OPTUMRx®

RxOutlook®

4th Quarter 2019

Welcome to the RxOutlook®, OptumRx's quarterly report summarizing the latest pipeline drug information, upcoming generic launches, and emerging therapies in today's pharmaceutical market.

In this edition, we take our first look at potential new drugs in 2020, concentrating on a select group of 11 near-term drugs that are expected to receive a Food and Drug Administration (FDA) approval decision by the end of the first quarter of 2020. These drugs are notable because of their potential clinical or financial impact, and/or public health interest. This selection of drugs gives a glimpse of what lies ahead in 2020: an emphasis on rare diseases, inherited conditions, oncology, and neurology. For example, six of these drugs have an orphan designation for treating rare diseases, including two new treatments (crizanlizumab and voxelotor) for sickle cell disease, a condition where the standard of care, hydroxyurea, was first approved by the FDA over 50 years ago. Trikafta™, originally anticipated in March 2020, was approved by the FDA almost 5 months earlier than expected and is intended for the treatment of cystic fibrosis (CF) patients that harbor at least one copy of *F508del*, a genetic mutation found in roughly 90% of the CF population. Similarly, the two cancer drugs (tazemetostat and avapritinib) target niche populations defined by either a biomarker or failure of available treatments. We expect this focus on orphan drugs to continue in 2020. According to our estimates, cancer will account for the largest therapeutic area in the pipeline, and 70% of the cancer drugs that could reach the market in 2020 will have orphan designations for small targeted populations. Conversely, orphan drugs account for roughly 30% of non-oncology pipeline drugs with potential approval in 2020.

The second largest therapeutic area in the 2020 pipeline is neurology. The list of migraine therapies continues to grow with two more calcitonin gene-related peptide (CGRP) inhibitors for migraine headache. Rimegepant is likely to be the second oral CGRP inhibitor product for acute migraines. Eptinezumab is likely to be the first intravenous CGRP inhibitor and offers dosing every 3 months for prophylaxis of migraine. These products will join the currently approved, self-administered, subcutaneous CGRP inhibitors. The third neurology drug is a new drug for multiple sclerosis: ozanimod, the third agent in the sphingosine 1-phosphate (S1P) modulator class.

Rounding out our selection are three drugs with unique places in therapy. AR-101 could be the first drug approved for reducing the incidence and severity of allergic reactions in patients with food allergy to peanuts. Considering the large number of people with food allergies, AR-101 is likely to receive a large amount of attention in the media if approved by the FDA. Givlaari® (givosiran), another product approved ahead of its expected approval date, is the first drug for reduction of attacks in patients with acute hepatic porphyria (AHP). AHP is a rare genetic disorder characterized by painful acute attacks caused by the accumulation of metabolic neurotoxins. Givosiran is an RNA interference (RNAi) drug that silences the gene responsible for production of excessive neurotoxic substances. Givosiran is the second RNAi drug approved by the FDA, and a third RNAi drug could reach the market later in 2020. Finally, bempedoic acid could be the first ATP-citrate lyase inhibitor approved for primary hyperlipidemia. This drug targets an enzyme upstream from the statins and could offer an oral alternative to injectable proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitors for patients who need additional low-density lipoprotein-cholesterol (LDL-C) lowering despite maximum-tolerated doses of statins.

Key pipeline drugs with FDA approval decisions expected by the end of the 1st quarter 2020

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Elexacaftor/ tezacaftor/ ivacaftor; ivacaftor	Vertex	Cystic fibrosis*	10/21/2019 (Approved)
Crizanlizumab	Novartis	Sickle cell disease*	11/15/2019 (Approved)
Givosiran	Alnylam	Acute hepatic porphyria*	11/20/2019 (Approved)
Voxelotor	Global Blood Therapeutics	Sickle cell disease*	11/25/2019 (Approved)
AR-101	Aimmune Therapeutics	Peanut allergy	1/2020
Tazemetostat	Epizyme	Epithelioid sarcoma*	1/23/2020
Rimegepant	Biohaven	Acute migraine	2/2020
Avapritinib	Blueprint Medicines	Gastrointestinal stromal tumors*	2/14/2020
Eptinezumab	Alder	Migraine prophylaxis	2/21/2020
Ozanimod	Celgene	Multiple sclerosis	3/25/2020

* 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 in the following manner:

Detailed Drug Insights

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

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 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 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 1st 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.

Drugs reviewed in detail in the 1Q, 2Q, and 3Q 2019 reports

- Afamelanotide (Scenesse®)
- Brolucizumab (Beovu®)
- Cabotegravir/rilpivirine
- Celiprolol
- Darolutamide (Nubeqa™)
- Diroximel fumarate (Vumerity™)
- Dolutegravir/lamivudine (Dovato®)
- Entrectinib (Rozlytrek™)
- Esketamine (Spravato™)
- Fedratinib (Inrebic®)
- Golodirsen
- Lasmiditan (Reyvow™)
- Lemborexant
- Lumateperone
- Luspatercept (Reblozyl®)
- Mannitol (inhaled formulation)
- Metoclopramide (intranasal formulation)
- NKTR-181
- Onasemnogene abeparvovec (Zolgensma®)
- Pexidartinib (Turalio™)
- Pitolisant (Wakix®)
- Polatuzumab vedotin (Polivy™)
- Quizartinib
- Risankizumab (Skyrizi™)
- RVT-802
- Selinexor (Xpovio™)
- Semaglutide (Rybelsus®)
- Tafamidis (Vyndaqel®) and tafamidis meglumine (Vyndamax®)
- Tenapanor (Ibsrela®)
- Ubrogepant
- Upadacitinib (Rinvoq™)

Past issues of RxOutlook can be found at https://professionals.optumrx.com/publications.html.

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

Elexacaftor/tezacaftor/ivacaftor; ivacaftor (Brand Name: Trikafta™)

Manufacturer: Vertex
Regulatory designations: Orphan Drug, Breakthrough Therapy, Fast Track
FDA approval date: 10/21/2019 *(approved ahead of anticipated approval date)*

Therapeutic use

Trikafta was approved for the treatment of cystic fibrosis (CF) in patients aged 12 years and older who have at least one *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.

CF, a rare, progressive, life-threatening disease, results in the formation of thick mucus that builds up in the lungs, digestive tract, and other parts of the body. In the lungs, CF can cause chronic lung infections and progressive lung damage which in many patients eventually leads to early death.

CF is caused by a defective protein that results from mutations in the *CFTR* gene. Approximately 2,000 mutations of the *CFTR* gene have been identified, and in order to be diagnosed with CF, patients need to have mutations in two defective *CFTR* genes. The *F508del* mutation is the most common with up to 90% of CF patients (approximately 27,000 people in the U.S.) carrying at least one gene with this mutation.

- Treatment of CF in patients aged 12 years and older who have at least one *F508del* mutation in the *CFTR* gene

Elexacaftor/tezacaftor/ivacaftor; ivacaftor (Brand Name: Trikafta™) ***(continued...)***

Clinical profile

Trikafta is a combination of three drugs that target the defective CFTR protein. It helps the protein made by the *CFTR* gene mutation function more effectively. Elexacaftor is a novel drug while tezacaftor and ivacaftor are the active ingredients for Symdeko®. Currently available therapies, including Symdeko, are treatment options for some patients with CF, but many patients have mutations that are ineligible for treatment.

Pivotal trial data:
The efficacy of Trikafta was established in two randomized, double-blind studies in CF patients aged 12 years and older. The primary efficacy endpoint in both studies was the mean absolute change in percent predicted forced expiratory volume in 1 second ($ppFEV_1$). Study 1 was a 24-week, placebo-controlled study in 403 patients who had a *F508del* mutation on one allele and a mutation on the second allele that results in either no CFTR protein or a CFTR protein that is not responsive to ivacaftor and tezacaftor/ivacaftor. The treatment difference between Trikafta and placebo for the mean absolute change from baseline in $ppFEV_1$ at week 4 was 13.8% (95% CI: 12.1, 15.4; $p < 0.0001$). The treatment difference was sustained through week 24. The number of pulmonary exacerbation events (event rate per year calculated based on 48 weeks per year) from baseline through week 24 was 0.37 and 0.98 for Trikafta and placebo, respectively ($p < 0.0001$).

Study 2 was a 4-week, active-controlled study in 107 patients who were homozygous for the *F508del* mutation. In study 2, patients were randomized to receive Trikafta or Symdeko. Treatment with Trikafta resulted in a statistically significant improvement in $ppFEV_1$ of 10.0% (95% CI: 7.4, 12.6; $p < 0.0001$) vs. Symdeko.

Safety:
The most common adverse reactions with Trikafta use were headache, upper respiratory tract infection, abdominal pain, diarrhea, rash, increased alanine aminotransferase (ALT), nasal congestion, increased blood creatine phosphokinase, increased aspartate aminotransferase (AST), rhinorrhea, rhinitis, influenza, sinusitis, and increased blood bilirubin.

Dosing:
The recommended dose of Trikafta is two tablets (each containing elexacaftor 100 mg, tezacaftor 50 mg and ivacaftor 75 mg) taken orally in the morning and one ivacaftor tablet (containing ivacaftor 150 mg) taken orally in the evening.

- CFTR modulators
- Oral formulation
- $ppFEV_1$: 13.8% improvement vs. placebo (heterozygous *F508del* mutation population); 10.0% improvement vs. Symdeko (homozygous *F508del* mutation population)
- Common AEs: headache, upper respiratory tract infection, abdominal pain, diarrhea, rash, increased ALT/AST, nasal congestion, increased blood creatine phosphokinase, rhinorrhea, rhinitis, influenza, sinusitis and increased blood bilirubin
- Dosing: Two combination tablets (elexacaftor tezacaftor, and ivacaftor) in the morning; one ivacaftor tablet in the evening

Competitive environment

Approximately 60% of CF patients have mutations amenable to treatment with previously approved CFTR modulators (Kalydeco® [ivacaftor], Orkambi® [ivacaftor/lumacaftor], and Symdeko). Trikafta has demonstrated efficacy in patients carrying two *F508del* mutations, as well as patients with just one *F508del* mutation, which expands the treatable population to approximately 90% of all CF patients. Trikafta also provided an improvement in lung function in a short-term, head-to-head study against Symdeko in patients with two *F508del* mutations.

However, Trikafta's current indication is limited to patients 12 years of age and older, whereas Kalydeco, Orkambi, and Symdeko are indicated in younger patients (6 months, 2 years, and 6 years of age and older, respectively). While the majority of patients with CF will be eligible for treatment with Trikafta, a CF mutation test should still be used to confirm the presence of at least one *F508del* mutation.

The Wholesale Acquisition Cost (WAC) for Trikafta is $311,500 per year.

- Advantages: expands the patient population eligible for treatment with CFTR modulators, improved lung function vs. standard of care (Symdeko)
- Disadvantages: initial indication limited to patients 12 years and older, lack of long-term data vs. Symdeko, CF mutation test still required
- WAC = $311,500 per year

Crizanlizumab (Brand Name: Adakveo®)

Manufacturer: Novartis
Regulatory designations: Orphan Drug, Breakthrough Therapy
FDA approval date: 11/15/2019 *(approved ahead of anticipated approval date)*

Therapeutic use

Crizanlizumab was approved for the reduction of the frequency of vasoocclusive crises (VOCs) in adults and pediatric patients aged 16 years and older with sickle cell disease (SCD).

- Reduce the frequency of VOCs in adults and pediatric patients aged 16 years and older with SCD

SCD is a rare genetic blood disorder that affects about 100,000 people in the U.S. A genetic mutation results in the production of sickle hemoglobin. When these sickle hemoglobin molecules become deoxygenated, they can polymerize in red blood cells (RBCs) which leads to the formation of abnormally shaped RBCs that have a crescent or sickle shape.

Due to the abnormal properties of the sickled RBCs, patients can develop hemolytic anemia and vascular obstructions (commonly called vaso-occlusions). Vascular occlusions cause intense pain (VOCs) that require early and aggressive treatment with analgesics, including opioids, and can be accompanied by tissue ischemia and inflammation. VOCs can occur as early as 6 months old and SCD patients are at risk for these events throughout their life. Common sites of VOC include the back, chest, abdomen, and long bones. VOCs are the main reason patients with SCD seek medical care.

The combination of hemolytic anemia and vaso-occlusion can ultimately lead to multi-organ damage as well as early death.

Crizanlizumab (Brand Name: Adakveo®) (continued...)

Clinical profile

Crizanlizumab is a novel monoclonal antibody that binds to a molecule called P-selectin on the surface of platelets and endothelium in the blood vessels, and has been shown to inhibit interactions between endothelial cells, platelets, RBCs, and leukocytes. P-selectin is an adhesion molecule and one of the major drivers of the vaso-occlusive process.

Pivotal trial data:
The efficacy of crizanlizumab was evaluated in SUSTAIN, a Phase 2, randomized, double-blind, placebo-controlled study in 198 patients with SCD. Patients received low-dose crizanlizumab (2.5 mg/kg), high-dose crizanlizumab (5.0 mg/kg), or placebo. The primary endpoint was the annual rate of sickle cell-related pain crises with high-dose crizanlizumab vs. placebo.

The median rate of crises per year was 1.63 with high-dose crizanlizumab vs. 2.98 with placebo (indicating a 45.3% lower rate with high-dose crizanlizumab, $p = 0.010$). The median time to the first crisis was significantly longer with high-dose crizanlizumab than with placebo (4.1 months vs. 1.4 months), as was the median time to the second crisis (10.32 vs. 5.09 months, $p = 0.02$). Low-dose crizanlizumab did not demonstrate a significant change in sickle cell-related pain crises compared with placebo (2.01 vs. 2.98 respectively; $p = 0.18$).

Safety:
The most common adverse events with crizanlizumab use were nausea, arthralgia, back pain, and pyrexia.

Dosing:
The recommended dose of crizanlizumab is 5 mg/kg administered via intravenous (IV) infusion over a period of 30 minutes at week 0, week 2, and every 4 weeks thereafter. Crizanlizumab may be given with or without hydroxyurea.

