2). Median PFS was 6.8 months (95% CI: 5.1, 8.2).

<u>Safety:</u>
The most common adverse events with sotorasib use were diarrhea, nausea, increased alanine and aspartate transaminase (ALT/AST).

<u>Dosing:</u>
In the pivotal trial, sotorasib was administered orally daily.

Competitive environment

If approved, sotorasib would be the first KRASG12C inhibitor for advanced and/or metastatic NSCLC. Currently, patients have limited treatment options following first-line therapy. Sotorasib is also being studied for colorectal cancers and other solid tumors.

Sotorasib was only studied in a small initial patient population and is targeting a narrow patient population, which may limit its early use. In addition, there are other products in the pipeline, such as Mirati Therapeutics' adagrasib, which is another oral KRAS inhibitor for NSCLC. Mirati is expected to file an NDA for adagrasib in the second half of 2021. However, adagrasib has been associated with QT prolongation, which was not detected in sotorasib clinical trials.

For reference, the WAC for Tepmetko® (tepotinib), a recently approved oral MET inhibitor used for NSCLC, is approximately ~$21,000 per 30 days.

- Advantages: Potentially first approved therapy for NSCLC with KRAS G12C mutation, unmet need, also in development for colorectal cancer and other solid tumors
- Disadvantages: Narrow initial target population, potential future competition with Mirati Therapeutics' adagrasib
- Reference WAC (Tepmetko): ~$21,000 per 30 days

Difelikefalin (Brand Name: Korsuva®)

Manufacturer: Cara Therapeutics
Regulatory designations: Breakthrough Therapy
Expected FDA decision: 8/23/2021

Therapeutic use

Difelikefalin is in development for the treatment of moderate-to-severe pruritus in adult patients on hemodialysis.

- Treatment of moderate-to-severe pruritus in adult patients on hemodialysis

Pruritus in hemodialysis occurs with high frequency and intensity in patients with chronic kidney disease undergoing dialysis. The pathogenesis is not completely understood, but is thought to be due to metabolic disturbances, dysregulated immune response, and imbalances in the endogenous opioid system. It is present in approximately 40% of patients with end-stage renal disease (ESRD) and is associated with poor sleep quality, depression, reduced quality of life, increased risk of infection and increased risk of death.

It is estimated that approximately 468,000 individuals in the U.S. are on dialysis with 40% of those patients reporting moderate-to-severe pruritus.

Clinical profile

Difelikefalin is a peripherally restricted and selective agonist of kappa opioid receptors. Activation of kappa opioid receptors on peripheral neurons and immune cells creates an antipruritic effect. Difelikefalin's chemical structure prevents diffusion to the central nervous system and brain, minimizing any potential abuse potential.

- Peripherally restrictive selective agonist of kappa opioid receptors
- IV formulation
- Percentage with a decrease of ≥ 3 points in the WI-NRS score: 49% to 54% vs. 28% to 42% with placebo
- Common AEs: Diarrhea, falling, dizziness, vomiting, nausea
- Dosing: Three times a week

The manufacturer has not provided any information at this time to suggest that difelikefalin would be a controlled substance.

Difelikefalin (continued...)

Pivotal trial data:
The efficacy of difelikefalin was evaluated in two pivotal clinical trials: KALM-1 and KALM-2. Both were Phase 3, randomized, double-blind, placebo-controlled studies in 378 and 473 hemodialysis patients respectively, who had moderate-to-severe pruritus. Patients were randomized to difelikefalin or placebo three times per week for 12 weeks. The primary outcome was the percentage of patients with an improvement (decrease) of at least 3 points from baseline at week 12 on the 24-hour Worst Itching Intensity Numerical Rating Scale (WI-NRS). The WI-NRS is a patient-reported survey that is used to record the severity of itch symptoms during the previous 24-hour period. Scores range from 0 to 10, with higher scores indicating greater itch intensity.

In KALM-1, the percentage of patients who had a decrease of at least 3 points in the WI-NRS score was 49.1% with difelikefalin and 27.9% with placebo ($p < 0.001$). In KALM-2, the percentage of patients who had a decrease of at least 3 points in the WI-NRS score was 54% with difelikefalin and 42% with placebo ($p = 0.02$).

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

Dosing:
In the pivotal trial, difelikefalin was administered IV three times per week after dialysis.

Competitive environment

Difelikefalin could be the first approved therapy for the treatment for pruritus in hemodialysis. Current off-label treatments include topical options such as emollients, and UV radiation. Systemic off-label treatment options include antihistamines, corticosteroids, naltrexone, sertraline, and gabapentin. Current treatments are all off-label and have been reported to have moderate efficacy in treating pruritus throughout the years. Difelikefalin is also being studied as an oral formulation and for other indications such as pruritus in chronic liver disease, notalgia paresthetica (a condition characterized by severe itching of the back), and atopic dermatitis.

However, while difelikefalin has demonstrated efficacy in treating hemodialysis associated pruritus, there are many different treatments options currently available (many of which have generic alternatives or are over-the-counter). Although off-label, these alternatives may offer more lower cost options to treating pruritus in hemodialysis, as compared to difelikefalin.

- Advantages: Potentially the first approved treatment for pruritus in hemodialysis, unmet need, also in development other conditions (eg, chronic liver disease, notalgia paresthetica, atopic dermatitis)
- Disadvantages: Primary outcome was a patient reported outcome, off-label alternatives (eg, emollients, antihistamines, gabapentin) are currently used with modest effectiveness

Atogepant (Brand Name: To be determined)

Manufacturer: AbbVie
Expected FDA decision: 3Q 2021

Therapeutic use

- Prevention of episodic and chronic migraine in adults

Atogepant is in development for the prevention of episodic and chronic migraine in adults.

Migraines are a debilitating headache disorder that are characterized by moderate-to-severe unilateral and pulsating headaches that can last 4 to 72 hours. Symptoms may or may not also be associated with visual disturbances, nausea/vomiting, photophobia or phonophobia. These symptoms can greatly decrease quality of life and productivity.

Migraines most commonly affect individuals aged 18 to 44 and disproportionately impacts women. Approximately 39 million individuals in the U.S. are affected by migraines, with approximately 5 million currently receiving therapy for migraine prevention.

Clinical profile

- CGRP receptor antagonist
- Oral formulation
- Change from baseline in mean monthly migraine days: -3.69 to -4.20 days vs. -2.48 days with placebo
- Percentage of patients with ≥ 50% reduction in mean monthly migraine days: 56% to 61% with vs. 29% with placebo
- Common AEs: Constipation, nausea, upper respiratory tract infection
- Dosing: Once daily

Atogepant is a calcitonin gene-related peptide (CGRP) receptor antagonist.

Although not completely understood, it is thought that migraines are caused by signaling in the trigeminovascular system and neurogenic inflammation. In addition, studies have found that CGRP and its receptors are expressed in regions of the nervous system associated with migraine pathophysiology and are elevated during migraine attacks. By binding to CGRP receptors, atogepant inhibits the trigeminocervical pain transmission pathway and vasodilatory component of neurogenic inflammation.

Atogepant (continued...)

Pivotal trial data:
The efficacy of atogepant was evaluated in a Phase 3, randomized, double-blind, placebo-controlled study in 910 patients with chronic migraine (≥ 15 migraine days per month). Patients were randomized to atogepant 10 mg, atogepant 30 mg, atogepant 60 mg and placebo taken once daily. The primary endpoint was the change from baseline in mean monthly migraine days across 12 weeks.

The change from baseline in mean monthly migraine days was -4.2 days with atogepant 60 mg, -3.86 days with atogepant 30 mg, -3.69 days with atogepant 10 mg ($p < 0.0001$ for all atogepant strengths vs placebo) and -2.48 days with placebo. The percentage of patients with ≥ 50% reduction in mean monthly migraine days at week 12 was 60.8% with atogepant 60 mg, 58.7% with atogepant 30 mg, 55.6% with atogepant 10 mg ($p < 0.0001$ for all atogepant strengths vs placebo) and 29% with placebo.

Atogepant was also evaluated in a Phase 2/3, randomized, double-blind study in patients with episodic migraine (4 to 14 migraine days per month). Patients were randomized to atogepant 10 mg once daily (QD), atogepant 30 mg QD, atogepant 30 mg twice daily (BID), atogepant 60 mg QD, atogepant 60 mg BID, and placebo. The primary endpoint was the change from baseline in mean monthly migraine days across 12 weeks.

The mean change in monthly migraine days (p-values vs. placebo) were: placebo (-2.85), atogepant 10 mg QD (-4.00, $p = 0.0236$), 30 mg QD (-3.76, $p = 0.0390$), 30 mg BID (-4.23, $p = 0.0034$), 60 mg QD (-3.55, $p = 0.0390$), and 60 mg BID (-4.14, $p = 0.0031$).

Safety:
The most common adverse events with atogepant use were constipation, nausea, and upper respiratory tract infection.

Dosing:
In the pivotal trials, atogepant was administered orally once daily.

Atogepant (continued...)

Competitive environment

If approved, atogepant would offer an additional oral treatment option for migraine prophylaxis. Current oral treatments for migraine prophylaxis include beta blockers (eg propranolol), antidepressants (eg, amitriptyline), and anticonvulsants (eg, topiramate, valproic acid). Injectable treatments include Botox® (onabotulinumtoxinA) and CGRP antagonists (ie, Aimovig® [erenumab], Ajovy® [fremanezumab], Emgality® [galcanezumab], Vyepti™ [eptinezumab]).

However, Biohaven's Nurtec® ODT (rimegepant) is another oral CGRP antagonist approved for the treatment of acute migraine. It is currently being evaluated for the preventative treatment of migraines and an FDA decision is expected earlier than atogepant (May to June 2021). Nurtec may potentially be more convenient than atogepant, as it comes in an ODT formulation (which aids in swallowing as migraine patients often suffer from severe nausea) and is dosed every other day for migraine prevention. If approved, Nurtec ODT would represent the first CGRP antagonist to be approved for both the treatment and prevention of migraines, unlike atogepant which would be indicated for migraine prevention only. Atogepant is entering a crowded market and will likely compete with not only Nurtec ODT but with the other current treatment options for migraine prevention.

