OPTUMRx®

RxOutlook®

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Orphan drugs continue to feature prominently in the drug development pipeline

In 1983 the Orphan Drug Act was signed into law. Thirty seven years later, what was initially envisioned as a minor category of drugs has become a major part of the drug development pipeline.

The Orphan Drug Act was passed by the United States Congress in 1983 in order to spur drug development for rare conditions with high unmet need. The legislation provided financial incentives to manufacturers if they could demonstrate that the target population for their drug consisted of fewer than 200,000 persons in the United States, or that there was no reasonable expectation that commercial sales would be sufficient to recoup the developmental costs associated with the drug. These "Orphan Drug" approvals have become increasingly common over the last two decades. In 2000, two of the 27 (7%) new drugs approved by the FDA had Orphan Designation, whereas in 2019, 20 of the 48 new drugs (42%) approved by the FDA had Orphan Designation.

Since the passage of the Orphan Drug Act, 37 years ago, additional regulations and FDA designations have been implemented in an attempt to further expedite drug development for certain serious and life threatening conditions. Drugs with a Fast Track designation can use Phase 2 clinical trials to support FDA approval. Drugs with Breakthrough Therapy designation can use alternative clinical trial designs instead of the traditional randomized, double-blind, placebo-controlled trial. Additionally, drugs may be approved via the Accelerated Approval pathway using surrogate endpoints in clinical trials rather than clinical outcomes. Although the net effect of these regulatory actions supports drug development for important and rare conditions, one unintended consequence is that the data generated to support FDA approval is often insufficient for healthcare decision makers, leaving them with unanswered questions about the value of these drugs, their relative place in therapy, and, because of cost, how to balance appropriate patient access with overall affordability.

In this edition of RxOutlook, we highlight ten key pipeline drugs with a potential launch by the end of the second quarter of 2020. Of this list, six drugs have an Orphan Designation for the management of a rare disease and one is an approved orphan drug seeking broader use for a non-rare disease. Obeticholic acid has Orphan Designation and FDA approval for the rare disease primary biliary cholangitis; however, it is expected to receive approval for nonalcoholic steatohepatitis, a relatively common condition. Fenfluramine, a drug originally developed for weight loss but removed from the market due to cardiovascular safety concerns, is finding new life as an orphan drug for Dravet syndrome, a very serious seizure disorder that is commonly resistant to existing therapies. Some of the orphan drugs we review will be the first approved treatments for a particular condition (eg, selumetinib for neurofibromatosis) but others will be options for when patients have failed existing drug options (eg, isatuximab for multiple myeloma).

Looking forward to 2020, OptumRx has identified 30 drugs with Orphan Designation that have already been filed and accepted for review by the FDA. While all of these drugs may not be approved by the FDA, this number gives further evidence of the continued growth of orphan drugs and the impact that the Orphan Drug Act has had on the current and future drug development pipeline.

Key pipeline drugs with FDA approval decisions expected by the end of the 2nd quarter 2020

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Fenfluramine	Zogenix	Dravet syndrome*	3/25/2020
Opicapone	Neurocrine Biosciences	Parkinson's disease	4/24/2020
Isatuximab	Sanofi	Multiple myeloma*	4/30/2020
Dasotraline	Sunovion Pharmaceuticals	Binge eating disorder	5/14/2020
Risdiplam	Genentech/ Roche	Spinal muscular atrophy*	5/22/2020
Amphora (L-lactic acid, citric acid and potassium bitartrate)	Evofem Biosciences	Prevention of pregnancy	5/25/2020
Pemigatinib	Incyte	Cholangiocarcinoma*	5/30/2020
Viltolarsen	Nippon Shinyaku	Duchenne muscular dystrophy*	6/2020
Obeticholic acid	Intercept Pharmaceuticals	Nonalcoholic steatohepatitis	6/26/2020
Selumetinib	AstraZeneca/Merck	Neurofibromatosis*	2Q 2020

* Orphan Drug Designation

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

Detailed Drug Insights

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

Read more

Extended Generic Pipeline Forecast

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

Read more

Extended Brand Pipeline Forecast

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

Read more

Key Pending Indication Forecast

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

Read more

Past and future reviews

Please note that RxOutlook highlights select near-term approvals. Some drugs may not appear in this issue because they have been reviewed in previous editions of RxOutlook. Drugs of interest that are earlier in development or with expected approvals beyond 2nd quarter 2020 may appear in future reports; however, for those who need an initial look at the full pipeline, please refer to the Brand Pipeline Forecast Table found later in this report.

