OPTUMRx®

RxOutlook®

2nd Quarter 2020

While COVID-19 dominates the present day, a large number of drug approvals looms for 3Q:2020

This issue of RxOutlook is set against the backdrop of the COVID-19 pandemic. As a nation, we are still struggling with the urgent health needs of those who are sick, with the social isolation needed to "flatten the curve", and with the question of how and when to safely open up the country again. Recognizing that these issues are at the forefront of our minds, we turn our attention to the drug development pipeline for the latest quarterly briefing on what drugs are on the horizon. While this publication is dedicated to investigational drugs, we have decided not to include drugs for COVID-19 in this edition. Much remains unknown and research is occurring at such a rapid pace for COVID-19 that this edition will be obsolete the moment it is published. What we do know is that the pipeline for COVID-19 includes over 275 investigational products and at least 100 vaccines. The vast majority of them are still in the pre-clinical stage of development, and while any potential Food and Drug Administration (FDA) approvals seem far off, with so many compounds in development there is hope that some will eventually prove to be useful in treating and/or preventing COVID-19 infections.

As we hopefully begin to emerge from the COVID-19 pandemic, a full slate of pipeline drugs is expected to be approved in the second half of the year. In this edition of RxOutlook, we feature 17 key pipeline drugs with potential launch dates by the end of the third quarter of 2020. This is a much larger sample than we usually cover in RxOutlook because an unusually larger number of drugs have expected approval dates from July to September. While each drug is important in its own right, a few items are important to highlight. Eleven of the 17 drugs (65%) have a Breakthrough Therapy Designation from the FDA. Drugs with Breakthrough Designation have the benefit of early FDA guidance, which helps expedite development and regulatory review. To get this designation, the manufacturer must show that their drug is intended to treat a serious condition and preliminary clinical evidence must indicate substantial improvement over available therapies on clinically significant endpoints. These endpoints measure an effect on irreversible morbidity or mortality or on symptoms that represent serious consequences of the disease. However, of the 11 breakthrough drugs, eight of them are supported by only a single trial making it difficult to fully measure the true nature of their impact.

It is also important to note that eight drugs in this report are used for cancer indications including two drugs for lung cancer, both of which received FDA approval several months ahead of their expected decision dates, and two for metastatic breast cancer. Continuing with a theme that was discussed in the last RxOutlook report, 10 of the 17 drugs have Orphan Drug Designations for the treatment of rare diseases or indications. Among these drugs, one is particularly notable: valoctocogene roxaparvovec – the long-awaited gene therapy for hemophilia type A, a drug with potential to impact the treatment of hemophilia A financially and clinically.

Key pipeline drugs with FDA approval decisions expected by the end of the 3rd quarter 2020

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Capmatinib	Novartis	Non-small cell lung cancer*	5/6/2020 (Approved)
Selpercatinib	Eli Lilly	Non-small cell lung cancer/ thyroid cancer*	5/8/2020 (Approved)
Ofatumumab (SC)	Novartis	Multiple sclerosis	6/2020
Abicipar pegol	Allergan	Neovascular age-related macular degeneration	6/2020 – 7/2020
Triheptanoin	Ultragenyx Pharmaceutical	Long-chain fatty acid oxidation disorders*	7/31/2020
Fostemsavir	ViiV Healthcare/ GlaxoSmithKline	HIV-1 infection	8/5/2020
Viaskin Peanut	DBV Technologies	Peanut allergy	8/5/2020
KTE-X19	Gilead Sciences/Kite Pharma	Mantle cell lymphoma*	8/10/2020
Ripretinib	Deciphera Pharmaceuticals	Gastrointestinal stromal tumors*	8/13/2020
Belantamab mafodotin	GlaxoSmithKline	Multiple myeloma*	8/14/2020
Satralizumab	Roche/Genentech/ Chugai	Neuromyelitis optica spectrum disorder*	8/14/2020
Tucatinib	Seattle Genetics	Breast cancer*	4/17/2020 (Approved)
Margetuximab	MacroGenics	Breast cancer	8/19/2020
Filgotinib	Gilead Sciences	Rheumatoid arthritis	8/19/2020
Valoctocogene roxaparvovec	BioMarin	Hemophilia A*	8/21/2020
Veverimer	Tricida	Metabolic acidosis associated with chronic kidney disease	8/22/2020
Tafasitamab	MorphoSys	Diffuse large B-cell lymphoma*	8/30/2020

* Orphan Drug Designation

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

Detailed Drug Insights

This section reviews the important characteristics (eg, therapeutic use, clinical profile, competitive environment and regulatory timeline) for key pipeline drugs with potential FDA approvals by the end of the 2nd quarter 2020.

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 2020 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

Capmatinib (Brand Name: Tabrecta™)

Manufacturer: Novartis
Regulatory designations: Orphan Drug, Breakthrough Therapy
Approval date: 5/6/2020 (PDUFA date was originally 8/11/2020)

Therapeutic use

Capmatinib was approved for the treatment of adult patients with metastatic non-small cell lung cancer (NSCLC) whose tumors have a mutation that leads to mesenchymal-to-epithelial transition (MET) exon 14 skipping as detected by an FDA-approved test.

In the U.S., an estimated 228,820 new cases of lung cancer will be diagnosed in 2020 and about 135,720 deaths are expected to occur. NSCLC accounts for about 84% of all lung cancer cases.

About 3% to 4% of NSCLC cases are MET exon 14 skipping mutations. Currently, there are no therapies that specifically target the MET exon 14 mutation of NSCLC.

- Treatment of locally advanced or metastatic MET exon 14 skipping mutated NSCLC

Clinical profile

Capmatinib is a selective MET tyrosine kinase inhibitor. It is designed to inhibit MET, an enzyme involved in cell growth and proliferation in different cancers when deregulated.

Pivotal trial data:
The efficacy of capmatinib was evaluated in a Phase 3, non-randomized, open-label study in 97 patients with NSCLC. The primary endpoint was overall response rate (ORR). The ORR was 68% (95% CI: 48, 84) for treatment-naïve patients and 41% (95% CI: 29, 53) for previously treated patients. The secondary endpoint of duration of response (DOR) was 12.6 months (95% CI: 5.5, 25.3) and 9.7 months (95% CI: 5.5, 13) for the treatment-naïve and treatment-experienced patients, respectively.

Safety:
The most common adverse events with capmatinib use were peripheral edema, nausea, fatigue, vomiting, dyspnea, and decreased appetite.

Dosing:
Capmatinib is administered orally twice daily.

- MET inhibitor
- Oral formulation
- ORR (treatment-naïve): 68%
- ORR (previously treated): 41%
- Common AEs: peripheral edema, nausea, fatigue, vomiting, dyspnea, decreased appetite
- Dosing: twice daily

Capmatinib (Brand Name: Tabrecta™) (continued...)

Competitive environment

Capmatinib is the first approved therapy to specifically target the MET exon-14 skipping mutation in NSCLC. With the relatively high ORR for both treatment-naïve and previously treated patients, capmatinib shows promising early stage data for this patient population.

However, while the data is encouraging, the overall survival (OS) data for the pivotal trial was not reported. In addition, capmatinib will only treat a small patient population because the initial indication only covers patients with a specific NSCLC mutation and whose cancer is metastatic or locally advanced. Finally, capmatinib will potentially compete with Xalkori® (crizotinib), which is approved for other mutations of lung cancer, as well as pipeline products such as tepotinib and savolitinib. Merck may file for regulatory approval for tepotinib in 2020.

The Wholesale Acquisition Cost (WAC) price for capmatinib is approximately $19,230 per 30 days.

- Advantages: novel targeted therapy for MET exon 14 skipping mutated NSCLC, promising early stage data based on response rates, oral administration
- Disadvantages: lack of OS data, small eligible patient population, potentially competing with Xalkori and additional pipeline agents (tepotinib and savolitinib)
- WAC: ~$19,230 per 30 days

Selpercatinib (Brand Name: Retevmo™)

Manufacturer: Eli Lilly
Regulatory designations: Orphan Drug, Breakthrough Therapy
Approval date: 5/8/2020 (PDUFA date was originally 7/2020 – 8/2020)

Therapeutic use

Selpercatinib was approved for the treatment of adult patients with metastatic *RET (REarranged during Transfection)* fusion-positive non-small cell lung cancer (NSCLC); adult and pediatric patients 12 years of age and older with advanced or metastatic *RET*-mutant medullary thyroid cancer (MTC) who require systemic therapy; and adult and pediatric patients 12 years of age and older with advanced or metastatic *RET* fusion-positive thyroid cancer who require systemic therapy and who are radioactive iodine-refractory (if radioactive iodine is appropriate).

In the U.S., an estimated 228,820 new cases of lung cancer will be diagnosed in 2020 and about 135,720 deaths are expected to occur. NSCLC accounts for about 84% of all lung cancer cases. An estimated 52,890 new cases of thyroid cancer are expected in 2020 and about 2,180 deaths.

Genomic alterations in *RET* kinase, which include fusions and activating point mutations, lead to overactive *RET* signaling and uncontrolled cell growth. *RET* fusions have been identified in approximately 2% of NSCLC and 10 to 20% of papillary and other thyroid cancers. Activating *RET* point mutations account for approximately 60% of MTC cases.

- Treatment of patients with advanced RET fusion-positive NSCLC, RET-mutant MTC and RET fusion-positive thyroid cancer

Selpercatinib (Brand Name: Retevmo™) (continued...)

Clinical profile

Selpercatinib is a highly selective inhibitor of RET signaling.

Pivotal trial data:
The efficacy of selpercatinib was evaluated in a single-arm, multi-cohort study (LIBRETTO-001) in patients with *RET*-driven cancers. The study included both treatment-naive patients and heavily pretreated patients with a variety of advanced solid tumors including *RET* fusion-positive NSCLC, *RET*-mutant MTC, and *RET* fusion-positive thyroid cancer. The primary efficacy outcome was ORR. In treatment-naïve *RET* fusion-positive NSCLC patients (n = 39), the ORR was 85% (95% CI: 70, 94) and in treatment-experienced patients (n = 105), the ORR was 64% (95% CI: 54, 73).

The *RET*-altered thyroid cancer portion of the LIBRETTO-001 study included two different populations: *RET*-mutant MTC and *RET* fusion-positive thyroid cancers. The *RET*-mutant MTC dataset consisted of 55 patients with prior Cometriq® (cabozantinib) and/or Caprelsa® (vandetanib) therapy. Treatment with selpercatinib resulted in an ORR of 69% (95% CI: 55, 81). In 88 *RET*-mutant MTC patients who received neither Cometriq nor Caprelsa, selpercatinib treatment resulted in an ORR of 73% (95% CI: 62, 82).

In 19 treatment-experienced *RET* fusion-positive thyroid cancer patients, selpercatinib treatment resulted in a 79% ORR (95% CI: 54, 94). In the 8 treatment-naïve patients, the ORR was 100% (95% CI: 63, 100).

Safety:
The most common adverse events with selpercatinib use were increased aspartate aminotransferase (AST), increased alanine aminotransferase (ALT), increased glucose, decreased leukocytes, decreased albumin, decreased calcium, dry mouth, diarrhea, increased creatinine, increased alkaline phosphatase, hypertension, fatigue, edema, decreased platelets, increased total cholesterol, rash, decreased sodium, and constipation.

Dosing:
Selpercatinib is administered orally twice daily.

- RET inhibitor
- Oral formulation
- NSCLC: ORR = 85% (treatment-naïve) and 64% (treatment-experienced)
- MTC: ORR = 73% (cabozantinib and/or vandetanib-naïve) and 69% (cabozantinib and/or vandetanib-experienced)
- Thyroid cancer: ORR = 79% (treatment-experienced)
- Common AEs: increased AST, increased ALT, increased glucose, decreased leukocytes, decreased albumin, decreased calcium, dry mouth, diarrhea, increased creatinine, increased alkaline phosphatase, hypertension, fatigue, edema, decreased platelets, increased total cholesterol, rash, decreased sodium, constipation
- Dosing: twice daily

Competitive environment

Selpercatinib offers a targeted therapy for the treatment of NSCLC and thyroid cancer. The initial indication for selpercatinib is narrow as *RET* fusions and mutations only represent a small subset of patients with NSCLC and thyroid cancer. In addition, the approval is based on an early phase study in mostly treatment-experienced patients and there is a lack of robust OS data. In December of 2019, Eli Lilly opened two selpercatinib Phase 3 trials in patients with treatment-naïve *RET* fusion-positive NSCLC and patients with treatment-naïve *RET*-mutant MTC.

Selpercatinib may also face future competition in the *RET* inhibitor space; Blueprint Medicines' recently announced that they completed a rolling FDA submission for pralsetinib for the treatment of *RET* fusion-positive NSCLC.

The WAC price for selpercatinib is approximately $20,600 per 30 days.

- Advantages: promising early stage data, targeted therapy, well tolerated
- Disadvantages: narrow indication, lack of late stage data, potential future competition
- WAC: ~$20,600 per 30 days

Ofatumumab (Brand Name: To be determined)

Manufacturer: Novartis
Expected FDA decision: 6/2020

Therapeutic use

Subcutaneous (SC) ofatumumab is in development for the treatment of relapsing forms of multiple sclerosis (MS).

Ofatumumab is currently available in an intravenous (IV) formulation as Arzerra® and is FDA approved for the treatment of chronic lymphocytic leukemia.

MS is a chronic, autoimmune disease of the central nervous system that disrupts the normal functioning of the brain, optic nerves and spinal cord through inflammation and tissue loss. Patients with relapsing forms of MS have episodes of worsening function (relapses) followed by recovery periods (remissions). These remissions may not be complete and may leave patients with some degree of residual disability.

According to the National Multiple Sclerosis Society, up to 913,925 adults are affected by MS in the U.S. and approximately 85% of patients initially present with the relapsing form of the disease.

- Treatment of relapsing forms of MS

Ofatumumab (continued...)

Clinical profile

Ofatumumab is a fully human anti-CD20 monoclonal antibody. Ofatumumab works by binding to the CD20 molecule on the B-cell surface and induces potent B-cell lysis and depletion.

Pivotal trial data:
The efficacy of ofatumumab was evaluated in ASCLEPIOS I and II, two identical design, flexible duration (up to 30 months), double-blind, double-dummy, randomized Phase 3 studies in 1,882 patients with relapsing forms of MS. Patients received either ofatumumab 20 mg monthly SC injections or Aubagio® (teriflunomide) 14 mg taken orally once daily. The primary endpoint was the reduction in the number of confirmed relapses, evaluated as the annualized relapse rate (ARR).

In both studies, ofatumumab showed a statistically significant reduction in the ARR. In ASCLEPIOS I, patients treated with ofatumumab had an ARR of 0.11 vs. 0.22 with Aubagio, corresponding to a 50.5% reduction in ARR ($p < 0.001$). Similarly, in ASCLEPIOS II, ofatumumab treated patients had an ARR of 0.10 vs. 0.25 with Aubagio, corresponding to a 58.8% reduction in ARR ($p < 0.001$).

Safety:
The safety data for SC ofatumumab from the Phase 3 trials is limited; however, the most common adverse event with SC ofatumumab use in earlier stage trials in MS was injection-related reactions which occurred in 52% of patients (vs. 15% for placebo). These reactions were mild to moderate in nature, and tended to diminish with subsequent dosing. Serious adverse events reported in the early stage trials also included cholelithiasis, cytokine-related syndrome and hypokalemia.

Dosing:
In the pivotal trials, ofatumumab was administered SC once monthly.

- anti-CD20 monoclonal antibody
- SC formulation
- Overall reduction in ARR: 50.5% and 58.8 reduction vs. Aubagio ($p < 0.001$)
- Common AE: injection-related reactions
- Dosing: once monthly

Competitive environment

If approved, ofatumumab would be the first SC administered anti-CD20 therapy for MS and it can be self-administered once a month. The only other CD20 targeting therapy for MS is Ocrevus® (ocrelizumab) which is administered via IV infusion.

However, ofatumumab will be entering a crowded marketplace for the treatment of MS, competing not only with Ocrevus, but other well established oral and injectable products with different mechanisms of action (MOA). While this formulation of ofatumumab is expected to be self-administered, it still requires monthly injections whereas Ocrevus can be administered every 6 months as a maintenance dose.

The IV formulation of ofatumumab, Arzerra, has a boxed warning for hepatitis B virus (HBV) reactivation and progressive multifocal leukoencephalopathy (PML) and these warnings may apply for the SC formulation.

For reference, the WAC price for Ocrevus is approximately $65,000 per year.

- Advantages: first SC administered anti-CD20 therapy for MS, self-administered
- Disadvantages: alternatives available - including Ocrevus which shares the same MOA and other oral and injectable MS products, limited safety data, IV formulation of ofatumumab has a boxed warning for HBV reactivation and PML
- Reference WAC (Ocrevus): ~$65,000 per year

Abicipar pegol (Brand Name: To be determined)

Manufacturer: Allergan
Expected FDA decision: 6/2020 – 7/2020

Therapeutic use

- Treatment of neovascular AMD

Abicipar pegol is in development for the treatment of neovascular (wet) age-related macular degeneration (AMD).

The American Academy of Ophthalmology estimates that 15 million North Americans currently have AMD with about 10% to 15% suffering from wet AMD. Wet AMD is a degenerative disease of the central portion of the retina characterized by growth of abnormal vessels in the subretinal space; this results in loss of central vision and, if untreated, can lead to blindness.

Clinical profile

- VEGF inhibitor
- Intravitreal formulation
- Stable vision at week 52: 91.2% to 94.8% vs. 95.5 to 96.0% with Lucentis (noninferiority met)
- Common AE: intraocular inflammation
- Maintenance dose: once every 8 or 12 weeks

Abicipar pegol is a vascular endothelial growth factor (VEGF) inhibitor. Increased signaling through the VEGF pathway is associated with pathologic ocular angiogenesis and retinal edema. Inhibition of the VEGF pathway has been shown to reduce the growth of neovascular lesions, resolve retinal edema and improve vision in patients with retinal vascular diseases.

Abicipar pegol differs from currently available VEGF inhibitors in that it is associated with higher binding affinity and low molecular weight which may result in a longer duration of effect.