While it is difficult to compare across clinical trials, the efficacy of atogepant does appear to be similar or incrementally better than the injectable CGRP antagonists and Nurtec ODT. In clinical trials, treatment with atogepant resulted in an approximately 30% improvement in the percentage of patients with at least a 50% reduction in mean monthly migraine days, whereas a 22 to 24% improvement was achieved with the injectable CGRP antagonists. Moreover, treatment with atogepant resulted in a 1.7-day reduction in mean monthly migraine days compared to a 0.8-day reduction achieved with Nurtec ODT.

For reference, the WAC for Aimovig, and Nurtec are approximately $638 and $1,673 per month, respectively.

- Advantages: Currently no oral CGRP antagonists approved for migraine prevention, well tolerated
- Disadvantages: Daily use vs. monthly injections for other CGRP antagonists (eg, Aimovig, Ajovy, Emgality), not indicated for the treatment of migraines, Biohaven's Nurtec ODT is an oral CGRP likely to be approved for migraine prevention in 2Q 2021
- Reference WAC (Aimovig): ~$638 per month
- Reference WAC (Nurtec): ~ $1,673 per month

2nd Quarter 2021

Extended generic pipeline forecast

OptumRx generic pipeline forecast

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
2021 Possible launch date					
BEPREVE	bepotastine	Bausch Health	Ophthalmic	All	2021
THALOMID	thalidomide	Celgene	Oral	All	2021
DALIRESP	roflumilast	AstraZeneca	Oral	All	2021
RESTASIS	cyclosporine	Allergan	Ophthalmic	All	2021
BYETTA	exenatide	AstraZeneca	Subcutaneous	All	2021
DUREZOL	difluprednate	Alcon	Ophthalmic	All	2021
TOVIAZ	fesoterodine	Pfizer	Oral	All	2021
SYNDROS	dronabinol	Insys Therapeutics	Oral	All	2021
CUVPOSA	glycopyrrolate	Merz	Oral	All	2021
EPIDUO FORTE	adapalene/benzoyl peroxide	Galderma	External	All	2021
RESCULA	unoprostone isopropyl	R-Tech Ueno	Ophthalmic	All	2021
CHANTIX	varenicline	Pfizer	Oral	All	1H-2021
FORTEO	teriparatide	Eli Lilly	Injection	All	2Q-2021
PERFOROMIST	formoterol fumarate	Mylan	Inhalation	All	06-2021
NARCAN	naloxone	Emergent BioSolutions	Intranasal	All	2H-2021
JEVTANA KIT	cabazitaxel	Sanofi	Intravenous	All	2H-2021
FERAHEME	ferumoxytol	AMAG Pharmaceuticals	Intravenous	All	07-2021
SUTENT	sunitinib	Pfizer	Oral	All	08-2021
BYSTOLIC	nebivolol	Allergan	Oral	All	09-2021
LUCENTIS	ranibizumab	Roche	Intravitreal	All	09-2021
INNOPRAN XL	propranolol	Ani Pharmaceuticals	Oral	All	10-2021
BROVANA	arformoterol	Sunovion	Inhalation	All	11-2021
CAYSTON	aztreonam lysine	Gilead	Inhalation	All	12-2021
EXPAREL	bupivacaine	Pacira	Injection	All	12-2021
2022 Possible launch date					
DULERA	formoterol fumarate/mometasone furoate	Merck	Inhalation	All	2022

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
FLOVENT HFA	fluticasone propionate	GlaxoSmithKline	Inhalation	All	2022
POMALYST	pomalidomide	Celgene	Oral	All	2022
DEXILANT	dexlansoprazole	Takeda	Oral	All	2022
IXEMPRA Kit	ixabepilone	R-Pharm	Intravenous	All	1H-2022
NATPARA	parathyroid hormone 1-84	NPS/Nycomed	Subcutaneous	All	01-2022
NPLATE	romiplostim	Amgen	Subcutaneous	All	01-2022
OXAYDO	oxycodone	Egalet	Oral	All	01-2022
EPANED KIT	enalapril	Silvergate	Oral	All	01-2022
ONEXTON	clindamycin/benzoyl peroxide	Bausch Health	External	All	01-2022
NEUPRO	rotigotine	UCB	External	All	01-2022
AFINITOR DISPERZ	everolimus	Novartis	Oral	All	01-2022
SUPREP BOWEL PREP KIT	magnesium sulfate anhydrous/ potassium sulfate / sodium sulfate	Braintree	Oral	All	01-2022
BALCOLTRA	levonorgestrel/ethinyl estradiol/ ferrous bisglycinate	Avion	Oral	All	01-2022
SELZENTRY	maraviroc	ViiV Healthcare	Oral	All	02-2022
VIMPAT	lacosamide	UCB	Intravenous; oral	All	03-2022
ZIPSOR	diclofenac potassium	Depomed	Oral	All	03-2022
CHOLBAM	cholic acid	Retrophin	Oral	All	03-2022
ABRAXANE	paclitaxel	Celgene/Abraxis	Injection	All	03-2022
REVLIMID	lenalidomide	Bristol-Myers Squibb/Celgene	Oral	All	03-2022
ARESTIN	minocycline hydrochloride	Bausch Health	Subgingival	All	03-2022
PRADAXA	dabigatran etexilate mesylate	Boehringer Ingelheim	Oral	All	2Q-2022
LEXISCAN	regadenoson	Astellas	Intravenous	All	04-2022
COMBIGAN	brimonidine/timolol	Allergan	Ophthalmic	All	04-2022
ZOLADEX	goserelin	TerSera Therapeutics	Subcutaneous	All	04-2022
ALIMTA	pemetrexed disodium	Eli Lilly	Intravenous	All	05-2022
VELCADE	bortezomib	Takeda	Intravenous	All	05-2022
TARGINIQ ER	oxycodone/naloxone	Purdue	Oral	All	05-2022
CAPRELSA	vandetanib	Genzyme/Sanofi	Oral	All	06-2022
VIIBRYD	vilazodone	Forest/Allergan	Oral	All	06-2022
ELESTRIN	estradiol	Mylan	External	All	06-2022

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
ACTEMRA	tocilizumab	Roche/Chugai	Intravenous; subcutaneous	All	2H-2022
IRESSA	gefitinib	AstraZeneca	Oral	All	07-2022
EVAMIST	estradiol	Perrigo/Elan	External	All	07-2022
KEVEYIS	dichlorphenamide	Strongbridge Biopharma	Oral	All	08-2022
ORAVIG	miconazole	Galt Pharmaceuticals	Oral	All	09-2022
ORENCIA	abatacept	Bristol-Myers Squibb	Intravenous; subcutaneous	All	11-2022
XERESE	acyclovir/hydrocortisone	Bausch Health	External	All	11-2022
NAGLAZYME	galsulfase	BioMarin	Intravenous	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	75 mg, 150 mg, 300 mg	2023
PROLENSA	bromfenac	Bausch Health	Ophthalmic	All	2023
ALPHAGAN P	brimonidine	Allergan	Ophthalmic	All	2023
KOMBIGLYZE XR	saxagliptin/metform	Astra Zeneca	Oral	All	1H-2023
ONGLYZA	saxagliptin	AstraZeneca	Oral	All	1H-2023
AMZEEQ	minocycline	Foamix	External	All	1Q-2023
NOXAFIL	posaconazole	Merck	Intravenous	All	01-2023
HUMIRA	adalimumab	AbbVie	Subcutaneous	All	01-2023
APIDRA	insulin glulisine recombinant	Sanofi	Subcutaneous	All	01-2023
DUEXIS	ibuprofen/famotidine	Horizon Pharma	Oral	All	01-2023
XYREM	sodium oxybate	Jazz	Oral	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
GLOPERBA	colchicine	Avion Pharmaceuticals	Oral	All	01-2023

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
FIRVANQ KIT	vancomycin	Azurity	Oral	All	01-2023
LUMIZYME	alglucosidase alfa	Genzyme	Intravenous	All	02-2023
LATUDA	lurasidone	Sunovion	Oral	All	02-2023
GATTEX	teduglutide recombinant	Takeda	Subcutaneous	All	03-2023
AGGRASTAT	tirofiban	Medicure	Intravenous	All	03-2023
AUBAGIO	teriflunomide	Sanofi/Genzyme	Oral	All	03-2023
DEFITELIO	defibrotide	Jazz	Intravenous	All	03-2023
PROVAYBLUE	methylene blue	Provepharm/American Regent	Intravenous	All	04-2023
KEPIVANCE	palifermin	Swedish Orphan Biovitrum	Intravenous	All	04-2023
CLINDESSE	clindamycin phosphate	Perrigo	Vaginal	All	04-2023
CORLANOR	ivabradine	Amgen	Oral	All	04-2023
DALVANCE	dalbavancin	Amgen	Intravenous	All	05-2023
LIVALO	pitavastatin	Eli Lilly/Kowa Pharmaceuticals	Oral	All	05-2023
KYNMOBI	apomorphine	Sunovion	Sublingual	All	05-2023
BIJUVA	estradiol/progesterone	TherapeuticsMD	Oral	All	06-2023
XURIDEN	uridine	Wellstat Therapeutics	Oral	All	07-2023
TOLAK	fluorouracil	Pierre Fabre	External	All	07-2023
MOZOBIL	plerixafor	Sanofi/Genzyme	Subcutaneous	All	07-2023
MYRBETRIQ	mirabegron	Astellas	Oral	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
TEMODAR	temozolomide	Merck	Injection	All	09-2023
STELARA	ustekinumab	Janssen	Subcutaneous	All	09-2023
VIBATIV	telavancin	Theravance	Intravenous	All	09-2023
LEXETTE	halobetasol	Mayne	External	All	09-2023
VOTRIENT	pazopanib	Novartis	Oral	All	10-2023
OZURDEX	dexamethasone	Allergan	Ophthalmic	All	11-2023
AMTURNIDE	aliskiren/amlodipine/hydrochlorothiazide	Novartis	Oral	All	11-2023
VESICARE LS	solifenacin	Astellas	Oral	All	11-2023