- P-selectin inhibitor
- IV formulation
- Median rate of pain crises per year: 1.63 with high-dose crizanlizumab vs. 2.98 with placebo (45.3% reduction)
- Common AEs: nausea, arthralgia, back pain, pyrexia
- Dosing: every 4 weeks (maintenance)

Competitive environment

Crizanlizumab offers a new mechanism of action (MOA) for the treatment of SCD, a condition where there is a high unmet need for treatments. Hydroxyurea is the current standard of care; however, a substantial proportion of patients with SCD continue to have complications associated with the disease and could benefit from additional treatment options. An estimated 5% to 10% of children and 25% to 30% of adults have true lack of response from hydroxyurea. While the sample size was small, the pivotal trial did include patients with or without concomitant hydroxyurea use and the data suggests that crizanlizumab would be beneficial in both subgroups.

However, crizanlizumab would likely be a second-line therapy since hydroxyurea can be taken orally and is supported by years of clinical experience with demonstrated clinical benefit. In contrast, the support for crizanlizumab's efficacy was based on a small Phase 2 trial and it must be dosed via IV infusion.

Crizanlizumab may soon face competition from other near-term pipeline drugs being developed for the treatment of SCD. An FDA approval decision is expected for Global Blood Therapeutics' SCD drug, voxelotor, in February 2020, and bluebird bio's gene therapy for SCD may also be available by the end of 2022.

The estimated WAC for crizanlizumab is $85,000 to $113,000 per year, depending on dosing.

- Advantages: novel MOA, high unmet need, once monthly dosing
- Disadvantages: likely second-line behind hydroxyurea, lack of late stage trial data, potential competition in the pipeline (eg, voxelotor, bluebird bio's gene therapy), IV administration
- WAC = $85,000 to $113,000 per year

Voxelotor (Brand Name: Oxbryta™)

Manufacturer: Global Blood Therapeutics
Regulatory designations: Orphan Drug, Breakthrough Therapy, Fast Track
FDA approval date: 11/25/2019 (approved ahead of anticipated approval date)

Therapeutic use

Voxelotor was approved for the treatment of sickle cell disease (SCD) in adults and pediatric patients 12 years of age and older.

This indication is approved under accelerated approval based on increase in hemoglobin. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).

- Treatment of SCD in adults and pediatric patients 12 years of age and older

Clinical profile

Voxelotor is a novel hemoglobin polymerization inhibitor that increases hemoglobin's affinity for oxygen. Since oxygenated sickle hemoglobin does not polymerize, voxelotor blocks polymerization and the resultant sickling and destruction of RBCs.

- Hemoglobin polymerization inhibitor
- Oral formulation

<u>Pivotal trial data:</u>
The efficacy of voxelotor was evaluated in the HOPE trial, a Phase 3, randomized, double-blind, placebo-controlled study in 274 patients with SCD. Patients received 1500 mg of voxelotor, 900 mg of voxelotor, or placebo. The primary endpoint was the percentage of patients who had a hemoglobin response, which was defined as an increase of more than 1.0 g/dL in hemoglobin from baseline at week 24.

After 24 weeks of treatment, 51% of patients receiving 1500 mg voxelotor had a hemoglobin response compared to 7% in the placebo group ($p < 0.001$). In the 900 mg voxelotor group, 33% of patients had a hemoglobin response at week 24 ($p < 0.001$ vs. placebo). As a secondary endpoint, the study also evaluated the annualized incidence rate of VOCs (per person-year); the rate was 2.77 with voxelotor 1500 mg and 2.76 with voxelotor 900 mg vs. 3.19 with placebo.

- Hemoglobin response (hemoglobin improvement ≥ 1.0 g/dL): 51% with high-dose voxelotor vs. 7% with placebo

<u>Safety:</u>
The most common adverse events with voxelotor use were headache, diarrhea, abdominal pain, nausea, fatigue, rash, and pyrexia.

- Common AEs: headache, diarrhea, abdominal pain, nausea, fatigue, rash, pyrexia

<u>Dosing:</u>
The recommended dosage of Oxbryta is 1,500 mg taken orally once daily with or without food.

- Dosing: once daily

Voxelotor (continued...)

Competitive environment

Voxelotor offers another drug with a novel MOA for the treatment of SCD and is believed to target the underlying molecular pathogenesis of the condition. In contrast to crizanlizumab, voxelotor can be taken orally and has shown benefit in improving hemoglobin and preventing worsening of anemia. Voxelotor showed benefit in hemoglobin response regardless of concurrent use of hydroxyurea at baseline.

Similar to crizanlizumab, voxelotor would likely be a second-line therapy for SCD behind hydroxyurea. However, the incidence of VOCs did not differ significantly between voxelotor and placebo, and VOCs are a primary cause for hospitalization or medical care for patients with SCD. In addition, there is a lack of long-term safety data with use of voxelotor, which is a concern because the mechanism by which voxelotor improves anemia is by increasing hemoglobin oxygen affinity. This may adversely impact tissue oxygen delivery; however, no significant safety signals were highlighted in the pivotal trial.

- Advantages: novel MOA, high unmet need, oral, once daily
- Disadvantages: will likely be second-line behind hydroxyurea, lack of benefit in reducing VOCs, lack of long-term safety data, potential competition in the pipeline (eg, crizanlizumab, bluebird bio's gene therapy)

Givosiran (Brand Name: Givlaari®)

Manufacturer: Alnylam
Regulatory designations: Orphan Drug, Breakthrough Therapy
FDA approval date: 11/20/2019 *(approved ahead of anticipated approval date)*

Therapeutic use

- Treatment of AHP

Givosiran was approved for the treatment of adults with acute hepatic porphyria (AHP).

AHP are a rare sub-group of porphyrias characterized by the occurrence of neuro-visceral attacks. AHP is a comprised of four major subtypes, with each subtype resulting from a genetic defect leading to deficiency in one of the enzymes of the heme biosynthesis pathway in the liver. These enzymatic defects cause the accumulation of neurotoxic heme intermediates aminolevulinic acid (ALA) and porphobilinogen (PBG). Common symptoms of AHP include severe, diffuse abdominal pain, weakness, nausea, and fatigue.

The estimated prevalence of AHP is about 3 to 5 per 100,000 in the U.S.

Clinical profile

- RNAi targeting ALAS1
- SC formulation
- Mean composite attack rate: 1.9 vs. 6.5 for with placebo (70% reduction)
- Common AEs: nausea and injection site reactions
- Dosing: once monthly

Givosiran is a novel RNA interference (RNAi) agent that targets aminolevulinic acid synthase 1 (*ALAS1*). Givosiran is believed to work by significantly lowering induced liver ALAS1 levels and thereby decreasing neurotoxic heme intermediates, ALA and PBG.

<u>Pivotal trial data:</u>
The efficacy of givosiran was evaluated in ENVISION, a Phase 3, randomized, double-blind, placebo-controlled study in 94 patients with AHP. The primary endpoint was the rate of attacks requiring hospitalization, urgent care, or home IV hemin use. The mean rate of porphyria attacks was 1.9 and 6.5 for givosiran and placebo, respectively. This represented a 70% (95% CI: 60, 80) reduction in porphyria attacks. In addition, 50% of givosiran-treated patients were attack-free during the 6-month treatment period vs. 16.3% of placebo-treated patients.

<u>Safety:</u>
The most common adverse events with givosiran use were nausea and injection site reactions.

<u>Dosing:</u>
The recommended dose of givosiran is 2.5 mg/kg administered via subcutaneous (SC) injection once monthly.

Givosiran (continued...)

Competitive environment

Givosiran is the first product approved with evidence of reduction of AHP attacks and it offers a novel MOA for the treatment of the condition. There is a high unmet need for preventative treatments for AHP as the only other approved therapy is Panhematin® (hemin for injection), which is only approved for the amelioration of recurrent attacks of acute intermittent porphyria (the most common subtype of AHP). The use of Panhematin use is also limited due to safety concerns and IV administration via a large arm vein or a central venous catheter.

However, givosiran must be administered via SC injection and requires administration by a healthcare professional. In the pivotal study, givosiran was also associated with increased adverse events related to the kidney and liver, which will require additional monitoring from a provider.

The estimated WAC for givosiran is $575,000 per year.

- Advantages: novel MOA, first approved product for prevention of AHP attacks, high unmet need, monthly administration
- Disadvantages: SC administration, safety concerns that will require additional provider monitoring
- WAC = $575,000 per year

AR-101 (Brand name: Palforzia™)

Manufacturer: Aimmune Therapeutics
Regulatory designations: Breakthrough Therapy, Fast Track
Expected FDA decision: 1/2020

Therapeutic use

AR-101 is in development to reduce the incidence and severity of allergic reactions, including anaphylaxis after accidental exposure to peanut in patients aged 4 through 17 years with a confirmed diagnosis of peanut allergy.

Peanut allergy is the most common food allergy in the U.S. with about 2% of children affected and it accounts for the majority of deaths related to food allergy. The current standard of care is peanut avoidance and access to self-injectable epinephrine in cases of accidental exposure.

- To reduce the incidence and severity of allergic reactions after accidental exposure to peanut in patients aged 4 through 17 years with a confirmed diagnosis of peanut allergy

Clinical profile

AR-101 is an oral immunotherapy. It is a naturally derived peanut protein product that contains the key allergens found in peanuts. The goal of treatment is to desensitize patients to peanut allergens through a dose escalation of AR-101 followed by maintenance therapy with the drug.

- Immunotherapy containing naturally derived peanut protein
- Oral formulation
- Exit food challenge (ingest a dose of 600 mg or more of peanut protein, without dose-limiting symptoms): 67.2% vs. 4.0% with placebo
- Common AEs: abdominal pain, vomiting, nausea
- Dosing: once daily

<u>Pivotal trial data:</u>
The efficacy of AR-101 was evaluated in PALISADE, a Phase 3, randomized, double-blind, placebo-controlled study in patients with peanut allergy. The prespecified primary analysis population included 496 patients aged 4 to 17 years. Patients randomized to AR-101 proceeded through a 1-day initial dose-escalation phase (from 0.5 mg to 6 mg); an increasing-dose phase, during which the dose was increased gradually every 2 weeks from 3 mg to 300 mg; and a 24-week maintenance phase where patients received 300 mg/day of AR-101. The primary efficacy endpoint was the proportion of patients who could ingest a peanut protein challenge dose of 600 mg or more, without dose-limiting symptoms, in an exit food challenge at the end of the maintenance phase.

At the exit food challenge, 67.2% of patients treated with AR-101 were able to ingest a dose of 600 mg or more of peanut protein, without dose-limiting symptoms vs. 4.0% of patients treated with placebo (difference 63.2%; 95% CI: 53.0, 73.3; $p < 0.001$). During the exit food challenge, the maximum severity of symptoms was moderate in 25% of the participants in the AR-101 group vs. 59% of those in the placebo group and severe in 5% and 11%, respectively.

<u>Safety:</u>
The most common adverse events with AR-101 use were abdominal pain, vomiting, and nausea.

<u>Dosing:</u>
In the pivotal trial, AR-101 was administered orally once a day.

AR-101 (Brand name: Palforzia™)(continued...)

Competitive environment

If approved, AR-101 would be the first standardized immunotherapy for peanut allergy. There is a high unmet need for a treatment as peanut allergy is one of the most common food allergies in the U.S. and accidental exposure can cause severe allergic reactions, including anaphylaxis.

Provider and parental acceptance of AR-101 as a treatment for peanut allergy may be limited due to several factors. First, treatment with AR-101 requires approximately 6 months of dose titration with clinical observation every 2 weeks. Second, AR-101 was associated with a higher discontinuation rate due to adverse events vs. placebo (11.6% vs. 2.4%) and a higher rate of epinephrine use during the course of the trial, excluding during the food challenges (14.0% vs. 6.5%). If approved, a boxed warning and REMS program is highly likely for AR-101 because of the safety concerns. Third, while AR-101 was shown to be effective in immunological desensitization vs. placebo, there is a lack of data showing AR-101 as effective in reducing allergic reactions due to accidental peanut exposure.

Finally, AR-101 may also face future competition as DBV Technologies' transdermal product for peanut allergy, Viaskin Peanut, could become available in the second half 2020. Compared indirectly, AR-101 appears to be more effective in achieving peanut desensitization, but Viaskin Peanut has a more favorable safety and tolerability profile and does not require a challenging dose titration.

The projected WAC price for AR-101 is $5,000 to $10,000 for the initial 6 month titration period and then $300 to $400 per 30 days.

- Advantages: potentially first approved product for peanut allergy, high unmet need, large market, oral
- Disadvantages: challenging dose titration, safety/tolerability concerns, lack of demonstrated benefit in reducing allergic reactions due to accidental exposure, potential future competition (ie, Viaskin Peanut)
- Projected WAC = $5,000 to $10,000 x 6 months, followed by $300 to $400 per 30 days

Tazemetostat (Brand Name: To be determined)

Manufacturer: Epizyme
Regulatory designations: Orphan Drug
Expected FDA decision: 1/23/2020

Therapeutic use

Tazemetostat is in development for the treatment of metastatic or locally advanced epithelioid sarcoma in patients not eligible for curative surgery.

Epithelioid sarcoma is a rare soft tissue sarcoma that most often starts in tissues under the skin of the hands, forearms, feet, or lower legs. Patients are most commonly diagnosed as young adults, between 20 and 40 years of age, and median overall survival from initial diagnosis is 30 months.

About 12,750 new soft tissue sarcomas will be diagnosed in 2019 and epithelioid sarcoma only accounts for approximately 1% of all cases.

- Treatment of metastatic or locally advanced epithelioid sarcoma in patients not eligible for curative surgery

Clinical profile

Tazemetostat is a first-in-class inhibitor of enhancer of zeste homolog 2 (EZH2). Activating mutations and overexpression of EZH2 are found in various solid tumors and hematologic malignancies.

Pivotal trial data:
Tazemetostat was evaluated in a single-arm, Phase 2 study in 24 treatment-naïve and 38 relapsed and/or refractory patients with epithelioid sarcoma. The primary endpoint was overall response rate, which was comprised of complete or partial responses as measured by RECIST criteria. The overall objective response rate (ORR) for all tazemetostat patients was 15%, and 26% of patients achieved the secondary endpoint of disease control. Although the numbers are small, treatment naïve patients appeared to respond better to tazemetostat (ORR = 25%) than did those patients with relapsed and/or refractory disease (ORR = 8%). Median overall survival at the last reported data cut-off was 82.4 weeks (95% CI: 47.4, not reached).

Safety:
The most common adverse events with tazemetostat use were fatigue, cancer pain, decreased appetite, nausea/vomiting, constipation, diarrhea, anemia, cough, headache, and plural effusion.

Dosing:
In the pivotal trial, tazemetostat was administered orally twice a day.