Pivotal trial data:
The efficacy of abicipar pegol was evaluated in two identical, randomized, double-masked, Phase 3 studies (CEDAR and SEQUOIA) in treatment-naïve patients with wet AMD. The studies included 3 treatment arms: (1) abicipar pegol 2 mg monthly for 3 doses followed by a dose every 8 weeks (abicipar pegol 2Q8 arm); (2) abicipar pegol 2 mg monthly for 2 doses, followed by a dose after 8 weeks, followed by a dose every 12 weeks (abicipar pegol 2Q12 arm); or (3) Lucentis® (ranibizumab) 0.5 mg monthly. The primary endpoint was based on a proportion of patients with stable vision at week 52. Stable vision was defined as the proportion of patients with vision loss of fewer than or equal to 15 letters in best-corrected visual acuity (BCVA) from baseline.

Each abicipar pegol regimen (2Q8 and 2Q12) met the primary endpoint of noninferiority to Lucentis in stable vision at week 52 in both trials. The proportion of patients with stable vision at week 52 was 94.8%, 91.3%, 96.0% (SEQUOIA) and 91.7%, 91.2%, 95.5% (CEDAR) in the abicipar pegol 2Q8, abicipar pegol 2Q12, and Lucentis arms, respectively. Mean gain in BCVA from baseline at week 52 was 8.3, 7.3, 8.3 letters (SEQUOIA) and 6.7, 5.6, 8.5 letters (CEDAR) in the treatment arms, respectively.

Safety:
The most common adverse event with abicipar pegol use was intraocular inflammation.

Dosing:
In the pivotal trials, abicipar pegol was administered via intravitreal injection and dosed either (1) monthly for 3 doses followed by a dose every 8 weeks, or (2) monthly for 2 doses, followed by a dose after 8 weeks, followed by a dose every 12 weeks.

Abicipar pegol (continued...)

Competitive environment

If approved, abicipar pegol would provide an additional VEGF inhibitor treatment option for wet AMD. Other approved VEGF inhibitors for wet AMD include Lucentis, Eylea® (aflibercept), and the recently approved Beovu® (brolucizumab). The primary advantage for abicipar pegol is that it can be administered every 12 weeks which could reduce the number of annual intravitreal injections.

However, abicipar pegol is a relatively late market entry for the treatment of wet AMD and one of its competitors, Beovu, can also be dosed every 12 weeks. Eylea and Lucentis are well-established treatments in the ophthalmology space with FDA approvals for other indications (eg, macular edema following retinal vein occlusion, diabetic macular edema, diabetic retinopathy).

In addition, while abicipar pegol may offer a convenience benefit in terms of the frequency of injections, the incidence of intraocular inflammation events was significantly higher among patients receiving abicipar pegol vs. Lucentis (approximately 15% vs. less than 1%). Allergan is working on an optimized formulation of abicipar pegol which may reduce the incidence of ocular inflammation to about 9%.

For reference, the WAC price for Beovu is approximately $7,400 to $11,000 per year, following the loading dose.

- Advantages: long duration of effect and potential reduction in the number of intravitreal injections per year
- Disadvantages: late market entry, currently available VEGF inhibitors are also approved for other ophthalmic indications, higher incidence of intraocular inflammation vs. Lucentis
- Reference WAC (Beovu): ~$7,400 to $11,000 per year

Triheptanoin (Brand Name: To be determined)

Manufacturer: Ultragenyx Pharmaceutical
Regulatory designations: Orphan Drug, Fast Track
FDA approval date: 7/31/2020

Therapeutic use

Triheptanoin is in development for the treatment of long-chain fatty acid oxidation disorders (LC-FAOD).

LC-FAODs are a group of rare genetic disorders characterized by metabolic deficiencies in which the body is unable to convert long-chain fatty acids into energy. The inability to produce energy from fat can lead to severe depletion of glucose in the body, and serious liver, muscle, and heart disease. LC-FAODs are a very rare disease with only 2,000 to 3,500 patients in the U.S.

The current standard of care for patients with LC-FAOD is low-fat/high carbohydrate diets, medium-chain triglyceride (MCT) oil (medical food product), and frequent feeding (ie, avoidance of prolonged fasting). Despite lifestyle and dietary changes, symptoms can still be triggered by acute illness or other physiologic stress, resulting in significant morbidity and life-threatening metabolic decompensation.

- Treatment of LC-FAOD

Triheptanoin (continued...)

Clinical profile

Triheptanoin is a highly purified, pharmaceutical-grade, MCT consisting of three 7-carbon fatty acids on a glycerol backbone. It is designed to provide patients with an alternative energy source that can be metabolized to increase intermediate substrates in the Krebs cycle, a key energy-generating process. Unlike typical even-chain fatty acids, one of the Krebs cycle intermediates generated specifically by triheptanoin also can be converted to glucose.

Pivotal trial data:
The filing with the FDA was supported by data including results from 3 small trials: a company-sponsored Phase 2, single-arm, open-label study in 29 patients; a long-term safety and efficacy extension study in 75 patients; and a randomized controlled investigator-sponsored study of 32 patients.

In the Phase 2 study, after a four-week run-in on current regimen, triheptanoin was titrated to a target dose of 25% to 35% of total daily caloric intake. Patients were eligible for a 12-minute walk test (12MWT) if they were at least 6 years old during the study and met skeletal myopathy requirements. Eligible patients (n = 8) demonstrated a 28% increase (least squares mean 181.9 meters; p = 0.087) from baseline (673.4 meters) in 12MWT distance at week 18.

A total of 75 patients were enrolled in the long-term safety and efficacy study including 24 patients who were previously enrolled in the company-sponsored Phase 2 study, 20 naïve patients who had not previously been treated, and 31 patients from expanded access or investigator-sponsored studies. The primary endpoint was the annualized major clinical event rate (eg, hospitalizations and emergency room visits). Patients who previously completed the Phase 2 received treatment for an additional 78 weeks (minimum of 3 years of total treatment). Over the entire treatment period, patients had a 67% reduction in median annualized event rate. Patients who were naïve to treatment (n = 20) at study entry and received up to 78 weeks of treatment had a 70% reduction in the median annualized event rate.

In the investigator-sponsored study, patients were randomized to a diet containing 20% of their total daily energy from either triheptanoin or trioctanoin (alternative MCT) for 4 months. Patients in the triheptanoin group increased left ventricular (LV) ejection fraction by 7.4% (p = 0.046) while experiencing a 20% (p = 0.041) decrease in LV wall mass on their resting echocardiogram.

Safety:
The most common adverse events with triheptanoin use were diarrhea, vomiting, abdominal pain, and gastroenteritis.

Dosing:
In the pivotal trials, triheptanoin was administered orally with the goal of 20% of their total daily energy coming from triheptanoin.

- Medium-chain triglyceride
- Oral formulation
- 12MWT distance: 28% increase from baseline
- Major clinical event rate: 67% to 70% reduction compared to baseline
- Common AEs: diarrhea, vomiting, abdominal pain, gastroenteritis
- Dosing: titrated to total daily caloric intake goals

Triheptanoin (continued...)

Competitive environment

Triheptanoin could be the first drug approved by the FDA for the treatment of LC-FAOD and would provide a pharmaceutical-grade MCT for treatment of the condition. Despite current treatment approaches, there is a high unmet need for additional options as patients can still experience significant disease manifestations such as rhabdomyolysis and cardiomyopathy and can experience life-threatening metabolic decompensation. These metabolic events can lead to frequent hospitalizations.

While there is a need for treatment options, clinical studies for triheptanoin were generally non-randomized and uncontrolled. The current standard of care includes lifestyle and diet modifications and supplementation with medical food products. Considering the ultra-rare nature of LC-FAODs, the potential number of patients that would be candidates for triheptanoin is very small.

- Advantages: potentially the first approved therapy for LC-FAOD, high unmet need (patients with LC-FAOD can experience life-threatening metabolic decompensation)
- Disadvantages: lack of robust clinical data, current standard of care are lifestyle/diet modifications and medical food products (ie, MCT oil), small eligible patient population

Fostemsavir (Brand Name: To be determined)

Manufacturer: ViiV Healthcare/GlaxoSmithKline
Regulatory designations: Fast Track, Breakthrough Therapy
Expected FDA decision: 8/5/2020

Therapeutic use

Fostemsavir is in development, in combination with other antiretroviral agents, for the treatment of heavily treatment-experienced adults with multidrug-resistant human immunodeficiency virus (HIV)-1 infection who are unable to form a suppressive regimen due to resistance, intolerance or safety considerations.

As of 2016, an estimated 1.1 million people aged 13 and older had HIV-1 infection in the U.S., including an estimated 162,500 (14%) people whose infections had not been diagnosed.

While antiretroviral therapies are available for HIV-1 infection, patients may develop multidrug resistance or have contraindications to or unacceptable side effects with various drugs.

- In combination with other antiretroviral agents, for the treatment of heavily treatment-experienced adults with multidrug-resistant HIV-1 infection

Clinical profile

Fostemsavir, a prodrug of temsavir, is a first-in-class HIV-1 attachment inhibitor that works by binding directly to the glycoprotein 120 subunit on the surface of the virus. By binding to this location on the virus, fostemsavir blocks HIV from attaching to host immune system CD4+ T-cells and other immune cells, thereby preventing HIV from infecting those cells and multiplying.

Pivotal trial data:
The efficacy of fostemsavir was evaluated in the BRIGHTE study, a Phase 3, partially-randomized, double-blind, placebo-controlled study in 371 heavily treatment-experienced adults living with HIV-1 infection with multidrug resistance. In the first cohort (randomized cohort) patients who had the option of using at least one fully active, approved antiretroviral drug in at least one but no more than two antiretroviral classes added either fostemsavir or placebo to their failing regimen for 8 days. Patients were then included in an open-label phase where they received fostemsavir plus optimized background therapy. In the second cohort (nonrandomized cohort), patients who had no remaining antiretroviral options were started on open-label fostemsavir plus optimized background therapy on day 1. The primary endpoint was the mean change in the HIV-1 RNA level from day 1 through day 8 in the randomized cohort.

At day 8, the mean decrease in the HIV-1 RNA level was 0.79 log10 copies/mL in the fostemsavir group vs. 0.17 log10 copies/mL in the placebo group ($p < 0.001$). At week 48, a virologic response (defined as a HIV-1 RNA level < 40 copies/mL) occurred in 54% of the patients in the randomized cohort and in 38% of those in the nonrandomized cohort.

Safety:
The most common adverse events with fostemsavir use were nausea and diarrhea.

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

- Oral formulation
- Day 8: mean decrease of 0.79 log10 copies/mL in HIV-1 RNA level vs. 0.17 log10 copies/mL with placebo ($p < 0.001$)
- Week 48 (open-label): 38% to 54% virologic response
- Common AEs: nausea and diarrhea
- Dosing: twice daily

Fostemsavir (continued...)

Competitive environment

If approved, fostemsavir would offer an oral, first-in-class antiretroviral therapy for patients with multidrug-resistant HIV-1 infection. There is a high unmet need for treatments in this patient population and fostemsavir has no in vitro cross-resistance to currently available classes of antiretroviral drugs. Even partial virologic suppression of HIV RNA to > 0.5 log10 copies/mL from baseline correlates with clinical benefit.

The proposed initial indication for fostemsavir is narrow and it would be reserved for patients who have exhausted other antiretroviral therapies. GlaxoSmithKline estimates that only about 2 to 4% of HIV-1 infected patients would be candidates for fostemsavir. In the multidrug resistant HIV-1 subpopulation, fostemsavir will be competing with Trogarzo® (ibalizumab-uiyk). Trogarzo is administered IV as a single loading dose followed by a maintenance dose every 2 weeks.

For reference, the WAC price for Trogarzo is approximately $118,000 per year.

- Advantages: first-in-class antiretroviral, high unmet need for therapies for multidrug-resistant HIV-1 infection, no in vitro cross resistance with other classes of antiretroviral drugs, oral administration
- Disadvantages: narrow indication, competing with Trogarzo
- Reference WAC (Trogarzo): ~$118,000 per year

DBV-712 (Brand Name: Viaskin Peanut)

Manufacturer: DBV Technologies
Regulatory designations: Fast Track, Breakthrough Therapy
Expected FDA decision: 8/5/2020 (additional data was submitted to the FDA in response to a question about efficacy, so the decision could be delayed if FDA deems the submission a major amendment)

Therapeutic use

Viaskin Peanut is in development for the treatment of peanut allergy in children ages 4 to 11 years.

Peanut allergy is the most common food allergy in children in the U.S., affecting approximately 1.2 million children and teens, with an estimated prevalence of 2.2%. Peanut allergy accounts for the majority of deaths related to food allergy. Historically, the standard of care has been peanut avoidance and access to self-injectable epinephrine in cases of accidental exposure.

- Treatment of peanut allergy in children ages 4 to 11 years

Clinical profile

Viaskin Peanut is an epicutaneous immunotherapy. It is a patch designed to deliver peanut antigens in small quantities to the immune system through the outer layers of the skin.

- Epicutaneous immunotherapy
- Patch formulation

Pivotal trial data:
The efficacy of Viaskin Peanut was evaluated in peanut allergic children ages 4 to 11 years in two studies, PEPITES and PEOPLE. PEPITES was a Phase 3, randomized, double-blind, placebo-controlled trial in which 365 patients received Viaskin Peanut 250 µg or placebo. The primary outcome was the percentage difference in responders between Viaskin Peanut and placebo based on eliciting dose (ED) at 12 months. The ED was defined as the highest dose at which patient had signs and symptoms of an immediate hypersensitivity reaction. The proportion of patients that could tolerate an increased amount of peanuts compared to baseline was 35.3% in the Viaskin Peanut group vs. 13.6% in the placebo group (difference 21.7; 95% CI: 12.4, 29.8; $p<0.001$).

- PEPITES (12 months): proportion that could tolerate increased amount of peanut vs. baseline: 35.3% vs. 13.6% with placebo ($p<0.001$)

PEOPLE is an ongoing open-label extension study that evaluates the long-term safety and efficacy of Viaskin Peanut. In the study, 213 patients were given Viaskin Peanut for 36 months. After 36 months, 75.9% of the patients increased their ED from baseline, and the proportion of patients that reached an ED of at least 1,000 mg (equivalent to 3 to 4 peanut kernels) was 51.8%.

- PEOPLE (36 months; open-label): 75.9% increased their ED vs. baseline; 51.8% had an ED of ≥ 1000 mg

Safety:
The most common adverse event with Viaskin Peanut use was application site reaction.

- Common AEs: application site reactions

Dosing:
In the pivotal trials, the patch was applied for 6 hours a day for the first week (day 1 through day 7), 12 hours a day for the second week (day 8 through day 14), and then 24 hours (± 4 hours of allowance) a day from the third week onwards (day 15). The patch was applied to the back, rotating the location every day.

- Dosing: one patch every 24 hours

DBV-712 (Brand Name: Viaskin Peanut) (continued...)

Competitive environment

If approved, Viaskin Peanut would offer a novel therapy for the treatment of peanut allergy in children. There is a potential unmet need for this condition because oral Palforzia™ (peanut [*Arachis hypogaea*] allergen powder) is the only other FDA-approved treatment option, and its use is limited since not all peanut allergic patients are able to tolerate the medication. Compared to Palforzia, Viaskin appears to offer a better safety profile with its most common adverse event being application site reactions, and it is not associated with the gastrointestinal side effects seen with Palforzia. Furthermore, Viaskin Peanut offers a more convenient and shorter titration phase compared to Palforzia. Palforzia initially requires a 20-week titration phase that involves dose escalations every 2 weeks, in which supervision in a healthcare setting is needed for the first dose and each subsequent initial dose when a dose is increased. Conversely, Viaskin Peanut's titration phase is only 2 weeks and only requires medical supervision once for the first dose.

Viaskin Peanut targets children ages 4 to 11 years, while Palforzia has been approved for children ages 4 to 17 years. Like Palforzia, lack of adherence to Viaskin Peanut allows the sensitivity to peanuts to return and could result in serious allergic reaction when the therapy is restarted. A major disadvantage for both Palforzia and Viaskin Peanut is the uncertainty about their use in clinical practice due to the lack of real world data demonstrating a reduction in anaphylaxis, which is the main goal of these therapies.

The projected WAC price for Viaskin Peanut is approximately $6,500 per year.

- Advantages: potential unmet need, better safety profile vs. Palforzia, only initial dose requires supervision by medical professional, patch and once daily
- Disadvantages: narrow age range vs. Palforzia, lack of adherence may result in serious allergic reaction when therapy restarts, lack of data for real world reduction of anaphylaxis
- Projected WAC: ~$6,500 per year

KTE-X19 (Brand Name: To be determined)

Manufacturer: Gilead Sciences/Kite Pharma
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 8/10/2020

Therapeutic use

KTE-X19 is in development for the treatment of adult patients with relapsed or refractory mantle cell lymphoma (MCL).

MCL is a rare and aggressive form of non-Hodgkin's lymphoma (NHL) that affects cells originating in the mantle zone of lymphatic nodules. In the U.S., 1 in 100,000 individuals are diagnosed with MCL each year. The 5-year overall survival rate ranges from 20% to 60% depending on the extent of the cancer.

- Treatment of adult patients with relapsed or refractory MCL

Clinical profile

KTE-X19 is a chimeric antigen receptor (CAR) T cell therapy designed to target CD19. CD19 is a surface antigen broadly expressed on the surface of B cells, making it a viable target for the treatment of B cell malignancies, such as MCL.

- CAR T cell therapy
- IV formulation

Pivotal trial data:
KTE-X19 was evaluated in a single-arm, open label Phase 2 study, in which a single infusion of KTE-X19 were given to 60 patients whose disease is refractory to or has relapsed up to five prior lines of therapy. The ORR was 85% (95% CI: 67, 96) and the complete remission (CR) rate was 57% (95% CI: 37, 76). The 12-month estimates for other clinical endpoints were DOR 83% (95% CI: 60, 93), PFS 71% (95% CI: 50, 84), and OS 86% (95% CI: 66, 94). The medians for PFS and OS were not reached.

- ORR: 86%
- 12-month estimated PFS: 71%
- 12-month estimated OS: 86%

Safety:
The most common adverse events with KTE-X19 use were cytokine release syndrome (CRS), neurologic events, anemia, decreased platelet count, neutropenia, and decreased neutrophil count.

- Common AEs: CRS, neurologic events, anemia, decreased platelet count, neutropenia, decreased neutrophil count

Dosing:
In the pivotal trial, KTE-X19 was administered as a one-time IV infusion.