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
KOGENATE FS	octocog alpha	Bayer	Intravenous	All	11-2023
HELIXATE FS	antihemophilic factor VIII	CSL Behring/Bayer	Intravenous	All	11-2023
MULTAQ	dronedarone	Sanofi	Oral	All	12-2023
GIAZO	balsalazide disodium	Bausch Health	Oral	All	12-2023
KALBITOR	ecallantide	Dyax	Subcutaneous	All	12-2023
GILENYA	fingolimod	Novartis	Oral	0.5 mg	12-2023
LANTUS SOLOSTAR	insulin glargine	Sanofi Aventis	Subcutaneous	All	12-2023
LANTUS	insulin glargine	Sanofi	Subcutaneous	All	12-2023

Extended brand pipeline forecast

OptumRx Brand Pipeline Forecast

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
2021 Possible launch date									
FP-001 (LMIS)	leuprolide mesylate	Foresee	gonadotropin-releasing hormone analog	Prostate cancer	SC	Filed NDA	5/27/2021	Yes	No
DS-100	dehydrated alcohol	Eton	undisclosed	Methanol poisoning	SC	Filed NDA	5/27/2021	No	Yes
BGJ-398	infigratinib	BridgeBio	FGFR1-3 selective inhibitor	Cholangiocarcinoma	PO	Filed NDA	5/2021 - 6/2021	Yes	Yes
ET-104	zonisamide	Eton	anticonvulsant	Seizures	PO	Filed NDA	5/30/2021	No	No
ALKS-3831	olanzapine/ samidorphan	Alkermes	dopamine receptor antagonist/ opioid receptor antagonist	Schizophrenia/ Bipolar disorder	PO	Filed NDA	6/1/2021	No	No
SCY-078 (MK-3118)	ibrexafungerp	Scynexis	glucan synthase inhibitor	Vulvovaginal candidiasis	PO	Filed NDA	6/1/2021	No	Yes
relugolix/ estradiol/ norethindrone acetate	relugolix/ estradiol/ norethindrone acetate	Myovant Sciences	gonadotropin-releasing hormone receptor antagonist	Uterine fibroids	PO	Filed NDA	6/1/2021	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Ryplazim	human plasminogen	Liminal BioSciences	plasminogen	Plasminogen deficiency	IV	Filed BLA	6/5/2021	Yes	Yes
BIIB-037	aducanumab	Biogen	amyloid beta-protein inhibitor	Alzheimer's disease	IV	Filed BLA	6/7/2021	Yes	No
TAK-721 (SHP-621)	budesonide	Takeda	corticosteroid	Eosinophilic esophagitis	PO	Filed NDA	6/15/2021	Yes	Yes
arimoclomol	arimoclomol	Orphazyme	cytoprotectives	Niemann-Pick disease	PO	Filed NDA	6/17/2021	Yes	Yes
INC-424	ruxolitinib	Incyte	janus kinase inhibitor	Atopic dermatitis	TOP	Filed NDA	6/21/2021	Yes	No
VP-102	cantharidin	Verrica	antiviral	Molluscum	TOP	Filed NDA	6/23/2021	No	No
ACP-001 (TransCon Growth Hormone)	lonapegsomatropin	Ascendis Pharma	growth hormone prodrug	Short stature/ growth hormone deficiency	SC	Filed BLA	6/25/2021	Yes	Yes
Verkazia	cyclosporine	Santen Pharmaceutical	immunosuppressant	Vernal keratoconjunctivitis	OPH	Filed NDA	6/26/2021	No	Yes
CPP-1X/ sulindac (DFMO)	eflornithine/ sulindac	Cancer Prevention Pharma	ornithine decarboxylase inhibitor/ non-steroidal anti-inflammatory drug	Familial adenomatous polyposis	PO	Filed NDA	6/29/2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
NexoBrid	bromelain	Vericel	peptide hydrolase replacement agent	Burns/ skin injury	TOP	Filed BLA	6/29/2021	No	Yes
PF-06482077	multivalent group B streptococcus vaccine	Pfizer	vaccine	Bacterial infection	IM	Filed BLA	6/2021	Yes	No
tanezumab	tanezumab	Pfizer/ Eli Lilly	nerve growth factor inhibitor	Osteoarthritis	SC	Filed BLA	2Q2021	Yes	No
SPI-2012	eflapegrastim	Spectrum	granulocyte colony-stimulating factor	Chemotherapy-induced neutropenia	SC	Filed BLA	2Q2021	Yes	No
S5G4T-1	benzoyl peroxide	Sol-Gel Technologies	benzoyl peroxide	Rosacea	TOP	Filed NDA	2Q2021	No	No
tramadol	tramadol	Avenue Therapeutics	opioid receptor agonist	Pain	IV	Filed NDA	2Q2021	No	No
StrataGraft Skin Tissue	StrataGraft Skin Tissue	Mallinckrodt	autologous skin tissue	Burn injury	TOP	Filed BLA	1H2021	Yes	Yes
Leukotac	inolimomab	ElsaLys Biotech	IL-2 monoclonal antibody	Graft vs. host disease	IM	Filed BLA	1H2021	Yes	Yes
RT-002 (Daxi)	daxibotulinumtoxinA	Revance Therapeutics	botulinum toxins	Glabellar lines (frown lines)	IM	Filed BLA	Mid-2021	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
PRV-031	teplizumab	Provention Bio/ MacroGenics	CD3 antigen inhibitor	Diabetes mellitus	IV	Filed BLA	7/2/2021	Yes	No
CMX-001	brincidofovir	Chimerix	DNA-directed DNA polymerase inhibitor	Smallpox	PO	Filed NDA	7/7/2021	No	Yes
CCX-168	avacopan	ChemoCentryx	C5a receptor antagonist	Vasculitis	PO	Filed NDA	7/7/2021	Yes	Yes
BAY-948862	finerenone	Bayer	mineralocorticoid receptor antagonist	Diabetic nephropathy	PO	Filed NDA	7/9/2021	No	No
V-114	pneumococcal conjugate vaccine	Merck	vaccine	Bacterial infection	IM	Filed BLA	7/18/2021	Yes	No
odevixibat	odevixibat	Albireo Pharma	ileal bile acid transporter inhibitor	Progressive familial intrahepatic cholestasis	PO	Filed NDA	7/20/2021	Yes	Yes
sulopenem	sulopenem	Iterum Therapeutics	carbapenem	Urinary tract infections	PO	Filed NDA	7/25/2021	No	No
MGA-012	retifanlimab	Incyte	programmed cell death protein 1 inhibitor	Anal cancer	IV	Filed BLA	7/25/2021	Yes	Yes
Uptravi (IV)	selexipag	Janssen	non-prostanoid prostacyclin agonist	Pulmonary arterial hypertension	IV	Filed NDA	7/30/2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
PF-04965842	abrocitinib	Pfizer	janus kinase inhibitor	Atopic dermatitis	PO	Filed NDA	Early 3Q2021	Yes	No
TWIN (S6G5T-1; S6G5T-3)	benzoyl peroxide/ tretinoin	Sol-Gel Technologies	retinoid	Acne vulgaris	TOP	Filed NDA	8/1/2021	No	No
JNJ-6372	amivantamab	Johnson & Johnson	EGFR and cMET antibody	Non-small cell lung cancer	IV	Filed BLA	8/3/2021	Yes	No
ET-101	topiramate	Eton	undisclosed	Seizure disorders	PO	Filed NDA	8/6/2021	No	No
AMG-510	sotorasib	Amgen	KRAS inhibitor	Non-small cell lung cancer	PO	Filed NDA	08/16/2021	Yes	Yes
Vicineum	oportuzumab monatox	Sesen Bio	anti-ECAM exotoxin A fusion protein	Bladder cancer	Intravesical	Filed BLA	8/18/2021	Yes	No
GZ-402666 (NeoGAA)	avalglucosidase alfa	Sanofi	enzyme replacement therapy	Pompe disease	IV	Filed BLA	8/18/2021	Yes	No
AXS-05	dextromethorphan/ bupropion	Axsome	N-methyl-D-aspartate antagonist/ antidepressant	Treatment-resistant depression	PO	Filed NDA	8/22/2021	No	No
CR-845	difelikefalin	Cara Therapeutics/ Vifor Pharma	opioid receptor agonist	Pruritus	IV/PO	Filed NDA	8/23/2021	No	No
KD-025	belumosudil	Kadmon	Rho-associated coiled-coil kinase 2 inhibitor	Graft vs. Host disease	PO	Filed NDA	8/30/2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
TicoVac	tick-borne encephalitis vaccine	Pfizer	vaccine	Tick-borne encephalitis	IM	Filed BLA	8/31/2021	No	No
Ovastat	treosulfan	Medexus Pharmaceuticals	alkylating agent	Hematopoietic stem cell transplantation	IV	Filed NDA	8/31/2021	Yes	Yes
paliperidone palmitate (6-month)	paliperidone palmitate	Johnson & Johnson	atypical antipsychotic	Schizophrenia	IM	Filed NDA	9/2/2021	Yes	No
INP-104	POD-dihydroergotamine mesylate (POD-DHE)	Impel NeuroPharma	ergot derivative	Acute migraines	Intranasal	Filed NDA	9/6/2021	No	No
PL-56	budesonide	Calliditas	corticosteroid	Nephropathy	PO	Filed NDA	9/15/2021	No	Yes
belzutifan	belzutifan	Merck	hypoxia-inducible factor-2 alpha inhibitor	Renal cell carcinoma	IV	Filed NDA	9/15/2021	Yes	Yes
Zydena	udenafil	Mezzion Pharma	phosphodiesterase type 5 inhibitor	Congenital single ventricle heart disease	PO	Filed NDA	9/21/2021	No	Yes
Doria	risperidone	Laboratorios Farmacéuticos Rovi	atypical antipsychotic	Schizophrenia	IM	Filed NDA	9/24/2021	Yes	No
SHP-625 (LUM-001)	maralixibat	Mirum Pharmaceuticals	apical sodium-dependent bile acid transporter inhibitor	Alagille syndrome	PO	Filed NDA	9/29/2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
AB-103	reltecimod	Atox Bio	CD-28 co-stimulatory receptor modulator	Necrotizing soft tissue infections	IV	Filed NDA	9/30/2021	Yes	Yes
FG-4592 (ASP-1517)	roxadustat	FibroGen/ AstraZeneca	hypoxia-inducible factor prolyl hydroxylase inhibitor	Anemia	PO	Filed NDA	3Q2021	Yes	No
MK-8031	atogepant	AbbVie	calcitonin gene-related peptide receptor antagonist	Migraine prophylaxis	PO	Filed NDA	3Q2021	No	No