- EZH2 inhibitor
- Oral formulation
- ORR = 15%
- Common AEs: fatigue, cancer pain, decreased appetite, nausea/vomiting, constipation, diarrhea, anemia, cough, headache, plural effusion
- Dosing: twice a day

Tazemetostat (continued...)

Competitive environment

Tazemetostat offers a novel MOA for the treatment of epithelioid sarcoma. Patients with this rare type of sarcoma have limited treatment options beyond surgery or radiation and tazemetostat has a relatively favorable adverse event profile compared to more cytotoxic drugs used in the advanced or metastatic setting. Tazemetostat is also being evaluated for other cancers, including follicular lymphoma and solid tumors.

However, support for the current FDA submission for tazemetostat is limited to a small, single arm, Phase 2 clinical trial with a modest response rate and a lack of robust overall survival benefit. The initial indication is also limited as epithelioid sarcoma represents only a small proportion of the overall soft tissue sarcoma population.

- Advantages: novel MOA, high unmet need, also in development for other cancers, oral
- Disadvantages: lack of late stage data, modest efficacy, limited initial indication

Avapritinib (Brand Name: To be determined)

Manufacturer: Blueprint Medicines
Regulatory designations: Orphan Drug, Breakthrough Therapy, Fast Track
Expected FDA decision: 2/14/2020 (PDGFRα exon 18 mutant gastrointestinal stromal tumors [GIST]); 2nd quarter 2020 (fourth-line therapy for GIST)

Therapeutic use

Avapritinib is in development for the treatment of adult patients with PDGFRα exon 18 mutant GIST, regardless of prior therapy, and fourth-line therapy for GIST.

GIST is a sarcoma of the gastrointestinal tract. The tumors can start anywhere in the gastrointestinal tract but occur most often in the stomach or small intestine. GISTs are typically diagnosed between the ages of 50 to 80. Most GIST cases are caused by a spectrum of clinically relevant mutations that force the KIT or PDGFRα protein kinases into an increasingly active state.

Current estimates for the total number of GIST cases each year in the U.S. range from about 4,000 to about 6,000. Approximately 10% of GIST cases are due to PDGFRα-mutations.

- Treatment of adult patients with PDGFRα exon 18 mutant GIST, regardless of prior therapy, and fourth-line therapy for GIST

Avapritinib (continued...)

Clinical profile

Avapritinib selectively and potently inhibits KIT and PDGFRα mutant kinases. It is a type 1 inhibitor designed to target the active kinase conformation; all oncogenic kinases signal via this conformation. Avapritinib has demonstrated broad inhibition of KIT and PDGFRα mutations associated with GIST, including potent activity against activation loop mutations that are associated with resistance to current therapies.

Pivotal trial data:
The efficacy of avapritinib was evaluated in the ongoing Phase 1 NAVIGATOR study. As of the data cutoff date of November 16, 2018, 43 patients with PDGFRα exon 18 mutant GIST (including 38 patients with PDGFRα D842V-driven GIST) and 111 patients with fourth-line GIST were treated with avapritinib.

In evaluable patients with PDGFRα exon 18 mutant GIST, the ORR was 86% and the median duration of response was not reached. In evaluable patients with fourth-line GIST, the ORR was 22% and the median duration of response was 10.2 months.

Avapritinib is also being evaluated in VOYAGER, a Phase 3, open-label, randomized study comparing avapritinib vs. Stivarga® (regorafenib) in patients with third- or fourth-line GIST. Topline data from that study are expected in the second quarter of 2020 and the results will likely be submitted in time for the FDA review of the fourth-line GIST indication.

Safety:
The most common adverse events with avapritinib use were nausea, fatigue, anemia, cognitive effects, periorbital edema, vomiting, decreased appetite, diarrhea, increased lacrimation, and peripheral edema.

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

- KIT and PDGFRα kinase inhibitor
- Oral formulation
- ORR (PDGFRα exon 18 mutant GIST) = 86%
- ORR (fourth-line GIST) = 22%
- Common AEs: nausea, fatigue, anemia, cognitive effects, periorbital edema, vomiting, decreased appetite, diarrhea, increased lacrimation, peripheral edema
- Dosing: once daily

Competitive environment

If approved, avapritinib would be the first therapy for PDGFRα exon 18 mutant GIST and the only therapy for patients with KIT-driven GIST whose disease progresses beyond Gleevec® (imatinib), Sutent® (sunitinib), and Stivarga. The early stage data is promising with relatively high response rates, particularly in the PDGFRα subpopulation. Avapritinib would offer an additional oral, once a day oncology therapy.

However, the proposed initial indications for avapritinib are narrow. Of note, PDGFRα-mutated GISTs only account for approximately 10% of the overall GIST population. In addition, the initial submission is based on an early stage study and there is a lack of robust overall survival data.

For reference, the WAC price for Stivarga is approximately $17,500 per 28-day cycle.

- Advantages: promising early stage data, unmet need, oral, once daily dosing
- Disadvantages: narrow initial indication, lack of late stage data
- Reference WAC (Stivarga) = ~$17,500 per 28-day cycle

Rimegepant (Brand Name: To be determined)

Manufacturer: Biohaven
Expected FDA decision: 2/2020

Therapeutic use

Rimegepant is in development for the acute treatment of migraine headaches in adults.

Patients suffering from migraines have recurrent episodes of severe headache accompanied by other symptoms including nausea, vomiting, sensitivity to light and sound, and changes in vision. An estimated 30 million adults in the U.S. experience migraine headaches.

- Acute treatment of migraine headaches in adults

Clinical profile

Rimegepant is an orally dosed calcitonin gene-related peptide (CGRP) receptor antagonist. CGRP and its receptors are expressed in regions of the nervous system associated with migraine pathophysiology.

- CGRP receptor antagonist
- Oral formulation

Pivotal trial data:
The efficacy of rimegepant was evaluated in three Phase 3, randomized, double-blind, placebo-controlled studies. In two of the studies, a rimegepant oral tablet formulation was evaluated and in the other study an orally dissolving tablet (ODT) was evaluated. The co-primary endpoints were the proportion of patients headache pain-free and most bothersome symptom (MBS)-free (eg, sensitivity to light or sound, or nausea) at 2 hours post-dose.

In all three trials, rimegepant met both co-primary endpoints. Across the studies, more patients dosed with rimegepant were free of headache pain at 2 hours after dosing vs. placebo (19.2% to 21.2% vs. 10.9% to 14.2%, respectively). More patients treated with rimegepant were also free of their MBS vs. placebo (35.1 to 37.6% vs. 25.2% to 27.7%, respectively).

- Headache pain-free at 2 hrs post-dose: 19.2% to 21.2% vs. 10.9% to 14.2% with placebo
- MBS-free at 2 hrs post-dose: 35.1 to 37.6% vs. 25.2% to 27.7% with placebo

Safety:
The most common adverse events with rimegepant use were nausea and urinary tract infections.

- Common AEs: nausea, urinary tract infections

Dosing:
In the pivotal trials, rimegepant was administered orally as needed after onset of migraine headache.

- Dosing: as needed after onset of migraine headache

Rimegepant (continued...)

Competitive environment

If approved, rimegepant would provide an additional alternative to triptan drugs for the treatment of acute migraine headaches. Rimegepant was well tolerated and could be a treatment option in patients who either have contraindications or are non-responders to triptan therapy. An ODT formulation would also provide a convenience benefit in this patient population, as migraine sufferers often report nausea as well.

Rimegepant would likely be reserved as a second- or third-line agent due to the availability of generic triptan alternatives and a lack of head-to-head data vs. triptans, the well-established standard of care.

Rimegepant would also likely be the third novel therapy approved for acute migraine treatment in the span of a few months. Reyvow™ (lasmiditan), a selective serotonin 5-HT1F receptor agonist, was approved in October 2019 and ubrogepant, another oral CGRP antagonist, is likely to be approved in December 2019.

- Advantages: alternative to triptan therapies, well tolerated, ODT formulation
- Disadvantages: generically available alternatives, lack of head-to-head data vs. triptans, competing with Reyvow (lasmiditan) and potentially ubrogepant as an alternative to triptan therapies

Eptinezumab (Brand Name: To be determined)

Manufacturer: Alder BioPharmaceuticals
Expected FDA decision: 2/21/2020

Therapeutic use

Eptinezumab is in development for the preventive treatment of migraine in adults.

- Preventive treatment of migraine in adults

Clinical profile

Eptinezumab is another antagonist of CGRP; however, it is an IV administered monoclonal antibody.

- CGRP antagonist
- IV formulation
- Reduction in MMDs in episodic migraine patients: 3.9 to 4.3 vs. 3.2 with placebo
- Reduction in MMDs in chronic migraine patients: 7.7 to 8.2 vs. 5.6 with placebo
- Common AEs: nasopharyngitis, upper respiratory infection, urinary tract infection, nausea
- Dosing: once every 12 weeks

Pivotal trial data:
The efficacy of eptinezumab was evaluated in two Phase 3, randomized, double-blind, placebo-controlled studies (PROMISE 1 and PROMISE 2). PROMISE 1 was conducted in 888 patients with episodic migraine and PROMISE 2 was conducted in 1,072 patients with chronic migraine. The primary endpoint was the mean change from baseline in monthly migraine days (MMDs) over the 12-week treatment period.

In PROMISE 1, eptinezumab demonstrated a statistically significant reduction in MMDs from baseline over weeks 1 through 12 vs. placebo. Following the first quarterly infusion, patients treated with eptinezumab 300 mg experienced 4.3 fewer MMDs from baseline ($p = 0.0001$ vs. placebo) vs. 3.9 fewer MMDs with eptinezumab 100 mg ($p = 0.0179$ vs. placebo) vs. 3.2 fewer MMDs for placebo.

In PROMISE 2, eptinezumab also demonstrated a statistically significant reduction in MMDs vs. placebo. Patients treated with eptinezumab 300 mg experienced 8.2 fewer MMDs from baseline ($p < 0.0001$ vs. placebo) vs. 7.7 fewer MMDs with eptinezumab 100 mg ($p < 0.0001$ vs. placebo) vs. 5.6 fewer MMDs for placebo.

Safety:
The most common adverse events with eptinezumab use were nasopharyngitis, upper respiratory infection, urinary tract infection, and nausea.

Dosing:
In the pivotal trials, eptinezumab was administered via IV infusion once every 12 weeks.

Competitive environment

Eptinezumab would offer an additional option for the preventative treatment of migraine headaches and it can be dosed every 12 weeks.

However, eptinezumab requires IV administration and would be competing with self-injectable SC formulations of CGRP antagonists that were approved in 2018 for preventative treatment of migraine. SC administered CGRP antagonists include Aimovig® (erenumab), Ajovy® (fremanezumab), and Emgality® (galcanezumab), and these products are administered once a month. Ajovy can also be administered once every 3 months. Compared indirectly, eptinezumab does not appear to be clinically differentiated in efficacy or safety vs. existing CGRP antagonists.

For reference, the price of SC administered CGRP antagonists are $6,900 per year.

- Advantages: additional option for preventative migraine treatment, dosing every 12 weeks
- Disadvantages: alternatives available via self-administered SC injection, IV formulation appears to be similarly effective as SC formulations
- Reference WAC (SC formulations of CGRP antagonists) = $6,900 per year

Bempedoic acid (Brand Name: To be determined)

Manufacturer: Esperion
Expected FDA decision: 2/21/2020 (single-ingredient bempedoic acid); 2/26/2020 (fixed-dose combination of bempedoic acid/ezetimibe [Zetia®])

Therapeutic use

Bempedoic acid is in development as an adjunct to diet in combination with other lipid-lowering therapies (eg, maximally tolerated statins or ezetimibe) for the treatment of adults with primary hyperlipidemia who require additional lowering of low-density lipoprotein-cholesterol (LDL-C).

In the U.S., approximately 37% of the adult population has elevated LDL-C and an estimated 18 million individuals with atherosclerotic cardiovascular disease (ASCVD) live with elevated levels of LDL-C despite taking maximally tolerated lipid-modifying therapy.

- Treatment of adults with primary hyperlipidemia who require additional lowering of LDL-C

Clinical profile

Bempedoic acid is a novel ATP-citrate lyase inhibitor, a component of the cholesterol biosynthesis pathway. It works by reducing cholesterol biosynthesis and lowers LDL-C by up-regulating the LDL receptor. Bempedoic acid acts on the same pathway as statin therapies but has a different molecular target.

- ATP-citrate lyase inhibitor
- Oral formulation
- Placebo-controlled LDL-C reduction with single-ingredient bempedoic acid: 17% to 28%
- Placebo-controlled LDL-C reduction with bempedoic acid/ezetimibe: 29%
- Common AEs: uric acid and liver transaminase elevations
- Dosing: once daily

Pivotal trial data:
The efficacy of bempedoic acid was evaluated in four studies using a single-ingredient bempedoic acid tablet and one study evaluated a bempedoic acid/ezetimibe fixed-dose combination tablet. Two studies evaluated bempedoic acid in 3,008 patients with ASCVD on maximally-tolerated statins; two studies evaluated bempedoic acid in 613 patients with ASCVD, or at a high risk for ASCVD, considered statin averse; and one study evaluated the bempedoic acid/ezetimibe fixed-dose combination in 382 patients with ASCVD, or at high risk for ASCVD, on maximally tolerated statins.

Across the four studies evaluating the single-ingredient bempedoic acid, placebo-corrected LDL-C reduction was 17% to 28% with bempedoic acid. In the study evaluating the combination of bempedoic acid/ezetimibe, placebo-corrected LDL-C reduction was 29%.

Safety:
The most common adverse events with bempedoic acid use were uric acid and liver transaminase elevations.

Dosing:
In the pivotal trials, bempedoic acid was dosed orally once a day.

Bempedoic acid (continued...)

Competitive environment

Bempedoic acid would offer a novel MOA for the treatment of hyperlipidemia and adds an additional oral treatment option in patients who have contraindications to statins or who require additional lowering of LDL-C.

However, oral LDL-C lowering therapies are currently available generically, including different statin products and ezetimibe. The LDL-C reduction was also very modest compared to injectable proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitors, which can provide over 50% reduction in LDL-C.

An improvement in cardiovascular outcomes (eg, myocardial infarction, stroke, etc) has not yet been demonstrated with bempedoic acid and results of Esperion's cardiovascular outcomes study are not expected until 2022.

The projected WAC price for bempedoic acid is approximately $3,500 per year.