Getting acquainted with pipeline forecast terms

Clinical trial phases

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

Pipeline acronyms

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

Detailed insights on key drugs

Fenfluramine (Brand Name: Fintepla®)

Manufacturer: Zogenix
Regulatory designations: Orphan Drug, Fast Track
Expected FDA decision: 3/25/2020

Therapeutic use

Fenfluramine is in development for the treatment of seizures associated with Dravet syndrome.

Dravet syndrome is a rare, severe form of epilepsy characterized by frequent, prolonged seizures, neurodevelopmental delay, and neurologic disability. Seizures are often resistant to existing antiepileptic agents and most patients require two or more drugs to achieve seizure control. Dravet syndrome affects an estimated 1 in 15,700 individuals in the U.S. and it generally begins during the first year of life.

Patients are at increased risk for early death with the most common causes of death is sudden unexpected death in epilepsy and status epilepticus.

- Treatment of seizures associated with Dravet syndrome

Fenfluramine (Brand Name: Fintepla®) ***(continued...)***

Clinical profile

Fenfluramine is a serotonin modulator. The exact mechanism in epilepsy is unknown. High doses of fenfluramine were previously available in the U.S. for obesity and it was used off-label in combination with phentermine; however, fenfluramine was withdrawn from the market in 1997 due to cardiovascular adverse events.

Pivotal trial data:
The efficacy of fenfluramine was evaluated in two randomized, double-blind, Phase 3 studies in patients with Dravet syndrome. In the first study, 119 patients received fenfluramine 0.2 mg/kg per day, fenfluramine 0.7 mg/kg per day, or placebo, added to existing antiepileptic agents for 14 weeks. The primary outcome was the change in mean monthly convulsive seizure frequency (MCSF) during the treatment period compared with baseline in the 0.7 mg/kg per day group vs. placebo; 0.2 mg/kg per day vs. placebo was assessed as a key secondary outcome. The study met its primary efficacy endpoint, with fenfluramine 0.7 mg/kg per day demonstrating a 62.3% greater reduction in mean MCSF vs. placebo (95% CI: 47.7, 72.8; $p < 0.0001$); fenfluramine 0.2 mg/kg per day demonstrated a 32.4% reduction in mean MCSF vs. placebo (95% CI: 6.2, 52.3; $p = 0.0209$). The median seizure frequency went from 20.7 seizures per 28 days to 4.7 seizures per 28 days in the fenfluramine 0.7 mg/kg group, from 17.5 seizures per 28 days to 12.6 per 28 days in the fenfluramine 0.2 mg/kg group, and from 27.3 per 28 days to 22.0 per 28 days in the placebo group.

In the second study, 87 patients received fenfluramine 0.4 mg/kg per day or placebo, added to an existing Diacomit® (stiripentol)-containing antiepileptic drug regimen. The primary efficacy end point was the change in mean MCSF between fenfluramine and placebo during the combined titration and maintenance periods. Patients treated with fenfluramine achieved a 54.0% (95% CI: 35.6, 67.2; $p < 0.001$) greater reduction in mean MCSF vs. placebo.

Safety:
The most common adverse events with fenfluramine use were decreased appetite/weight, diarrhea, fatigue, lethargy, and somnolence.

Dosing:
In the pivotal trials, fenfluramine was administered orally twice a day.

- Serotonin modulator
- Oral formulation
- Mean MCSF: 54.0% to 62.3% greater reduction vs. placebo
- Common AEs: appetite, diarrhea, fatigue, lethargy, somnolence, decreased weight
- Dosing: twice a day

Competitive environment

If approved, fenfluramine would offer a novel therapy for the treatment of Dravet syndrome, a condition for which there is a significant unmet need. Historically, treatment options for Dravet syndrome were limited and many patients are refractory to existing therapies. The efficacy of fenfluramine was demonstrated in both patients with or without concomitant use of Diacomit, another product recently approved for Dravet syndrome. In addition, fenfluramine is being evaluated for Lennox-Gastaut syndrome, another rare and severe child-onset epilepsy.

Fenfluramine will be the third product recently approved for Dravet syndrome, behind Diacomit and Epidiolex® (cannabidiol), both of which were approved in 2018. Fenfluramine has a different mechanism of action as a serotonin modulator, whereas Diacomit modulates GABA, and Epidiolex is a cannabinoid. Fenfluramine was previously removed from the market due to cardiovascular safety concerns (eg, heart valve abnormalities). Although cardiovascular adverse events were associated with higher doses of fenfluramine, and not observed in the Dravet syndrome trials, these studies were short-term in nature and longer term follow-up will be needed to better understand the risks. Fenfluramine is likely to be reserved as add-on therapy to existing antiepileptic regimens. Fenfluramine will also likely be a controlled substance as the previously approved formulation was as well.

For reference, the Wholesale Acquisition Cost (WAC) price for Epidiolex is approximately $24,000 per year.