- Dosing: one-time dose

KTE-X19 (continued...)

Competitive environment

If approved, KTE-X19 would become the first CAR T cell therapy for MCL, offering an additional treatment option for patients with relapsed or refractory MCL that only requires a one-time dose. The early stage data for KTE-X19 are promising.

However, there is a lack of long-term remission data for CAR T cell therapies, which is important considering the high cost of the one-time administration. KTE-X19 will be competing against five other FDA-approved drugs for the same indication, four of which are oral formulations. Similar to other FDA-approved CAR T cell therapies, KTE-X19 could potentially receive a boxed warning and REMS program for CRS, which may require close monitoring for four weeks post-administration. Furthermore, treatment delays may also occur due to the long preparation process needed to produce the cells for administration to the patient.

For reference, the WAC price for two other CAR T cell therapies, Kymriah® (tisagenlecleucel) and Yescarta® (axicabtagene ciloleucel), ranges from $373,000 to $475,000.

- Advantages: potentially the first CAR T cell therapy for MCL, additional treatment option for relapsed/ refractory MCL, one-time infusion, promising early stage data
- Disadvantages: lack of long-term remission data, crowded marketplace, high cost, possible boxed warning and REMS program, treatment delays due to preparation needed for cells
- Reference WAC (Kymriah, Yescarta): ~$373,000 to $475,000 per one-time dose

Tafasitamab (Brand Name: To be determined)

Manufacturer: MorphoSys
Regulatory designations: Orphan Drug, Fast Track, Breakthrough Therapy
Expected FDA decision: 8/30/2020

Therapeutic use

Tafasitamab is in development as a treatment of relapsed or refractory diffuse large B cell lymphoma (DLBCL) as part of a combination regimen with lenalidomide.

- Treatment of relapsed or refractory DLBCL as part of a combination regimen with lenalidomide

Clinical profile

Tafasitamab is a humanized monoclonal antibody that targets CD19. CD19 is a surface antigen broadly expressed on the surface of B cells, making it a viable target for the treatment of B cell malignancies, such as DLBCL.

Pivotal trial data:
The combination regimen of tafasitamab and lenalidomide was evaluated in a single-arm, open-label Phase 2 study in 80 patients with DLBCL who had received at least three prior lines of therapy. The ORR was 60% (95% CI: 48.4, 70.8). Median PFS was 12.1 months (95% CI: 5.7, not reached) and median OS has not been reached.

Safety:
The most common adverse events with tafasitamab use were neutropenia, thrombocytopenia, and anemia.

Dosing:
In the pivotal trial, tafasitamab was administered intravenously once weekly in four-week cycles up to 24 cycles or until disease progression or unacceptable toxicity.

- Anti-CD19 monoclonal antibody
- IV formulation
- ORR: 60%
- Median PFS: 12.1 months
- Common AEs: neutropenia, thrombocytopenia, anemia
- Dosing: every week in four-week cycles up to 24 cycles or until disease progression or unacceptable toxicity

Competitive environment

Tafasitamab would offer a novel targeted immunotherapy for the treatment of refractory or relapsed DLBCL. The early stage data shows promising efficacy for this treatment, and it also has a favorable side effect profile. Tafasitamab may also be an alternative to CAR T therapies, which are costly and have delays in therapy due to the long production process.

The proposed initial indication for tafasitamab is expected to be narrow and limited to the relapsed and refractory DLBCL subpopulation. In addition to CAR T cell therapies, tafasitamab will potentially be competing with Polivy® (polatuzumab vedotin), which was approved in June 2019 for a similar indication. Tafasitamab and Polivy both have a similar side effect profile and are both IV administered. However, tafasitamab is dosed once weekly, while Polivy is dosed once every 21 days.

For reference, the WAC price for Polivy is approximately $15,000 per 21-day cycle.

- Advantages: novel targeted immunotherapy for DLBCL, promising early stage data, favorable side effect profile, alternative to CAR T therapies
- Disadvantages: competing with recently approved Polivy (polatuzumab vedotin) and CAR T therapies, IV administration, narrow initial indication
- Reference WAC (Polivy): ~$15,000 per 21-day cycle

Ripretinib (Brand Name: To be determined)

Manufacturer: Deciphera Pharmaceuticals
Regulatory designations: Orphan Drug, Fast Track, Breakthrough Therapy
Expected FDA decision: 8/13/2020

Therapeutic use

Ripretinib is in development for the treatment of patients with advanced gastrointestinal stromal tumors (GIST) who have received prior treatment with imatinib, sunitinib, and regorafenib.

GIST is an uncommon cancer that forms in the gastrointestinal tract, most commonly in the stomach or small intestine. In the U.S., 4,000 to 6,000 patients are diagnosed with GIST each year. The 5-year relative survival rate for patients with GIST (all stages) is about 83%.

Clinical profile

Ripretinib is an inhibitor of platelet-derived growth factor alpha (PDGFRα) and KIT kinases, which both play a role in cell growth. Mutations in the KIT or PDGFRα genes have been found in the majority of GIST tumors.

Pivotal trial data:
The efficacy of ripretinib was evaluated a Phase 3, double-blind, placebo-controlled trial (INVICTUS) that compared ripretinib and placebo in 129 GIST patients previously treated with imatinib, sunitinib, and regorafenib. The primary endpoint was improved PFS compared to placebo. Median PFS was 6.3 months for the ripretinib group vs. 1.0 month for the placebo group ($p < 0.0001$). The secondary endpoint of ORR was 9.4% for the treatment group vs. 0% in the placebo group ($p = 0.0504$). According to the pre-specified hierarchical testing procedure of the endpoints, a formal analysis of the OS data was not conducted because ORR was not found to be statistically significant.

Safety:
The most common adverse events with ripretinib use were anemia, abdominal pain, hypertension, alopecia, fatigue, nausea, constipation, and myalgia.

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

- Treatment of patients with advanced GIST who have received prior treatment with imatinib, sunitinib, and regorafenib
- PDGFRα and KIT inhibitor
- Oral formulation
- Median PFS: 6.3 months vs. 1.0 month with placebo ($p < 0.0001$)
- ORR: 9.4% vs. 0% with placebo ($p = 0.0504$)
- Common AEs: anemia, abdominal pain, hypertension. alopecia, fatigue, nausea, constipation, myalgia
- Dosing: once daily

Ripretinib (continued...)

Competitive environment

If approved, ripretinib would provide a treatment option in the fourth-line setting for GIST where no other FDA-approved therapies exist. Ripretinib may have a role in other cancers as it is being evaluated in advanced systemic mastocytosis, gliomas, and solid tumors driven by KIT or PDGFRα.

However, the proposed initial indication for ripretinib is narrow. GIST is an uncommon disease, and patients who need to go to fourth-line therapy are an even smaller subset of patients. Additionally, statistical significance of the ORR in the pivotal trial was not achieved, leading to a lack of formal analysis of the OS data.

For reference, the WAC price for Ayvakit™ (avapritinib), another recently approved kinase inhibitor for GIST, is approximately $32,000 per 30 days.

- Advantages: no approved drugs for fourth-line GIST therapy, potential future cancer indications (advanced systemic mastocytosis, gliomas, and solid tumors driven by KIT or PDGFRα), oral and once a day
- Disadvantages: narrow initial indication, competing with Ayvakit, ORR was not achieved leading to lack of formal OS data
- Reference WAC (Ayvakit): ~$32,000 per 30 days

Belantamab mafodotin (Brand Name: To be determined)

Manufacturer: GlaxoSmithKline
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 8/14/2020

Therapeutic use

Belantamab mafodotin is in development for the treatment of patients with relapsed or refractory multiple myeloma whose prior therapy included an immunomodulatory agent, a proteasome inhibitor, and an anti-CD38 antibody.

Multiple myeloma is a cancer of the plasma cells (white blood cells that produce antibodies). Multiple myeloma is a relatively uncommon cancer with a lifetime risk of 1 in 132 (0.76%). In the U.S., about 32,270 new cases are estimated in 2020 and about 12,830 deaths are expected to occur.

Almost all patients with multiple myeloma who survive initial treatment will eventually relapse and require further therapy. The 5-year survival rate is approximately 50%.

- Treatment of patients with relapsed or refractory multiple myeloma whose prior therapy included an immunomodulatory agent, a proteasome inhibitor and an anti-CD38 antibody

Clinical profile

Belantamab mafodotin is an antibody-drug conjugate comprised of a humanized anti-B cell maturation antigen (BCMA) monoclonal antibody conjugated to the cytotoxic agent auristatin F. The normal function of BCMA is to promote plasma cell survival and BCMA is expressed at varying levels in myeloma patients.

- Antibody-drug conjugate targeting BCMA
- IV formulation
- ORR (2.5 mg/kg dose): 31%
- Common AEs: keratopathy, thrombocytopenia, anemia
- Dosing: every 3 weeks on day 1 of each cycle until disease progression or unacceptable toxicity

<u>Pivotal trial data:</u>
The efficacy of belantamab mafodotin was evaluated in the DREAMM-2 study, a Phase 2, open-label study in 196 patients with relapsed or refractory multiple myeloma. Patients were randomized to receive 2.5 mg/kg or 3.4 mg/kg belantamab mafodotin via IV infusion. As of June 21, 2019 (the primary analysis data cutoff date), the ORR was 31% (97.5% CI: 20.8, 42.6) in the 2.5 mg/kg cohort and 34% (97.5% CI: 23.9, 46.0) in the 3.4 mg/kg cohort. Currently, GlaxoSmithKline is only pursuing approval for the 2.5 mg/kg dose.

<u>Safety:</u>
The most common adverse events with belantamab mafodotin use were keratopathy (corneal disease), thrombocytopenia, and anemia.

<u>Dosing:</u>
In the pivotal trial, belantamab mafodotin was administered as an IV infusion every 3 weeks on day 1 of each cycle until disease progression or unacceptable toxicity.

Belantamab mafodotin (continued...)

Competitive environment

If approved, belantamab mafodotin would be the first BCMA targeting therapy. Multiple drugs are available for the treatment of multiple myeloma; however, the cancer often relapses or becomes refractory to treatment. Due to its tolerability profile, belantamab mafodotin may become a candidate to be used in combination with other treatment options or to be used in earlier settings in multiple myeloma. Phase 3 studies are currently evaluating belantamab mafodotin as part of a combination regimen in newly diagnosed multiple myeloma patients.

While belantamab mafodotin may have future expanded uses, the initial indication will be narrow and limited to heavily pretreated patients. There is a lack of OS data and the initial FDA filing is based on early stage trial results.

Additional pipeline therapies are in development targeting BCMA, including Bristol-Myers Squibb's CAR-T cell therapy, idecabtagene vicluecel, which could be approved by late 2020 or early 2021. While CAR T cell therapies require a more complex administration procedure and are associated with some serious adverse events, the numerical response rates appear higher compared to belantamab mafodotin.

For reference, the WAC price for Xpovio® (selinexor), another product recently approved for a heavily pretreated multiple myeloma population, is approximately $22,000 per 30 days.

- Advantages: first BCMA targeting therapy, high unmet need, potential for use in earlier lines of therapy for multiple myeloma
- Disadvantages: narrow initial indication, potential future competition with therapies targeting BCMA, lack of late stage data
- Reference WAC (Xpovio): ~$22,000 per 30 days

Satralizumab (Brand Name: To be determined)

Manufacturer: Roche/Genentech/Chugai
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 8/14/2020

Therapeutic use

Satralizumab is in development for the treatment of neuromyelitis optica spectrum disorder (NMOSD).

NMOSD is a rare, lifelong, and debilitating autoimmune disease of the central nervous system that damages the optic nerve and spinal cord, leading to blindness, muscle weakness, and paralysis. Unpredictable and severe relapses can directly cause cumulative, permanent, neurological damage, disability, and sometimes death.

NMOSD affects 15,000 people in the U.S. Approximately two thirds of NMOSD patients have antibodies to aquaporin-4 (AQP4), which are involved with the disease pathogenesis.

- Treatment of NMOSD

Clinical profile

Satralizumab is a humanized monoclonal antibody that targets the interleukin (IL)-6 receptor. IL-6 is thought to play a key role in NMOSD as it triggers the inflammation cascade, which leads to damage and disability.

- IL-6 monoclonal antibody
- SC formulation

Pivotal trial data:
The FDA filing for satralizumab is based on the results from the SAkuraSky and SAkuraStar pivotal studies. SAkuraSky was a Phase 3, randomized, double-blind, placebo-controlled trial of satralizumab added to baseline immunosuppressant treatment. A total of 83 patients were randomly assigned to receive 120 mg of satralizumab or placebo, administered SC at weeks 0, 2, and 4 and every 4 weeks thereafter. Results showed the satralizumab group had a 62% reduction in the risk of relapses vs. placebo in the overall population (95% CI: 0.16, 0.88), and a 79% reduction in the risk of relapses vs. placebo in AQP4-antibody positive patients (95% CI: 0.06, 0.75). However, in AQP4-antibody negative patients, 36% of the treatment group had a protocol-defined relapse vs. 43% with placebo (HR 0.66; 95% CI: 0.20, 2.24). Additionally, there was no significant difference in effect between the groups in the key secondary endpoints of pain and fatigue.

- SAkuraSky (add-on therapy): 62% reduction in risk of relapses vs. placebo
- SAkuraStar (monotherapy): 55% reduction in risk of relapses vs. placebo

SAkuraStar was a Phase 3, randomized, double-blind, placebo-controlled study evaluating monotherapy satralizumab in which 95 patients receive 120 mg of satralizumab or placebo, administered SC at weeks 0, 2, and 4 and every 4 weeks thereafter. Results showed that monotherapy satralizumab achieved a 55% reduction in the risk of relapses vs. placebo (95% CI: 0.23, 0.89). In the AQP4-antibody positive subgroup, 17.1% had a relapse at 48 weeks and 23.5% had a relapse at 96 weeks when treated with satralizumab, vs. 44.6% and 58.9% with placebo, respectively. However, similarly to SakuraSky, the HR for AQP4-antibody negative patients was 1.19 and not statistically significant (95% CI: 0.30, 4.78).

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

- Common AEs: urinary tract infections, upper respiratory tract infections, nasopharyngitis, headache

Dosing:
In the pivotal trials, satralizumab was administered via SC injection at weeks 0, 2, and 4 and every 4 weeks thereafter.

- Dosing: weeks 0, 2, 4, and every 4 weeks thereafter

Satralizumab (continued...)

Competitive environment

If approved, satralizumab would offer a novel treatment approach for NMOSD. Satralizumab will potentially compete with two other drugs, Soliris® (eculizumab) (approved for NMOSD patients that are AQP4-antibody positive and other indications) and inebilizumab (expected to be approved mid-2020). All three products have different mechanisms, dosing frequencies, and routes of administration. In comparison, satralizumab offers a monthly dosing regimen, whereas Soliris is dosed every two weeks and inebilizumab is dosed every six months. Satralizumab is a self-administered SC injection, while the other two products require IV infusions.

However, these three drugs will be competing against each other for a small patient population as NMOSD is an orphan disease affecting 15,000 people in the U.S. The proposed indication for satralizumab is for all NMOSD patients, positive or negative for AQP4-antibody. However, the lack of efficacy in AQP4-antibody negative patients could further limit its use to a smaller patient population. In addition, the secondary endpoints for pain and fatigue in the pivotal trial, SAkuraSky, were also not achieved.

For reference, the WAC price for Soliris is approximately $550,000 per year.

- Advantages: novel treatment approach for NMOSD, monthly dosing, self-administered SC injection vs. IV competitors
- Disadvantages: competing with Soliris and potentially inebilizumab, small eligible patient population, lack of efficacy data for AQP4-antibody negative patients, secondary endpoints were not met for pain and fatigue vs. placebo
- Reference WAC (Soliris): ~$550,000 per year

Tucatinib (Brand Name: Tukysa™)

Manufacturer: Seattle Genetics
Regulatory designations: Orphan Drug, Fast Track, Breakthrough Therapy
Approval date: 4/17/2020 (PDUFA date was originally 8/20/2020)

Therapeutic use

Tucatinib was approved, in combination with Herceptin® (trastuzumab) and capecitabine, for treatment of adult patients with advanced unresectable or metastatic human epidermal growth factor receptor 2 (HER2)-positive breast cancer, including patients with brain metastases, who have received one or more prior anti-HER2-based regimens in the metastatic setting.

Breast cancer is the most common cancer in American women, except for skin cancers. Currently, the average risk of a woman in the U.S. developing breast cancer sometime in her life is about 13%. In 2020, about 276,480 new cases of invasive breast cancer will be diagnosed in women and about 42,170 women will die from breast cancer.

HER2 is a protein found on the surface of some cancer cells that promotes growth and is associated with aggressive disease and poor prognosis. Approximately 15 to 20% of breast cancer cases are HER2-positive.

- In combination with trastuzumab and capecitabine, for treatment of adult patients with advanced unresectable or metastatic HER2-positive breast cancer, including patients with brain metastases, who have received one or more prior anti-HER2-based regimens in the metastatic setting

Clinical profile

Tucatinib is a potent tyrosine kinase inhibitor that is highly selective for HER2 with minimal inhibition of epidermal growth factor receptor (EGFR). Inhibition of EGFR has been associated with significant toxicities, including skin rash and diarrhea.

- Tyrosine kinase inhibitor
- Oral formulation

<u>Pivotal trial data:</u>
The efficacy of tucatinib was evaluated in the HER2CLIMB study, a Phase 2, randomized, double-blind, placebo-controlled study in 612 HER2-positive metastatic breast cancer patients who were previously treated with Herceptin, Perjeta® (pertuzumab), and Kadcyla® (ado-trastuzumab emtansine). Patients received tucatinib or placebo, in combination with Herceptin and capecitabine. The primary endpoint was PFS. Key secondary endpoints included OS and PFS in patients with brain metastases at baseline.

Median PFS was 7.8 months in the Tukysa arm vs. 5.6 months in the control arm (HR 0.54; 95% CI: 0.42, 0.71; $p < 0.00001$). Median OS was 21.9 months in the Tukysa arm vs. 17.4 months in the control arm (HR 0.66; 95% CI: 0.50, 0.87; $p = 0.0048$). For patients with brain metastases, median PFS was 7.6 months in the Tukysa arm vs. 5.4 months in the control arm (HR 0.48; 95% CI: 0.34, 0.69; $p < 0.00001$).