- Advantages: novel MOA, additional oral therapy for hyperlipidemia
- Disadvantages: generically available oral alternatives, modest LDL-C lowering vs. PCSK9 inhibitors, lack of cardiovascular outcomes data
- Projected WAC = ~$3,500 per year

Ozanimod (Brand Name: To be determined)

Manufacturer: Celgene
Expected FDA decision: 3/25/2020

Therapeutic use

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

MS is a chronic disorder of the central nervous system (CNS). MS typically starts with a relapsing-remitting course, in which episodes of worsening function (relapses) are followed by recovery periods (remissions). These remissions may not be complete and may leave patients with some degree of residual disability.

The overall estimated prevalence of MS in the U.S. may be as high as 1 million individuals and about 85% of patients are initially diagnosed with a relapsing form of MS.

- Treatment of relapsing forms of MS

Clinical profile

Ozanimod is a sphingosine 1-phosphate (S1P) receptor modulator that binds with high affinity selectively to S1P subtypes 1 (S1P1) and 5 (S1P5). Similar to other S1P receptor modulators, ozanimod causes lymphocyte retention in lymphoid tissues. The exact mechanism by which ozanimod exerts therapeutic effects in MS is unknown, but is believed to involve the reduction of lymphocyte migration into the CNS.

- S1P receptor modulator
- Oral formulation
- Adjusted ARR: 21% to 48% reduction vs. Avonex
- Common AEs: nasopharyngitis, headache, upper respiratory tract infection, hypertension, pharyngitis, urinary tract infections, increased ALT
- Dosing: once daily

Pivotal trial data:
The efficacy of ozanimod was evaluated in two, Phase 3, randomized, double-blind, active-controlled studies (SUNBEAM and RADIANCE) in a total of 2,666 MS patients. In both trials, oral ozanimod 0.5 mg and 1 mg were compared against intramuscular Avonex® (interferon beta-1a). In SUNBEAM, the primary endpoint was annualized relapse rates (ARR) during the 12-month treatment period. The primary endpoint of the RADIANCE trial was ARR over 24 months.

In the SUNBEAM trial, adjusted ARRs were 0.18 (95% CI: 0.14, 0.24) for ozanimod 1.0 mg, 0.24 (95% CI: 0.19 to 0.31) for ozanimod 0.5 mg, and 0.35 (95% CI: 0.28, 0.44) for Avonex. This represented a 31% ($p = 0.0013$) and 48% ($p < 0.0001$) reduction in ARR for ozanimod 0.5 mg and 1 mg, respectively vs. Avonex.

Similar results were found in the RADIANCE trial, with adjusted ARRs of 0.17 (95% CI: 0.14, 0.21) for ozanimod 1.0 mg, 0.22 (95% CI: 0.18, 0.26) for ozanimod 0.5 mg, and 0.28 (95% CI: 0.23, 0.32) for Avonex. This represented a 21% ($p = 0.0167$) and 38% ($p < 0.0001$) reduction in ARR for ozanimod 0.5 mg and 1 mg, respectively vs. Avonex.

Safety:
The most common adverse events with ozanimod use were nasopharyngitis, headache, upper respiratory tract infection, hypertension, pharyngitis, urinary tract infections, and increased ALT.

Dosing:
In the pivotal trials, ozanimod was dosed orally once a day.

Ozanimod (continued...)

Competitive environment

If approved, ozanimod would offer a selective S1P receptor modulator for the treatment of MS and can be dosed orally once a day. Ozanimod is also currently being evaluated for other conditions such as ulcerative colitis and Crohn's disease and if the results of the clinical trials are positive, it could be the first drug in this class with those indications.

However, there are many oral and injectable alternative products for relapsing forms of MS, including drugs in the same class. Ozanimod would also be a relatively late market entry – Novartis' non-selective S1P modulator, Gilenya® (fingolimod), has been available since 2010, and their selective S1P modulator, Mayzent® (siponimod), was launched in March 2019. In addition, ozanimod was not compared head-to-head against the other S1P modulators or any other oral MS drug.

For reference, the WAC price for Mayzent is approximately $90,000 per year.

- Advantages: additional oral selective S1P receptor modulator for MS, also in development for ulcerative colitis and Crohn's disease
- Disadvantages: alternatives available, late market entry, lack of head-to-head data vs. other oral MS drugs
- Reference WAC (Mayzent) = ~$90,000 per year

4th Quarter 2019

Extended generic pipeline forecast

OptumRx generic pipeline forecast

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
2019 Possible launch date					
CUVPOSA	Merz	glycopyrrolate	Oral solution	All	4Q-2019
PREPOPIK	Ferring Pharmaceuticals	citric acid/magnesium oxide/sodium picosulfate	Oral packet	All	4Q-2019
TRAVATAN Z	Alcon	travoprost	Ophthalmic	All	4Q-2019
DESONATE	LEO Pharma	desonide	Gel	All	4Q-2019
SUPRENZA	Citius/Akrimax	phentermine	Tablet, orally disintegrating	All	4Q-2019
VIVLODEX	Iroko/iCeutica	meloxicam	Capsule	All	4Q-2019
PRESTALIA	Symplmed	perindopril/amlodipine	Tablet	All	4Q-2019
APTENSIO XR	Rhodes	methylphenidate	Capsule, extended-release	All	4Q-2019
SAMSCA	Otsuka	tolvaptan	Tablet	All	4Q-2019
PYLERA	Allergan/Aptalis	bismuth subcitrate potassium/metronidazole/ tetracycline	Capsule	All	4Q-2019
FORTEO	Eli Lilly	teriparatide	Injection	All	4Q-2019
FERRIPROX	ApoPharma/Apotex	deferiprone	Tablet	All	4Q-2019
ZOHYDRO ER	Persion/Currax	hydrocodone	Capsule, extended-release	All	4Q-2019
NUVARING	Merck	etonogestrel/ethinyl estradiol	Vaginal ring	All	4Q-2019
RITUXAN	Genentech/Roche/Biogen Idec	rituxumab	Intravenous	All	4Q-2019
JADENU	Novartis	deferasirox	Tablet; oral granules	All	4Q-2019
RESTASIS	Allergan	cyclosporine	Ophthalmic	All	4Q-2019
OSMOPREP	Bausch Health	sodium biphosphate/sodium phosphate	Tablet	All	11-2019
AMELUZ	Biofrontera	aminolevulinic acid	Gel	All	11-2019
OMNARIS	Covis	ciclesonide	Intranasal	All	12-2019
THALOMID	Celgene	thalidomide	Capsule	All	12-2019
2020 Possible launch date					

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
MYCAMINE	Astellas	micafungin	Intravenous	All	2020
CIPRODEX	Alcon	ciprofloxacin/dexamethasone	Otic	All	2020
DORYX MPC	Mayne	doxycycline hyclate	Tablet, delayed-release	All	2020
SYNDROS	Insys Therapeutics	dronabinol	Oral solution	All	2020
DUREZOL	Alcon	difluprednate	Ophthalmic	All	2020
BYETTA	AstraZeneca	exenatide	Subcutaneous	All	2020
SAPHRIS	Allergan	asenapine	Tablet, sublingual	All	1H-2020
DALIRESP	AstraZeneca	roflumilast	Tablet	All	01-2020
SILENOR	Currax	doxepin	Tablet	All	01-2020
ELIGARD	QLT/Tolmar	leuprolide	Subcutaneous	All	03-2020
TAYTULLA	Allergan	ethinyl estradiol/ norethindrone/ferrous fumarate	Tablet	All	03-2020
MOXEZA	Alcon	moxifloxacin	Ophthalmic	All	03-2020
ZORTRESS	Novartis	everolimus	Tablet	All	03-2020
RENOVA	Bausch Health	tretinoin	Cream	All	03-2020
TOTECT	Cumberland	dexrazoxane	Injection	All	03-2020
APTIVUS	Boehringer Ingelheim	tipranavir	Capsule; oral solution	All	04-2020
DEPO-SUBQ PROVERA	Pfizer	medroxyprogesterone	Subcutaneous	All	05-2020
NYMALIZE	Arbor	nimodipine	Oral solution	All	05-2020
DEXILANT	Takeda	dexlansoprazole	Capsule, extended-release	All	06-2020
DENAVIR	Mylan	penciclovir	Cream	All	06-2020
ENTEREG	Merck	alvimopan	Capsule	All	2H-2020
TIROSINT	IBSA Institut Biochemique	levothyroxine	Capsule	All	2H-2020
ENBREL	Amgen	etanercept	Subcutaneous	All	2H-2020
VELPHORO	Fresenius	sucroferric oxyhydroxide	Tablet, chewable	All	3Q-2020
SYNERA	Galen	lidocaine/tetracaine	Transdermal patch	All	07-2020
PEGASYS	Roche	peginterferon alfa-2A	Subcutaneous	All	08-2020
PEG-INTRON	Merck	peginterferon alfa-2B	Subcutaneous	All	08-2020

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
MARQIBO KIT	Talon Therapeutics/Spectrum	vincristine	Intravenous	All	09-2020
TYKERB	Novartis	lapatinib	Tablet	All	09-2020
BIDIL	Arbor	isosorbide dinitrate/hydrazaline	Tablet	All	09-2020
TRUVADA	Gilead	emtricitabine/tenofovir	Tablet	200 mg/300 mg	09-2020
ATRIPLA	Gilead/Bristol-Myers Squibb	efavirenz/ emtricitabine/ tenofovir	Tablet	All	09-2020
KUVAN	BioMarin	sapropterin	Tablet; oral solution	All	10-2020
RISPERDAL CONSTA	Janssen	risperidone	Injection, extended-release	All	11-2020
XOLEGEL	Almirall	ketoconazole	Gel	All	11-2020
DULERA	Merck	formoterol fumarate/ mometasone furoate	Inhalation	All	11-2020
EPIDUO FORTE	Galderma	adapalene/benzoyl peroxide	Gel	All	12-2020
OFIRMEV	Mallinckrodt	acetaminophen	Intravenous	All	12-2020
ABSORICA	Sun	isotretinoin	Capsule	All	12-2020
TOVIAZ	Pfizer	fesoterodine	Tablet, extended-release	All	12-2020
MYDAYIS	Shire	amphetamine/ dextroamphetamine mixture	Capsule, extended-release	All	12-2020
2021 Possible launch date					
BEPREVE	Bausch Health	bepotastine	Ophthalmic	All	2021
ACTEMRA	Roche/Chugai	tocilizumab	Intravenous; subcutaneous	All	2021
KERYDIN	Pfizer	tavaborole	Topical solution	All	2021
VIIBRYD	Forest/Allergan	vilazodone	Tablet	All	2021
EMTRIVA	Gilead	emtricitabine	Capsule	All	1H-2021
AMITIZA	Sucampo/Takeda	lubiprostone	Capsule	All	01-2021
CRIXIVAN	Merck	indinavir	Capsule	All	02-2021
NORTHERA	H. Lundbeck	droxidopa	Capsule	All	02-2021
MYALEPT	Aegerion	metreleptin	Subcutaneous	All	02-2021

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
FORTICAL	Upsher-Smith	calcitonin salmon recombinant	Intranasal	All	02-2021
YONSA	Sun	abiraterone	Tablet	All	03-2021
IMPAVIDO	Knight Therapeutics	miltefosine	Capsule	All	03-2021
ACTOPLUS MET XR	Takeda	pioglitazone/metformin	Tablet, extended-release	All	03-2021
OVIDREL	EMD Serono/Merck	choriogonadotropin	Intramuscular; subcutaneous	All	03-2021
NEUPRO	UCB	rotigotine	Transdermal patch	All	03-2021
LYRICA CR	Pfizer	pregabalin	Tablet, extended-release	All	04-2021
ERAXIS	Pfizer	anidulafungin	Intravenous	All	04-2021
TECFIDERA	Biogen	dimethyl fumarate	Capsule, delayed-release	All	05-2021
ZOMIG	Impax/Grunenthal	zolmitriptan	Intranasal	All	05-2021
QUTENZA	Grunenthal	capsaicin	Transdermal patch	All	06-2021
PERFOROMIST	Mylan	formoterol fumarate	Inhalation	All	06-2021
APTIOM	Sunovion/Bial	eslicarbazepine	Tablet	All	06-2021
SEEBRI NEOHALER	Novartis	glycopyrrolate	Inhalation	All	06-2021
INTELENCE	Janssen	etravirine	Tablet	All	06-2021
FLOVENT HFA	GlaxoSmithKline	fluticasone propionate	Inhalation	All	2H-2021
ORENCIA	Bristol-Myers Squibb	abatacept	Intravenous; subcutaneous	All	07-2021
FERAHEME	AMAG Pharmaceuticals	ferumoxytol	Intravenous	All	07-2021
RESCULA	R-Tech Ueno	unoprostone isopropyl	Ophthalmic	All	07-2021
ALTRENO	Bausch Health	tretinoin	Lotion	All	08-2021
BALCOLTRA	Avion	levonorgestrel/ethinyl estradiol/ferrous bisglycinate	Tablet	All	08-2021
SUTENT	Pfizer	sunitinib	Capsule	All	08-2021
SELZENTRY	ViiV Healthcare	maraviroc	Tablet	All	08-2021
POMALYST	Celgene	pomalidomide	Capsule	All	08-2021
VERAMYST	GlaxoSmithKline	fluticasone fumarate	Intranasal	All	08-2021
JEVTANA KIT	Sanofi	cabazitaxel	Intravenous	All	09-2021
BYSTOLIC	Allergan	nebivolol	Tablet	All	09-2021

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
PRADAXA	Boehringer Ingelheim	dabigatran etexilate mesylate	Capsule	All	4Q-2021
INNOPRAN XL	Ani Pharmaceuticals	propranolol	Capsule, extended-release	All	10-2021
BIJUVA	TherapeuticsMD	estradiol/progesterone	Capsule	All	10-2021
APRISO	Bausch Health	mesalamine	Capsule, extended-release	All	10-2021
MIRCERA	Roche/Royalty Pharma	methoxy polyethylene glycol-epoetin beta	Subcutaneous	All	11-2021
ENTYVIO	Takeda	vedolizumab	Intravenous	All	11-2021
BRYHALI	Bausch Health	halobetasol	Lotion	All	11-2021
BROVANA	Sunovion	arformoterol	Inhalation	All	11-2021
ONEXTON	Bausch Health	clindamycin/benzoyl peroxide	Gel	All	12-2021
EPANED KIT	Silvergate	enalapril	Oral solution	All	12-2021
CHANTIX	Pfizer	varenicline	Tablet	All	12-2021
CAYSTON	Gilead	aztreonam lysine	Inhalation	All	12-2021
BETHKIS	Chiesi	tobramycin	Inhalation	All	12-2021
MYTESI	Napo	crofelemer	Tablet, delayed-release	All	12-2021
EXPAREL	Pacira	bupivacaine	Injection	All	12-2021
SUPREP BOWEL PREP KIT	Braintree	magnesium sulfate anhydrous/potassium sulfate / sodium sulfate	Oral solution	All	12-2021