RVT-802	RVT-802	Enzyvant/Roivant	Tissue-based therapy	Congenital athymia	Implant	Filed BLA	10/08/2021	Yes	Yes
HuMax-TF ADC	tisotumab vedotin	Seagen/ Genmab	tissue factor antibody	Cervical cancer	IV	Filed BLA	10/10/2021	Yes	No
UCB-4940 (CDP-4940)	bimekizumab	UCB	interleukin-17 receptor inhibitor	Plaque psoriasis	SC	Filed BLA	10/15/2021	Yes	No
FT-218	sodium oxybate extended-release	Avadel	dopamine receptor agonist	Narcolepsy	PO	Filed NDA	10/15/2021	Yes	Yes
OMS-721	narsoplimab	Omeros	anti-MASP-2 monoclonal antibody	Hematopoietic stem cell transplant-associated thrombotic microangiopathy	IV	Filed BLA	10/17/2021	Yes	Yes
OS-01 nasal spray	varenicline	Oyster Point Pharma	nicotinic acetylcholine receptor agonist	Dry eye disease	Intranasal	Filed NDA	10/17/2021	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
TAK-788	mobocertinib	Takeda	tyrosine kinase inhibitor	Non-small cell lung cancer	PO	Filed NDA	10/26/2021	Yes	Yes
MOD-401	somatrogon	Pfizer/ Opko	human growth hormone	Growth hormone deficiency	SC	Filed BLA	10/2021	Yes	Yes
Kyzatrex	testosterone undecanoate	Marius Pharmaceuticals	testosterone replacement therapy	Hypogonadism	PO	Filed NDA	10/31/2021	No	No
SH-111	SH-111	Shorla Pharma	unknown	T-cell leukemia	undisclosed	Filed NDA	10/2021 - 11/2021	Yes	No
CLS-1001	triamcinolone acetonide	Clearside	corticosteroid	Macular edema	Intraocular/ subretinal	Filed NDA	11/03/2021	Yes	No
BXCL-501	dexmedetomidine	BioXcel Therapeutics	selective alpha 2a receptor agonist	Schizophrenia and bipolar disorder	PO	InTrial	11/11/2021	No	No
JNJ-4528 (LCAR-B38M)	ciltacabtagene autoleucel	Legend Biotech/ Janssen	chimeric antigen receptor (CAR) T cell therapy	Multiple myeloma	IV	Filed BLA	11/2021	Yes	Yes
DE-117	omidenepag isopropyl	Santen Pharmaceutical	prostaglandin E Receptor 2 agonist	Glaucoma	OPH	Filed NDA	11/19/2021	No	No
BMN-111	vosoritide	BioMarin	C-type natriuretic peptide analog	Achondroplasia	SC	Filed NDA	11/20/2021	Yes	Yes
TG-1303	ublituximab	TG Therapeutics	CD-20 monoclonal antibody/	Chronic lymphocytic leukemia	IV	Filed NDA	11/29/2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
			phosphoinositide-3 kinase delta inhibitor						
Sci-B-Vac	hepatitis B vaccine	VBI Vaccines	vaccine	Hepatitis B	IM	Filed BLA	11/30/2021	No	No
pacritinib	pacritinib	CTI BioPharma	janus associated kinase-2 inhibitor	Myelofibrosis	PO	Filed NDA	11/30/2021	Yes	Yes
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension	INH	Filed NDA	11/30/2021	Yes	No
Filsuvez (AP-101)	episalvan	Amryt Pharma	triterpene	Epidermolysis bullosa	TOP	InTrial	11/30/2021	No	Yes
ARGX-113	efgartigimod	Argenx	neonatal Fc receptor antibody	Myasthenia gravis	IV	Filed BLA	12/17/2021	Yes	Yes
ALN-TTRsc02	vutrisiran	Alnylam	siRNA/RNAi	Transthyretin-mediated amyloidosis	SC	Filed BLA	12/19/2021	Yes	Yes
Tyvaso DPI	treprostinil	United Therapeutics	prostacyclin mimetic	Pulmonary arterial hypertension/ pulmonary hypertension	INH	Filed NDA	12/19/2021	Yes	No
R-1646 (RO-4926219, AF-219, MK-7264)	gefapixant	Merck/ Roche	P2X3 antagonist	Chronic cough	PO	Filed NDA	12/21/2021	No	No
dextroamphetamine	dextroamphetamine	Noven Pharmaceuticals	CNS stimulant	Attention deficit hyperactivity disorder	TOP	Filed NDA	12/22/2021	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
transdermal system									
TadFin	tadalafil and finasteride	Veru	phosphodiesterase type 5 inhibitor /5-alpha-reductase inhibitor	Benign prostatic hyperplasia	PO	Filed NDA	12/23/2021	No	No
AGN-190584	pilocarpine	Allergan	cholinergic muscarinic receptor agonist	Presbyopia	OPH	InTrial	12/25/2021	No	No
ublituximab (TGTX-1101, TG-1101, Utuxin)	ublituximab	TG Therapeutics	CD-20 monoclonal antibody	Chronic lymphocytic leukemia; multiple sclerosis	IV	InTrial	4Q2021	Yes	Yes
Rizaport (VersaFilm)	rizatriptan	IntelGenx	triptans	Acute migraines	PO	CRL	4Q2021	No	No
CAM-2038	buprenorphine	Braeburn	opioid receptor agonist (partial)	Opioid use disorder/ Pain	SC	CRL	2H2021	Yes	No
Contepo	fosfomycin	Nabriva Therapeutics	cell wall inhibitor	Bacterial infections	IV	CRL	2H2021	Yes	No
ALN-PCSsc (PCSK9si)	inclisiran	Novartis	RNA interfering therapeutic targetting proprotein convertase subtilisin–kexin type 9	Hyperlipidemia	SC	CRL	2H2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Taclantis	paclitaxel injection concentrate for suspension	Sun Pharma Advanced Research Company (SPARC)	taxane	Breast cancer; lung cancer; pancreatic cancer	IV	CRL	2H2021	No	No
MEDI-546	anifrolumab	AstraZeneca/ BMS	interferon receptor antagonist	Systemic lupus erythematosus	IV	Filed BLA	2H2021	Yes	No
ropeginterferon alfa-2b	ropeginterferon alfa-2b	PharmaEssentia	interferon	Polycythemia vera	SC	CRL	2H2021	Yes	Yes
JZP-458	recombinant crisantaspase	Jazz Pharmaceuticals	asparaginase	Acute lymphoblastic leukemia	IM/IV	InTrial	2H2021	Yes	No
ET-105	lamotrigine	Eton	anticonvulsant	Epilepsy	PO	CRL	2021	No	No
E-58425 (MR-308)	celecoxib/tramadol	Esteve	non-steroid anti-inflammatory drug/ opioid	Acute pain	PO	Filed NDA	2021	No	No
LN-145	LN-145	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Cervical Cancer	IV	InTrial	Late 2021	Yes	No
2022 Possible launch dates									
COR-003	levoketoconazole	Strongbridge Biopharma	cortisol synthesis inhibitor	Cushing's syndrome	PO	Filed NDA	1/2/2022	No	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
HMPL-012	surufatinib	Hutchison China MediTech	angio-immunokinase inhibitor	Neuroendocrine tumors	PO	Filed NDA	1/3/2022	Yes	Yes
ACT-541468	daridorexant	Idorsia Pharmaceuticals	orexin receptor antagonist	Insomnia	PO	Filed NDA	1/8/2022	No	No
MYK-461 (SAR-439152)	mavacamten	MyoKardia	cardiac myosin allosteric modulator	Cardiomyopathy	PO	Filed NDA	1/28/2022	Yes	Yes
AGEN-2034	balstilimab	Agenus	PD-1 antagonist	Cervical cancer	IV	Filed BLA	02/19/2022	Yes	No
RTA-402	bardoxolone methyl	Reata Pharmaceuticals/ AbbVie	Nrf2 activator	Alport syndrome	PO	Filed NDA	2/25/2022	Yes	Yes
GC-5107	human immunoglobulin	GC Pharma	human immunoglobulin	Primary immunodeficiencies	IV	Filed BLA	2/25/2022	Yes	No
Tlando	testosterone	Lipocine	androgen	Hypogonadism	PO	Tentative Approval	3/27/2022	No	No
AKB-6548	vadadustat	Akebia Therapeutics/ Vifor Pharma/ Otsuka	hypoxia-inducible factor-prolyl hydroxylase inhibitor	Chronic kidney disease-related anemia	PO	Filed NDA	3/30/2022	Yes	No
NPI-2358	plinabulin	BeyondSpring	tumor vascular disrupting agent	Chemotherapy-induced neutropenia	IV	InTrial	3/31/2022	Yes	No
F-627	benegrastim	Evive Biotech	granulocyte colony-stimulating factor	Chemotherapy-induced neutropenia	SC	Filed BLA	3/31/2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
EBV-CTL (ATA-129)	tabelecleucel	Atara Biotherapeutics	cell therapy	Lymphoproliferative disorder	IV	InTrial	1Q2022	Yes	Yes
AG-348	mitapivat	Agios	pyruvate kinase-R activator	Pyruvate kinase deficiency	PO	InTrial	1Q2022	Yes	Yes
AT-GAA	cipaglucosidase alfa	Amicus	enzyme therapy	Pompe disease	IV	InTrial	1Q2022	Yes	Yes
SGX-301	synthetic hypericin	Access Pharmaceuticals	synthetic hypericin	Cutaneous T-cell lymphoma	TOP	InTrial	1Q2022	Yes	Yes
Purified Cortrophin Gel	corticotropin	ANI Pharmaceuticals	adrenocorticotropic hormone	Multiple sclerosis/ rheumatoid arthritis/ systemic lupus erythematosus/ ulcerative colitis	IV	InTrial	1Q2022	Yes	No
SYD-985	[vic-] trastuzumab duocarmazine	Synthon	HER2-targeting antibody-drug conjugate	Breast cancer	IV	InTrial	1Q2022	Yes	No
BIVV-009 (TNT-009)	sutimlimab	Sanofi	complement C1s subcomponent inhibitor	Cold agglutinin disease	IV	CRL	1Q2022	Yes	Yes
ABL-001	asciminib	Novartis	allosteric Bcr-Abl inhibitor	Chronic myeloid leukemia	PO	InTrial	1Q2022	Yes	Yes
PRO-140	leronlimab	CytoDyn	C-C chemokine receptor 5 antagonist	HIV	SC	InTrial	1Q2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
R-667 (RG-667)	palovarotene	Ipsen	selective retinoic acid receptor agonist	Fibrodysplasia ossificans progressiva (FOP)	PO	InTrial	1Q2022	Yes	Yes