- Advantages: significant unmet need, benefit demonstrated with or without concomitant Diacomit use, potential future indication for Lennox-Gastaut syndrome
- Disadvantages: recently approved competition (Diacomit, Epidiolex), cardiovascular safety concerns, possible DEA scheduling
- Reference WAC (Epidiolex): ~$24,000 per year

Opicapone (Brand Name: Ongentys®)

Manufacturer: Neurocrine Biosciences
Expected FDA decision: 4/24/2020

Therapeutic use

Opicapone is in development as an adjunctive treatment to levodopa/carbidopa in patients with Parkinson's disease experiencing "OFF" episodes.

Parkinson's disease is a chronic, progressive and debilitating neurodegenerative disorder that affects approximately one million people in the U.S. Parkinson's disease is characterized by a loss of dopamine and its function. As the disease progresses, dopamine production steadily decreases resulting in slowed movement (bradykinesia), tremor, rigidity, impaired posture and balance, and speech and writing difficulty.

The primary treatment for Parkinson's disease is administration of dopaminergic therapies, including levodopa. While levodopa improves patients' motor symptoms, as the disease progresses, the beneficial effects of levodopa begin to wear off more quickly, causing symptoms to worsen as patients experience motor fluctuations throughout the day ("OFF" episodes).

Opicapone (Brand Name: Ongentys®) (continued...)

- Adjunctive treatment to levodopa/carbidopa in patients with Parkinson's disease experiencing "OFF" episodes

Clinical profile

Opicapone is a selective catechol-O-methyltransferase (COMT) inhibitor. Opicapone prolongs the clinical effect of levodopa by decreasing its metabolism and allows for greater availability in the brain.

Pivotal trial data:
The efficacy of opicapone was evaluated in two randomized, double-blind, Phase 3 studies (BIPARK-1 and BIPARK-2) in patients with Parkinson's disease and motor fluctuations. BIPARK-1 was a placebo- and active-controlled study of opicapone as an adjunct to levodopa therapy in which approximately 600 patients received once-daily opicapone (5 mg, 25 mg, or 50 mg), placebo, or 200 mg doses of the COMT inhibitor entacapone for 14 to 15 weeks. BIPARK-2 was a placebo-controlled study of opicapone as an adjunct to levodopa therapy in which approximately 400 patients received once-daily opicapone (25 mg or 50 mg) or placebo for 14 to 15 weeks.

In both trials, opicapone 50 mg demonstrated a statistically significant increase in absolute "ON" time without troublesome dyskinesia from baseline to week 14/15. In BIPARK-1, the increase was 1.9 hours with opicapone 50 mg vs. 0.9 hours for placebo ($p = 0.002$) and in BIPARK-2 the difference was 1.7 hours for opicapone 50 mg vs. 0.9 hours for placebo ($p = 0.025$). In addition, a significantly higher percentage of patients treated with opicapone 50 mg had an increase in total "ON" time of an hour or longer at week 14/15 in both BIPARK-1 (65.2%, $p < 0.01$) and BIPARK-2 (61.9%, $p < 0.01$). There was no statistically significant difference between opicapone 5 mg or 25 mg vs. placebo. Treatment with opicapone 50 mg was non-inferior to entacapone.

Safety:
The most common adverse events with opicapone use were dyskinesia, constipation, and dry mouth.

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

- COMT inhibitor
- Oral formulation
- "ON" time without troublesome dyskinesia: 1.7 to 1.9 hours with opicapone 50 mg vs. 0.7 to 0.9 hours for placebo
- Common AEs: dyskinesia, constipation, dry mouth
- Dosing: once daily

Competitive environment

If approved, opicapone would offer an additional COMT inhibitor for the treatment of Parkinson's disease. Currently approved COMT inhibitors include entacapone and tolcapone. Tolcapone is generally considered more efficacious than entacapone, however entacapone is preferred as a first-line COMT inhibitor because of its tolerability and safety profile (eg, tolcapone is associated with liver toxicity). In one of the pivotal trials, opicapone demonstrated non-inferiority vs. entacapone and did provide numerically improved "ON" time while maintaining a mild adverse event profile. Entacapone and tolcapone are dosed multiple times a day, whereas opicapone is given once daily.

Opicapone enters a crowded category of drugs used for "OFF" episodes. The other COMT inhibitors are both available generically and have been used for decades for the treatment of Parkinson's disease. Other maintenance drugs include the monoamine oxidase type B (MAO B) inhibitors and the recently approved Nourianz™ (istradefylline). Rescue therapies are also available for treating acute "OFF" episodes, including inhaled Inbrija® (levodopa) and injectable Apokyn® (apomorphine). In addition, there is a lack of robust head-to-head trial data comparing opicapone vs. its competitors with an active control only being included in one of the two pivotal trials.