+ = 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
2019 Possible launch date									
RVT-802	RVT-802	Enzyvant/Roivant	Tissue-based therapy	Congenital athymia	Implant	Filed NDA	12/2019	Yes	Yes
Zimhi	naloxone	Adamis	opioid antagonist	Opioid dependence	IM	Filed NDA	12/2019	No	No
MK-1602 (AGN-241689)	ubrogepant	Allergan/ Merck	calcitonin gene-related peptide (CGRP) receptor antagonist	Acute migraines	PO	Filed NDA	12/2019	No	No
IDP-123	IDP-123	Bausch Health	retinoid	Acne	TOP	Filed NDA	12/22/2019	No	No
Brinavess	vernakalant	Correvio	potassium channel blocker	Arrhythmia	IV	Filed NDA	12/24/2019	Yes	No
E-2006	lemborexant	Eisai/ Purdue	orexin receptor antagonist	Insomnia	PO	Filed NDA	12/27/2019	No	No
ITI-007	lumateperone	Intra-Cellular Therapies	antipsychotic	Schizophrenia	PO	Filed NDA	12/27/2019	No	No
TMC-278-LA	cabotegravir (long-acting)/ rilpivirine (long-acting)	ViiV Healthcare	HIV integrase inhibitor/ non-nucleoside reverse transcriptase inhibitor (NNRTI)	HIV	IM	Filed NDA	12/29/2019	Yes	No
S-265744 (S/GSK-1265744)	cabotegravir	ViiV Healthcare	HIV integrase inhibitor	HIV	PO	Filed NDA	12/29/2019	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
NRL-1	diazepam	Neurelis	benzodiazepine	Seizures	Intranasal	Filed NDA	2H2019	No	Yes
2020 Possible launch date									
Entyvio (SC formulation)	vedolizumab	Takeda	integrin receptor antagonist	Ulcerative colitis	SC	Filed sBLA	12/2019 - 1/2020	Yes	No
E-7438 (EPZ-6438)	tazemetostat	Epizyme/ Eisai	methyltransferase EZH2 inhibitor	Sarcoma	PO	Filed NDA	1/23/2020	Yes	Yes
Rykindo	risperidone ER	Luye	atypical antipsychotic	Schizophrenia/ Schizoaffective disorder	IM	Filed NDA	1/28/2020	Yes	No
FP-001 (LMIS)	leuprolide mesylate	Foresee	gonadotropin-releasing hormone (GnRH) analog	Prostate cancer	SC	Filed NDA	1/29/2020	Yes	No
AR-101	AR-101	Aimmune	peanut protein capsule	Peanut allergy	PO	Filed BLA	1/2020	Yes	No
BLU-285	avapritinib	Blueprint Medicines	selective KIT and PDGFRa inhibitor	Gastrointestinal stromal tumors	PO	Filed NDA	2/14/2020	Yes	Yes
Twirla	ethinyl estradiol/ levonorgestrel	Agile Therapeutics	hormonal combination contraceptive	Pregnancy prevention	TOP	Filed NDA	2/16/2020	No	No
BMS-927711 (BHV-3000)	rimegepant sulfate	Biohaven	calcitonin gene-related peptide (CGRP) receptor antagonist	Acute migraines	PO	Filed NDA	2/20/2020	No	No
ALD-403	eptinezumab	Alder	calcitonin gene-related peptide (CGRP) receptor antagonist	Migraine prevention	IV	Filed BLA	2/21/2020	No	No
ETC-1002	bempedoic acid	Esperion Therapeutics	ATP citrate lyase inhibitor	Hypercholesterolemia	PO	Filed NDA	2/21/2020	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ETC-1002/ ezetimibe	bempedoic acid/ ezetimibe	Esperion Therapeutics	ATP citrate lyase inhibitor/ cholesterol absorption inhibitor	Hypercholesterolemia	PO	Filed NDA	2/26/2020	No	No
APD-421	amisulpride	Acacia	dopamine receptor antagonist	Nausea/ vomiting	IV	Filed NDA	2/26/2020	No	No
RV-001 (Roche-1, R-1507)	teprotumumab	Horizon Therapeutics	insulin-like growth factor 1 (IGF-1) receptor antagonist	Thyroid eye disease	IV	Filed BLA	3/8/2020	Yes	Yes
ITCA-650 (sustained release exenatide)	exenatide sustained-release	Intarcia	glucagon-like peptide-1 (GLP-1) receptor agonist	Diabetes mellitus	SC implant	Filed NDA	3/9/2020	No	No
ZEBOV	VS-EBOV (rVSV-EBOV; rVSV-ZEBOV-GP)	Merck/ NewLink Genetics	vaccine	Ebola	IM	Filed BLA	3/14/2020	Yes	No
naloxone	naloxone	Insys Therapeutics	opioid antagonist	Opioid dependence	Intranasal	Filed NDA	3/15/2020	No	No
ASG-22M6E	enfortumab vedotin	Astellas/ Seattle Genetics	nectin-4 antagonist	Bladder cancer	IV	Filed BLA	3/15/2020	Yes	No
ET-105	lamotrigine	Eton	anticonvulsant	Epilepsy	PO	Filed NDA	3/17/2020	No	No
ozanimod	ozanimod	Celgene	sphingosine 1-phosphate 1 (S1PR1) and 5 (S1PR5) receptor modulator	Multiple sclerosis	PO	Filed NDA	3/25/2020	Yes	No
obeticholic acid	obeticholic acid	Intercept Pharmaceuticals	farnesoid X receptor (FXR) agonist	Nonalcoholic steatohepatitis	PO	Filed NDA	3/26/2020	Yes	No
Rizaport (VersaFilm)	rizatriptan	IntelGenx / Red Hill Biopharma	triptans	Acute migraines	PO	Filed NDA	3/26/2020	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
HTX-011	bupivacaine/ meloxicam	Heron Therapeutics	anesthetic/ Nonsteroidal Anti-inflammatory Drug (NSAID)	Pain	Instillation	Filed NDA	3/26/2020	No	No
SPARC-1028	paclitaxel injection concentrate for suspension	Sun Pharma Advanced Research Company (SPARC)	taxane	Breast Cancer; Lung Cancer; Pancreatic Cancer	IV	Filed NDA	1Q2020	No	No
Posidur	SABER-bupivacaine CR	Novartis/ Durect	local anesthetic	Pain	SC	Filed NDA	1Q2020	No	No
LCI-699	osilodrostat	Novartis	aldosterone synthase inhibitor	Cushing's syndrome	PO	Filed NDA	1Q2020	No	Yes
empagliflozin, linagliptin, metformin XR	empagliflozin, linagliptin, metformin XR	Eli Lilly/ Boehringer Ingelheim	sodium glucose co-transporter-2 (SGLT-2) inhibitor, dipeptidyl peptidase 4 (DPP4) inhibitor, biguanide	Diabetes mellitus	PO	Filed NDA	1Q2020	No	No
bimatoprost sustained release	bimatoprost sustained release	Allergan	prostaglandin agonist	Glaucoma	Implant	Filed NDA	4/1/2020	No	No
CNS-7056	remimazolam	Cosmo	benzodiazepine	Procedural sedation	IV	Filed NDA	4/3/2020	Yes	No
Men Quad TT	meningococcal polysaccharide (serogroups A, C, Y, and W135) tetanus toxoid conjugate vaccine	Sanofi	antibacterial	meninococcus/ tetanus	IM	Filed BLA	4/25/2020	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Ongentys	opicapone	Neurocrine Biosciences/ Bial/ Ono	catechol-O-methyltransferase (COMT) inhibitor	Parkinson's disease	PO	Filed NDA	4/26/2020	No	No
Trevyent	treprostinil	SteadyMed	prostacyclin analog	Pulmonary arterial hypertension	SC	Filed NDA	4/27/2020	Yes	Yes
DS-8201	trastuzumab deruxtecan	Daiichi Sankyo	HER2-targeting antibody-drug conjugate	Breast cancer	IV	Filed BLA	4/29/2020	Yes	No
isatuximab	isatuximab	Sanofi/ ImmunoGen	CD38 antagonist	Multiple myeloma/ Acute lymphoblastic leukemia or lymphoblastic lymphoma	IV	Filed BLA	4/30/2020	Yes	Yes
selumetinib	selumetinib	AstraZeneca/ Merck	selective MEK kinase inhibitor	Neurofibromatosis	PO	Filed NDA	4/2020 – 5/2020	Yes	Yes
SEP-225289 (DSP-225289, SEP-289)	dasotraline	Sumitomo Dainippon/ Sunovion	triple reuptake inhibitor	Eating disorders	PO	Filed NDA	5/14/2020	No	No
INCB-54828	pemigatinib	Incyte	selective FGFR1/2/3 inhibitor	Biliary tract cancer	PO	Filed NDA	5/2020	Yes	Yes
FMX-103	minocycline	Foamix	tetracyclines	Rosacea	TOP	Filed NDA	6/2/2020	No	No
NS-065	viltolarsen	Nippon Shinyaku	morpholino antisense oligonucleotide	Duchenne muscular dystrophy	IV	Filed BLA	6/2/2020	Yes	Yes
Bafiertam	monomethyl fumarate	Banner Life Sciences	Nrf2 pathway activator	Multiple sclerosis	PO	Tentative Approval	6/20/2020	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
abicipar pegol	abicipar pegol	Allergan	VEGF-A inhibitor	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	6/13/2020 - 7/13/2020	Yes	Yes
EVK-001	metoclopramide	Evoke Pharma	antidopiminergics	Diabetic gastroparesis	Intranasal	CRL	2Q2020	No	No
Bronchitol	mannitol	Pharmaxis	osmotic gradient enhancer; mucus clearance enhancer	Cystic fibrosis	INH	CRL	2Q2020	No	Yes
Rexista XR	oxycodone ER	IntelliPharmaCeutics	opioid agonist	Pain	PO	Not Approved	1H2020	No	No
insulin glargine	insulin glargine	Mylan/ Biocon	Long-acting insulin	Diabetes mellitus	SC	CRL	1H2020	No	No
IMMU-132	sacituzumab govitecan	Immunomedics	RS7-SN-38 antibody-drug conjugate	Breast cancer	IV	CRL	1H2020	Yes	No
NKTR-181	NKTR-181	Nektar	opioid agonist	Pain	PO	Not Approved	1H2020	No	No
SRP-4045	casimersen	Sarepta	morpholino antisense oligonucleotide	Duchenne muscular dystrophy	IV	InTrial	Mid-2020	Yes	Yes
Contepo	fosfomycin	Nabriva Therapeutics	cell wall inhibitor	Bacterial infections	IV	CRL	Mid-2020	Yes	No
RG-7916	risdiplam	Roche/ PTC Therapeutics	SMN2 splicing modifier	Spinal muscular atrophy	PO	InTrial	Mid-2020	Yes	Yes
Apealea (Paclical)	paclitaxel	Oasmia	taxane	Ovarian cancer	IV	InTrial	Mid-2020	Yes	Yes
TGR-1202	umbralisib	TG Therapeutics/ Rhizen	phosphoinositide-3 kinase (PI3K) delta inhibitor	Diffuse large B-cell lymphoma/ Chronic lymphocytic leukemia	PO	InTrial	Mid-2020	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Prochymal	remestemcel-L	Mesoblast/ JCR/ Mallinckrodt/ Osiris Therapeutics	mesenchymal stem cells	Graft vs. Host disease	IV	InTrial	Mid-2020	Yes	Yes
MOR-208 (MOR-00208, XmAB-5574)	tafasitamab	MorphoSys/ Xencor	CD-19 antagonist	Diffuse large B-cell lymphoma	IV	InTrial	Mid-2020	Yes	Yes
LOXO-292	selpercatinib	Eli Lilly/ Loxo Oncology	RET inhibitor	Solid tumors; non-small cell lung cancer; thyroid cancer	PO	InTrial	Mid-2020	Yes	No
Amphora	Amphora	Evofem Biosciences	spermicidal agent	Pregnancy prevention	VG	CRL	Mid-2020	No	No
PRO-140	leronlimab	CytoDyn	C-C chemokine receptor 5 (CCR5) antagonist	HIV; Graft vs. host disease	SC	InTrial	Mid-2020	Yes	Yes
QVM-149	indacaterol/ glycopyrronium bromide/ mometasone furoate	Novartis/ Sosei	long-acting beta 2 adrenergic receptor agonist (LABA)/ long-acting muscarinic receptor antagonist (LAMA)/ corticosteroid	Asthma	INH	InTrial	Mid-2020	No	No
JCAR-017	lisocabtagene maraleucel	Juno/ Celgene	chimeric antigen receptor (CAR) T cell therapy	Diffuse large B-cell lymphoma	IV	InTrial	Mid-2020	Yes	Yes
GSP-301	mometasone furoate/ olopatadine HCl	Glenmark	corticosteroid/ antihistamine	Allergic rhinitis	Intranasal	CRL	Mid-2020	No	No
Zepsyre	lurbinectidin (lurbinectedin)	PharmaMar/ Myriad Genetics	alkylating agent	Small cell lung cancer	IV	InTrial	Mid-2020	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
MitoGel	mitomycin C	UroGen	alkylating agent	Bladder cancer	Intravesical	InTrial	Mid-2020	No	Yes
3-F8 (Hu-3F8)	naxitamab	Y-mAbs Therapeutics	GD2 antagonist	Neuroblastoma	IV	InTrial	Mid-2020	Yes	Yes
Zynteglo (LentiGlobin)	lentiviral beta-globin gene transfer	Bluebird Bio	gene therapy	Beta-thalassemia	IV	InTrial	Mid-2020	Yes	Yes
EBP-994 (rEBP-994)	lonafarnib	Eiger Biopharmaceuticals	prenylation inhibitor	Hutchinson-Gilford Progeria Syndrome (HGPS or progeria) and progeroid laminopathies; Hepatitis D (HDV)	PO	InTrial	Mid-2020	Yes	Yes
Darzalex	daratumumab (with recombinant human hyaluronidase)	Johnson & Johnson / Genmab	humanized anti-CD38 monoclonal antibody	Multiple myeloma/ Amyloidosis	SC	Filed BLA	7/10/2020	Yes	Yes
Fintepla	fenfluramine	Zogenix	serotonin receptor agonist	Dravet syndrome	PO	Filed NDA	7/24/2020	Yes	Yes
MC2-01	calcipotriene/ betamethasone	MC2 Therapeutics	vitamin D analog/ corticosteroid	Psoriasis	TOP	Filed NDA	7/24/2020	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
Viaskin Peanut	Viaskin Peanut	DBV Technologies	Immunotherapy	Peanut allergy	TOP	Filed BLA	8/5/2020	No	No
TRC-101	veverimer	Tricida	carrier protein modulator	Chronic kidney disease	PO	Filed NDA	8/22/2020	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Winlevi	clascoterone	Cassiopea	androgen antagonist	Acne vulgaris	TOP	Filed NDA	8/27/2020	No	No
SA-237 (RG-6168)	satralizumab	Roche/ Chugai	interleukin-6 (IL-6) monoclonal antibody	Neuromyelitis optica	SC	Filed BLA	8/2020 – 9/2020	Yes	Yes
collagenase clostridium histolyticum	collagenase clostridium histolyticum	Endo	protease enzyme	Cellulite	SC	Filed BLA	9/6/2020	Yes	No
VP-102	VP-102	Verrica	antiviral	Molluscum	TOP	Filed NDA	9/16/2020	No	No
NNC-0195-0092 (NN-8640)	somapacitan	Novo Nordisk	recombinant human growth hormone (rhGH)	Growth hormone deficiency	SC	Filed BLA	9/21/2020	Yes	No
LY-900014 (URLi)	insulin lispro	Eli Lilly	insulins	Diabetes mellitus	SC	Filed BLA	3Q2020	No	No
BMN-270	valoctocogene roxaparvovec	BioMarin	gene therapy	Hemophilia	IV	InTrial	3Q2020	Yes	Yes
efavirenz	efavirenz	Micro Labs	non-nucleoside reverse transcriptase inhibitor (NNRTI)	HIV	PO	Tentative Approval	3Q2020	Yes	No
GSK-2857916	belantamab mafodotin	GlaxoSmithKline/ Seattle Genetics	anti-BCMA antibody-drug conjugate	Multiple myeloma	SC	InTrial	3Q2020	Yes	Yes
PPP-001	delta-9-tetrahydrocannabinol/ cannabidiol	PhytoPain Pharma	cannabinoid product	Pain	INH	InTrial	3Q2020	Yes	Yes
BCX-7353	BCX-7353	BioCryst	kallikrein inhibitor	Hereditary angioedema	PO	InTrial	3Q2020	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
SPI-2012	eflapegrastim	Spectrum/ Hanmi	granulocyte colony-stimulating factor (GCSF)	Neutropenia	SC	Filed BLA	10/25/2020	Yes	No
CAM-2038	buprenorphine	Camurus/ Braeburn	opioid receptor agonist (partial)	Opioid use disorder/ Pain	SC	Tentative Approval	11/1/2020	Yes	No
SPN-812	SPN-812	Supernus	selective norepinephrine reuptake inhibitor	Attention deficit hyperactivity disorder	PO	Filed NDA	11/11/2020	No	No
Melflufen (Ygalo)	melphalan-flufenamide	Oncopeptides AB	alkylating agent/ DNA synthesis inhibitor	Multiple myeloma/ Non-small cell lung cancer/ Ovarian cancer	IV	InTrial	4Q2020	No	Yes
DCC-2618	ripretinib	Deciphera	PDGFR-alpha kinase inhibitor	Gastrointestinal stromal tumors	PO	InTrial	4Q2020	Yes	Yes
ALN-PCSsc (PCSK9si)	inclisiran	The Medicines Company/ Alnylam	proprotein convertase subtilisin/kexin 9 (PCSK-9) inhibitor	Hyperlipidemia	SC	InTrial	4Q2020	Yes	Yes
ARRY-380 (ONT-380)	tucatinib	Seattle Genetics/ Array BioPharma	ErbB-2 (Her-2/neu) inhibitor	Breast cancer	PO	InTrial	4Q2020	Yes	Yes
BLU-667	pralsetinib	Blueprint Medicines	RET inhibitor	Non-small cell lung cancer	PO	InTrial	4Q2020	Yes	Yes
GLPG-0634	filgotinib	Gilead/ Galapagos	janus associated kinase-1 (JAK) inhibitor	Rheumatoid arthritis	PO	InTrial	4Q2020	Yes	No
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension	INH	InTrial	4Q2020	Yes	No
ofatumumab (OMB-157)	ofatumumab	Novartis	CD20 monoclonal antibody	Multiple sclerosis	SC	InTrial	4Q2020	Yes	No
Infacort	hydrocortisone	Diurnal Group	corticosteroid	Adrenal insufficiency	PO	InTrial	4Q2020	No	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
TAK-385	TAK-385	Myovant Sciences/ Roivant Sciences/ Takeda	gonadotropin-releasing hormone (GnRH) receptor antagonist	Uterine fibroids/ Endometriosis	PO	InTrial	4Q2020	Yes	No
tramadol	tramadol	Avenue Therapeutics	opioid receptor agonist	Pain	IV	InTrial	4Q2020	No	No
AmnioFix	dehydrated human amnion/chorion membrane (dHACM)	MiMedx	amniotic tissue membrane	Plantar fasciitis/ Achilles tendonitis/ Osteoarthritis	INJ	InTrial	4Q2020	Yes	No
Estelle	estetrol/ drospirenone	Mithra/ Fuji/ Zhejian Xianju	estrogen receptor agonist	Pregnancy prevention	PO/SL	InTrial	4Q2020	No	No
Qtrypta	zolmitriptan	Zosano	triptans	Acute migraines	TOP	InTrial	4Q2020	No	No
R-667 (RG-667)	palovarotene	Clementia/ Roche	selective retinoic acid receptor agonist (RAR-gamma)	Fibrodysplasia ossificans progressiva (FOP)	PO	InTrial	4Q2020	Yes	Yes
ALKS-3831	olanzapine/ samidorphan	Alkermes	dopamine receptor antagonist/ opioid receptor antagonist	Schizophrenia/ Bipolar disorder	PO	InTrial	4Q2020	No	No
REGN-EB3	REGN-EB3	Regeneron	anti-Ebola virus	Ebola	IV	InTrial	4Q2020	Yes	Yes
FG-4592	roxadustat	FibroGen/ AstraZeneca	hypoxia-inducible factor prolyl hydroxylase (HIF-PHI)	Anemia	PO	InTrial	2H2020	Yes	No
Doria	risperidone	Laboratorios Farmacéuticos Rovi	atypical antipsychotic	Schizophrenia	IM	InTrial	2H2020	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Traumakine	interferon-beta - 1a	Faron/ Maruishi	interferon	Acute respiratory distress syndrome	IV	InTrial	2H2020	Yes	No
NX-1207 (NYM-4805, REC 0482)	fexapotide triflutate	Nymox	pro-apoptotic	Benign prostatic hyperplasia	Intratumoral	InTrial	2H2020	Yes	No
bb-2121	idecabtagene vicluecel	Celgene/ Bluebird Bio	chimeric antigen receptor (CAR) T cell therapy	Multiple myeloma	IV	InTrial	2H2020	Yes	Yes
131I-8H9	omburtamab	Y-mAbs Therapeutics	B7-H3 antagonist	Brain cancer	Undisclosed	InTrial	2H2020	Yes	Yes
IdeS	imlifidase	Hansa Medical	bacterial enzyme	Kidney transplant	IV	InTrial	2H2020	Yes	Yes
ASTX-727	decitabine and E-7727	Otsuka/ Astex Pharmaceuticals	nucleoside metabolic inhibitor	Myelodysplastic syndrome	PO	InTrial	2H2020	Yes	Yes
Sci-B-Vac	hepatitis B vaccine	VBI Vaccines	vaccine	Hepatitis B	IM	InTrial	2H2020	No	No
LJPC-0118	LJPC-0118	La Jolla Pharmaceutical	protozoacide	Malaria	Undisclosed	InTrial	2H2020	No	No
BMS-663068	fostemsavir	Bristol-Myers Squibb	HIV attachment inhibitor	HIV	PO	InTrial	2H2020	Yes	No
Zeftera	ceftobiprole	Basilea	cephalosporin antibiotic	Bacterial infections	IV	InTrial	2H2020	Yes	No
AGIL-AADC	AGIL-AADC	Agilis Biotherapeutics	gene therapy	Aromatic L-amino acid decarboxylase deficiency	Intracerebral	InTrial	2H2020	Yes	Yes
KTE-X19	KTE-X19	Gilead	chimeric antigen receptor (CAR) T cell therapy	Mantle cell lymphoma	IV	InTrial	2H2020	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
CLS-1001	triamcinolone acetonide	Clearside	corticosteroid	Macular edema	intraocular/ subretinal	CRL	2H2020	Yes	No
EM-100	ketotifen	Eton	antihistamine	Allergic conjunctivitis/ Dry eyes	OP	CRL	2H2020	No	No
MAGH-22	margetuximab	MacroGenics/ Green Cross	HER2 oncoprotein antagonist	Breast cancer	IV	InTrial	2H2020	Yes	No
KPI-121 0.25%	loteprednol etabonate	Kala	corticosteroid	Dry eyes	OP	CRL	2H2020	No	No
ropeginterferon alfa-2b	ropeginterferon alfa-2b	PharmaEssentia/ AOP Orphan	interferon	Polycythemia vera	SC	InTrial	2H2020	Yes	Yes
KP-415	D-threo-methylphenidate controlled-release	KemPharm	CNS stimulant	Attention deficit hyperactivity disorder	PO	InTrial	2H2020	No	No
Iomab-B	iodine I 131 monoclonal antibody BC8	Actinium	anti-CD45 monoclonal antibody	Acute myeloid leukemia/ Myelodysplastic syndrome	IV	InTrial	2H2020	Yes	Yes
quizartinib	quizartinib	Daiichi Sankyo	FLT-3 receptor tyrosine kinase inhibitor	Acute myeloid leukemia	PO	CRL	2H2020	Yes	Yes
EGP-437	dexamethasone phosphate (iontophoretic)	EyeGate	corticosteroid	Uveitis	OP	InTrial	2H2020	Yes	No
Libervant	diazepam	Aquestive Therapeutics	benzodiazepine	Seizures	SL	InTrial	2H2020	No	Yes
TRV-130	oliceridine	Trevena	opioid receptor agonist	Pain	IV	CRL	2H2020	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
arimoclomol	arimoclomol	Orphazyme	cytoprotectives	Niemann-Pick Disease/ Sporadic Inclusion Body Myositis/ Amyotrophic lateral sclerosis	PO	InTrial	2H2020	Yes	Yes
Mycapssa (Octreolin)	octreotide	Chiasma	somatostatin analog	Acromegaly	PO	CRL	2H2020	Yes	Yes
PRT-201	vonapanitase	Proteon Therapeutics	human elastase (recombinant)	End stage renal diease/ Peripheral artery disease/ Vascular access in hemodialysis	TOP	InTrial	2H2020	Yes	Yes
PRX-102	alpha galactosidase	Protalix	enzyme replacement	Fabry disease	IV	InTrial	2H2020	Yes	No
INCB-028060 (INC-280)	capmatinib	Novartis/ Incyte	cMET inhibitor	Non-small cell lung cancer	PO	InTrial	2H2020	Yes	Yes
ET-103	levothyroxine	Eton Pharmaceuticals	L-thyroxine	Hypothyroidism	PO	InTrial	2H2020	No	No
Vicinium (VB-4-845)	oportuzumab monatox	Eleven Biotherapeutics	anti-ECAM exotoxin A fusion protein	Bladder cancer	Intravesical	InTrial	2H2020	Yes	No
RT-002	daxibotulinumtoxinA	Revance Therapeutics	botulinum toxins	Glabellar lines (frown lines)	IM	InTrial	2H2020	Yes	Yes
N-1539	meloxicam	Recro Pharma/ Alkermes	nonsteroidal anti-inflammatory drug (NSAID)	Pain	IV	CRL	2020	Yes	No
QMF-149	indacaterol maleate/ mometasone furoate	Novartis/ Merck	long-acting beta 2 agonist/ corticosteroid	Asthma	INH	InTrial	2020	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ND-0612L	levodopa/ carbidopa	NeuroDerm	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	SC	InTrial	2020	Yes	No
CPP-1X/ sulindac (DFMO)	eflornithine/ sulindac	Cancer Prevention Pharma/ Zeria	ornithine decarboxylase inhibitor/ non-steroidal anti-inflammatory drug (NSAID)	Familial adenomatous polyposis/ Colorectal cancer	PO	InTrial	2020	Yes	Yes
Zalviso	sufentanil, ARX-01	AcelRx	opioid analgesic	Pain	SL	CRL	2020	No	No
Deltyba	delamanid	Otsuka	mycolic acid biosynthesis inhibitor	Tuberculosis	PO	InTrial	2020	No	No
Pedmark (STS)	sodium thiosulfate	Fennec	reducing agent	Hearing loss	IV	InTrial	2020	Yes	Yes
ND-0612H	levodopa/ carbidopa	NeuroDerm	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	SC	InTrial	2020	Yes	No
DS-200	DS-200	Eton	undisclosed	Ophthalmological disease	SC	InTrial	2020	No	No
ELI-200	oxycodone/ naltrexone	Elite	opioid agonist	Pain	PO	CRL	2020	No	No
VivaGel	astodrimer sodium (SPL-7013)	Starpharma	viral attachment inhibitor	Bacterial infections	VG	CRL	2020	No	No
Dexasite	dexamethasone	InSite Vision	corticosteroid	Blepharitis/ Ocular inflammation	TOP	InTrial	2020	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ublituximab (LFB-R603, TG20, TGTX-1101, TG-1101, Utuxin)	ublituximab	TG Therapeutics	CD-20 monoclonal antibody	Chronic lymphocytic leukemia/ Small cell lymphocytic lymphoma/ Mantle cell lymphoma/ Multiple sclerosis	IV	InTrial	2020	Yes	Yes
CCP-08	CCP-08	Tris Pharma	undisclosed	Viral rhinitis	PO	CRL	2020	No	No
NeoCart	autologous chondrocyte tissue implant	Histogenics/ Purpose	autologous chondrocyte tissue implant	Joint repair	Undisclosed	InTrial	2020	Yes	No
APN-311	dinutuximab beta	EUSA Pharma	disialoganglioside	Neuroblastoma	SC	InTrial	2020	Yes	Yes
tamsulosin DRS	tamsulosin delayed-release	Veru	alpha-adrenergic antagonist	Benign prostatic hyperplasia	PO	InTrial	2020	No	No
Oralair Mites (Actair)	dust mite peptide	Stallergenes/ Shionogi	vaccine	Dust mite allergic rhinitis	SL	InTrial	2020	Yes	No
Travivo	gepirone ER	GSK/Fabre-Kramer	5-HT-1A receptor agonist	Major depressive disorder	PO	CRL	2020	No	No
MNK-812	oxycodone	Mallinckrodt	opioid agonist	Pain	PO	CRL	2020	No	No
APL-130277	apomorphine	Sumitomo Dainippon/ MonoSol Rx/ Sunovion	non-ergoline dopamine agonist	Parkinson's disease	SL	CRL	2020	No	No
tadalafil VersaFilm	tadalafil VersaFilm	IntelGenx	phosphodiesterase-5 (PDE-5) inhibitor	Erectile dysfunction	PO	Filed NDA	2020	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Multikine	Leukocyte Interleukin (CS-001P3)	CEL-SCI	immunomodulator	Head and Neck cancer/ Squamous cell carcinoma	SC	InTrial	2020	Yes	Yes
APC-8000	tadalafil	Adamis	phosphodiesterase-5 (PDE-5) inhibitor	Erectile dysfunction	PO	CRL	2020	Yes	No
Corplex donepezil	donepezil	Corium International	anticholinergic	Alzheimer's disease	TOP	InTrial	Late 2020	No	No
CC-486	azacitidine	Celgene	DNA methylation inhibitor	Acute myeloid leukemia/ Myelodysplastic syndromes	PO	InTrial	Late 2020	Yes	Yes
Linhaliq	ciprofloxacin	Aradigm/ Grifols	fluoroquinolone	Non-cystic fibrosis bronchiectasis/ Cystic fibrosis	INH	CRL	Late 2020	Yes	Yes
MLN-4924 (TAK-92)	pevonedistat	Takeda	Nedd 8 Activating Enzyme (NAE) antagonist	Acute myeloid leukemia/ Chronic myelogenous leukemia/ Myelodysplastic syndrome	PO	InTrial	Late 2020	Yes	No
tanezumab	tanezumab	Pfizer/ Eli Lilly	neurotrophic tyrosine kinase receptor type 1 (TrkA) antagonist (monoclonal antibody)	Osteoarthritis/ Pain	IV/SC	InTrial	Late 2020	Yes	No
BIVV-009 (TNT-009)	sutimlimab	Sanofi	complement C1s subcomponent inhibitor	Cold agglutinin disease	IV	InTrial	Late 2020	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Lucassin	terlipressin	Orphan Therapeutics/ Ikaria	V-1 (vasopressin) agonist	Hepato-renal syndrome	IV	CRL	Late 2020	Yes	Yes
iclaprim	iclaprim	Motif Bio	tetrahydrofolate dehydrogenase inhibitor	Bacterial infections	IV	CRL	Late 2020	Yes	Yes
SRP-4053	golodirsen	Sarepta	morpholino antisense oligonucleotide	Duchenne muscular dystrophy	IV	CRL	Late 2020	Yes	Yes
NS-2 (ALDX-1E1, ALDX-1E2, ADX-102)	reproxalap	Aldeyra Therapeutics	aldehyde antagonist	Allergic conjunctivitis/ Dry eyes	OP	InTrial	Late 2020	No	No
Ryplazim	human plasminogen	ProMetic/ Hematech	plasminogen	Plasminogen deficiency	IV	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
Lenti-D	elivaldogene tavalentivec	Bluebird Bio	gene therapy	Cerebral adreno-myeloneuropathy	IV	InTrial	Late 2020	Yes	Yes
TG-1303	ublituximab/ TGR-1202	TG Therapeutics	CD-20 monoclonal antibody/ phosphoinositide-3 kinase (PI3K) delta inhibitor	Chronic lymphocytic leukemia/ Diffuse large B-cell lymphoma/ Non-Hodgkin lymphoma	IV/PO	InTrial	Late 2020	Yes	Yes
MVA-MUC1-IL2	TG-4010	Transgene	vaccine	Non-small cell lung cancer	SC	InTrial	Late 2020	No	No
SHP-621	budesonide	Shire	corticosteroid	Eosinophilic esophagitis	PO	InTrial	Late 2020	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
HuMax-TF ADC	tisotumab vedotin	Genmab/ Seattle Genetics	tissue factor antibody	Solid tumors	Undisclosed	InTrial	Late 2020	Yes	No
BMN-111	vosoritide (vasoritide)	BioMarin/ Chugai	C-type natriuretic peptide (CNP) analog	Achondroplasia	SC	InTrial	Late 2020	Yes	Yes
TSR-042	dostarlimab	AnaptysBio	PD-1 checkpoint inhibitor	Endometrial cancer	IV	InTrial	Late 2020	Yes	No
JZP-258	sodium oxybate extended-release	Jazz	dopamine receptor agonist	Narcolepsy	PO	InTrial	Late 2020	Yes	No
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	CRL	Late 2020	Yes	Yes
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
Oraxol	HM-30181A/ paclitaxel	Athenex	P-glycoprotein pump inhibitor/ taxane	Breast cancer	PO	InTrial	Late 2020	Yes	Yes
BIM-22493 (RM-493)	setmelanotide	Rhythm/ Camurus/ Ipsen	melanocortin 4 receptor (MC4R) agonist	Rare genetic disorders of obesity	SC	InTrial	Late 2020	Yes	Yes
RG-3477 (ACT-128800)	ponesimod	Johnson & Johnson	sphingosine 1 phosphate receptor agonist	Multiple sclerosis	PO	InTrial	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
2021 Possible launch date									
ALNG-01 (ALN-G-01)	lumasiran	Alnylam	glycolate oxidase antagonist	Hyperoxaluria	Intranasal	InTrial	1Q2021	Yes	Yes
MK-4618 (KRP-114V, RVT-901)	vibegron	Roivant Sciences/ Urovant/ Kissei/ Kyorin/ Merck	selective beta 3 adrenergic receptor agonist	Overactive bladder	PO	InTrial	1Q2021	No	No
BIIB-037	aducanumab	Biogen Idec/ Eisai	amyloid beta-protein inhibitor	Alzheimer's disease	IV	InTrial	1Q2021	Yes	No
KX-01 (KX2-391)	tirbanibulin	Athenex	Src kinase and tubulin inhibitor	Actinic keratosis	PO	InTrial	1Q2021	Yes	No
ZP-4207 (ZP-GA-1)	dasiglucagon	Zealand Pharma	glucagon analog	Diabetes mellitus	SC	InTrial	1Q2021	No	Yes
Furoscix	furosemide	scPharmaceuticals	diuretic	Heart failure	SC	CRL	1Q2021	Yes	No
SDP-037, SDN-037	SDP-037, SDN-037	Sun Pharma Advanced Research Company (SPARC)	Corticosteroid	Ocular inflammation/pain	OP	InTrial	2Q2021	No	No
SCY-078	ibrexafungerp	Scynexis	glucan synthase inhibitors	Fungal infections	IV/PO	InTrial	1H2021	No	Yes
ACP-001	TransCon Growth Hormone	Ascendis	growth hormone prodrug	Short stature/ growth hormone deficiency	SC	InTrial	1H2021	Yes	No
RGN-259 (GBT-201; RGN-352)	thymosin beta 4	RegeneRx	actin regulating peptide	Neurotrophic keratitis/ Dry eyes	OP	InTrial	1H2021	No	Yes
CM-AT	CM-AT	Curemark	protein absorption enhancer	Autism	PO	InTrial	1H2021	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
GFT-505	elafibranor	Genfit	selective peroxisome proliferator-activated receptor (PPAR) modulator	Non-alcoholic steatohepatitis/ Primary biliary cirrhosis	PO	InTrial	1H2021	Yes	No
NexoBrid	bromelain	MediWound/ BL&H/ CrystalGenomics/ Kaken	peptide hydrolase replacement agent	Burns/ Skin injury	TOP	InTrial	1H2021	No	Yes
PDR-001	PDR-001	Novartis	PD-1 checkpoint inhibitor	Melanoma	IV	InTrial	1H2021	Yes	No
CCX-168	avacopan	ChemoCentryx/ Galencia	C5a receptor (C5aR) antagonist	Vasculitis	PO	InTrial	1H2021	Yes	Yes
StrataGraft Skin Tissue	StrataGraft Skin Tissue	Mallinckrodt	autologous skin tissue	Burn injury	TOP	InTrial	1H2021	Yes	Yes
NPI-2358	plinabulin	BeyondSpring	tumor vascular disrupting agent	Neutropenia	IV	InTrial	1H2021	Yes	No
GZ-402665	olipudase alfa	Sanofi	sphingomyelinase	Niemann-Pick disease type B	IV	InTrial	Mid-2021	Yes	Yes
AT-132 (AAV8-MTM1)	AT-132 (AAV8-MTM1)	Audentes Therapeutics	gene therapy	X-linked myotubular myopathy	IV	InTrial	Mid-2021	Yes	Yes
GZ-402666 (NeoGAA)	avalglucosidase alfa	Sanofi	enzyme therapy	Pompe disease	IV	InTrial	Mid-2021	Yes	No
EBV-CTL (ATA-129)	tabelecleucel	Atara Biotherapeutics/ Memorial Sloan-Kettering Cancer Center	cell therapy	Lymphoproliferative disorder	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
RSV-F (ResVax)	respiratory syncytial virus vaccine	Novavax	vaccine	Respiratory syncytial virus infection	IM	InTrial	Mid-2021	Yes	No
entinostat	entinostat	Syndax	histone deacetylase (HDAC) inhibitor	Breast cancer	PO	InTrial	Mid-2021	Yes	No
ADCT-402	loncastuximab tesirine	ADC Therapeutics	antibody drug conjugate	Diffuse large B-cell lymphoma	IV	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
AXS-07	meloxicam/rizatriptan	Axsome Therapeutics	non-steroidal anti-inflammatory drug/triptan	Migraine	PO	InTrial	Mid-2021	No	No
ALN-APC (ALN-AT3)	fitusiran	Alnylam/ Sanofi	RNAi therapeutic	Hemophilia	SC	InTrial	Mid-2021	Yes	Yes
WVE-210201	suvodirsen	Wave Life Sciences	oligonucleotide	Duchenne muscular dystrophy	IV	InTrial	Mid-2021	Yes	Yes
PDP-716	brimonidine	Sun Pharma Advanced Research Company (SPARC)	alpha-2 agonist	Glaucoma	OP	InTrial	3Q2021	No	No
PRV-031	teplizumab	MacroGenics/ Provention Bio	CD3 antigen inhibitor	Diabetes mellitus	IV	InTrial	4Q2021	Yes	Yes
TBR-652	cenicriviroc	Tobira Therapeutics/ Takeda	C-C chemokine receptor 5 (CCR5) and receptor 2 antagonist	Non-alcoholic steatohepatitis	PO	InTrial	4Q2021	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
BXCL-501	dexmedetomidine	BioXcel Therapeutics	selective alpha 2a receptor agonist	Schizophrenia and bipolar disorder	PO	InTrial	2H2021	No	No
UCB-4940 (CDP-4940)	bimekizumab	UCB	interleukin-17 (IL-17) receptor inhibitor	Psoriasis/ Psoriatic arthritis/ Ankylosing spondylitis/ Rheumatoid arthritis	IV	InTrial	2H2021	Yes	No
177Lu-PSMA-617	Lutetium	Endocyte	Radiopharmaceutical	Prostate cancer	IV	InTrial	2H2021	Yes	No
OS-01	OC-01	Oyster Point Pharma	nicotinic acetylcholine receptor (nAChR) agonist	Dry eye disease	Intranasal	InTrial	2H2021	No	No
SHP-647	SHP-647	Shire	MAdCAM-1 antagonist	Irritable bowel disease	IV/SC	InTrial	2H2021	Yes	Yes
IDP-124	pimecrolimus	Bausch Health	calcineurin Inhibitor	Atopic dermatitis	TOP	InTrial	2H2021	No	No
glatiramer acetate depot	glatiramer acetate long-acting	Mylan/ Mapi Pharma	immunosuppressant	Multiple sclerosis	IM	InTrial	2H2021	Yes	No
KD-025	KD-025	Kadmon	ROCK2 (Rho-associated coiled-coiled kinase 2) inhibitor	Graft vs. Host disease	PO	InTrial	2H2021	No	Yes
AXS-05	dextromethorphan/ bupropion	Axsome	N-methyl-D-aspartate (NMDA) antagonist	Treatment-resistant depression	PO	InTrial	2H2021	No	No
Otividex	dexamethasone sustained-release	Otonomy	corticosteroid	Meniere's disease	Intratympanic	InTrial	2H2021	Yes	No
MEDI-546	anifrolumab	AstraZeneca/ BMS	interferon receptor antagonist	Systemic lupus erythematosus	IV	InTrial	2H2021	Yes	No
LN-145	lifileucel	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Cervical Cancer	IV	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
INP-104	POD-dihydroergotamine mesylate (POD-DHE)	Impel/ 3M	ergot derivative	Acute migraines	Intranasal	InTrial	2H2021	No	No
SPN-810	molindone	Supernus	atypical antipsychotic	Attention deficit hyperactivity disorder	PO	InTrial	2H2021	No	No
GS-010	GS-010	GenSight Biologics	gene therapy	Optic neuropathy	Intraocular	InTrial	2H2021	Yes	Yes
TWIN (S6G5T-1; S6G5T-3)	benzoyl peroxide/ tretinoin	Sol-Gel Technologies	retinoid	Acne vulgaris	TOP	InTrial	2H2021	No	No
AMAG-423	digoxin immune fab (DIF)	AMAG/ Velo	digitalis-like factor antagonist	Preeclampsia	IV	InTrial	2H2021	Yes	Yes
CR-845	difelikefalin	Cara Therapeutics	opioid receptor agonist	Pruritus	IV/PO	InTrial	2H2021	No	No
pIL-12	tavokinogene telsaplasmid	OncoSec Medical	gene therapy	Melanoma	Intratumoral	InTrial	2H2021	Yes	Yes
MD-1003	MD-1003	MedDay	biotin	Multiple sclerosis	PO	InTrial	2H2021	Yes	No
VBP-15	vamorolone	Santhera	corticosteroid	Duchenne muscular dystrophy	PO	InTrial	2H2021	Yes	Yes
ZYN-002	ZYN-002	Zynerba	cannabinoid product	Fragile X syndrome	TOP	InTrial	2H2021	Yes	Yes
PL-56	budesonide	Calliditas/ Kyowa Hakko Kirin	corticosteroid	Nephropathy	PO	InTrial	2H2021	No	No
dovitinib	dovitinib	Oncology Venture	fibroblast growth factor receptor 3 (FGFR3) inhibitor	Renal cell carcinoma	PO	InTrial	2H2021	Yes	No
Edsivo	celiprolol HCl	Acer Therapeutics	alpha-2/beta-1 adrenergic agent	vascular Ehlers-Danlos Syndrome	PO	CRL	2021	Yes	Yes
Tlando	testosterone	Lipocine	androgen	Hypogonadism	PO	CRL	2021	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
PRO-145223	etrolizumab	Genentech	IgG1 monoclonal antibody	Ulcerative colitis	SC	InTrial	2021	Yes	No
POL-6326	balixafortide	Polyphor	chemokine (CXCR4) antagonist	Transplant/ Breast cancer	IV	InTrial	2021	Yes	No
idebenone	idebenone	Santhera	co-enzyme Q-10 analog	Duchenne muscular dystrophy	PO	InTrial	2021	Yes	Yes
ABL-001	asciminib	Novartis	allosteric Bcr-Abl inhibitor	Chronic myelogenous leukemia	PO	InTrial	2021	Yes	Yes
PPP-002	PPP-002	Tetra Bio-Pharma	botanical drug	Pain	Undisclosed	InTrial	2021	No	No
Zynquista	sotagliflozin	Sanofi/ Lexicon	sodium-dependent glucose transporter 1 (SGLT-1) and SGLT-2 inhibitor	Diabetes mellitus	PO	CRL	2021	No	No
JNJ-3872 (VX-787)	pimodivir	Johnson & Johnson/ Vertex	viral protein inhibitor	Influenza	PO	InTrial	2021	No	No
RG-7314 (RO-5285119)	balovaptan	Roche	V1A vasopressin receptor antagonist	Autism spectrum disorder	PO	InTrial	2021	Yes	No
RG-6264	trastuzumab/ pertuzumab	Roche	HER2/neu receptor antagonist	Breast cancer	SC	InTrial	2021	Yes	No
IMO-2125	tilsotolimod	Idera	toll-like receptor 9 (TLR-9) agonist	Melanoma	SC/ intratumoral	InTrial	2021	Yes	Yes
ATI-5923	tecarfarin	ARYx Therapeutics/ Armetheon	vitamin K epoxide reductase enzyme inhibitor	Anticoagulation	PO	InTrial	2021	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
RG-7388 (RO-5503781)	idasanutlin	Roche	MDM2 antagonist	Acute myelogenous leukemia	PO	InTrial	2021	Yes	No
CMX-001	brincidofovir hexadecyloxypropyl ester	Chimerix	DNA-directed DNA polymerase inhibitor	Adenovirus/ Cytomegalovirus/ Smallpox	PO	InTrial	2021	No	Yes
LY-686017	tradipitant	Vanda Pharmaceuticals	neurokinin 1 receptor (NK-1R) antagonist	Motion sickness	PO	InTrial	2021	No	No
RG-7440	ipatasertib	Roche	pan-Akt inhibitor	Prostate cancer; breast cancer	PO	InTrial	2021	Yes	No
EMD-1214063	tepotinib	Merck	c-Met receptor tyrosine kinase inhibitor	Non-small cell lung cancer	PO	InTrial	2021	Yes	No
RTA-408	omaveloxolone	Reata Pharmaceuticals	Nrf2 activator	Friedreich's ataxia	PO	InTrial	2021	Yes	Yes
Recentin	cediranib	AstraZeneca	vascular endothelial growth factor receptor (VEGF) antagonists	Ovarian cancer	PO	InTrial	2021	Yes	Yes
MOD-401	somatrogon	OPKO Health/ Pfizer	enzyme replacement	Growth hormone deficiency	SC	InTrial	2021	Yes	Yes
DS-100	DS-100	Eton	undisclosed	Ophthalmological disease	SC	InTrial	2021	No	No
S5G4T-1 (DER-45-EV)	benzoyl peroxide	Sol-Gel Technologies	benzoyl peroxide	Rosacea	TOP	InTrial	2021	No	No
OTL-101	ADA-transduced autologous stem cell therapy	Orchard Therapeutics	gene therapy	Adenosine deaminase-deficient severe combined immunodeficiency	Undisclosed	InTrial	2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
CAT-1004	edasalonexent	Catabasis	NF-kB inhibitor	Duchenne muscular dystrophy	PO	InTrial	Late 2021	Yes	Yes
ONS-5010	bevacizumab	Outlook Therapeutics	anti-VEGF antibody	wet age-related macular degeneration	Intravitreal	InTrial	Late 2021	Yes	No
PF-06482077	multivalent group B streptococcus vaccine	Pfizer	vaccine	Bacterial infection	IM	InTrial	Late 2021	Yes	No
REGN-1500	evinacumab	Regeneron	angiopoietin-like 3 (ANGPTL3) antagonist	Hyperlipidemia	IV/SC	InTrial	Late 2021	Yes	No
AMT-061	etranacogene dezaparvovec	uniQure	gene therapy	Hemophilia B	IV	InTrial	Late 2021	Yes	No
PDS-1.0	ranibizumab	Roche/ Genentech	Anti-VEGF (vascular endothelial growth factor)	Wet age-related macular degeneration	Intravitreal implant	InTrial	Late 2021	Yes	No
Humacyl	human acellular vessel	Humacyte	cellular therapy	End-stage renal disease/ Peripheral artery disease	Implant	InTrial	Late 2021	Yes	No
NNZ-2566	trofinetide	Neuren	insulin-like growth factor 1 (IGF-1) derivative	Rett syndrome/ Fragile X syndrome/ Brain injury	IV/PO	InTrial	Late 2021	Yes	Yes
MK-0594 (VPD-737)	serlopitant	Menlo	NK-1 receptor antagonist	Pruritus	PO	InTrial	Late 2021	Yes	No
QAW-039 (NVP-QAW-039)	fevipiprant	Novartis	chemoattractant receptor-homologous molecule (CRTH2) antagonist	Asthma	PO	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
ADV-7103	tripotassium citrate monohydrate/ potassium hydrogen carbonate	Advicenne	Undisclosed	Distal rental tubular acidosis	PO	InTrial	Late 2021	Yes	No
PW-4142 (T-111)	nalbuphine ER	Trevi Therapeutics/ Endo	opioid agonist/ antagonist	Prurigo nodularis	PO	InTrial	Late 2021	No	No
GSK-2696274 (OTL-200)	GSK-2696274 (OTL-200)	GlaxoSmithKline	gene therapy	Leukodystrophy	IV	InTrial	Late 2021	Yes	Yes
Ultomiris SC	ravulizumab-cwvz	Alexion	C5 complement inhibitor	paroxysmal nocturnal hemoglobinuria; Hemolytic uremic syndrome	SC	InTrial	Late 2021	Yes	Yes
PF-04965842	abrocitinib	Pfizer	janus kinase 1 (JAK-1) inhibitor	Atopic dermatitis	PO	InTrial	Late 2021	Yes	No
COR-003	levoketoconazole	Strongbridge	azole antifungal	Cushing's syndrome	PO	InTrial	Late 2021	No	Yes
AKB-6548	vadadustat	Akebia Therapeutics	hypoxia-inducible factor-prolyl hydroxylase (HIF-PH) inhibitor	Anemia	PO	InTrial	Late 2021	Yes	No