AGEN-1884	zalifrelimab	Agenus	immune checkpoint modulator antibody	Cervical cancer	IV	InTrial	1Q2022	Yes	No
AMG-157 (MEDI-9929)	tezepelumab	AstraZeneca/ Amgen	thymic stromal lymphopoietin antagonist	Asthma	IV/SC	Filed BLA	5/10/2022	Yes	No
Zynteglo (LentiGlobin)	betibeglogene autotemcel	Bluebird Bio	gene therapy	Beta-thalassemia; sickle cell disease	IV	InTrial	2Q2022	Yes	Yes
AXS-07	meloxicam/rizatriptan	Axsome Therapeutics	non-steroidal anti-inflammatory drug/triptan	Migraine	PO	InTrial	2Q2022	No	No
IDP-120	tretinoin/ benzoyl peroxide	Bausch	retinoid	Acne	TOP	InTrial	2Q2022	No	No
OBE-2109 (KLH-2109)	linzagolix	ObsEva	gonadotropin-releasing hormone antagonist	Uterine fibroids	PO	InTrial	2Q2022	No	No
Sativex	nabiximols	GW Pharmaceuticals/ Otsuka	cannabinoid product	Spasticity	SL/ SPR	InTrial	1H2022	No	No
dovitinib	dovitinib	Oncology Venture	fibroblast growth factor receptor 3 inhibitor	Renal cell carcinoma	PO	InTrial	1H2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Iomab-B	iodine I 131 monoclonal antibody BC8	Actinium	anti-CD45 monoclonal antibody	Acute myeloid leukemia/ Myelodysplastic syndrome	IV	InTrial	1H2022	Yes	Yes
Lenti-D	elivaldogene tavalentivec	Bluebird Bio	gene therapy	Adrenomyeloneuropathy	IV	InTrial	1H2022	Yes	Yes
VT-1161	oteseconazole	Mycovia Pharmaceuticals	lanosterol demethylase inhibitor	Fungal infections	PO	InTrial	1H2022	No	No
AmnioFix	dehydrated human amnion/chorion membrane (dHACM)	MiMedx	amniotic tissue membrane	Plantar fasciitis/ achilles tendonitis	INJ	InTrial	1H2022	Yes	No
MLN-4924 (TAK-92)	pevonedistat	Ligand	Nedd 8 activating enzyme antagonist	Myelodysplastic syndrome	IV	InTrial	1H2022	Yes	No
ACER-001	sodium phenylbutyrate	Acer Therapeutics	BCKDC kinase inhibitor	Urea cycle disorders	PO	InTrial	1H2022	No	No
CERC-801	CERC-801	Cerecor	D-galactose	Phosphoglucomutase 1 (PGM1) deficiency	PO	InTrial	1H2022	Yes	Yes
HM781-36B	poziotinib	Spectrum Pharmaceuticals	pan-HER inhibitor	Non-small cell lung cancer	PO	InTrial	1H2022	Yes	No
Botulax	botulinum toxin type A	Hugel Pharma	botulinum toxin	Wrinkles	IM	InTrial	1H2022	Yes	No
DARE-BV1	clindamycin	Daré Bioscience	lincosamide	Bacterial vaginosis	Intravaginal	InTrial	1H2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory 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	1H2022	No	No
Libervant	diazepam	Aquestive Therapeutics	benzodiazepine	Seizures	PO	CRL	1H2022	No	Yes
AGIL-AADC	AGIL-AADC	PTC Therapeutics	gene therapy	Aromatic L-amino acid decarboxylase deficiency	Intracerebral	InTrial	1H2022	Yes	Yes
131I-8H9	omburtamab	Y-mAbs Therapeutics	B7-H3 antagonist	Brain cancer	Intrathecal	InTrial	1H2022	Yes	Yes
pIL-12 (DNA IL-12)	tavokinogene telsaplasmid	OncoSec Medical	gene therapy	Melanoma	Intratumoral	InTrial	1H2022	Yes	Yes
TAK-003	Dengue fever vaccine	Takeda	vaccine	Dengue fever	SC	InTrial	1H2022	Yes	No
Furoscix	furosemide	scPharmaceuticals	diuretic	Heart failure	SC	CRL	1H2022	Yes	No
Zimhi	naloxone	Adamis	opioid antagonist	Opioid overdose	IM	CRL	1H2022	No	No
PDS-1.0	ranibizumab	Roche/ Genentech	vascular endothelial growth factor inhibitor	Wet age-related macular degeneration	Intravitreal implant	InTrial	1H2022	Yes	No
FT-2102	olutasidenib	Forma Therapeutics	dehydrogenase 1 inhibitor	Acute myeloid leukemia	PO	InTrial	1H2022	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ABI-009	sirolimus and albumin	Aadi Bioscience	mTOR kinase inhibitor	Epithelioid cell caricnoma	IV	InTrial	1H2022	Yes	Yes
SHP-620	maribavir	Shire	benzimidazole	Cytomegalovirus	PO	InTrial	1H2022	No	Yes
Adstiladrin	nadofaragene firadenovec	FerGene	gene therapy	Bladder cancer	Intravesical	CRL	1H2022	Yes	No
ESN-364	fezolinetant	Astellas	NK3 receptor antagonist	Menopause	PO	InTrial	1H2021	No	No
SP-02	darinaparsin	Solasia Pharma	organic arsenical	Peripheral T-cell lymphoma	IV	InTrial	1H2022	Yes	Yes
JS-001	toripalimab	Shanghai Junshi Biosciences/ Coherus BioSciences	anti-PD-1 monoclonal antibody	Nasopharyngeal carcinoma	IV	InTrial	1H2022	Yes	Yes
S-265744 LAP (S/GSK-1265744 LAP; GSK-744 LA)	cabotegravir	ViiV Healthcare	HIV integrase inhibitor	HIV pre-exposure prophylaxis	IM	InTrial	1H2022	No	No
GS-010	GS-010	GenSight Biologics	gene therapy	Optic neuropathy	Intraocular	InTrial	Mid-2022	Yes	Yes
ERY-ASP (ERY-001)	L-asparaginase (eryaspase)	Erytech/ Recordati	L-asparaginase	Pancreatic cancer	IV	InTrial	Mid-2022	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
IMGN-853 (M-9346A-sulfo-SPDB-DM4)	mirvetuximab soravtansine	ImmunoGen	folate receptor-1 antagonist	Ovarian cancer	IV	InTrial	Mid-2022	Yes	Yes
PF-06838435 (SPK-9001)	fidanacogene elaparvovec	Pfizer/ Spark Therapeutics	gene therapy	Hemophilia B	IV	InTrial	Mid-2022	Yes	Yes
FCX-007 (GM-HDF-COL7, INXN-3002)	FCX-007 (GM-HDF-COL7, INXN-3002)	Castle Creek Pharmaceutical	gene-modified autologous fibroblast	Epidermolysis bullosa	Intradermal	InTrial	Mid-2022	Yes	Yes
MIN-102	hydroxypioglitazone	Minoryx Therapeutics	PPAR gamma agonist	Adrenomyeloneuropathy	undisclosed	InTrial	Mid-2022	Yes	Yes
OTL-200 (GSK-2696274)	OTL-200 (GSK-2696274)	Orchard Therapeutics	gene therapy	Leukodystrophy	IV	InTrial	Mid-2022	Yes	Yes
AMT-061	etranacogene dezaparvovec	CSL Behring/ uniQure	gene therapy	Hemophilia B	IV	InTrial	Mid-2022	Yes	Yes
Ultomiris SC	ravulizumab-cwvz	Alexion	C5 complement inhibitor	paroxysmal nocturnal hemoglobinuria; Hemolytic uremic syndrome	SC	InTrial	Mid-2022	Yes	Yes
AT-007	AT-007	Applied Therapeutics	aldose reductase inhibitor	Galactosemia	undisclosed	InTrial	Mid-2022	Yes	Yes
KB-103	beremagene geperpavec	Krystal Biotech	gene therapy	Epidermolysis bullosa	Topical	InTrial	Mid-2022	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
SPR-994	tebipenem	Spero Therapeutics	carbapenem	Urinary tract infections	PO	InTrial	Mid-2022	No	No
DCR-PHXC	nedosiran	Dicerna/ Alnylam	glycolate oxidase antagonist	hyperoxaluria	SC	InTrial	Mid-2022	Yes	Yes
MRTX-849	adagrasib	Mirati Therapeutics	KRAS inhibitor	Non-small cell lung cancer	PO	InTrial	Mid-2022	Yes	No
RG-7828	mosunetuzumab	Roche	anti-CD20/CD3 monoclonal antibody	Follicular lymphoma	IV/SC	InTrial	Mid-2022	Yes	Yes
GS-CA1 (GS-6207)	lenacapavir	Gilead	HIV capsid inhibitor	HIV-1	SC	InTrial	Mid-2022	No	No
NX-1207 (NYM-4805, REC 0482)	fexapotide triflutate	Nymox	pro-apoptotic	Benign prostatic hyperplasia	Intratumoral	InTrial	Mid-2022	Yes	No
GZ-402665	olipudase alfa	Sanofi	enzyme replacement therapy	Acid sphingomyelinase deficiency	IV	InTrial	Mid-2022	Yes	Yes
PS-433540 (RE-021; DARA)	sparsentan	Retrophin/ Bristol-Myers Squibb/ Ligand	dual-acting angiotensin/endothelin receptor antagonist	Focal segmental glomerulosclerosis	PO	InTrial	Mid-2022	No	Yes
CCD-1042	ganaxolone	Marinus Pharmaceuticals	allosteric modulator of GABA receptors	Seizures	PO	InTrial	Mid-2022	No	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
MTP-131 (SS-31)	elamipretide	Stealth Biotherapeutics	mitochondrial permeability transition pore inhibitor	Barth syndrome	IV/PO/SC	InTrial	Mid-2022	Yes	Yes
WTX-101	bis-choline tetrathiomolybdate (TTM)	Alexion	chelating agent	Wilson's disease	PO	InTrial	Mid-2022	Yes	Yes
GSK-2894512 (WBI-1001)	tapinarof	Dermavant Sciences	therapeutic aryl hydrocarbon receptor modulating agent	Plaque psoriasis	TOP	InTrial	Mid-2022	Yes	No
IMC-gp100	tebentafusp	Immunocore	anti-CD3 antibody	Uveal melanoma	IV	InTrial	Mid-2022	Yes	Yes
INCB-050465	parsaclisib	Incyte	PI3K-delta inhibitor	Follicular lymphoma/ mantle cell lymphoma/ marginal zone lymphoma	PO	InTrial	Mid-2022	Yes	Yes
NiCord	omidubicel	Gamida	cellular therapy	Hematological cancers	IV	InTrial	3Q2022	Yes	Yes
omecamtiv mecarbil	omecamtiv mecarbil	Amgen	mysoin activator	Heart failure	PO	InTrial	3Q2022	No	No
CUTX-101	copper histidinate	Fortress Biotech	copper replacement	Menkes Disease	SC	InTrial	3Q2022	Yes	Yes