For reference, the WAC price for Nourianz is approximately $18,000 per year.

- Advantages: potential alternative to entacapone and tolcapone due to efficacy and safety, oral and once daily administration
- Disadvantages: crowded marketplace (including generic alternatives), lack of robust comparative efficacy data
- Reference WAC (Nourianz): ~$18,000 per year

Isatuximab (Brand Name: To be determined)

Manufacturer: Sanofi
Regulatory designations: Orphan Drug
Expected FDA decision: 4/30/2020

Therapeutic use

Isatuximab is in development for the treatment of patients with relapsed/refractory multiple myeloma (RRMM) as part of a combination regimen with pomalidomide (Pomalyst®) and dexamethasone.

- In combination with pomalidomide and dexamethasone, for the treatment of patients with RRMM

Multiple myeloma is a cancer of the plasma cells (white blood cells that produce antibodies). Multiple myeloma is a relatively uncommon cancer with a lifetime risk of 1 in 132 (0.76%). In the U.S., about 32,110 new cases were diagnosed in 2019 and about 12,960 deaths were expected to occur.

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

Clinical profile

Isatuximab is a monoclonal antibody that targets multiple myeloma cells that express the CD38 glycoprotein. Isatuximab is designed to trigger multiple, distinct mechanisms that are believed to directly promote programmed tumor cell death and immunomodulatory activity. CD38 is highly and uniformly expressed on multiple myeloma cells.

- Monoclonal antibody targeting CD38
- IV formulation

<u>Pivotal trial data:</u>
The efficacy of isatuximab was evaluated in ICARIA-MM, a randomized, open-label, Phase 3 study in 307 patients with RRMM who had received at least two previous lines of treatment. Patients received isatuximab plus pomalidomide/dexamethasone or pomalidomide/dexamethasone alone.

At a median follow-up of 11.6 months, median progression-free survival (PFS) was 11.5 months in the isatuximab-pomalidomide-dexamethasone group vs. 6.5 months in the pomalidomide-dexamethasone group (hazard ratio [HR] 0.596, 95% CI: 0.44, 0.81; p = 0.001). Isatuximab combination therapy also demonstrated a significantly greater overall response rate (ORR) vs. pomalidomide plus dexamethasone alone (60% vs. 35%, p < 0.0001).

- Median PFS = 11.5 months with isatuximab combination therapy vs. 6.5 months with pomalidomide plus dexamethasone
- ORR = 60% with isatuximab combination therapy vs. 35% with pomalidomide plus dexamethasone

<u>Safety:</u>
The most common adverse events with isatuximab use were infusion reactions, upper respiratory tract infections, and diarrhea.

- Common AEs: infusion reactions, upper respiratory tract infections, and diarrhea

<u>Dosing:</u>
In the pivotal trial, isatuximab was administered intravenously (IV) on days 1, 8, 15, and 22 in the first 28-day cycle, then on days 1 and 15 in subsequent cycles until disease progression and unacceptable toxicity.

- Dosing: days 1, 8, 15, and 22 in the first 28-day cycle, then on days 1 and 15 in subsequent cycles until disease progression and unacceptable toxicity

Isatuximab (continued...)

Competitive environment

Multiple drugs are available for the treatment of multiple myeloma; however, the cancer often relapses or becomes refractory to treatment so there is an unmet need in this condition. The only other product targeting CD38 is Darzalex® (daratumumab); it is also approved for multiple myeloma as monotherapy, or as part of different combination regimens for the treatment multiple myeloma, in the first line, second line, third line, or fourth line setting. Isatuximab will compete with Darzalex and may ultimately be used in a similar manner if future studies with isatuximab are positive. Until then, it will probably be reserved for third line and later. Compared to Darzalex, isatuximab does offer shorter IV infusion durations.

Darzalex has been available since 2015 and it is an established backbone therapy for multiple myeloma. While isatuximab may have future expanded uses, the initial indication will be limited to combination therapy with pomalidomide plus dexamethasone in the relapsed/refractory setting. The PFS data was promising, but overall survival (OS) data was not yet mature for the pivotal trial. Finally, isatuximab does require IV administration and could face future competition with a subcutaneously (SC) administered Darzalex formulation that is expected to be available in 2021.

For reference, the WAC price for Darzalex is approximately $13,750 per 30 days.

- Advantages: significant unmet need, future potential for expanded use in earlier settings for multiple myeloma, shorter infusion time vs. Darzalex
- Disadvantages: established alternative with the same MOA (Darzalex), narrow initial indication, lack of OS data, IV administration
- Reference WAC (Darzalex): ~$13,750 per 30 days

Dasotraline (Brand Name: To be determined)

Manufacturer: Sunovion Pharmaceuticals
FDA approval date: 5/14/2020

Therapeutic use

Dasotraline is in development for the treatment of patients with moderate-to-severe binge eating disorder.