IM = intramuscular, INH = inhalation, INJ = injection, IUD = intrauterine device, IV = intravenous, OP = ophthalmic, PO = oral, SC = subcutaneous, SL = sublingual, 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
Belviq/ Belviq XR	lorcaserin	Arena/Eisai	5-HT-2C receptor agonist	Obesity	Label update: to include long-term efficacy and safety data and remove the limitation of use related to the effect of Belviq on cardiovascular morbidity and mortality	PO	11/2019 – 12/2019
Tecentriq	atezolizumab	Genentech	PD-L1 monoclonal antibody	Non-small cell lung cancer	In combination with Abraxane (albumin-bound paclitaxel; nab-paclitaxel) and carboplatin for the initial (first-line) treatment of people with metastatic non-squamous non-small cell lung cancer (NSCLC) who do not have EGFR or ALK genomic tumor aberrations	IV	12/2/2019
Vascepa	icosapent ethyl	Amarin	ethyl ester of eicosapentaenoic acid	Hyperlipidemia	To reduce the risk of cardiovascular events in high-risk patients	PO	12/28/2019
Lynparza	olaparib	AstraZeneca	poly (ADP-ribose) polymerase (PARP) inhibitor	Pancreatic cancer	Monotherapy for maintenance of gBRCA mutated metastatic pancreatic cancer	PO	12/31/2019
Xtandi	enzalutamide	Astellas/ Pfizer	androgen receptor inhibitor	Prostate cancer	Treatment of metastatic hormone-sensitive prostate cancer	PO	12/31/2019