ONS-5010	bevacizumab-vikg	Outlook Therapeutics	anti-VEGF antibody	Wet age-related macular degeneration	Intravitreal	InTrial	4Q2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
BHV-3500	vazegepant	Biohaven	calcitonin gene-related peptide receptor antagonist	Migraine	Intranasal	InTrial	4Q2022	No	No
VBP-15	vamorolone	Santhera	corticosteroid	Duchenne muscular dystrophy	PO	InTrial	4Q2022	Yes	Yes
Takecab	vonoprazan fumarate	Phathom Pharmaceuticals	potassium-competitive acid blocker	H. pylori infection	PO	InTrial	4Q2022	No	No
REGN-475 (SAR-164877)	fasinumab	Regeneron/ Sanofi-Aventis/ Teva	selective anti-nerve growth factor monoclonal antibody	Osteoarthritis	IV/SC	InTrial	2H2022	Yes	No
DBV-712 (Viaskin Peanut)	DBV-712	DBV Technologies	Immunotherapy	Peanut allergy	TOP	CRL	2H2022	No	No
Oxabact (IxOC-3)	oxalobacter	OxThera	probiotic	Hyperoxaluria	PO	InTrial	2H2022	No	Yes
ALT-803	nogapendekin alfa inbakicept	ImmunityBio	interleukin-15 super agonist/ IL-15R alpha-Fc fusion complex	Bladder cancer	Intravesical	InTrial	2H2022	Yes	No
GLPG-0634	filgotinib	Gilead/ Galapagos	janus associated kinase-1 inhibitor	Rheumatoid arthritis	PO	CRL	2H2022	Yes	No
RG-7716 (RO-6867461)	faricimab	Roche/ Chugai	bispecific VEGF-A/ angiopoietin-2 antagonist	Diabetic macular edema; age-related macular degeneration	Intravitreal	InTrial	2H2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
LY-686017	tradipitant	Vanda Pharmaceuticals	neurokinin 1 receptor antagonist	Motion sickness/ gastroparesis	PO	InTrial	2H2022	No	No
CDZ-173	leniolisib	Pharming/ Novartis	phosphatidylinositol-3-4-5-trisphosphate inhibitor	Primary immunodeficiencies	PO	InTrial	2H2022	Yes	Yes
AAI-101	cefepime/enmetazobactam	Allecra	beta-lactam/b-lactamase inhibitor	Urinary tract infection	IV	InTrial	2H2022	No	No
RG-7440 (GDC-0068)	ipatasertib	Roche	pan-Akt inhibitor	Prostate cancer	PO	InTrial	2H2022	Yes	No
MBG-453	MBG-453	Novartis	anti-TIM-3	Myelodysplastic syndrome	IV	InTrial	2H2022	Yes	No
PAX-101	suramin	PaxMedica	unknown	trypanosomiasis	IV	InTrial	2H2022	No	No
OPNT-003	nalmefene	Opiant	opioid receptor antagonist	Opioid overdose	Intranasal	InTrial	2H2022	No	No
ABBV-951	levodopa/carbidopa	AbbVie	aromatic amino acid/ aromatic amino acid decarboxylation inhibitor	Parkinson's disease	SC	InTrial	2H2022	Yes	No
Zynquista	sotagliflozin	Lexicon	sodium-dependent glucose transporter 1 (SGLT-1) and SGLT-2 inhibitor	Diabetes mellitus	PO	CRL	2H2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Qtrypta	zolmitriptan	Zosano	triptans	Acute migraines	TOP	CRL	2H2022	No	No
Hepcludex	bulevirtide	Gilead	HBV receptor binder	Hepatitis delta virus	SC	InTrial	2H2022	No	Yes
glatiramer acetate depot	glatiramer acetate long-acting	Mylan	immunosuppressant	Multiple sclerosis	IM	InTrial	2H2022	Yes	No
177Lu-PSMA-617	Lutetium	Novartis	radiopharmaceutical	Prostate cancer	IV	InTrial	2H2022	Yes	No
BMS-986165	deucravacitinib	Bristol-Myers Squibb	tyrosine kinase 2 inhibitor	Plaque psoriasis	PO	InTrial	2H2022	Yes	No
FMXIN-001	naloxone	Nasus Pharma	opioid antagonist	Opioid overdose	Intranasal	InTrial	2H2022	No	No
SAR-439859	amcenestrant	Sanofi	selective estrogen receptor degrader	Breast cancer	PO	InTrial	2H2022	Yes	No
magrolimab	magrolimab	Gilead	CD47 monoclonal antibody	Myelodysplastic syndrome	IV	InTrial	2H2022	Yes	Yes
RGN-259 (GBT-201; RGN-352)	timbetasin	RegeneRx	actin regulating peptide	Dry eyes	OP	InTrial	2022	No	Yes
BGB-A317 (BGB-A-317)	tislelizumab	Celgene/ BeiGene	programmed death-1 inhibitor	Hepatocellular cancer	IV	InTrial	2022	Yes	No
VGX-3100	VGX-3100	Inovio	vaccine	Cervical cancer/dysplasia	IM	InTrial	2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
CNTX-4975	CNTX-4975	Centrexion Therapeutics	TRPV1 agonist	Osteoarthritis	Intraarticular	InTrial	2022	Yes	No
pentoxifylline	pentoxifylline	Eton	phosphodiesterase inhibitor	Peyronie's disease	PO	InTrial	2022	No	No
NNZ-2566	trofinetide	Neuren	insulin-like growth factor 1 derivative	Rett syndrome	IV/PO	InTrial	2022	Yes	Yes
POL-6326	balixafortide	Polyphor	chemokine antagonist	Breast cancer	IV	InTrial	2022	Yes	No
OTL-101	OTL-101	Orchard Therapeutics	gene therapy	Adenosine deaminase-deficient severe combined immunodeficiency	IV	InTrial	2022	Yes	Yes
ADP-A2M4 (MAGE-A4)	ADP-A2M4 (MAGE-A4)	Adaptimmune	SPEAR T-cell therapy	Sarcoma	IV	InTrial	2022	Yes	Yes
HY-01	minocycline	Hovione	tetracycline	Rosacea	TOP	InTrial	2022	No	No
IBI-308	sintilimab	Eli Lilly	programmed death-1 receptor inhibitor	Non-small cell lung cancer	IV	InTrial	2022	Yes	No
obeticholic acid	obeticholic acid	Intercept Pharmaceuticals	farnesoid X receptor agonist	Nonalcoholic steatohepatitis	PO	CRL	2022	Yes	No
Nanoflu	influenza vaccine	Novavax	vaccine	Influenza	IM	InTrial	2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ADV-7103	tripotassium citrate monohydrate/ potassium hydrogen carbonate	Advicenne	undisclosed	Distal rental tubular acidosis	PO	InTrial	2022	Yes	No
LN-144	lifileucel	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Melanoma	IV	InTrial	2022	Yes	Yes
SPN-830	apomorphine	Supernus Pharmaceuticals	non-ergoline dopamine agonist	Parkinson's disease	SC infusion	InTrial	2022	Yes	No
KN-046	KN-046	Alphamab Oncology	PD-L1/CTLA-4 bispecific monoclonal antibody	Thymic cancer	IV	InTrial	2022	Yes	Yes
CERC-802	CERC-802	Cerecor	D-mannose	Mannose-phosphate isomerase deficiency	PO	InTrial	2022	Yes	Yes
scCeftriaxone	ceftriaxone	scPharmaceuticals	Penicillin binding protein inhibitor	Bacterial infections	SC	InTrial	2022	No	No
CAT-354	tralokinumab	Leo Pharma	interleukin-13 inhibitor	Atopic dermatitis	SC	CRL	2022	Yes	No
TAK-609	idursulfase-IT	Takeda	enzyme replacement	Hunter syndrome	Intrathecal	InTrial	2022	Yes	Yes
PRX-102	pegunigalsidase alfa	Protalix	enzyme replacement	Fabry disease	IV	CRL	2022	Yes	No
Entyvio (SC formulation)	vedolizumab	Takeda	integrin receptor antagonist	Ulcerative colitis/ Crohn's disease	SC	CRL	2022	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
PTX-022	rapamycin	Palvella Therapeutics	mTOR kinase inhibitor	Pachyonychia congenita	TOP	InTrial	2022	No	Yes
MOR-103 (GSK-165)	otilimab	MorphoSys/ GlaxoSmithKline	granulocyte macrophage colony-stimulating factor antibody	Rheumatoid arthritis	IV	InTrial	2022	Yes	No
NuThrax	anthrax vaccine adsorbed/ CPG-7909	Emergent Biosolutions	vaccine/ oligodeoxynucleotide	Anthrax	IM	InTrial	Late 2022	Yes	No
NS-2 (ALDX-1E1, ALDX-1E2, ADX-102)	reproxalap	Aldeyra Therapeutics	aldehyde antagonist	Dry eyes	OP	InTrial	Late 2022	No	No
R-1658 (RG-1658, JTT-705, RO-4607381)	dalcetrapib	DalCor	cholesteryl ester transfer protein inhibitor	Acute coronary syndrome	PO	InTrial	Late 2022	Yes	No
RP-L102 (RPL-102)	RP-L102	Rocket Pharmaceuticals	gene therapy	Fanconi anemia	IV	InTrial	Late 2022	Yes	Yes
LY-3298176	tirzepatide	Eli Lilly	glucose-dependent insulinotropic polypeptide /glucagon-like peptide-1 receptor agonist	Diabetes mellitus	SC	InTrial	Late 2022	No	No
GSK-2140944	gepotidacin	GlaxoSmithKline	bacterial Type II topoisomerase inhibitor	Bacterial infections	PO/IV	InTrial	Late 2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
RG-7433 (ABT-263)	navitoclax	AbbVie	Bcl-2 inhibitor	Myelofibrosis	PO	InTrial	Late 2022	Yes	Yes
MT-7117	MT-7117	Mitsubishi Tanabe Pharma	undisclosed	Erythropoietic protoporphyria	PO	InTrial	Late 2022	Yes	No
ARQ-151	roflumilast	Arcutis Biotherapeutics	phosphodiesterase-4 inhibitor	Plaque psoriasis	TOP	InTrial	Late 2022	Yes	No
Roctavian	valoctocogene roxaparvovec	BioMarin	gene therapy	Hemophilia A	IV	CRL	Late 2022	Yes	Yes
resminostat	resminostat	4SC AG	pan histone deacetylase inhibitor	Mycosis fungoides/ Sézary syndrome	PO	InTrial	Late 2022	Yes	No
iMAB-362	zolbetuximab	Astellas	GC182 monoclonal antibody	Gastric adenocarcinoma	IV	InTrial	Late 2022	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