- Median PFS: 7.8 months vs. 5.6 months in the placebo-combination group ($p < 0.00001$)
- Median OS: 21.9 months vs. 17.4 months in the placebo-combination group ($p = 0.0048$)

<u>Safety:</u>
The most common adverse events with tucatinib use were diarrhea, palmar-plantar erythrodysesthesia, nausea, fatigue, hepatotoxicity, vomiting, stomatitis, decreased appetite, abdominal pain, headache, anemia, and rash.

- Common AEs: diarrhea, palmar-plantar erythrodysesthesia, nausea, fatigue, hepatotoxicity, vomiting, stomatitis, decreased appetite, abdominal pain, headache, anemia, rash

<u>Dosing:</u>
Tucatinib is administered orally twice daily.

- Dosing: twice daily

Tucatinib (Brand Name: Tukysa™)

Competitive environment

Tucatinib offers an additional treatment option in the heavily pretreated HER2-positive breast cancer population. The data for tucatinib is promising, particularly in patients with brain metastases, where there is an especially high unmet need. Compared to other tyrosine kinase inhibitor (Nerlynx® [neratinib]), tucatinib's selectivity for HER2 may provide an improved safety profile. Tucatinib is also being evaluated for colorectal cancer.

The initial indication for tucatinib will be narrow and its use will likely be limited to the heavily pretreated population. Tucatinib is currently being evaluated in a Phase 3 trial (HER2CLIMB-02) in combination with Kadcyla in patients with unresectable locally advanced or metastatic HER2-positive breast cancer, who received prior treatment with a taxane and Herceptin.

Drug development in the heavily pretreated HER2-positive breast cancer population is becoming increasingly more competitive. Other currently available tyrosine kinase inhibitors can be used in this niche. Daiichi Sankyo's Enhertu® (fam-trastuzumab deruxtecan-nxki) was approved in December 2019 for a similar indication and margetuximab may be approved later this year.

The WAC price for tucatinib is approximately $18,500 per 30 days.

- Advantages: high unmet need in this niche of breast cancer patients, improved tolerability profile vs. other tyrosine kinase inhibitors, also being evaluated for colorectal cancer, oral administration
- Disadvantages: narrow initial indication, increasingly competitive pipeline for heavily pretreated breast cancer
- WAC: ~$18,500 per 30 days

Margetuximab (Brand Name: To be determined)

Manufacturer: MacroGenics
Regulatory designations: Fast Track
Expected FDA decision: 8/19/2020

Therapeutic use

Margetuximab is in development for the treatment of patients with metastatic human epidermal growth factor receptor 2 (HER2)-positive breast cancer in combination with chemotherapy.

Clinical profile

Margetuximab is a monoclonal antibody that targets the HER2 oncoprotein.

Pivotal trial data:
The efficacy of margetuximab was evaluated in the SOPHIA study, a Phase 3, open-label trial comparing margetuximab plus chemotherapy vs. Herceptin plus chemotherapy in 536 patients with HER2-positive metastatic breast cancer. All patients were previously treated with anti-HER2-targeted therapies. The primary endpoints were PFS and OS. Intent-to-treat PFS analysis occurred after 265 PFS events. The first and second interim OS analyses occurred after 158 and 270 OS events, respectively. Final OS analysis is planned after 385 events and is expected to occur in the second half of 2020.

Median PFS with margetuximab plus chemotherapy was 5.8 months vs. 4.9 months with trastuzumab plus chemotherapy (HR 0.76; 95% CI: 0.59, 0.98; p = 0.033). Among the approximately 85% of patients carrying the CD16A 158F allele, a pre-specified exploratory subpopulation in the study, PFS was 6.9 months vs. 5.1 months, respectively (HR 0.68; 95% CI: 0.52, 0.90; p = 0.005).

At the second interim OS analysis, median OS with margetuximab plus chemotherapy was 21.6 months vs. 19.8 months with trastuzumab plus chemotherapy (HR 0.885; 95% CI: 0.693, 1.130; p = 0.326). Among patients carrying the CD16A 158F allele, the median OS was prolonged by 4.3 months in the margetuximab arm vs. trastuzumab arm (23.7 months vs. 19.4 months; HR 0.793; 95% CI: 0.607, 1.035; p = 0.087).

Safety:
The most common adverse events with margetuximab use were infusion-related reactions, fatigue, diarrhea, and AST increase.

Dosing:
In the pivotal trial, margetuximab was administered via IV infusion every three weeks in combination with one of four chemotherapy agents (capecitabine, eribulin, gemcitabine or vinorelbine) given at the standard dose.

- In combination with chemotherapy, for the treatment of patients with metastatic HER2-positive breast cancer
- Monoclonal antibody targeting HER2
- IV formulation
- Median PFS: 5.8 months with margetuximab plus chemotherapy vs. 4.9 months with trastuzumab plus chemotherapy (p = 0.005)
- Median OS (interim analysis): 21.6 months vs. 19.8 months (failed to achieve statistically significant difference)
- Common AEs: infusion-related reactions, fatigue, diarrhea, AST increase
- Dosing: once every 3 weeks

Margetuximab (continued...)

Competitive environment

Similar to tucatinib, margetuximab would offer an additional treatment option in the heavily pretreated HER2-positive breast cancer population where there is an unmet need. Margetuximab is also being evaluated for gastric cancer which could potentially increase the market potential for the drug.

However, the initial indication for margetuximab will limit its use to patients who have failed multiple HER2 targeted therapies. In the pivotal trial, all patients had previously received Herceptin and Perjeta, and approximately 90% had previously received Kadcyla. While margetuximab did show a marginal improvement in PFS, it did not demonstrate a statistically significant improvement in OS vs. Herceptin at the last interim analysis.

As mentioned above, drug development in the heavily pretreated HER2-positive breast cancer population is also becoming increasingly more competitive and margetuximab will be competing with products such as Enhertu and tyrosine kinase inhibitors such as tucatinib.

For reference, Enhertu has a WAC price of approximately $13,300 per 30 days.

- Advantages: unmet need in this niche of breast cancer patients, also being evaluated for gastric cancer
- Disadvantages: narrow initial indication, marginal benefit vs. Herceptin, competing with recently approved Enhertu in the heavily pretreated population
- Reference WAC (Enhertu): ~$13,300 per 30 days

Filgotinib (Brand Name: To be determined)

Manufacturer: Gilead Sciences
Expected FDA decision: 8/19/2020

Therapeutic use

Filgotinib is in development as a treatment of adults who are living with moderate-to-severe rheumatoid arthritis (RA).

RA is an inflammatory disease that affects the joints and affects 1.3 million people in the U.S.

- Treatment of adults who are living with moderate-to-severe RA

Clinical profile

Filgotinib is a janus kinase (JAK)1-selective inhibitor. JAKs are enzymes that are essential in cell signaling for cytokines, which are involved in the pathogenesis of RA.

- JAK1 inhibitor
- Oral formulation

Pivotal trial data:
The efficacy for filgotinib was evaluated in three pivotal trials. FINCH 1 was a Phase 3 randomized, double-blind, active-controlled study that evaluated filgotinib vs. Humira® (adalimumab) vs. placebo in patients who had inadequate response to methotrexate. The proportion of patients that achieved 20% improvement in RA signs and symptoms (ACR20) at week 12 was 76.6% in the filgotinib 200 mg group vs. 69.8% in the filgotinib 100 mg group vs. 49.9% in the placebo group ($p < 0.001$). Filgotinib also met all key secondary endpoints vs. placebo, including clinical remission and ACR70. Filgotinib was found to be non-inferior to Humira for the various clinical endpoints, including ACR20, ACR50, ACR70, and clinical remission.

- FINCH 1 (MTX-experienced) and FINCH 2 (DMARD-experienced): ACR20 improvement vs. placebo at week 12 ($p < 0.001$)

FINCH 2 was a Phase 3, randomized, double-blind, placebo-controlled trial evaluating filgotinib vs. placebo in patients who did not respond to any biologic disease-modifying anti-rheumatic drugs (DMARDs). At week 12, 66.0% patients receiving filgotinib 200 mg vs. 31.1% patients receiving placebo achieved ACR20 response (difference 34.9; 95% CI: 23.5, 46.3; $p<0.001$). More patients receiving 100 mg (57.5%) also achieved ACR20 response vs. placebo (31.1%) (difference 26.4; 95% CI: 15.0, 37.9; $p<0.001$). Filgotinib also met all key secondary endpoints, including clinical remission and ACR70.

- FINCH 3 (MTX-naïve): ACR20 improvement vs. MTX alone at week 24 ($p < 0.05$)

FINCH 3 was a Phase 3, randomized, double-blind, and active-controlled trial that evaluated filgotinib vs. filgotinib plus methotrexate vs. methotrexate alone in methotrexate-naïve patients. At week 24, 81.0% vs. 80.2% vs. 71.4% achieved ACR20 in the filgotinib 200 mg plus methotrexate group, filgotinib 100 mg plus methotrexate group ($p < 0.05$), and methotrexate monotherapy group, respectively. The proportion of patients achieving ACR50, ACR70, and clinical remission at week 24 was also significantly higher for patients receiving filgotinib plus methotrexate vs. patients receiving methotrexate alone. The proportion of patients that achieved ACR20 in the filgotinib monotherapy group was 78.1%, but statistical significance vs. methotrexate alone was not achieved.

Safety:
The most common adverse events with filgotinib use were nasopharyngitis, headache, and upper respiratory infection. Preclinical tests of filgotinib resulted in reduced production of sperm cells, which led to the FDA's concern of testicular toxicity and prompted the manufacturer to include a dedicated male patient testicular safety study.

- Common AEs: nasopharyngitis, headache, upper respiratory infection

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

- Dosing: once daily

Filgotinib (continued...)

Competitive environment

If approved, filgotinib would be the fourth oral, selective JAK1 inhibitor treatment for rheumatoid arthritis. The selective JAK1 inhibition may confer safety advantages over Xeljanz® (tofacitinib) and Olumiant® (baricitinib), which are non-selective JAK inhibitors. Filgotinib is also in development for Crohn's disease, which could expand its market potential.

Filgotinib would be joining three other JAK inhibitors for RA, and there are also other RA treatment options aside from JAK inhibitors. Filgotinib demonstrated non-inferiority to Humira; however, Rinvoq® (upadacitinib), another recently approved JAK1 inhibitor, has demonstrated superiority against Humira.

Filgotinib appears to have a similar safety profile to other JAK inhibitors and may receive a boxed warning for serious infections, malignancy, and thrombosis. Preclinical studies have also shown that the 200 mg strength may have a potential risk for testicular toxicity, which is unique to filgotinib.

For reference, Rinvoq has a WAC price of approximately $59,000 per year.

- Advantages: additional oral selective JAK1 inhibitor for RA, also in development for Crohn's disease
- Disadvantages: crowded marketplace, lack of superiority data vs. Humira, potential boxed warning for infection, malignancies, and thrombosis, potential testicular toxicity with 200 mg strength
- Reference WAC (Rinvoq): ~$59,000 per year

Valoctocogene roxaparvovec (Brand Name: To be determined)

Manufacturer: BioMarin
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 8/21/2020

Therapeutic use

Valoctocogene roxaparvovec is in development for the treatment of hemophilia A in adult patients.

Hemophilia A is a blood disorder in which patients lack functioning Factor VIII protein to help their blood clot. These patients are at risk for painful and/or potentially life-threatening bleeds and often experience painful, spontaneous bleeds into their muscles or joints.

Hemophilia A affects 1 in 5,000 male births and about 400 babies are born with hemophilia A each year. Approximately 40 to 50% of patients suffer from severe disease.

- Treatment of hemophilia A in adult patients

Valoctocogene roxaparvovec (continued...)

Clinical profile

Valoctocogene roxaparvovec is an adeno-associated virus (AAV)–mediated gene therapy delivering a functional factor VIII gene.

Pivotal trial data:
The efficacy of valoctocogene roxaparvovec was evaluated in a three-year, multi-cohort, Phase 1/2 study in 15 adult patients with severe hemophilia A. Cohort 1 included one patient who received 6 x 1012 vector genomes (vg)/kg of body weight; cohort 2 included one patient who received 2 x 1013 vg/kg; cohort 3 included seven patients, who received 6 x 1013 vg/kg; and cohort 4 included six patients, who received 4 x 1013 vg/kg. Factor VIII level, annualized bleeding event rate (ABR), and the use of factor VIII was evaluated for up to 3 years.

In cohort 3, the seven patients who had received 6 x 1013 vg/kg had the following mean factor VIII activity levels (as measured by chromogenic assay) at the end of years 1, 2, and 3: 64 IU/dL (median, 60 IU/dL), 36 IU/deciliter (median, 26 IU/dL), and 33 IU/dL (median, 20 IU/dL), respectively. The mean ABR decreased by 96%, from a mean (±SD) of 16.3 ± 15.7 events per year (median, 16.5 events per year) at baseline to 0.7 ± 1.6 events per year (median, 0.0 events per year) at the end of year 3. In the year before study entry, the mean annualized number of factor VIII infusions per patient was 136.7 ± 22.4 (median, 138.5); at the end of year 3, the mean annualized use of exogenous factor VIII decreased by 96% to a mean of 5.5 ± 9.4 infusions (median, 0.0 infusions).

In cohort 4, the six patients who received 4 x 1013 vg/kg had a mean factor VIII activity level of 21.0 IU/dL (median, 23 IU/dL) at the end of year 1 and 15 IU/dL (median, 13 IU/dL) at the end of year 2. The ABR decreased by 92%, from a mean of 12.2 ± 15.4 events per year (median, 8.0 events per year) in the year before study entry to a mean of 1.2 ± 2.4 events per year (median, 0.0 events per year) at the end of year 2. In the year before study entry, the mean annualized number of factor VIII infusions per participant was 146.5 ± 41.6 (median, 155.5). At end of year 2, the mean annual use of exogenous factor VIII decreased by 95%, to a mean of 6.8 ± 15.6 infusions (median, 0.5 infusions).

In addition to the Phase 1/2 study, valoctocogene roxaparvovec is currently being evaluated in an ongoing Phase 3, open-label, single-arm study. For the 16 patients who had reached week 26 by an April 30, 2019 cutoff, the estimated mean ABR was 1.5, representing a reduction of 85% from baseline levels. In addition, there was an 84% reduction in median annualized factor VIII usage and a 94% reduction in mean FVIII usage annualized between week 5 and 26. In the 23 to 26 week time period the mean factor VIII level was 36 IU/dL and the median was 33 IU/dL.

Safety:
The most common adverse events with valoctocogene roxaparvovec use were ALT elevation, nausea, headache, fatigue, arthralgia, AST elevation, nasopharyngitis, back pain, and oropharyngeal pain.

Dosing:
Valoctocogene roxaparvovec is administered as a one-time IV infusion.

- Viral gene therapy delivering a functional factor VIII gene
- IV formulation
- Mean ABR: Decreased by 96% at year 3 with 6 x 1013 vg/kg dose and by 92% at year 2 with 4 x 1013 vg/kg dose
- Safety: ALT elevation, nausea, headache, fatigue, arthralgia, AST elevation, nasopharyngitis, back pain, oropharyngeal pain
- Dosing: one-time dose

Valoctocogene roxaparvovec (continued...)

Competitive environment

The current standard of care for patients with severe hemophilia A is chronic prophylactic treatment, typically with Factor VIII replacement products or Hemlibra® (emicizumab-kxwh). Valoctocogene roxaparvovec would offer a one-time gene therapy that could replace the need for chronic prophylactic therapy in some patients. It has demonstrated efficacy for up to 3 years but the true durability of effect remains unclear. BioMarin projects that the efficacy could last as long as 8 years based on factor VIII expression modeling.

However, in addition to likely being reserved for patients with severe disease, the use of valoctocogene roxaparvovec will also be limited to patients without factor inhibitors (~70% of severe patients) and to patients that do not have existing antibodies to the AAV vector (~80% of patients). Patients with pre-existing liver disease will likely be excluded from treatment and all patients who receive the gene therapy must be monitored for liver enzyme increases and liver toxicity.

In addition, there is uncertainty about the long-term benefits with gene therapy since factor VIII activity levels do steadily drop after administration of the gene therapy. The potential benefit for up to 8 years is based on an extrapolation of three-year data from the small Phase 1/2 trial rather than real patient data. The questions about sustained efficacy is especially important with gene therapy because the projected cost for the one-time dose of valoctocogene roxaparvovec is in the range of $2 to $3 million. Valoctocogene roxaparvovec can reduce the need for chronic high cost factor replacement therapies, but the long-term value proposition of gene therapy will be heavily dependent on the durability of response.

- Advantages: one-time dose, potentially eliminate the need for chronic prophylactic therapy in select hemophilia A patients
- Disadvantages: likely reserved for patients with severe hemophilia A, lack of data in patients with factor inhibitors, cannot be used in patients with antibodies to the viral vector, full durability of response not yet known
- Projected WAC: $2 to $3 million for a one-time dose

Veverimer (Brand Name: To be determined)

Manufacturer: Tricida
Expected FDA decision: 8/22/2020

Therapeutic use

Veverimer is in development as a chronic treatment of metabolic acidosis in patients with chronic kidney disease (CKD).

- Chronic treatment of metabolic acidosis in patients with CKD

The kidneys are an essential part of maintaining normal balance of acids and bases in the blood. However, as kidney function declines in CKD, the balance can be disrupted. Metabolic acidosis is a chronic condition that can be caused by CKD and it involves the accumulation of acid in the body. This occurs because a patient's kidney is unable to secrete enough acid or produce enough bicarbonate to balance the body's acid levels. Chronic metabolic acidosis is associated with increased risk of muscle wasting, loss of bone density, CKD progression, and death.

In the U.S., approximately 31 to 34 million patients have CKD, and 15% to 19% of these have metabolic acidosis. There are currently no FDA approved therapies for the chronic treatment of metabolic acidosis.

Clinical profile

Veverimer is a non-absorbed, orally-administered polymer that selectively binds and removes hydrochloric acid from the gastrointestinal lumen.