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Qsymia	phentermine and topiramate extended-release	Vivus	sympathomimetic amine anorectic/ antiepileptic	Cardiovascular/ safety data	Label update: to include cardiovascular risk and post-marketing safety data	PO	12/31/2019
Fiasp	insulin aspart	Novo Nordisk	insulins	Diabetes mellitus	To improve glycemic control in children and adolescents with type 1 diabetes	SC	1/1/2020
Trulicity	dulaglutide	Eli Lilly	glucagon-like peptide-1 (GLP-1) receptor agonist	Cardiovascular outcomes	Cardiovascular risk reduction in adults with type 2 diabetes	SC	1/1/2020
Ozempic	semaglutide	Novo Nordisk	glucagon-like peptide-1 (GLP-1) receptor agonist	Cardiovascular risk reduction	Cardiovascular risk reduction in adults with type 2 diabetes	SC	1/20/2020
Rybelsus	semaglutide	Novo Nordisk	glucagon-like peptide-1 (GLP-1) receptor agonist	Cardiovascular risk reduction	Cardiovascular risk reduction in adults with type 2 diabetes	PO	1/20/2020
Dificid	fidaxomicin	Merck	macrolide	Clostridium difficile-associated diarrhea	Treatment of Clostridium difficile-associated diarrhea (CDAD) in patients 6 months of age and older	PO	1/24/2020