Binge eating disorder is characterized by recurrent binge eating episodes during which a person feels a loss of control and marked distress over his or her eating. Unlike other eating disorders (eg, bulimia nervosa), binge eating episodes are not followed by purging, excessive exercise, or fasting. People with binge eating disorder often are overweight or obese.

Binge eating disorder is the most common eating disorder in the U.S. with a past-year prevalence of 1.2% and a lifetime prevalence of 2.8%. It is about twice as common in females than males.

- Treatment of patients with moderate-to-severe binge eating disorder

Clinical profile

Dasotraline is a dopamine and norepinephrine reuptake inhibitor. It does not stimulate neuronal release of dopamine and norepinephrine.

Pivotal trial data:
The efficacy of dasotraline was evaluated in two 12-week, randomized, placebo-controlled studies. The first study was a Phase 2/3 trial in 317 adults with moderate to severe binge eating disorder. Patients received flexibly-dosed dasotraline 4 mg to 8 mg per day or placebo. The primary endpoint was the change from baseline in number of binge eating days (defined as days during which at least one binge episode occurs) per week at week 12. The difference between dasotraline vs. placebo was -0.99 days for the primary endpoint ($p < 0.0001$).

The second study was a Phase 3 trial which evaluated fixed once-daily doses of dasotraline 4 mg and dasotraline 6 mg vs. placebo. The trial met its primary endpoint, demonstrating a statistically significant decrease in number of binge days per week from baseline to week 12 in the group treated with dasotraline 6 mg/day vs. the placebo-treated group. The study did not meet its primary endpoint for the group treated with dasotraline 4 mg/day. Numerical differences between the groups have not been provided.

Safety:
The most common adverse events with dasotraline use were insomnia, dry mouth, headache, decreased appetite, nausea, and anxiety.

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

- Dopamine and norepinephrine reuptake inhibitor
- Oral formulation
- Change from baseline in number of binge eating days per week: -0.99 vs. placebo
- Common AEs: insomnia, dry mouth, headache, decreased appetite, nausea, anxiety
- Dosing: once daily

Dasotraline (continued...)

Competitive environment

Dasotraline would be the second approved product for the treatment of binge eating disorder and it would offer a different mechanism compared to the only other product approved for binge eating disorder, Vyvanse® (lisdexamfetamine), a stimulant. Vyvanse is a controlled substance but dasotraline is not likely to have abuse potential.

However, dasotraline would be a relatively late market entry as Vyvanse has been approved for binge eating disorder since early 2015 and generic alternatives for Vyvanse could be available by late 2023. When compared indirectly, dasotraline appears to be less effective than Vyvanse for binge eating disorder and it was associated with a higher discontinuation rate, although comparisons across different clinical trials are difficult. Off-label use of selective serotonin reuptake inhibitors and antiepileptic drugs is also common for the treatment of binge eating disorder; many of these options are also available as generics.

For reference, the WAC price for Vyvanse is approximately $3,700 per year.

- Advantages: novel therapy for binge eating disorder, less abuse potential vs. Vyvanse, oral and once a day
- Disadvantages: late market entry, potentially less effective and a higher discontinuation rate vs. Vyvanse
- Reference WAC (Vyvanse) = ~$3,700

Risdiplam (Brand Name: To be determined)

Manufacturer: Roche/Genentech/PTC Therapeutics
Regulatory designations: Orphan Drug, Fast Track, Breakthrough Therapy
Expected FDA decision: 5/22/2020

Therapeutic use

- Treatment of SMA

Risdiplam is in development for the treatment of spinal muscular atrophy (SMA).

SMA is a rare group of severe neuromuscular disorders characterized by the loss of motor neurons leading to progressive muscle weakness and atrophy. SMA is caused by a genetic defect in the *survival motor neuron 1 (SMN1)* gene, which is responsible for coding a protein necessary for survival of motor neurons. The defect leads to deficient or dysfunctional SMN protein, and the symptoms of muscle weakness. It is the most common genetic cause of infant mortality and one of the most common rare diseases, affecting approximately one in 11,000 babies.

Depending on the type of SMA, an individual's physical strength and their ability to walk, eat or breathe can be significantly diminished or lost. Type 1 SMA is a severe form of the disease that develops within the first few months of life and ultimately leads to death or the need for permanent ventilation support by 24 months of age for more than 90% of patients. Type 2 and 3 SMA patients experience later onset of disease and less severe symptoms. Approximately 70% of Type 2 patients are alive at age 25 and Type 3 patients have near normal life expectancy.

Risdiplam (continued...)