- Hydrochloric acid binder
- Oral formulation
- Proportion of patients with increased or normal serum bicarbonate: 59% vs. 22% with placebo
- Common AEs: diarrhea, flatulence, nausea, constipation
- Dosing: once daily

<u>Pivotal trial data:</u>
Veverimer was evaluated in a randomized, double-blind, placebo-controlled, Phase 3 study (TRCA-301) in 217 CKD patients with metabolic acidosis. Patients received either veverimer 6 grams per day or placebo as oral suspensions for 12 weeks. The primary endpoint was the difference in the proportion of responders at week 12. Responders were defined as achieving an increase of more than 4 mmol/L from baseline serum bicarbonate or a normal serum bicarbonate range of 22 to 29 mmol/L. The proportion of patients that were found to be responders was 59% in the veverimer group vs. 22% in the placebo group (difference 37; 95% CI: 23, 49).

<u>Safety:</u>
The most common adverse events with veverimer use were diarrhea, flatulence, nausea, and constipation.

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

Veverimer (continued...)

Competitive environment

Veverimer would potentially become the first and only FDA approved therapy with an indication for the chronic treatment of metabolic acidosis in CKD patients. Veverimer is taken orally once daily, which provides an easy dosing regimen for patients with CKD who often have several comorbidities and other pharmacotherapies.

The current standard of care for this condition is reducing dietary intake of acid-producing foods and using exogenous alkali. Examples include sodium bicarbonate or calcium carbonate, which are available as generics and over-the-counter. Veverimer does not require any diet restrictions, which is helpful for patients who have difficulty adhering to the recommended diet due to income or preference. However, some patients who can treat this condition by modifying their diets alone may not need pharmacological therapy.

- Advantages: potentially the first and only approved chronic therapy for metabolic acidosis in CKD patients, oral and once daily, allows for patient's preferred diet
- Disadvantages: generic alternatives available (eg, sodium bicarbonate), standard of care involves lifestyle modifications

2nd Quarter 2020

Extended generic pipeline forecast

OptumRx generic pipeline forecast

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
2020 Possible launch date					
CUVPOSA	glycopyrrolate	Merz	Oral solution	All	2020
PREPOPIK	citric acid/magnesium oxide/sodium picosulfate	Ferring Pharmaceuticals	Oral solution	All	2020
DESONATE	desonide	LEO Pharma	Gel	All	2020
SUPRENZA	phentermine	Citius/Akrimax	Tablet, orally disintegrating	All	2020
VIVLODEX	meloxicam	Iroko/iCeutica	Capsule	All	2020
PRESTALIA	perindopril/amlodipine	Symplmed	Tablet	All	2020
SAMSCA	tolvaptan	Otsuka	Tablet	All	2020
FERRIPROX	deferiprone	ApoPharma/Apotex	Tablet	All	2020
RESTASIS	cyclosporine	Allergan	Ophthalmic	All	2020
OMNARIS	ciclesonide	Covis	Intranasal	All	2020
THALOMID	thalidomide	Celgene	Capsule	All	2020
CIPRODEX	ciprofloxacin/dexamethasone	Alcon	Otic	All	2020
DORYX MPC	doxycycline hyclate	Mayne	Tablet, delayed-release	All	2020
SYNDROS	dronabinol	Insys Therapeutics	Oral solution	All	2020
DUREZOL	difluprednate	Alcon	Ophthalmic	All	2020
BYETTA	exenatide	AstraZeneca	Subcutaneous	All	2020
MOVIPREP	PEG-3350/sodium sulfate/sodium chloride/potassium chloride/sodium ascorbate/ascorbic acid	Salix/Bausch Health	Oral solution	All	2020
ULTRAVATE	halobetasol	Sun	Lotion	All	2020
ADRENALIN	epinephrine	Par/Endo	Intramuscular	All	2020
APTENSIO XR	methylphenidate	Rhodes	Capsule, extended-release	All	1H-2020
OSMOPREP	sodium biphosphate/sodium phosphate	Bausch Health	Tablet	All	1H-2020

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
DEPO-SUBQ PROVERA	medroxyprogesterone	Pfizer	Subcutaneous	All	05-2020
NYMALIZE	nimodipine	Arbor	Oral solution	All	05-2020
LANTUS	insulin glargine	Sanofi	Subcutaneous	All	06-2020
ENTEREG	alvimopan	Merck	Capsule	All	2H-2020
TIROSINT	levothyroxine	IBSA Institut Biochemique	Capsule	All	2H-2020
ENBREL	etanercept	Amgen	Subcutaneous	All	2H-2020
KORLYM	mifepristone	Corcept	Tablet	All	2H-2020
SYNERA	lidocaine/tetracaine	Galen	Transdermal patch	All	07-2020
PEGASYS	peginterferon alfa-2A	Roche	Subcutaneous	All	08-2020
PEG-INTRON	peginterferon alfa-2B	Merck	Subcutaneous	All	08-2020
POMALYST	pomalidomide	Celgene	Capsule	All	08-2020
MARQIBO KIT	vincristine	Talon Therapeutics/Spectrum	Intravenous	All	09-2020
TYKERB	lapatinib	Novartis	Tablet	All	09-2020
BIDIL	isosorbide dinitrate/hydrazaline	Arbor	Tablet	All	09-2020
TRUVADA	emtricitabine/tenofovir	Gilead	Tablet	200 mg/300 mg	09-2020
ATRIPLA	efavirenz/emtricitabine/ tenofovir	Gilead	Tablet	All	09-2020
KUVAN	sapropterin	BioMarin	Tablet; oral solution	All	10-2020
RISPERDAL CONSTA	risperidone	Janssen	Injection, extended-release	All	11-2020
XOLEGEL	ketoconazole	Almirall	Gel	All	11-2020
EPIDUO FORTE	adapalene/benzoyl peroxide	Galderma	Gel	All	12-2020
OFIRMEV	acetaminophen	Mallinckrodt	Intravenous	All	12-2020
ABSORICA	isotretinoin	Sun	Capsule	All	12-2020
TOVIAZ	fesoterodine	Pfizer	Tablet, extended-release	All	12-2020
DALIRESP	roflumilast	AstraZeneca	Tablet	All	12-2020
DEXILANT	dexlansoprazole	Takeda	Capsule, delayed-release	All	12-2020
VELPHORO	sucroferric oxyhydroxide	Fresenius	Tablet, chewable	All	12-2020

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
SAPHRIS	asenapine	Allergan	Tablet, sublingual	All	12-2020
FORTEO	teriparatide	Eli Lilly	Injection	All	12-2020
2021 Possible launch date					
BEPREVE	bepotastine	Bausch Health	Ophthalmic	All	2021
KERYDIN	tavaborole	Pfizer	Topical solution	All	2021
EMTRIVA	emtricitabine	Gilead	Capsule	All	1H-2021
AMITIZA	lubiprostone	Sucampo/Takeda	Capsule	All	01-2021
CRIXIVAN	indinavir	Merck	Capsule	All	02-2021
NORTHERA	droxidopa	H. Lundbeck	Capsule	All	02-2021
MYALEPT	metreleptin	Aegerion	Subcutaneous	All	02-2021
FORTICAL	calcitonin salmon recombinant	Upsher-Smith	Intranasal	All	02-2021
YONSA	abiraterone	Sun	Tablet	All	03-2021
IMPAVIDO	miltefosine	Knight Therapeutics	Capsule	All	03-2021
ACTOPLUS MET XR	pioglitazone/metformin	Takeda	Tablet, extended-release	All	03-2021
OVIDREL	choriogonadotropin	EMD Serono/Merck	Intramuscular; subcutaneous	All	03-2021
NEUPRO	rotigotine	UCB	Transdermal patch	All	03-2021
LYRICA CR	pregabalin	Pfizer	Tablet, extended-release	All	04-2021
ERAXIS	anidulafungin	Pfizer	Intravenous	All	04-2021
ZOMIG	zolmitriptan	Impax/Grunenthal	Intranasal	All	05-2021
PERFOROMIST	formoterol fumarate	Mylan	Inhalation	All	06-2021
APTIOM	eslicarbazepine	Sunovion/Bial	Tablet	All	06-2021
INTELENCE	etravirine	Janssen	Tablet	All	06-2021
FLOVENT HFA	fluticasone propionate	GlaxoSmithKline	Inhalation	All	2H-2021
TECFIDERA	dimethyl fumarate	Biogen	Tablet	All	2H-2021
FERAHEME	ferumoxytol	AMAG Pharmaceuticals	Intravenous	All	07-2021
RESCULA	unoprostone isopropyl	R-Tech Ueno	Ophthalmic	All	07-2021
ALTRENO	tretinoin	Bausch Health	Lotion	All	08-2021
BALCOLTRA	levonorgestrel/ethinyl estradiol/ferrous bisglycinate	Avion	Tablet	All	08-2021
SUTENT	sunitinib	Pfizer	Capsule	All	08-2021

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
SELZENTRY	maraviroc	ViiV Healthcare	Tablet	All	08-2021
JEVTANA KIT	cabazitaxel	Sanofi	Intravenous	All	09-2021
BYSTOLIC	nebivolol	Allergan	Tablet	All	09-2021
PRADAXA	dabigatran etexilate mesylate	Boehringer Ingelheim	Capsule	All	4Q-2021
INNOPRAN XL	propranolol	Ani Pharmaceuticals	Capsule, extended-release	All	10-2021
MIRCERA	methoxy polyethylene glycol-epoetin beta	Roche/Royalty Pharma	Subcutaneous	All	11-2021
BROVANA	arformoterol	Sunovion	Inhalation	All	11-2021
ONEXTON	clindamycin/benzoyl peroxide	Bausch Health	Gel	All	12-2021
EPANED KIT	enalapril	Silvergate	Oral solution	All	12-2021
CHANTIX	varenicline	Pfizer	Tablet	All	12-2021
CAYSTON	aztreonam lysine	Gilead	Inhalation	All	12-2021
BETHKIS	tobramycin	Chiesi	Inhalation	All	12-2021
MYTESI	crofelemer	Napo	Tablet, delayed-release	All	12-2021
EXPAREL	bupivacaine	Pacira	Injection	All	12-2021
SUPREP BOWEL PREP KIT	magnesium sulfate anhydrous/potassium sulfate / sodium sulfate	Braintree	Oral solution	All	12-2021
AFINITOR DISPERZ	everolimus	Novartis	Oral suspension	All	12-2021
2022 Possible launch date					
PREZISTA	darunavir	Janssen	Tablet	75 mg, 150 mg, 300 mg	2022
SOLIRIS	eculizumab	Alexion	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	Tablet	All	01-2022
VIMPAT	lacosamide	UCB	Intravenous; tablet; oral solution	All	03-2022
ZIPSOR	diclofenac potassium	Depomed	Capsule	All	03-2022
CHOLBAM	cholic acid	Retrophin	Capsule	All	03-2022
ABRAXANE	paclitaxel	Celgene/Abraxis	Injection	All	03-2022
REVLIMID	lenalidomide	Celgene	Capsule	All	03-2022

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
ARESTIN	minocycline hydrochloride	Bausch Health	Subgingival, sustained-release	All	03-2022
MAVENCLAD	cladribine	Serono	Tablet	All	03-2022
LEXISCAN	regadenoson	Astellas	Intravenous	All	04-2022
COMBIGAN	brimonidine/timolol	Allergan	Ophthalmic	All	04-2022
TEFLARO	ceftaroline fosamil	Allergan	Intravenous	All	04-2022
ZOLADEX	goserelin	TerSera Therapeutics	Subcutaneous	All	04-2022
DUOBRII	halobetasol propionate/tazarotene	Bausch Health	Lotion	All	04-2022
BANZEL	rufinamide	Eisai	Tablet; oral suspension	All	05-2022
ALIMTA	pemetrexed disodium	Eli Lilly	Intravenous	All	05-2022
VELCADE	bortezomib	Takeda	Intravenous	All	05-2022
TARGINIQ ER	oxycodone/naloxone	Purdue	Tablet, extended-release	All	05-2022
CAPRELSA	vandetanib	Genzyme/Sanofi	Tablet	All	06-2022
VIIBRYD	vilazodone	Forest/Allergan	Tablet	All	06-2022
ELESTRIN	estradiol	Mylan	Gel	All	06-2022
QBRELIS	lisinopril	Silvergate	Oral solution	All	06-2022
BIJUVA	estradiol/progesterone	TherapeuticsMD	Capsule	All	2H-2022
IRESSA	gefitinib	AstraZeneca	Tablet	All	07-2022
EYLEA	aflibercept	Regeneron	Intraocular	All	07-2022
ACTEMRA	tocilizumab	Roche/Chugai	Intravenous; subcutaneous	All	07-2022
EVAMIST	estradiol	Perrigo/Elan	Transdermal solution	All	07-2022
IXEMPRA Kit	ixabepilone	R-Pharm	Intravenous	All	07-2022
VOSEVI	sofosbuvir/velpatasvir/ voxilaprevir	Gilead	Tablet	All	07-2022
VIBATIV	telavancin	Theravance	Intravenous	All	08-2022
SOLOSEC	secnidazole	Symbiomix Therapeutics	Oral granules	All	09-2022
ORAVIG	miconazole	Midatech/R-Pharm	Tablet, buccal	All	09-2022
HALFLYTELY with BISACODYL	bisacodyl / polyethylene glycol 3350, potassium chloride, sodium bicarbonate, sodium chloride	Braintree	Tablet/oral solution	All	10-2022

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
ORENCIA	abatacept	Bristol-Myers Squibb	Intravenous; subcutaneous	All	11-2022
XERESE	acyclovir/hydrocortisone	Bausch Health	Cream	All	11-2022
NAGLAZYME	galsulfase	BioMarin	Intravenous	All	11-2022
FOLOTYN	pralatrexate	Acrotech/Aurobindo	Intravenous	All	11-2022
NASCOBAL	cyanocobalamin	Par/Endo	Intranasal	All	12-2022
MYRBETRIQ	mirabegron	Astellas	Tablet, extended-release	All	12-2022
DYLOJECT	diclofenac	Hospira/Pfizer/Javelin	Intravenous	All	12-2022
RAYOS	prednisone	Horizon	Tablet, delayed-release	All	12-2022
TREANDA	bendamustine	Cephalon/Teva	Intravenous	All	12-2022
ZIOPTAN	tafluprost	Akorn	Ophthalmic	All	12-2022