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Keytruda	pembrolizumab	Merck	anti-PD-1 inhibitor	Melanoma, classical Hodgkin lymphoma, primary mediastinal large B-cell lymphoma, gastric cancer, hepatocellular carcinoma and Merkel cell carcinoma	Updated dosing frequency: every-six-weeks dosing schedule option	IV	2/18/2020
Opdivo	nivolumab	Bristol-Myers Squibb	anti-PD-1 inhibitor	Hepatocellular carcinoma	In combination with ipilimumab for the treatment of patients with advanced hepatocellular carcinoma (HCC) previously treated with sorafenib	IV	3/10/2020
Reblozyl	luspatercept	Celgene	Erythroid maturation agent	Myelodysplastic syndromes	Treatment of adult patients with very low to intermediate risk myelodysplastic syndromes (MDS)-associated anemia who have ring sideroblasts and require red blood cell transfusions	SC	4/4/2020
Otezla	apremilast	Celgene	phosphodiesterase 4 inhibitor	Scalp psoriasis	Treatment of moderate to severe scalp psoriasis	PO	4/15/2020
Jardiance	empagliflozin	Boehringer Ingelheim/ Eli Lilly	sodium-dependent glucose transporter 2 (SGLT-2) inhibitor	Diabetes mellitus	Adjunct to insulin for treatment of type 1 diabetes mellitus (T1DM)	PO	4/15/2020

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Nerlynx	neratinib	Puma Biotechnology	irreversible pan-ErbB receptor tyrosine kinase inhibitor	Breast cancer	In combination with capecitabine for the treatment of patients with HER2-positive metastatic breast cancer who have failed two or more prior lines of HER2-directed treatment (third-line disease)	PO	4/30/2020
Cyramza	ramucirumab	Eli Lilly/ Shire/ AstraZeneca	vascular endothelial growth factor 2 (VEGF-2) receptor antagonist	Non-small cell lung cancer	First-line treatment of patients with metastatic EGFR-mutated non-small cell lung cancer (NSCLC)	IV	5/15/2020
Orilissa	elagolix	AbbVie	gonadotropin-releasing hormone (GnRH) receptor antagonist	Uterine fibroids	Management of heavy menstrual bleeding associated with uterine fibroids in women	PO	6/5/2020
Taltz	ixekizumab	Eli Lilly	IL-17 monoclonal antibody	Non-radiographic axial spondyloarthritis	Treatment of non-radiographic axial spondyloarthritis	SC	6/15/2020
Xolair	omalizumab	Genentech	IgE antagonist	Nasal polyps	Treatment of adults with chronic rhinosinusitis with nasal polyps (CRSwNP) who have not adequately responded to intranasal corticosteroids	SC	6/15/2020
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
Calquence	acalabrutinib	AstraZeneca	Bruton's tyrosine kinase inhibitor	Chronic lymphocytic leukemia	Treatment of high risk relapsed or refractory chronic lymphocytic leukemia (CLL)	PO	6/30/2020

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
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
Ofev	nintedanib	Boehringer Ingelheim	tyrosine kinase inhibitor	Interstitial lung diseases	Treatment of progressive fibrosing interstitial lung diseases	PO	7/15/2020
Tremfya	guselkumab	Janssen Biotech	interleukin-23 (IL-23) inhibitor	Psoriatic arthritis	Treatment of active psoriatic arthritis	SC	7/16/2020
Trelegy Ellipta	fluticasone furoate/ umeclidinium/ vilanterol	GSK/ Theravance Biopharma	corticosteroid (ICS)/ long-acting muscarinic agent (LAMA)/ long-acting beta agonist (LABA)	Asthma	Treatment of asthma	INH	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
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

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

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