OptumRx Key Pending Indication Forecast

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Opdivo	nivolumab	Bristol-Myers Squibb	anti-PD-1 antibody	Esophageal cancer	Treatment of patients with resected esophageal or gastroesophageal junction cancer in the adjuvant setting, after neoadjuvant chemoradiation therapy	IV	5/20/2021
Zeposia	ozanimod	Bristol Myers Squibb	sphingosine-1-phosphate receptor modulator	Ulcerative colitis	Treatment of adults with moderately to severely active ulcerative colitis	PO	5/30/2021
Jardiance	empagliflozin	Boehringer Ingelheim/ Eli Lilly	sodium-dependent glucose transporter 2 inhibitor	Heart failure	To reduce the risk of cardiovascular death and hospitalization for heart failure and to slow kidney function decline in adults with chronic heart failure with reduced ejection fraction, including those with and without type 2 diabetes	PO	5/30/2021
Esbriet	pirfenidone	Genentech	dual TGF-beta synthesis and TNG-alpha synthesis inhibitor	Unclassifiable interstitial lung disease	Treatment of unclassifiable interstitial lung disease	PO	5/31/2021
Nurtec ODT	rimegepant	Biohaven	calcitonin gene-related peptide inhibitor	Migraine prophylaxis	Preventive treatment of migraine in both episodic and chronic migraine patients	PO	6/1/2021
Ozempic	semaglutide	Novo Nordisk	glucagon-like peptide-1 receptor agonist	Obesity	Treatment of adults with obesity or overweight with at least one weight-related comorbidity, as an adjunct to reduced-calorie diet and increased physical activity	SC	6/4/2021

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Trikafta	elexacaftor/tezacaftor /ivacaftor; ivacaftor	Vertex	cystic fibrosis transmembrane conductance regulator modulators	Cystic fibrosis	Treatment of children with CF ages 6 to 11 years who have two F508del mutations and in children who have one F508del mutation and one minimal function mutation	PO	6/8/2021
Shingrix	zoster vaccine recombinant, adjuvanted	GlaxoSmithKline	vaccine	Herpes zoster	Prevention of herpes zoster in adults aged 18 years and older at increased risk of herpes zoster	IM	6/15/2021
Nucala	mepolizumab	GlaxoSmithKline	IL-5 antagonist monoclonal antibody	Nasal polyps	Treatment of chronic rhinosinusitis with nasal polyposis	SC	6/15/2021
Ayvakit	avapritinib	Blueprint Medicines	selective KIT and PDGFRa inhibitor	Systemic mastocytosis	Treatment of adult patients with advanced systemic mastocytosis	PO	6/17/2021
Jakafi	ruxolitinib	Incyte	janus associated kinase inhibitor	Graft-versus-host disease	Treatment of steroid-refractory chronic graft-versus-host disease in adult and pediatric patients 12 years and older	PO	6/22/2021
Rinvoq	upadacitinib	AbbVie	janus associated kinase inhibitor	Ankylosing spondylitis	Treatment of adult patients with active ankylosing spondylitis	PO	6/25/2021
Cosentyx	secukinumab	Novartis	IL-17 receptor antagonist	Pediatric psoriasis	Treatment of pediatric psoriasis	SC	6/27/2021
Rinvoq	upadacitinib	AbbVie	janus kinase inhibitor	Psoriatic arthritis	Treatment of adult patients with active psoriatic arthritis	PO	6/29/2021

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Solosec	secnidazole	Lupin Pharmaceuticals	nitroimidazole antimicrobial	Trichomoniasis	Treatment of trichomoniasis in adults and adolescents	PO	6/30/2021
Rinvoq	upadacitinib	AbbVie	janus kinase inhibitor	Atopic dermatitis	Treatment of adults and adolescents with moderate to severe atopic dermatitis	PO	7/19/2021
Ibsrela	tenapanor	Ardelyx	sodium-hydrogen exchanger-3 inhibitor	Hyper-phosphatemia	To control serum phosphorus in adult patients with chronic kidney disease on dialysis	PO	7/29/2021
Xeljanz	tofacitinib	Pfizer	janus kinase inhibitor	Axial spondyloarthritis	Treatment of axial spondyloarthritis	PO	7/30/2021
Olumiant	baricitinib	Eli Lilly	janus associated kinase 1/2 inhibitor	Atopic dermatitis	Treatment of adults with moderate-to-severe atopic dermatitis	PO	7/31/2021
Lenvima	lenvatinib	Eisai	tyrosine kinase inhibitor	Renal cell cancer	In combination with Keytruda (pembrolizumab), for advanced and/or metastatic renal cell carcinoma	PO	8/6/2021
Xywav	calcium, magnesium, potassium, and sodium oxybates	Jazz	dopamine receptor agonist	Idiopathic hypersomnia	Treatment of adult patients with idiopathic hypersomnia	PO	8/12/2021
Padcev	enfortumab vedotin-ejfv	Astellas/ Seagen	Nectin-4-directed antibody and microtubule inhibitor conjugate	Urothelial cancer	Treatment of patients with locally advanced or metastatic urothelial cancer who have been previously treated with a PD-1/L1 inhibitor and are ineligible for cisplatin	IV	8/17/2021
Keytruda	pembrolizumab	Merck	anti-PD-1 inhibitor	Renal cell carcinoma	In combination with Lenvima (lenvatinib), for advanced and/or metastatic renal cell carcinoma	IV	8/25/2021

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Xarelto	rivaroxaban	Janssen	factor Xa inhibitor	Peripheral arterial disease	Reduce the risk of major thrombotic vascular events such as heart attack, stroke and amputation in patients after recent lower-extremity revascularization in patients with peripheral arterial disease (PAD)	PO	8/26/2021
Tibsovo	ivosidenib	Agios	IDH1 inhibitor	Cholangio-carcinoma	Treatment for patients with previously treated isocitrate dehydrogenase 1 mutated cholangiocarcinoma	PO	9/1/2021
Opdivo	nivolumab	Bristol Myers Squibb	PD-1 inhibitor	Urothelial cancer	Adjuvant treatment of patients with surgically resected, high-risk muscle-invasive urothelial carcinoma	IV	9/3/2021
Keytruda	pembrolizumab	Merck	anti-PD-1 inhibitor	Endometrial carcinoma	In combination with Lenvima (lenvatinib), for the treatment of patients with advanced endometrial carcinoma who have disease progression following prior systemic therapy in any setting and are not candidates for curative surgery or radiation	IV	9/3/2021
Lenvima	lenvatinib	Eisai	tyrosine kinase inhibitor	Endometrial carcinoma	In combination with Keytruda (pembrolizumab), for the treatment of patients with advanced endometrial carcinoma who have disease progression following prior systemic therapy in any setting and are not candidates for curative surgery or radiation	IV	9/3/2021
Keytruda	pembrolizumab	Merck	anti-PD-1 inhibitor	Cutaneous squamous cell carcinoma	Treatment of patients with locally advanced cutaneous squamous cell carcinoma that is not curable by surgery or radiation	IV	9/9/2021

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Darzalex Faspro	daratumumab and hyaluronidase-fihj	Janssen/ Halozyme Therapeutics	humanized anti-CD38 monoclonal antibody	Multiple myeloma	In combination with pomalidomide and dexamethasone (D-Pd) for the treatment of patients with relapsed or refractory multiple myeloma who have received at least one prior line of therapy	SC	9/12/2021
Verzenio	abemaciclib	Eli Lilly	cyclin-dependent kinase 4 and 6 inhiibitor	Early breast cancer	Treatment of hormone receptor positive, HER2 negative, early breast cancer	PO	9/15/2021
Tecartus	brexucabtagene autoleucel	Gilead	CD19-directed genetically modified autologous T cell immunotherapy	Acute lymphoblastic leukemia	Treatment of adult patients with relapsed or refractory B-cell precursor acute lymphoblastic leukemia	IV	10/1/2021
Brukinsa	zanubrutinib	BeiGene	kinase inhibitor	Waldenström's Macroglobulinemia	Treatment of adult patients with Waldenström's Macroglobulinemia	PO	10/18/2021
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 inhibitor	Asthma	Add-on maintenance treatment in patients with moderate-to-severe asthma aged 6 years and older with an eosinophilic phenotype or with oral corticosteroid dependent asthma	SC	10/21/2021
Prograf	tacrolimus	Astellas Pharma	calcineurin inhibitor	Lung transplant	Prevention of rejection in lung transplantation	PO	10/30/2021
Andexxa	coagulation factor Xa (recombinant), inactivated-zhzo	Alexion	recombinant Factor Xa inhibitor antidote	Drug toxicity	In patients presenting with acute intracranial hemorrhage while taking an oral Factor Xa inhibitor	IV	10/31/2021
Caplyta	lumateperone	Intra-Cellular Therapies	antipsychotic	Bipolar I or II disorder	Treatment of bipolar depression in patients with bipolar I or II disorder as monotherapy and adjunctive therapy (with lithium or valproate)	PO	12/17/2021