+ = may launch during the stated date or later

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
2020 Possible launch date									
APL-130277	apomorphine	Sumitomo Dainippon/ Sunovion	non-ergoline dopamine agonist	Parkinson's disease	PO	Filed NDA	5/21/2020	No	No
Amphora	Amphora	Evofem Biosciences	spermicidal agent	Pregnancy prevention	VG	Filed NDA	5/25/2020	No	No
E-58425 (MR-308)	celecoxib/ tramadol	Esteve	non-steroid anti-inflammatory drug/opioid	Acute pain	PO	Filed NDA	5/2020	No	No
naloxone nasal spray	naloxone	Insys Therapeutics/ Hikma Pharmaceuticals	opioid antagonist	Opioid overdose	Intranasal	Filed NDA	5/2020	No	No
nadofaragene firadenovec	nadofaragene firadenovec	Ferring Pharmaceuticals/ Blackstone Life Sciences	gene therapy	Bladder cancer	Intravesical	Filed BLA	5/2020 - 6/2020	Yes	No
FMX-103	minocycline	Foamix	tetracyclines	Rosacea	TOP	Filed NDA	6/2/2020	No	No
EM-100	ketotifen	Eton	antihistamine	Allergic conjunctivitis	OP	Filed NDA	6/11/2020	No	No
Contepo	fosfomycin	Nabriva Therapeutics	cell wall inhibitor	Bacterial infections	IV	Filed NDA	6/19/2020	Yes	No
EVK-001	metoclopramide	Evoke Pharma	antidopiminergics	Diabetic gastroparesis	Intranasal	Filed NDA	6/19/2020	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Fintepla	fenfluramine	Zogenix	serotonin receptor agonist	Dravet syndrome	PO	Filed NDA	6/25/2020	Yes	Yes
HTX-011	bupivacaine/ meloxicam	Heron Therapeutics	anesthetic/ nonsteroidal anti-inflammatory drug	Pain	Instillation	Filed NDA	6/26/2020	No	No
obeticholic acid	obeticholic acid	Intercept Pharmaceuticals	farnesoid X receptor (FXR) agonist	Nonalcoholic steatohepatitis	PO	Filed NDA	6/26/2020	Yes	No
Mycapssa	octreotide	Chiasma	somatostatin analog	Acromegaly	PO	Filed NDA	6/26/2020	Yes	Yes
OMB-157	ofatumumab	Novartis	CD20 monoclonal antibody	Multiple sclerosis	SC	Filed BLA	6/2020	Yes	No
insulin glargine	insulin glargine	Mylan/ Biocon	Long-acting insulin	Diabetes mellitus	SC	Filed BLA	6/2020	No	No
Posidur	SABER-bupivacaine CR	Novartis/ Durect	local anesthetic	Pain	SC	Filed NDA	2Q2020	No	No
AGN-150998	abicipar pegol	Allergan	VEGF-A inhibitor	Wet age-related macular degeneration	Intravitreal	Filed BLA	6/2020 – 7/2020	Yes	No
MEDI-551	Inebilizumab	Viela Bio	CD-19 antagonist	Neuromyelitis optica spectrum disorder	IV	Filed BLA	6/2020 – 7/2020	Yes	Yes
Bronchitol	mannitol	Pharmaxis	osmotic gradient enhancer; mucus clearance enhancer	Cystic fibrosis	INH	Filed NDA	Mid-2020	No	Yes
CNS-7056 (ONO-2745)	Remimazolam	Acacia Pharma	Benzodiazepine	Procedural sedation	IV	Filed NDA	7/5/2020	Yes	No
collagenase clostridium histolyticum	collagenase clostridium histolyticum	Endo	protease enzyme	Cellulite	SC	Filed BLA	7/6/2020	Yes	No
VP-102	VP-102	Verrica	Antiviral	Molluscum	TOP	Filed NDA	7/13/2020	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
RVL-1201	oxymetazoline	Osmotica/ Vertical Pharmaceuticals	alpha-adrenergic receptor agonist	Acquired blepharoptosis (droopy eyelid)	OPH	Filed NDA	7/16/2020	No	No
MC2-01	calcipotriene/ betamethasone	MC2 Therapeutics	vitamin D analog/ corticosteroid	Plaque psoriasis	TOP	Filed NDA	7/20/2020	No	No
JZP-258	sodium oxybate extended-release	Jazz	dopamine receptor agonist	Narcolepsy	PO	Filed NDA	7/21/2020	Yes	Yes
ASTX-727	decitabine and E-7727	Astex Pharmaceuticals	nucleoside metabolic inhibitor	Myelodysplastic syndrome	PO	Filed NDA	7/2020 - 8/2020	Yes	Yes
Corplex donepezil	Donepezil	Corium International	acetylcholinesterase inhibitor	Alzheimer's disease	TOP	Filed NDA	7/30/2020	No	No
UX-007	Triheptanoin	Ultragenyx/ Baylor Research Institute/ Uniquest	medium chain fatty acid	Glucose transport type 1 deficiency syndrome	PO	Filed NDA	7/31/2020	Yes	Yes
BMS-663068	Fostemsavir	Bristol-Myers Squibb	HIV attachment inhibitor	HIV	PO	Filed NDA	8/5/2020	Yes	No
DBV-712 (Viaskin Peanut)	DBV-712	DBV Technologies	Immunotherapy	Peanut allergy	TOP	Filed BLA	8/5/2020	No	No
TRV-130	Oliceridine	Trevena	opioid receptor agonist	Pain	IV	Filed NDA	8/7/2020	Yes	No
Pedmark (STS)	sodium thiosulfate	Fennec	reducing agent	Ototoxicity	IV	Filed NDA	8/10/2020	Yes	Yes
KTE-X19	KTE-X19	Gilead	chimeric antigen receptor (CAR) T cell therapy	Mantle cell lymphoma	IV	Filed BLA	8/10/2020	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
DCC-2618	ripretinib	Deciphera	PDGFR-alpha kinase inhibitor	Gastrointestinal stromal tumors	PO	Filed NDA	8/13/2020	Yes	Yes
SA-237 (RG-6168)	satralizumab	Roche	interleukin-6 (IL-6) monoclonal antibody	Neuromyelitis optica	SC	Filed BLA	8/15/2020	Yes	Yes
Zepsyre	lurbinectidin (lurbinectedin)	PharmaMar/ Jazz Pharmaceuticals	alkylating agent	Small cell lung cancer	IV	Filed NDA	8/16/2020	Yes	Yes
GSK-2857916	belantamab mafodotin	GlaxoSmithKline/ Seattle Genetics	anti-BCMA antibody-drug conjugate	Multiple myeloma	SC	Filed BLA	8/16/2020	Yes	Yes
GLPG-0634	filgotinib	Gilead/ Galapagos	janus associated kinase-1 (JAK) inhibitor	Rheumatoid arthritis	PO	Filed NDA	8/18/2020	Yes	No
MAGH-22	margetuximab	MacroGenics	HER2 oncoprotein antagonist	Breast cancer	IV	Filed BLA	8/19/2020	Yes	No
BMN-270	valoctocogene roxaparvovec	BioMarin	gene therapy	Hemophilia A	IV	Filed BLA	8/21/2020	Yes	Yes
TRC-101	veverimer	Tricida	carrier protein modulator	Chronic kidney disease	PO	Filed NDA	8/22/2020	Yes	No
RG-7916 (RO-7034067)	risdiplam	Roche/ PTC Therapeutics	SMN2 splicing modifier	Spinal muscular atrophy	PO	Filed NDA	8/24/2020	Yes	Yes
XaraColl	bupivacaine implant	Innocoll	sodium channel blocker	Pain	implant	Filed NDA	8/26/2020	Yes	No
Winlevi	clascoterone	Cassiopea	androgen antagonist	Acne vulgaris	TOP	Filed NDA	8/27/2020	No	No
Tlando	testosterone	Lipocine	Androgen	Hypogonadism	PO	Filed NDA	8/28/2020	No	No
MOR-208	tafasitamab	MorphoSys/ Xencor	CD-19 antagonist	Diffuse large B-cell lymphoma	IV	Filed BLA	8/30/2020	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
CC-486	azacitidine	Bristol Myers Squibb/ Celgene	DNA methylation inhibitor	Acute myeloid leukemia	PO	Filed NDA	9/3/2020	Yes	Yes
Lucassin	terlipressin	Mallinckrodt	V-1 (vasopressin) agonist	Hepato-renal syndrome	IV	Filed BLA	9/12/2020	Yes	Yes
NNC-0195-0092 (NN-8640)	somapacitan	Novo Nordisk	recombinant human growth hormone (rhGH)	Growth hormone deficiency	SC	Filed BLA	9/21/2020	Yes	No
LJPC-0118	artesunate	La Jolla Pharmaceutical	Protozoacide	Malaria	Undisclosed	Filed NDA	9/25/2020	No	Yes
Libervant	diazepam	Aquestive Therapeutics	Benzodiazepine	Seizures	PO	Filed NDA	9/27/2020	No	Yes
Prochymal	remestemcel-L	Mesoblast	mesenchymal stem cells	Graft vs. Host disease	IV	Filed BLA	9/30/2020	Yes	Yes
Infacort	hydrocortisone	Diurnal Group	Corticosteroid	Adrenal insufficiency	PO	Filed NDA	9/29/2020	No	Yes
NS-065 (NCNP-01)	viltolarsen	Nippon Shinyaku	morpholino antisense oligonucleotide	Duchenne muscular dystrophy	IV	Filed BLA	3Q2020	Yes	Yes
LY-900014 (URLi)	insulin lispro	Eli Lilly	Insulins	Diabetes mellitus	SC	Filed BLA	3Q2020	No	No
tramadol	tramadol	Avenue Therapeutics	opioid receptor agonist	Pain	IV	Filed NDA	10/9/2020	No	No
RG-6264	trastuzumab/ pertuzumab	Roche	HER2/neu receptor antagonist	Breast cancer	SC	Filed BLA	10/18/2020	Yes	No
Qtrypta	zolmitriptan	Zosano	Triptans	Acute migraines	TOP	Filed NDA	10/20/2020	No	No
SPI-2012	eflapegrastim	Spectrum/ Hanmi	granulocyte colony-stimulating factor (GCSF)	Neutropenia	SC	Filed BLA	10/24/2020	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
REGN-EB3	REGN-EB3	Regeneron	anti-Ebola virus	Ebola	IV	Filed BLA	10/25/2020	Yes	Yes
KPI-121 0.25%	loteprednol etabonate	Kala Pharmaceuticals	corticosteroid	Dry eyes	OP	Filed NDA	11/4/2020	No	No
SPN-812	viloxazine	Supernus	selective norepinephrine reuptake inhibitor	Attention deficit hyperactivity disorder	PO	Filed NDA	11/8/2020	No	No
ALKS-3831	olanzapine/ samidorphan	Alkermes	dopamine receptor antagonist/ opioid receptor antagonist	Schizophrenia/ Bipolar disorder	PO	Filed NDA	11/15/2020	No	No
JCAR-017	lisocabtagene maraleucel	Bristol-Myers Squibb/ Celgene	chimeric antigen receptor (CAR) T cell therapy	Diffuse large B-cell lymphoma	IV	Filed BLA	11/16/2020	Yes	Yes
EBP-994 (rEBP-994)	lonafarnib	Eiger Biopharmaceuticals	prenylation inhibitor	Hutchinson-Gilford Progeria Syndrome (HGPS or progeria) and progeroid laminopathies	PO	Filed NDA	11/23/2020	Yes	Yes
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension	INH	Filed NDA	11/24/2020	Yes	No
RT-002 (Daxi)	daxibotulinumtoxinA	Revance Therapeutics	botulinum toxins	Glabellar lines (frown lines)	IM	Filed BLA	11/25/2020	Yes	No
bb-2121	idecabtagene vicluecel	Bristol-Myers Squibb/ bluebird Bio	chimeric antigen receptor (CAR) T cell therapy	Multiple myeloma	IV	Filed BLA	11/30/2020	Yes	Yes
BIM-22493 (RM-493)	setmelanotide	Rhythm Pharmaceuticals	melanocortin 4 receptor (MC4R) agonist	Rare genetic disorders of obesity	SC	Filed NDA	11/30/2020	Yes	Yes
3-F8 (Hu-3F8)	naxitamab	Y-mAbs Therapeutics	GD2 antagonist	Neuroblastoma	IV	Filed BLA	11/30/2020	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
nifurtimox	nifurtimox	Bayer	anti-parasitic, anti-protozoal	Chagas disease	PO	Filed NDA	11/30/2020	No	Yes
BLU-667	pralsetinib	Blueprint Medicines	RET inhibitor	Non-small cell lung cancer	PO	Filed NDA	12/1/2020	Yes	Yes
BCX-7353	berotralstat	BioCryst	kallikrein inhibitor	Hereditary angioedema	PO	Filed NDA	12/3/2020	Yes	Yes
ALNG-01 (ALN-G-01)	lumasiran	Alnylam	glycolate oxidase antagonist	Hyperoxaluria	Intranasal	Filed NDA	12/7/2020	Yes	Yes
FG-4592 (ASP-1517)	roxadustat	FibroGen/ AstraZeneca	hypoxia-inducible factor prolyl hydroxylase (HIF-PHI)	Anemia	PO	Filed NDA	12/23/2020	Yes	No
MK-4618 (KRP-114V, RVT-901)	vibegron	Urovant Sciences	selective beta 3 adrenergic receptor agonist	Overactive bladder	PO	Filed NDA	12/26/2020	No	No
LY-03005	ansofaxine	Luye Pharma	serotonin-norepinephrine-dopamine triple reuptake inhibitor	Major depressive disorder	PO	Filed NDA	12/26/2020	No	No
KX-01 (KX2-391)	tirbanibulin	Athenex	Src kinase and tubulin inhibitor	Actinic keratosis	TOP	Filed NDA	12/30/2020	No	No
ALN-PCSsc (PCSK9si)	inclisiran	The Medicines Company/ Novartis	proprotein convertase subtilisin/kexin 9 (PCSK-9) inhibitor	Hyperlipidemia	SC	Filed NDA	12/2020	Yes	Yes
tanezumab	tanezumab	Pfizer/ Eli Lilly	Nerve growth factor inhibitor	Osteoarthritis	SC	Filed BLA	12/2020	Yes	No
131I-8H9	omburtamab	Y-mAbs Therapeutics	B7-H3 antagonist	Brain cancer	Undisclosed	InTrial	4Q2020	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
TGR-1202	umbralisib	TG Therapeutics	phosphoinositide-3 kinase (PI3K) delta inhibitor	Marginal zone lymphoma/ follicular lymphoma	PO	InTrial	4Q2020	Yes	Yes
BIVV-009 (TNT-009)	sutimlimab	Sanofi	complement C1s subcomponent inhibitor	Cold agglutinin disease	IV	Filed BLA	4Q2020	Yes	Yes
GSP-301	mometasone furoate/ olopatadine HCl	Glenmark	corticosteroid/ antihistamine	Allergic rhinitis	Intranasal	CRL	2H2020	No	No
S-265744 (S/GSK-1265744)	cabotegravir	ViiV Healthcare	HIV integrase inhibitor	HIV	PO	CRL	2H2020	Yes	No
ET-105	lamotrigine	Eton	Anticonvulsant	Epilepsy	PO	CRL	2H2020	No	No
Remune	AG-1661	Immune Response BioPharma	Vaccine	HIV	IM	CRL	Late 2020	Yes	Yes
OMS-721	narsoplimab	Omeros	anti-MASP-2 monoclonal antibody	Hematopoietic stem cell transplant-associated thrombotic microangiopathy	IV/SC	InTrial	Late 2020	Yes	Yes
PRX-102	pegunigalsidase alfa	Protalix	enzyme replacement	Fabry disease	IV	InTrial	Late 2020	Yes	No
TMC-278-LA	cabotegravi / rilpivirine	ViiV Healthcare	HIV integrase inhibitor/ non-nucleoside reverse transcriptase inhibitor	HIV	IM	CRL	Late 2020	Yes	No
Entyvio (SC formulation)	vedolizumab	Takeda	integrin receptor antagonist	Ulcerative colitis/ Crohn's disease	SC	CRL	Late 2020	Yes	No
CAM-2038	buprenorphine	Camurus/ Braeburn	opioid receptor agonist (partial)	Opioid use disorder/ Pain	SC	Tentative Approval	Late 2020	Yes	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	InTrial	Late 2020	No	No
ALXN-1101	fosdenopterin	BridgeBio Pharma/ Origin Biosciences	molybdenum cofactor stimulant	Molybdenum cofactor deficiency	IV	InTrial	Late 2020	Yes	Yes
Zimhi	naloxone	Adamis	opioid antagonist	Opioid overdose	IM	CRL	Late 2020	No	No
BGF-MDI (PT-010)	budesonide/ glycopyrronium/ formoterol	AstraZeneca	corticosteroid/ long-acting muscarinic receptor antagonist (LAMA)/ long-acting beta 2 adrenergic receptor agonist (LABA)	Chronic obstructive pulmonary disease	INH	CRL	Late 2020	No	No
Ryplazim	human plasminogen	ProMetic/ Hematech	Plasminogen	Plasminogen deficiency	IV	CRL	Late 2020	Yes	Yes
2021 Possible launch date									
TSR-042	dostarlimab	GlaxoSmithKline	PD-1 checkpoint inhibitor	Endometrial cancer	IV	Filed BLA	1/14/2021	Yes	No
KP-415	D-threo-methylphenidate controlled-release	KemPharm	CNS stimulant	Attention deficit hyperactivity disorder	PO	Filed NDA	3/2/2021	No	No
RG-3477 (ACT-128800)	ponesimod	Johnson & Johnson	sphingosine 1 phosphate receptor agonist	Multiple sclerosis	PO	Filed NDA	3/18/2021	Yes	No
ZP-4207 (ZP-GA-1)	dasiglucagon	Zealand Pharma	glucagon analog	Diabetes mellitus	SC	Filed NDA	3/31/2021	No	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Tivopath	tivozanib	AVEO Oncology	VEGF inhibitor	Renal cell cancer	PO	Filed NDA	3/31/2021	Yes	No
Furoscix	furosemide	scPharmaceuticals	Diuretic	Heart failure	SC	CRL	1Q2021	Yes	No
AGIL-AADC	AGIL-AADC	PTC Therapeutics	gene therapy	Aromatic L-amino acid decarboxylase deficiency	Intracerebral	InTrial	1Q2021	Yes	Yes
PRO-140	leronlimab	CytoDyn	C-C chemokine receptor 5 (CCR5) antagonist	HIV; Graft vs. host disease	SC	InTrial	1Q2020	Yes	Yes
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	CRL	1Q2021	Yes	Yes
Estelle	estetrol/ drospirenone	Mayne Pharma/ Mithra Pharmaceuticals	estrogen receptor agonist	Pregnancy prevention	PO	Filed NDA	4/16/2021	No	No
TAK-385	relugolix	Myovant Sciences	gonadotropin-releasing hormone (GnRH) receptor antagonist	Prostate cancer/ uterine fibroids	PO	Filed NDA	4/21/2021	Yes	No
Melflufen (Ygalo)	melphalan-flufenamide	Oncopeptides AB	alkylating agent/ DNA synthesis inhibitor	Multiple myeloma	IV	InTrial	2Q2021	No	Yes
S5G4T-1 (DER-45-EV)	benzoyl peroxide	Sol-Gel Technologies	benzoyl peroxide	Rosacea	TOP	InTrial	2Q2021	No	No
ACP-001	TransCon Growth Hormone	Ascendis	growth hormone prodrug	Short stature/ Growth hormone deficiency	SC	InTrial	2Q2021	Yes	No
Vicinium (VB-4-845)	oportuzumab monatox	Sesen Bio	anti-ECAM exotoxin A fusion protein	Bladder cancer	Intravesical	InTrial	1H2021	Yes	No
ABT-888	veliparib	AbbVie	PARP inhibitor	Ovarian cancer; breast cancer	PO	InTrial	1H2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ISIS-304801	volanesorsen	Ionis	antisense drug	Familial chylomicronemia syndrome	SC	CRL	1H2021	Yes	Yes
Luveniq	voclosporin	Aurinia Pharmaceuticals	calcineurin inhibitor	Lupus nephritis	PO	InTrial	1H2021	Yes	No
Humacyl	human acellular vessel	Humacyte	cellular therapy	End-stage renal disease	Implant	InTrial	1H2021	Yes	No
arimoclomol	arimoclomol	Orphazyme	cytoprotectives	Niemann-Pick Disease	PO	InTrial	1H2021	Yes	Yes
Apealea (Paclical)	paclitaxel	Oasmia	taxane	Ovarian cancer	IV	InTrial	1H2021	Yes	Yes
Oraxol	HM-30181A/ paclitaxel	Athenex	P-glycoprotein pump inhibitor/ taxane	Breast cancer	PO	InTrial	1H2021	Yes	No
ET-103	levothyroxine	Eton Pharmaceuticals	L-thyroxine	Hypothyroidism	PO	InTrial	1H2021	No	No
SRP-4045	casimersen	Sarepta	morpholino antisense oligonucleotide	Duchenne muscular dystrophy	IV	InTrial	1H2021	Yes	Yes
ropeginterferon alfa-2b	ropeginterferon alfa-2b	PharmaEssentia/ AOP Orphan	interferon	Polycythemia vera	SC	InTrial	1H2021	Yes	Yes
NX-1207 (NYM-4805, REC 0482)	fexapotide triflutate	Nymox	pro-apoptotic	Benign prostatic hyperplasia	Intratumoral	InTrial	1H2021	Yes	No
StrataGraft Skin Tissue	StrataGraft Skin Tissue	Mallinckrodt	autologous skin tissue	Burn injury	TOP	InTrial	1H2021	Yes	Yes
sulopenem	sulopenem	Iterum Therapeutics	carbapenem	Bacterial infection	IV/PO	InTrial	1H2021	No	No
PL-56	budesonide	Calliditas/ Kyowa Hakko Kirin	corticosteroid	Nephropathy	PO	InTrial	1H2021	No	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
REGN-1500	evinacumab	Regeneron	angiopoietin-like 3 (ANGPTL3) antagonist	Homozygous familial hypercholesterolemia	IV/SC	InTrial	1H2021	Yes	No
TG-1303	ublituximab/ TGR-1202	TG Therapeutics	CD-20 monoclonal antibody/ phosphoinositide-3 kinase (PI3K) delta inhibitor	Chronic lymphocytic leukemia/ Non-Hodgkin lymphoma	IV/PO	InTrial	1H2021	Yes	Yes
SCY-078 (MK-3118)	ibrexafungerp	Scynexis	glucan synthase inhibitors	Fungal infections	IV/PO	InTrial	Mid-2021	No	Yes
pIL-12 (DNA IL-12)	tavokinogene telsaplasmid	OncoSec Medical	gene therapy	Melanoma	Intratumoral	InTrial	Mid-2021	Yes	Yes
NexoBrid	bromelain	Vericel/ MediWound	peptide hydrolase replacement agent	Burns/ Skin injury	TOP	InTrial	Mid-2021	No	Yes
CLS-1001	triamcinolone acetonide	Clearside	corticosteroid	Macular edema	intraocular/ subretinal	CRL	Mid-2021	Yes	No
ZYN-002	ZYN-002	Zynerba	cannabinoid product	Fragile X syndrome	TOP	InTrial	Mid-2021	Yes	Yes
TadFin	tadalafil and finasteride	Veru	phosphodiesterase type 5 inhibitor /5-alpha-reductase inhibitor	Benign prostatic hyperplasia	PO	InTrial	Mid-2021	No	No
BGJ-398	infigratinib	BridgeBio	FGFR inhibitor	Biliary tract cancer	PO	InTrial	Mid-2021	Yes	Yes
CCX-168	avacopan	ChemoCentryx/ Galencia	C5a receptor (C5aR) antagonist	Vasculitis	PO	InTrial	Mid-2021	Yes	Yes
UCB-4940 (CDP-4940)	bimekizumab	UCB	interleukin-17 (IL-17) receptor inhibitor	Plaque psoriasis	IV	InTrial	Mid-2021	Yes	No
Estybon	rigosertib (ON 01910.Na)	Onconova	non-ATP competitive kinase inhibitor	Myelodysplastic syndrome	IV	InTrial	Mid-2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
CMX-001	brincidofovir	Chimerix	DNA-directed DNA polymerase inhibitor	Smallpox	PO	InTrial	Mid-2021	No	Yes
Trevyent	treprostinil	United Therapeutics	prostacyclin analog	Pulmonary arterial hypertension	SC	CRL	Mid-2021	Yes	Yes