Clinical profile

Risdiplam is a *survival motor neuron-2 (SMN-2)* splicing modifier for SMA. The homologous *SMN2* gene is predominantly spliced to a truncated mRNA, and only produces small amounts of functional SMN protein. Risdiplam is designed to provide sustained increase in SMN protein centrally and peripherally by helping the *SMN2* gene produce more functional SMN protein throughout the body.

Pivotal trial data:
The FDA filing for risdiplam incorporates 12-month data from the FIREFISH and SUNFISH pivotal studies. FIREFISH is an open-label, single-arm trial in infants with Type 1 SMA. Part 1 of the study was a dose-escalation evaluation in 21 infants aged one to seven months. The primary objective was to assess the safety profile of risdiplam (efficacy was an exploratory endpoint) and to determine the dose for Part 2, which is ongoing and evaluating the efficacy of risdiplam in 41 infants with Type 1 SMA for 24 months. Data from Part 1 demonstrated that after 16 months of treatment, 82% (14/17) of patients receiving high-dose risdiplam had a Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND) score ≥ 40 and 86% (18/21) of all infants were event-free after receiving risdiplam. No infant has required tracheostomy or reached permanent ventilation.

SUNFISH is a double-blind, placebo-controlled trial in children and young adults (2 to 25 years old) with Type 2 or 3 SMA. Part 1 determined the dose for the confirmatory Part 2 section, and evaluated efficacy as an exploratory endpoint in 51 patients. SUNFISH Part 2 was a large placebo-controlled trial evaluating the efficacy of risdiplam in 180 patients. The primary endpoint was the change from baseline in the Motor Function Measure 32 (MFM-32) scale after one year of treatment. This scale is designed to detect motor function changes in a broad range of patients.

In Part 1 of the SUNFISH trial, in patients for which the MFM-32 test has been completed at all visits up to month 12, 58% saw an improvement of at least 3 points from baseline vs. 7.6% for a matched natural history cohort. In Part 2, change from baseline in the MFM-32 score was significantly greater in patients treated with risdiplam vs. placebo (1.55 point mean difference; p = 0.0156). An exploratory subgroup analyses showed that the strongest responses in MFM-32 vs. placebo were observed in the youngest age group (2 to 5 years) (78.1% vs. 52.9% achieving ≥ 3 point increase).

Safety:
The most common adverse events with risdiplam use were fever, cough, vomiting, upper respiratory tract infections, persistent sore throat, and nasopharyngitis.

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

Risdiplam (continued...)

- SMN-2 splicing modifier
- Oral formulation
- Type 1 SMA: 82% of patients achieved CHOP-INTEND score ≥ 40; 86% were event-free after receiving risdiplam
- Type 2 SMA: 58% with improvement ≥ 3 points on the MFM-32 scale from baseline vs. 7.6% for a matched natural history cohort
- Common AEs: fever, cough, vomiting, upper respiratory tract infections, persistent sore throat, nasopharyngitis
- Dosing: once daily

Competitive environment

If approved, risdiplam would offer a novel oral therapy for the treatment of SMA. There is an unmet need for treatments for SMA as the condition is associated with a high rate of morbidity and mortality and treatment options have historically been limited. The only other FDA-approved drugs for SMA are Spinraza® (nusinersen), which requires chronic intrathecal dosing and the one-time IV infused gene therapy, Zolgensma® (onasemnogene abeparvovec). While clinical trials are still ongoing, the early results for risdiplam are promising.

However, it will be a relatively late market entry as Spinraza has been available since late 2016 and Zolgensma was approved in May 2019. While Spinraza does require invasive intrathecal injections, it has been widely used in SMA patients since its market entry. There is also a lack of long-term efficacy and safety data with risdiplam use and it is unknown whether patients will achieve improved outcomes if risdiplam is used concomitantly or after other SMA therapies.

For reference, the WAC price for Spinraza is approximately $750,000 in year 1 and $375,000 annually thereafter. However, the manufacturer of risdiplam has stated an intent to target a lower price point for risdiplam.

- Advantages: novel oral therapy for SMA, significant unmet need, promising data
- Disadvantages: alternatives available (ie, Spinraza, Zolgensma), lack of long-term data and lack of data evaluating concomitant use of risdiplam with other SMA products
- Reference WAC (Spinraza): ~$750,000 in year 1 and $375,000 annually thereafter

L-lactic acid, citric acid and potassium bitartrate (Brand Name: Amphora®)

Manufacturer: Evofem Biosciences
Expected FDA decision: 5/25/2020

Therapeutic use

Amphora is in development for prevention of pregnancy.