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Otezla	apremilast	Amgen	phosphodieasterase 4 inhibitor	Plaque psoriasis (mild-to-moderate)	Treatment of adults with mild-to-moderate plaque psoriasis who are candidates for phototherapy or systemic therapy	PO	12/19/2021
Cabenuva	cabotegravir, rilpivirine	ViiV/ Janssen	integrase strand transfer inhibitor/ non-nucleoside reverse transcriptase inhibitor	HIV-1 infection	Dosing update: every 2 month administration (currently approved every month)	IM	12/24/2021
Skyrizi	risankizumab-rzaa	AbbVie	interleukin-23 antagonist	Psoriatic arthritis	Treatment of psoriatic arthritis	SC	2/6/2022

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

References:

Albireo Pharma. Albireo Web site. Albireo announces U.S. FDA acceptance of New Drug Application for odevixibat. https://ir.albireopharma.com/news-releases/news-release-details/albireo-announces-us-fda-acceptance-new-drug-application. January 25, 2021. Accessed March 10, 2021.

American Diabetes Association. 11. Microvascular complications and foot care: standards of medical care in diabetes-2021. Diabetes Care. 2021;44(Suppl 1):S151-S167.

American Cancer Society. Anal cancer. American Cancer Society Web site. https://www.cancer.org/cancer/anal-cancer.html. Accessed April 20, 2021.

American Lung Association. Lung Cancer Fact Sheet. American Lung Association Web site. https://www.lung.org/lung-health-diseases/lung-disease-lookup/lung-cancer/resource-library/lung-cancer-fact-sheet. Accessed April 14, 2021.

Amgen Press Release. Amgen Web Site. Amgen's investigational KRAS G12C inhibitor sotorasib demonstrated rapid, deep and durable responses in previously treated patients with advanced non-small cell lung cancer. https://www.amgen.com/newsroom/press-releases/2021/01/amgens-investigational-kras-g12c-inhibitor-sotorasib-demonstrated-rapid-deep-and-durable-responses-in-previously-treated-patients-with-advanced-nonsmall-cell-lung-cancer. January 28, 2021. Accessed April 9, 2021.

Bakris GL, Agarwal R, Anker SD, et al; FIDELIO-DKD Investigators. Effect of finerenone on chronic kidney disease outcomes in type 2 diabetes. N Engl J Med. 2020;383(23):2219-2229.

Berti A, Cornec D, Crowson CS, Specks U, Matteson EL. The Epidemiology of ANCA associated vasculitis in the U.S.: a 20 year population based study [abstract]. Arthritis Rheumatol. 2017; 69 (suppl 10). https://acrabstracts.org/abstract/the-epidemiology-of-anca-associated-vasculitis-in-the-u-s-a-20-year-population-based-study/. Accessed February 23, 2021.

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

Cara Therapeutics Press Release. Cara Therapeutics Web site. Cara Therapeutics submits New Drug Application to U.S. Food and Drug Administration for KORSUVA™ injection in hemodialysis patients with moderate-to-severe pruritus. https://ir.caratherapeutics.com/news-releases/news-release-details/cara-therapeutics-submits-new-drug-application-us-food-and-drug. December 28, 2020. April 17, 2021.

Centers for Disease Control and Prevention (CDC). National Diabetes Statistics Report, 2020. CDC Web site. https://www.cdc.gov/diabetes/pdfs/data/statistics/national-diabetes-statistics-report.pdf. Accessed March 5, 2021.

Centers for Disease Control and Prevention (CDC). Chronic kidney disease basics. CDC Web site. https://www.cdc.gov/kidneydisease/basics.html. Page last reviewed February 7, 2020. Accessed March 9, 2021.

ChemoCentryx Press Release. ChemoCentryx Web site. ChemoCentryx announces FDA acceptance of the avacopan New Drug Application (NDA) for the treatment of ANCA-associated vasculitis. https://ir.chemocentryx.com/news-releases/news-release-details/chemocentryx-announces-fda-acceptance-avacopan-new-drug. September 17, 2020. Accessed February 23, 2021.

Datamonitor Healthcare Platform. Datamonitor Healthcare Web site. http://www.datamonitorhealthcare.com/

Fishbane S, Jamal Aamir, Munera C, et al. A Phase 3 trial of difelikefalin in hemodialysis patients with pruritus. NEJM. 2020; 382:222-232.

Genetic and Rare Diseases Information Center (GARD). ANCA-associated vasculitis. GARD Web site. https://rarediseases.info.nih.gov/diseases/13011/anca-associated-vasculitis. Accessed February 23, 2021.

Herold KC, Bundy BN, Long SA, et al; Type 1 Diabetes TrialNet Study Group. An anti-CD3 antibody, teplizumab, in relatives at risk for type 1 diabetes. N Engl J Med. 2019;381(7):603-613.

Incyte Press Release. Incyte Web site. Incyte announces acceptance and priority review of BLA for retifanlimab as a potential treatment for patients with squamous cell carcinoma of the anal canal (SCAC). https://investor.incyte.com/press-releases/press-releases/2021/Incyte-Announces-Acceptance-and-Priority-Review-of-BLA-for-Retifanlimab-as-a-Potential-Treatment-for-Patients-with-Squamous-Cell-Carcinoma-of-the-Anal-Canal-SCAC/default.aspx. January 21, 2021. Accessed April 20, 2021.

Incyte Press Release. Incyte Web site. Incyte announces encouraging results from Phase 2 trial of retifanlimab (INCMGA0012) in patients with previously treated, advanced squamous cell carcinoma of the anal canal. https://investor.incyte.com/press-releases/press-releases/2020/Incyte-Announces-Encouraging-Results-From-Phase-2-Trial-of-Retifanlimab-INCMGA0012-in-Patients-With-Previously-Treated-Advanced-Squamous-Cell-Carcinoma-of-the-Anal-Canal/default.aspx. September 18, 2020. Accessed April 20, 2021.

Jayne DRW, Merkel PA, Schall TJ, Bekker P; ADVOCATE Study Group. Avacopan for the treatment of ANCA-associated vasculitis. N Engl J Med. 2021;384(7):599-609.

Katsarava Z, Buse DC, Manack AN, et al. Defining the differences between episodic migraine and chronic migraine. Curr Pain Headache Rep. 2012; 16(1): 86-92.

Lung Cancer Foundation of America. What is EGFR-positive lung cancer and how is it treated? LCFA Web site. https://lcfamerica.org/lung-cancer-info/types-lung-cancer/egfr-mutation/ Accessed April 8, 2021.

MedlinePlus. Progressive familial intrahepatic cholestasis. MedlinePlus Website. https://medlineplus.gov/genetics/condition/progressive-familial-intrahepatic-cholestasis. Accessed March 10, 2021.

Mirum Pharmacueticals. Alagille Syndrome (ALGS). Mirum Pharmaceuticals Web site. https://mirumpharma.com/patients-and-families/algs/. Accessed April 2, 2021.

National Cancer Institute. KRAS gene. NIH Web site. https://www.cancer.gov/publications/dictionaries/cancer-terms/def/kras-gene. Accessed April 12, 2021.

National Comprehensive Cancer Network (NCCN). NCCN Web site. https://www.ncn.org/. Accessed March 30, 2021.

National Institute of Diabetes and Digestive and Kidney Diseases. Kidney Disease Statistics for the United States. NIH Web site. https://www.niddk.nih.gov/health-information/health-statistics/kidney-disease#:~:text=More%20than%20661%2C000%20Americans%20have,with%20a%20functioning%20kidney%20transplant. Accessed March 31, 2021.

Provention Bio Press Release. Provention Bio Web site. Provention Bio announces U.S. FDA filing of a Biologics License Application (BLA) and priority review for teplizumab for the delay or prevention of clinical type 1 diabetes in at-risk individuals. http://investors.proventionbio.com/2021-01-04-Provention-Bio-Announces-U-S-FDA-Filing-of-a-Biologics-License-Application-BLA-and-Priority-Review-for-Teplizumab-for-the-Delay-or-Prevention-of-Clinical-Type-1-Diabetes-in-At-risk-Individuals. January 4, 2021. Accessed March 4, 2021.

Sims EK, Bundy BN, Stier K, et al; Type 1 Diabetes TrialNet Study Group. Teplizumab improves and stabilizes beta cell function in antibody-positive high-risk individuals. Sci Transl Med. 2021;13(583):eabc8980.

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

Wang F, Li C, Wu Q, et al. EGFR exon 20 insertion mutations in non-small cell lung cancer. Translational Cancer Research. 2020; 9(4). doi: 10.21037/tcr.2020.03.10

Wao H, Mhaskar R, Kumar A, et al. Survival of patients with non-small cell lung cancer without treatment: a systematic review and meta-analysis. Syst Rev. 2013; 2:10.

optum.com/optumrx

The information contained herein is compiled from various sources and is provided for informational purposes only. Due to factors beyond the control of OptumRx, information related to prospective drug launches is subject to change without notice. This information should not be solely relied upon for formulary decision-making purposes.

OptumRx specializes in the delivery, clinical management and affordability of prescription medications and consumer health products. We are an Optum® company — a leading provider of integrated health services. Learn more at **optum.com**.

RxOutlook® is published by the OptumRx Clinical Services Department.