ADCT-402	loncastuximab tesirine	ADC Therapeutics	antibody drug conjugate	Diffuse large B-cell lymphoma	IV	InTrial	Mid-2021	Yes	Yes
AGEN-2034	balstilimab	Agenus	PD-1 antagonist	Cervical cancer	IV	InTrial	Mid-2021	Yes	No
EMD-1214063	tepotinib	Merck	c-Met receptor tyrosine kinase inhibitor	Non-small cell lung cancer	PO	InTrial	Mid-2021	Yes	No
RG-7388 (RO-5503781)	idasanutlin	Roche	MDM2 antagonist	Acute myelogenous leukemia	PO	InTrial	Mid-2021	Yes	No
GZ-402666 (NeoGAA)	avalglucosidase alfa	Sanofi	enzyme therapy	Pompe disease	IV	InTrial	Mid-2021	Yes	No
CUTX-101	copper histidinate	Fortress Biotech	copper replacement	Menkes Disease	SC	InTrial	Mid-2021	Yes	Yes
Recentin	cediranib	AstraZeneca	vascular endothelial growth factor receptor (VEGF) antagonists	Ovarian cancer	PO	InTrial	Mid-2021	Yes	Yes
AGEN-1884	zalifrelimab	Agenus	immune checkpoint modulator (CPM) antibody	Cervical cancer	IV	InTrial	Mid-2021	Yes	No
CR-845	difelikefalin	Cara Therapeutics	opioid receptor agonist	Pruritus	IV/PO	InTrial	Mid-2021	No	No
RSV-F (ResVax)	respiratory syncytial virus vaccine	Novavax	vaccine	Respiratory syncytial virus infection	IM	InTrial	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
AT-GAA	recombinant human acid alpha-glucosidase + AT2220	Amicus	enzyme therapy	Pompe disease	IV	InTrial	Mid-2021	Yes	Yes
EBV-CTL (ATA-129)	tabelecleucel	Atara Biotherapeutics/ Memorial Sloan-Kettering Cancer Center	cell therapy	Lymphoproliferative disorder	IV	InTrial	Mid-2021	Yes	Yes
entinostat	entinostat	Syndax	histone deacetylase (HDAC) inhibitor	Breast cancer	PO	InTrial	Mid-2021	Yes	No
AT-132 (AAV8-MTM1)	AT-132 (AAV8-MTM1)	Audentes Therapeutics	gene therapy	X-linked myotubular myopathy	IV	InTrial	Mid-2021	Yes	Yes
Zynquista	sotagliflozin	Sanofi/ Lexicon	sodium-dependent glucose transporter 1 (SGLT-1) and SGLT-2 inhibitor	Diabetes mellitus	PO	CRL	Mid-2021	No	No
BIIB-037	aducanumab	Biogen	amyloid beta-protein inhibitor	Alzheimer's disease	IV	InTrial	3Q2021	Yes	No
ET-101	ET-101	Eton	undisclosed	Seizure disorders	PO	InTrial	3Q2021	No	No
BMN-111	vosoritide (vasoritide)	BioMarin/ Chugai	C-type natriuretic peptide (CNP) analog	Achondroplasia	SC	InTrial	3Q2021	Yes	Yes
LN-144	lifileucel	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Melanoma	IV	InTrial	3Q2021	Yes	Yes
APR-246	APR-246	Aprea Therapeutics	p53 tumor suppressor protein stimulator	Myelodysplastic syndrome	IV	InTrial	3Q2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
NiCord	omidubicel	Gamida	cellular therapy	Hematological cancers	IV	InTrial	3Q2021	Yes	Yes
PF-06482077	multivalent group B streptococcus vaccine	Pfizer	vaccine	Bacterial infection	IM	InTrial	4Q2021	Yes	No
OS-01 nasal spray	OC-01	Oyster Point Pharma	nicotinic acetylcholine receptor (nAChR) agonist	Dry eye disease	Intranasal	InTrial	4Q2021	No	No
AXS-07	meloxicam/ rizatriptan	Axsome Therapeutics	non-steroidal anti-inflammatory drug/triptan	Migraine	PO	InTrial	4Q2021	No	No
VBP-15	vamorolone	Santhera	corticosteroid	Duchenne muscular dystrophy	PO	InTrial	4Q2021	Yes	Yes
AXS-05	dextromethorphan/ bupropion	Axsome	N-methyl-D-aspartate (NMDA) antagonist/ antidepressant	Treatment-resistant depression	PO	InTrial	4Q2021	No	No
MOD-401	somatrogon	OPKO Health/ Pfizer	enzyme replacement	Growth hormone deficiency	SC	InTrial	2H2021	Yes	Yes
PF-04965842	abrocitinib	Pfizer	janus kinase 1 (JAK-1) inhibitor	Atopic dermatitis	PO	InTrial	4Q2021	Yes	No
INC-424	ruxolitinib	Incyte	janus kinase (JAK) inhibitor	Atopic dermatitis	TOP	InTrial	4Q2021	Yes	No
KD-025	KD-025	Kadmon	ROCK2 (Rho-associated coiled-coiled kinase 2) inhibitor	Graft vs. Host disease	PO	InTrial	4Q2021	No	Yes
PRV-031	teplizumab	Provention Bio/ MacroGenics	CD3 antigen inhibitor	Diabetes mellitus	IV	InTrial	4Q2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
OPNT-003	nalmefene	Opiant	opioid receptor antagonist	Opioid overdose	Intranasal	InTrial	4Q2021	No	No
JZP-458 (PF-743)	recombinant crisantaspase	Jazz Pharmaceutics/ Pfenex	asparaginase	Acute lymphoblastic leukemia	IM/IV	InTrial	2H2021	Yes	No
LCAR-B38M	LCAR-B38M	Janssen	chimeric antigen receptor (CAR) T cell therapy	Multiple myeloma	Undisclosed	InTrial	2H2021	Yes	Yes
ublituximab (LFB-R603, TG20, TGTX-1101, TG-1101, Utuxin)	ublituximab	TG Therapeutics	CD-20 monoclonal antibody	Chronic lymphocytic leukemia/ multiple sclerosis	IV	InTrial	2H2021	Yes	Yes
MEDI-546	anifrolumab	AstraZeneca/ BMS	interferon receptor antagonist	Systemic lupus erythematosus	IV	InTrial	2H2021	Yes	No
SGX-301	synthetic hypericin	Access Pharmaceuticals	synthetic hypericin	Cutaneous T-cell lymphoma	TOP	InTrial	2H 2021	Yes	Yes
PDR-001	spartalizumab	Novartis	PD-1 checkpoint inhibitor	Melanoma	IV	InTrial	2H2021	Yes	No
DS-100	DS-100	Eton	undisclosed	Ophthalmological disease	SC	InTrial	2H2021	No	No
AT-007	AT-007	Applied Therapeutics	aldose reductase inhibitor	Galactosemia	undisclosed	InTrial	2H2021	Yes	Yes
Sci-B-Vac	hepatitis B vaccine	VBI Vaccines	vaccine	Hepatitis B	IM	InTrial	2H2021	No	No
SHP-620	maribavir	Shire	benzimidazole	Cytomegalovirus	PO	InTrial	2H2021	No	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
IDP-120	tretinoin/ benzoyl peroxide	Bausch	retinoid	Acne	TOP	InTrial	2H2021	No	No
AKB-6548	vadadustat	Akebia Therapeutics/ Vifor Pharma	hypoxia-inducible factor-prolyl hydroxylase (HIF-PH) inhibitor	Anemia	PO	InTrial	2H2021	Yes	No
RG-7440 (GDC-0068)	ipatasertib	Roche	pan-Akt inhibitor	Prostate cancer; breast cancer	PO	InTrial	2H2021	Yes	No
Iomab-B	iodine I 131 monoclonal antibody BC8	Actinium	anti-CD45 monoclonal antibody	Acute myeloid leukemia/ Myelodysplastic syndrome	IV	InTrial	2H2021	Yes	Yes
TWIN (S6G5T-1; S6G5T-3)	benzoyl peroxide/ tretinoin	Sol-Gel Technologies	retinoid	Acne vulgaris	TOP	InTrial	2H2021	No	No
REGN-2477	garetosmab	Regeneron	Activin A antibody	Fibrodysplasia ossificans progressiva	IV/SC	InTrial	2H2021	Yes	Yes
LN-145	LN-145	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Cervical Cancer	IV	InTrial	2H2021	Yes	No
MLN-4924 (TAK-92)	pevonedistat	Ligand	Nedd 8 Activating Enzyme (NAE) antagonist	Myelodysplastic syndrome	IV	InTrial	2H2021	Yes	No
paliperidone palmitate	paliperidone palmitate	Johnson & Johnson	atypical antipsychotic	Schizophrenia	IM	InTrial	2H2021	Yes	No
S-110 (SGI-110)	guadecitabine	Otsuka	DNA methyltransferase inhibitor	Myelodysplastic syndrome	SC	InTrial	2H2021	Yes	No
BBI-608	napabucasin	Sumitomo Dainippon	stem cell inhibitor	Colorectal cancer	PO	InTrial	2H2021	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 (NK-1R) antagonist	Motion sickness	PO	InTrial	2H2021	No	No
CAT-354	tralokinumab	Leo Pharma	interleukin-13 (IL-13) inhibitor	Atopic dermatitis	SC	InTrial	2H2021	Yes	No
INP-104	POD-dihydroergotamine mesylate (POD-DHE)	Impel/ 3M	ergot derivative	Acute migraines	Intranasal	InTrial	2H2021	No	No
ARGX-113	efgartigimod	Argen NV	Fc antagonist	Myasthenia gravis	IV/SC	InTrial	2H2021	Yes	Yes
177Lu-PSMA-617	Lutetium	Novartis	Radiopharmaceutical	Prostate cancer	IV	InTrial	2H2021	Yes	No
MTP-131 (SS-31)	elamipretide	Stealth Biotherapeutics	mitochondrial permeability transition pore inhibitor	Barth syndrome	IV/PO/SC	InTrial	2H2021	Yes	Yes
dovitinib	dovitinib	Oncology Venture	fibroblast growth factor receptor 3 (FGFR3) inhibitor	Renal cell carcinoma	PO	InTrial	2H2021	Yes	No
Taclantis	paclitaxel injection concentrate for suspension	Sun Pharma Advanced Research Company (SPARC)	taxane	Breast cancer; lung cancer; pancreatic cancer	IV	CRL	2H2021	No	No
IDP-124	pimecrolimus	Bausch Health	calcineurin Inhibitor	Atopic dermatitis	TOP	InTrial	2H2021	No	No
PRO-145223	etrolizumab	Genentech	IgG1 monoclonal antibody	Ulcerative colitis	SC	InTrial	2H2021	Yes	Yes
BXCL-501	dexmedetomidine	BioXcel Therapeutics	selective alpha 2a receptor agonist	Schizophrenia and bipolar disorder	PO	InTrial	2H2021	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
AmnioFix	dehydrated human amnion/chorion membrane (dHACM)	MiMedx	amniotic tissue membrane	Plantar fasciitis/ achilles tendonitis	INJ	InTrial	2H2021	Yes	No
R-667 (RG-667)	palovarotene	Clementia/ Roche	selective retinoic acid receptor agonist (RAR-gamma)	Fibrodysplasia ossificans progressiva (FOP)	PO	InTrial	2H2021	Yes	Yes
NPI-2358	plinabulin	BeyondSpring	tumor vascular disrupting agent (tVDA)	Neutropenia/ non-small cell lung cancer	IV	InTrial	2H2021	Yes	No
SYD-985	[vic-] trastuzumab duocarmazine	Synthon	HER2-targeting antibody-drug conjugate	Breast cancer	IV	InTrial	2H2021	Yes	No
RVT-802	RVT-802	Enzyvant/Roivant	Tissue-based therapy	Congenital athymia	Implant	CRL	2021	Yes	Yes
Purified Cortrophin Gel	corticotropin	ANI Pharmaceuticals	adrenocorticotropic hormone (ACTH)	Multiple sclerosis/ rheumatoid arthritis/ systemic lupus erythematosus/ ulcerative colitis	IV	InTrial	2021	Yes	No
RTA-408	omaveloxolone	Reata Pharmaceuticals	Nrf2 activator	Friedreich's ataxia	PO	InTrial	2021	Yes	Yes
ATI-1501	metronidazole	Appili Therapeutics	nitroimidazole	Fungal infections, anaerobic bacterial infections	PO	InTrial	Late 2021	No	No
SHP-625 (LUM-001)	maralixibat	Mirum Pharmaceuticals	apical sodium-dependent bile acid transporter (ABST) inhibitor	Alagille syndrome	PO	InTrial	Late 2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
COR-003	levoketoconazole	Strongbridge Biopharma	azole antifungal	Cushing's syndrome	PO	InTrial	Late 2021	No	Yes
Ultomiris SC	ravulizumab-cwvz	Alexion	C5 complement inhibitor	paroxysmal nocturnal hemoglobinuria; Hemolytic uremic syndrome	SC	InTrial	Late 2021	Yes	Yes
ADV-7103	tripotassium citrate monohydrate/ potassium hydrogen carbonate	Advicenne	undisclosed	Distal rental tubular acidosis	PO	InTrial	Late 2021	Yes	No
MT-7117	MT-7117	Mitsubishi Tanabe Pharma	Undisclosed	Erythropoietic protoporphyria	PO	InTrial	Late 2021	Yes	No
AMAG-423	digoxin immune fab (DIF)	AMAG/ Velo	digitalis-like factor antagonist	Preeclampsia	IV	InTrial	Late 2021	Yes	Yes
glatiramer acetate depot	glatiramer acetate long-acting	Mylan	immunosuppressant	Multiple sclerosis	IM	InTrial	Late 2021	Yes	No
CAT-1004	edasalonexent	Catabasis	NF-kB inhibitor	Duchenne muscular dystrophy	PO	InTrial	Late 2021	Yes	Yes
AMT-061	etranacogene dezaparvovec	uniQure	gene therapy	Hemophilia B	IV	InTrial	Late 2021	Yes	Yes
DARE-BV1	clindamycin	Daré Bioscience	lincosamide	Bacterial vaginosis	Intravaginal	InTrial	Late 2021	No	No
JNJ-6372	amivantamab	Johnson & Johnson	EGFR and cMET antibody	Non-small cell lung cancer	IV	InTrial	Late 2021	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
PDS-1.0	ranibizumab	Roche/ Genentech	Anti-VEGF (vascular endothelial growth factor)	Wet age-related macular degeneration	Intravitreal implant	InTrial	Late 2021	Yes	No
ABL-001	asciminib	Novartis	allosteric Bcr-Abl inhibitor	Chronic myelogenous leukemia	PO	InTrial	Late 2021	Yes	Yes
CT-100	corticotrophin	Eton	adrenocorticotropic hormone (ACTH)	Rheumatoid arthritis	INJ	InTrial	Late 2021		No
CaPre	omega-3 fatty acids	Acasti Pharma	fatty acids	Hypertriglyceridemia	PO	InTrial	Late 2021	No	No
NNZ-2566	trofinetide	Neuren	insulin-like growth factor 1 (IGF-1) derivative	Rett syndrome	IV/PO	InTrial	Late 2021	Yes	Yes
RGN-259 (GBT-201; RGN-352)	timbetasin	RegeneRx	actin regulating peptide	Dry eyes	OP	InTrial	Late 2021	No	Yes
OTL-200 (GSK-2696274)	OTL-200 (GSK-2696274)	Orchard Therapeutics	gene therapy	Leukodystrophy	IV	InTrial	Late 2021	Yes	Yes
2022 Possible launch date									
ACER-001	sodium phenylbutyrate	Acer Therapeutics	BCKDC kinase inhibitor	Urea cycle disorders	PO	InTrial	1Q2022	No	No
Zynteglo (LentiGlobin)	lentiviral beta-globin gene transfer	Bluebird Bio	gene therapy	Beta-thalassemia	IV	InTrial	1Q2022	Yes	Yes
OBE-2109 (KLH-2109)	linzagolix	ObsEva/ Kissei	gonadotropin-releasing hormone (GnRH) antagonist	Uterine fibroids/ Endometriosis	PO	InTrial	1Q2022	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
TBR-652 (TAK-652, CVC)	cenicriviroc	Allergan	C-C chemokine receptor 5 (CCR5) and receptor 2 antagonist	Non-alcoholic steatohepatitis	PO	InTrial	1Q2022	Yes	No
SB-206	SB-206	Novan Therapeutics	nitric oxide-releasing compound	Molluscum contagiosum	TOP	InTrial	2Q2022	No	No
GFT-505	elafibranor	Genfit	selective peroxisome proliferator-activated receptor (PPAR) modulator	Non-alcoholic steatohepatitis	PO	InTrial	1H2022	Yes	No
Lenti-D	elivaldogene tavalentivec	Bluebird Bio	gene therapy	Adrenomyeloneuropathy	IV	InTrial	1H2022	Yes	Yes
ALN-APC (ALN-AT3)	fitusiran	Sanofi/ Alnylam	RNAi therapeutic	Hemophilia	SC	InTrial	1H2022	Yes	Yes
ONS-5010	bevacizumab-vikg	Outlook Therapeutics	anti-VEGF antibody	wet age-related macular degeneration	Intravitreal	InTrial	1H2022	Yes	No
CERC-801	CERC-801	Cerecor	D-galactose	Phosphoglucomutase 1 (PGM1) deficiency	PO	InTrial	1H2022	Yes	Yes
AG-348	mitapivat	Agios	pyruvate kinase-R (PKR) activator	Pyruvate kinase deficiency	PO	InTrial	Mid-2022	Yes	Yes
IMGN-853 (M-9346A-sulfo-SPDB-DM4)	mirvetuximab soravtansine	ImmunoGen	folate receptor-1 antagonist	Ovarian cancer	IV	InTrial	Mid-2022	Yes	Yes
RG-7433 (ABT-263)	navitoclax	AbbVie	Bcl-2 inhibitor	Myelofibrosis	PO	InTrial	Mid-2022	Yes	Yes
M-7824	bintrafusp alfa	GlaxoSmithKline	PD-L1 / TGF-beta immunoinhibition	Biliary tract cancer	IV	InTrial	Mid-2022	Yes	Yes
MIN-102	hydroxypioglitazone	Minoryx Therapeutics	PPAR gamma agonist	Adrenomyeloneuropathy	Undisclosed	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
idebenone	idebenone	Santhera	co-enzyme Q-10 analog	Duchenne muscular dystrophy	PO	InTrial	Mid-2022	Yes	Yes
GZ-402665	olipudase alfa	Sanofi	sphingomyelinase	Niemann-Pick disease type B	IV	InTrial	Mid-2022	Yes	Yes
GS-010	GS-010	GenSight Biologics	gene therapy	Optic neuropathy	Intraocular	InTrial	Mid-2022	Yes	Yes
OTL-103 (GSK-2696275)	OTL-103 (GSK-2696275)	Orchard Therapeutics	gene therapy	Wiskott-Aldrich syndrome	IV	InTrial	Mid-2022	Yes	Yes
CORT-125134	relacorilant	Corcept Therapeutics	Glucocorticoid receptor II (GR-II) antagonist	Cushing's syndrome	PO	InTrial	4Q2022	Yes	Yes
STS-101	dihydroergotamine	Satsuma Pharmaceuticals		Migraine	Intranasal	InTrial	2H2022	No	No
Oxabact (IxOC-3)	oxalobacter	OxThera	probiotic	Hyperoxaluria	PO	InTrial	2H2022	No	Yes
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
PDP-716	brimonidine	Sun Pharma Advanced Research Company (SPARC)	alpha-2 agonist	Glaucoma	OP	InTrial	2H2022	No	No
VGX-3100	VGX-3100	Inovio	vaccine	Cervical cancer/dysplasia	IM	InTrial	2022	Yes	No
QGE-031	ligelizumab	Novartis/ Roche	Anti-IgE antibody	Urticaria	SC	InTrial	2022	Yes	No
HY-01	minocycline	Hovione	tetracycline	Rosacea	TOP	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
FCX-007 (GM-HDF-COL7, INXN-3002)	FCX-007 (GM-HDF-COL7, INXN-3002)	Fibrocell Science/ Intrexon	gene-modified autologous fibroblast	Epidermolysis Bullosa	Undisclosed	InTrial	2022	Yes	Yes
AMG-157 (MEDI-9929)	tezepelumab	AstraZeneca/ Amgen	thymic stromal lymphopoietin antagonist	Asthma/ Atopic dermatitis	IV/SC	InTrial	2022	Yes	No
CM-AT	CM-AT	Curemark	protein absorption enhancer	Autism	PO	InTrial	2022	Yes	No
CNTX-4975	CNTX-4975	Centrexion Therapeutics	TRPV1 agonist	Osteoarthritis	Intra-articular	InTrial	2022	Yes	No
CERC-802	CERC-802	Cerecor	D-mannose	Mannose-phosphate isomerase deficiency	PO	InTrial	2022	Yes	Yes
PW-4142 (T-111)	nalbuphine ER	Trevi Therapeutics/ Endo	opioid agonist/ antagonist	Prurigo nodularis	PO	InTrial	2022	No	No
OTL-101	ADA-transduced autologous stem cell therapy	Orchard Therapeutics	gene therapy	Adenosine deaminase-deficient severe combined immunodeficiency	Undisclosed	InTrial	2022	Yes	Yes
IPX-203	carbidopa/ levodopa	Amneal	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	PO	InTrial	2022	No	No
pentoxifylline	pentoxifylline	Eton	phosphodiesterase inhibitor	Peyronie's disease	PO	InTrial	2022	No	No
Doria	risperidone	Laboratorios Farmacéuticos Rovi	atypical antipsychotic	Schizophrenia	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
BHV-3500	vazegepant	Biohaven	calcitonin gene-related peptide (CGRP) receptor antagonist	Migraine	Intranasal	InTrial	2022	No	No
JNJ-3872 (VX-787)	pimodivir	Johnson & Johnson/ Vertex	viral protein inhibitor	Influenza	PO	InTrial	2022	No	No
GSK-2894512 (WBI-1001)	tapinarof	Dermavant Sciences	therapeutic aryl hydrocarbon receptor modulating agent (TAMA)	Plaque psoriasis	TOP	InTrial	2022	Yes	No
MBG-453	MBG-453	Novartis	anti-TIM-3	Myelodysplastic syndrome	IV	InTrial	2022	Yes	No
ND-0612H	levodopa/ carbidopa	NeuroDerm	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	SC	InTrial	2022	Yes	No
ND-0612L	levodopa/ carbidopa	NeuroDerm	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	SC	InTrial	2022	Yes	No
REGN-475 (SAR-164877)	fasinumab	Regeneron/ Sanofi-Aventis/ Teva	selective anti-nerve growth factor (NGF) monoclonal antibody	Osteoarthritis	IV/SC	InTrial	2022	Yes	No
GSK-2140944	gepotidacin	GlaxoSmithKline	bacterial Type II topoisomerase inhibitor	Bacterial infections	PO/IV	InTrial	Late 2022	No	No
CSL-112	CSL-112	CSL Limited	plasma-derived apolipoprotein A-I (apoA-I).	Myocardial infarction	IV	InTrial	Late 2022	Yes	No
MK-8031	atogepant	Allergan/ Merck	calcitonin gene-related peptide (CGRP) receptor antagonist	Migraine	PO	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
R-1658 (RG-1658, JTT-705, RO-4607381)	dalcetrapib	DalCor/ Japan Tobacco/ Roche	cholesteryl ester transfer protein inhibitor	Acute coronary syndrome	PO	InTrial	Late 2022	Yes	No
NuThrax	anthrax vaccine adsorbed/ CPG-7909	Emergent Biosolutions	vaccine/ oligodeoxynucleotide	Anthrax	IM	InTrial	Late 2022	Yes	No
RP-L102 (RPL-102)	RP-L102	Rocket Pharmaceuticals	gene therapy	Fanconi anemia	IV	InTrial	Late 2022	Yes	Yes
SDP-037, SDN-037	difluprednate	Sun Pharma Advanced Research Company (SPARC)	Corticosteroid	Ocular inflammation/pain	OP	InTrial	Late 2022	No	No
pacritinib	pacritinib	CTI BioPharma/ Baxalta	janus associated kinase-2 (JAK2) inhibitor	Myelofibrosis	PO	InTrial	Late 2022	Yes	Yes