- Prevention of pregnancy

Clinical profile

Amphora is a non-hormonal contraceptive vaginal gel designed to keep the vaginal pH within the normal range of 3.5 to 4.5, even in the presence of semen, which normally raises the vaginal pH to 7.0 to 8.0. This maintains an acidic environment that is inhospitable to sperm, as well as certain viral and bacterial pathogens associated with sexually transmitted infections.

Pivotal trial data:
Amphora was evaluated in the AMPOWER study, a single-arm, open-label, Phase 3 trial in preventing pregnancy over seven menstrual cycles of use in approximately 1,400 women in the U.S. The cumulative pregnancy rate with typical use of Amphora in the intent-to-treat population was 13.7% over seven cycles of use (95% CI: 9.9, 17.4), which corresponds to an 86.3% efficacy rate. The cumulative pregnancy rate for women who correctly used Amphora as directed (ie, perfect use) was 6.7% (95% CI: 4.6, 8.7), which corresponds to a 93.3% efficacy rate.

Safety:
The most common adverse events with Amphora use were bacterial vaginitis, vulvovaginal mycotic infection and urinary tract infection.

Dosing:
In the pivotal trial, Amphora was administered as-needed up to one hour before sexual intimacy.

- Vaginal pH regulator
- Vaginal gel
- Cumulative pregnancy rate: 13.7% over seven cycles of use
- Common AEs: bacterial vaginitis, vulvovaginal mycotic infection, urinary tract infection
- Dosing: as-needed; up to one hour before sexual intimacy

Competitive environment

If approved, Amphora would offer a novel non-hormonal contraception method. Many women have a contraindication or are unwilling to use hormonal based contraception and in these patients, Amphora would be a potential treatment alternative. Amphora can be used as-needed while oral contraception must be taken daily and Amphora has minimal side effects compared to hormonal based therapy. Amphora is also being studied for the prevention of urogenital chlamydia and gonorrhea in women.

However, Amphora is entering a crowded marketplace with many other contraception methods available, including widely used generic oral contraceptives. While the efficacy will likely meet the threshold for FDA approval, the rate of pregnancy prevention with typical use of Amphora is slightly lower when compared indirectly to oral contraceptives.

- Advantages: novel non-hormonal contraception, as-needed administration, also being evaluated for prevention of urogenital chlamydia and gonorrhea
- Disadvantages: crowded marketplace, lower efficacy vs. oral contraception

Pemigatinib (Brand Name: To be determined)

Manufacturer: Incyte
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 5/30/2020

Therapeutic use

Pemigatinib is in development for the treatment of patients with previously treated, locally advanced or metastatic cholangiocarcinoma with fibroblast growth factor receptor (FGFR) 2 fusions or rearrangements.

Cholangiocarcinoma is a rare cancer that forms in the bile duct. It is further classified as intrahepatic cholangiocarcinoma and extrahepatic cholangiocarcinoma. About 8,000 people in the U.S. are diagnosed with cholangiocarcinoma each year and FGFR2 fusions or rearrangements occur in 10 to 16% of patients with intrahepatic cholangiocarcinoma. The overall 5-year relative survival rate for patients with intrahepatic bile duct cancers is about 8%.

- Treatment of patients with previously treated, locally advanced or metastatic cholangiocarcinoma with FGFR2 fusions or rearrangements

Pemigatinib (continued...)

Clinical profile

Pemigatinib is a potent selective inhibitor of FGFR isoforms 1, 2 and 3 which, in preclinical studies, has demonstrated selective pharmacologic activity against cancer cells with FGFR alterations. FGFRs play an important role in tumor cell proliferation and survival, migration and angiogenesis. Activating fusions, rearrangements, translocations and gene amplifications in FGFRs are closely correlated with the development of various cancers.

Pivotal trial data:
The efficacy of pemigatinib in 107 previously treated patients with cholangiocarcinoma and FGFR2 fusions or rearrangements was evaluated in cohort A of FIGHT-202, a Phase 2, single-arm, open-label study. The ORR with pemigatinib was 36% (95% CI: 27, 45) and PFS was 6.9 months (95% CI: 6.2, 9.6). Preliminary median OS was 21.1 months but follow-up will continue as the data are not yet mature.

Safety:
The most common adverse events with pemigatinib use were hyperphosphatemia, alopecia, diarrhea, decreased appetite, and fatigue.

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

- FGFR inhibitor
- Oral formulation
- ORR = 36%
- PFS = 6.9 months
- Common AEs: hyperphosphatemia, alopecia, diarrhea, decreased appetite, and fatigue
- Dosing: once daily

Competitive environment

If approved, pemigatinib would be the first therapy for patients with previously treated, locally advanced or metastatic cholangiocarcinoma with FGFR2 fusions or rearrangements. There is a significant unmet need as limited treatment options are available for this type of cancer. Surgical resection can be curative but most patients (approximately 70%) are unresectable at time of diagnosis. For patients with unresectable or metastatic disease, chemotherapy is first-line but response rates are low and no other targeted therapies are approved. Pemigatinib would offer an additional oral, once a day oncology therapy.