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

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
Ayvakit	avapritinib	Blueprint Medicines	selective KIT and PDGFRa inhibitor	Gastrointestinal stromal tumor (4th line)	Treatment of adults with fourth-line gastrointestinal stromal tumor (GIST)	PO	5/14/2020
Rubraca	rucaparib	Clovis Oncology	poly-ADP-ribose polymerase-1/2 (PARP-1/PARP-2) inhibitor	Metastatic castration-resistant prostate cancer	Treatment of BRCA1/2-mutant recurrent, metastatic castrate-resistant prostate cancer	PO	5/15/2020
Cyramza	ramucirumab	Eli Lilly	vascular endothelial growth factor 2 (VEGF-2) receptor antagonist	Non-small cell lung cancer	In combination with erlotinib, for first-line treatment of patients with metastatic non-small cell lung cancer whose tumors have epidermal growth factor receptor exon 19 deletions or exon 21 (L858R) substitution mutations	IV	5/15/2020
Opdivo	nivolumab	Bristol-Myers Squibb	anti-PD-1 antibody; T lymphocyte stimulator; protein kinase B (PKB/Akt) inhibitor	Non-small cell lung cancer	In combination with low-dose Yervoy for the treatment of first-line advanced non-small-cell lung cancer (NSCLC) in patients with no EGFR or ALK genomic tumor aberrations	IV	5/15/2020

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 (IL-4/IL-13) inhibitor	Atopic dermatitis	Add-on maintenance treatment for children aged 6 to 11 years with moderate-to-severe atopic dermatitis whose disease is not adequately controlled with topical prescription therapies or when those therapies are not advisable	SC	5/26/2020
Trelegy Ellipta	fluticasone furoate/ umeclidinium/ vilanterol	GlaxoSmithKline	inhaled corticosteroid (ICS)/ long-acting muscarinic agent (LAMA)/ long-acting beta agonist (LABA)	Chronic obstructive pulmonary disease	Reduction in all-cause mortality in patients with chronic obstructive pulmonary disease (COPD)	INH	5/30/2020
Recarbrio	imipenem/cilastatin/relebactam	Merck	Carbapenem/ dehydropeptidase-1 inhibitor/ beta-lactamase inhibitor	Hospital-acquired pneumonia and ventilator-associated bacterial pneumonia	Empiric treatment of hospital-acquired pneumonia (HAP) and ventilator-associated bacterial pneumonia (VABP)	IV	6/4/2020
Orilissa	elagolix	AbbVie	gonadotropin-releasing hormone (GnRH) receptor antagonist	Uterine fibroids	Management of heavy menstrual bleeding (HMB) associated with uterine fibroids in women	PO	6/5/2020
Gardasil	human papilloma virus vaccine	Merck	vaccine	Head and neck cancers	Prevention of certain head and neck cancers caused by vaccine-type HPV in females and males 9 through 45 years of age	IM	6/15/2020
Xolair	omalizumab	Novartis	IgE antagonist	Nasal polyps	Treatment of adults with chronic rhinosinusitis with nasal polyps who have not adequately responded to intranasal corticosteroids	SC	6/15/2020

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Taltz	ixekizumab	Eli Lilly	IL-17 monoclonal antibody	Non-radiographic axial spondyloarthritis	Treatment of non-radiographic axial spondyloarthritis	SC	6/15/2020
Keytruda	pembrolizumab	Merck	anti-PD-1 inhibitor	Solid tumors	Treatment of adult and pediatric patients with unresectable or metastatic solid tumors with tissue tumor mutational burden-high ≥ 10 mutations/megabase	IV	6/16/2020
Tazverik	tazemetostat	Epizyme	methyltransferase EZH2 inhibitor	Follicular lymphoma	Treatment of patients with relapsed or refractory follicular lymphoma (FL), both with or without EZH2 activating mutations, who have received at least two prior lines of systemic therapy	PO	6/18/2020
Crysvita	burosumab- twza	Ultragenyx/ Kyowa Kirin	fibroblast growth factor 23 antibody	Bone complications	Treatment of fibroblast growth factor 23 (FGF23)-related hypophosphatemia associated with phosphaturic mesenchymal tumors (tumor-induced osteomalacia) that cannot be curatively resected or localized	SC	6/18/2020
Tecentriq	atezolizumab	Genentech	PD-L1 monoclonal antibody	Non-small cell lung cancer	First-line monotherapy for people with advanced non-squamous and squamous non-small cell lung cancer (NSCLC) without EGFR or ALK mutations with high PD-L1 expression (TC3/IC3 wild-type [WT])	IV	6/19/2020
Xpovio	selinexor	Karyopharm Therapeutics	selective inhibitor of nuclear export	Diffuse large B-cell lymphoma	Treatment for patients with relapsed or refractory diffuse large B-Cell lymphoma (DLBCL) after at least two prior multi-agent therapies and who are ineligible for stem cell transplantation	PO	6/23/2020

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Alunbrig	brigatinib	Takeda	tyrosine kinase inhibitor	Non-small cell lung cancer	First-line treatment for patients with anaplastic lymphoma kinase-positive (ALK+) metastatic non-small cell lung cancer (NSCLC) as detected by an FDA-approved test	PO	6/23/2020
Keytruda	pembrolizumab	Merck	anti-PD-1 inhibitor	Cutaneous squamous cell carcinoma	Treatment of patients with recurrent and/or metastatic cutaneous squamous cell carcinoma (cSCC) that is not curable by surgery or radiation	IV	6/29/2020
Lynparza	olaparib	AstraZeneca/ Merck	poly (ADP-ribose) polymerase (PARP) inhibitor	Prostate cancer	Treatment of metastatic castration-resistant prostate cancer (mCRPC) and deleterious or suspected deleterious germline or somatic homologous recombination repair gene mutations, who have progressed following prior treatment with a new hormonal agent	PO	2Q 2020
Ryanodex	dantrolene sodium	Eagle Pharmaceuticals	ryanodine receptor inhibitor	Exertional heat stroke	Treatment of exertional heat stroke (EHS), in conjunction with external cooling methods	IV	7/9/2020
Brilinta	ticagrelor	AstraZeneca	Thienopyridine	Cardiovascular outcomes	Reduction in the incidence of cardiovascular death, myocardial infarction, or stroke in patients with type 2 diabetes mellitus	PO	7/15/2020
Tremfya	guselkumab	Janssen Biotech	interleukin-23 (IL-23) inhibitor	Psoriatic arthritis	Treatment of active psoriatic arthritis	SC	7/16/2020
Qutenza	capsaicin 8%	Averitas Pharma	transient receptor potential vanilloid 1 (TRPV-1) agonist	Diabetic peripheral neuropathy	Treatment of neuropathic pain associated with diabetic peripheral neuropathy	TOP	7/19/2020

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Tecentriq	atezolizumab	Roche/ Genentech	PD-L1 monoclonal antibody	Hepatocellular carcinoma	In combination with Avastin (bevacizumab), for the treatment of people with unresectable hepatocellular carcinoma (HCC) who have not received prior systemic therapy	IV	7/27/2020
Cosentyx	secukinumab	Novartis	IL-17 receptor antagonist	Axial spondyloarthritis	Treatment of non-radiographic axial spondyloarthritis	SC	7/29/2020
Trelegy Ellipta	fluticasone furoate/ umeclidinium/ vilanterol	GlaxoSmithKline	inhaled corticosteroid (ICS)/ long-acting muscarinic agent (LAMA)/ long-acting beta agonist (LABA)	Asthma	Treatment of asthma	INH	7/31/2020
Epidiolex	cannabidiol	Greenwich Biosciences	cannabinoid	Tuberous sclerosis complex	Treatment of tuberous sclerosis complex (TSC)	PO	7/31/2020
Spravato	esketamine	J&J/ Janssen	NMDA receptor antagonist	Major depressive disorder	For the rapid reduction of depressive symptoms in adult patients with major depressive disorder (MDD) who have active suicidal ideation with intent	Intranasal	8/2/2020
Opdivo	nivolumab	Bristol-Myers Squibb	anti-PD-1 antibody; T lymphocyte stimulator; protein kinase B (PKB/Akt) inhibitor	Non-small cell lung cancer	In combination with Yervoy (ipilimumab) and with a limited course of chemotherapy, for the first-line treatment of patients with metastatic or recurrent non-small cell lung cancer (NSCLC) with no EGFR or ALK genomic tumor aberrations	IV	8/6/2020

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Stelara	ustekinumab	Janssen	human interleukin-12 and -23 antagonist	Plaque psoriasis	Treatment of pediatric (ages 6 to 11) patients with moderate to severe plaque psoriasis (PsO).	SC	8/7/2020
Dovato	dolutegravir and lamivudine	GlaxoSmithKline (ViiV)	integrase inhibitor/nucleoside analogue reverse transcriptase	HIV-1	As a switch treatment for HIV-1 infection in virologically suppressed adults on a stable antiretroviral regimen with no treatment failure	PO	8/14/2020
Imbruvica	ibrutinib	AbbVie	kinase inhibitor	Chronic lymphocytic leukemia/small lymphocytic lymphoma	In combination with rituximab for the first-line treatment of younger patients (70 years old or younger) with chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL)	PO	9/8/2020
Bavencio	avelumab	EMD Serono/ Pfizer	PD-L1 monoclonal antibody	Urothelial carcinoma	First-line maintenance treatment of patients with locally advanced or metastatic urothelial carcinoma (UC)	IV	10/8/2020
Linzess	linaclotide	Allergan/ Ironwood Pharmaceuticals	guanylate cyclase C receptor agonist	Abdominal symptoms	Treatment of abdominal symptoms	PO	10/31/2020
Darzalex	daratumumab	Janssen	CD38-directed cytolytic antibody	Multiple myeloma (with Kyprolis and dexamethasone)	In combination with Kyprolis (carfilzomib) and dexamethasone (DKd) for relapsed/refractory multiple myeloma	IV	11/15/2020
Xofluza	baloxavir	Genentech/ Shionogi	polymerase acidic (PA) endonuclease inhibitor	Influenza	Treatment of acute uncomplicated influenza in otherwise healthy children aged one to less than 12 years of age who have been symptomatic for no more than 48 hours	PO	11/23/2020

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Xofluza	baloxavir	Genentech/ Shionogi	polymerase acidic (PA) endonuclease inhibitor	Influenza	Post-exposure prophylaxis of influenza in people one year of age and older	PO	11/23/2020

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

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RxOutlook® is published by the OptumRx Clinical Services Department.