However, the proposed initial indication for pemigatinib is narrow. Cholangiocarcinoma is a very rare cancer and patients with FGFR2 fusions or rearrangements are only a small subset of patients. In addition, the initial submission is based on an early stage study and there is a lack of robust OS data.

A Phase 3 trial is evaluating pemigatinib vs. gemcitabine/cisplatin in the first-line setting but that study is not expected to be completed until early 2023.

- 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

Viltolarsen (Brand Name: To be determined)

Manufacturer: Nippon Shinyaku
Regulatory designations: Orphan Drug, Fast Track
Expected FDA decision: 6/2020

Therapeutic use

- Treatment of DMD amenable to exon 53 skipping

Viltolarsen is in development for the treatment of Duchenne muscular dystrophy (DMD) amenable to exon 53 skipping.

DMD is a rare genetic disorder characterized by progressive muscle deterioration and weakness. It is an X-linked disorder that affects young boys with a prevalence of approximately 1.3 to 2.1 cases per 10,000 live male births and approximately 6,000 males total in the U.S.

DMD is caused by an absence of dystrophin, a protein that helps keep muscle cells intact. The onset of symptoms occurs between 3 and 5 years of age and worsens over time. Progressive muscle weakness leads to decreased ambulation, inability to perform activities independently and confinement to a wheelchair by the early teen age years. Later, patients experience life-threatening heart and respiratory conditions, with death commonly occurring in the late teens or twenties.

Clinical profile

- Exon-skipping antisense oligonucleotide
- IV formulation
- Drug-induced increases in dystrophin content of muscle: 5.8% of normal levels (range 1.1 to 14.4%)
- Safety: unknown
- Dosing: once weekly

Viltolarsen is an exon-skipping antisense oligonucleotide therapy. Exon-skipping therapies prevent mutated exons from being read (the mutated genetic sequences are skipped over) during the process of transcription. This approach produces a dystrophin protein that is shorter than normal but potentially still functional, to improve muscle function.

Viltolarsen is only appropriate for patients with genetic mutations that are amenable to exon 53 skipping (about 8% of all DMD patients).

Pivotal trial data:
The efficacy of viltolarsen was evaluated in a single-arm, U.S./Canada-based Phase 2 study in 16 boys with DMD. Patients received a low or high dose of viltolarsen. The primary efficacy outcome measure was Western blot assessment of muscle dystrophin expression, determined by sampling muscle tissue prior to drug treatment and following week 24 of treatment. Drug-induced increases in dystrophin content of muscle were seen in all patients, with a dystrophin increase averaging 5.8% of normal levels (range 1.1 to 14.4%). In addition, viltolarsen-treated patients showed improvements vs. age- and treatment-matched natural history controls in timed function tests such as the 6 minute walk test.

Safety:
To date, safety data has not been published or announced by Nippon Shinyaku.

Dosing:
In clinical trials, viltolarsen was administered as an IV infusion every week.

Viltolarsen (continued...)

Competitive environment

If approved, viltolarsen would be the third exon-skipping antisense drug for the treatment of DMD and the second specifically for patients with mutations amenable to exon 53 skipping (Vyondys 53™ [golodirsen] is also approved for this subset of patients). There is a significant unmet need for treatments for DMD since it is associated with substantial morbidity and mortality. While the data is very limited, other DMD drugs have been approved with exceedingly modest improvements in dystrophin levels. Compared indirectly, the dystrophin improvements as well as secondary clinical outcome benefits with viltolarsen were promising vs. previously approved exon skipping DMD agents, but comparisons across different clinical trials are difficult.

Data for viltolarsen are only available from early stage unpublished trials with small sample sizes. The FDA submission is based on data demonstrating an improvement in a surrogate endpoint (dystrophin levels) and the clinical significance of a small change in dystrophin has not been established. There is disagreement among experts with respect to the minimum level of dystrophin that might be reasonably likely to predict clinical benefit in patients with DMD. In addition, only an estimated 8% of patients with DMD have a mutation amendable to exon 53 and viltolarsen requires weekly IV infusion.

For reference, the average WAC price for Vyondys 53 is approximately $300,000 per year, but varies significantly due to weight-based dosing.

- Advantages: significant unmet need, promising early stage data compared to previously approved exon-skipping products
- Disadvantages: lack of late stage data, primary efficacy endpoint was improvement in surrogate of dystrophin improvement, small eligible patient population, IV administration
- Reference WAC (Vyondys 53): $300,000 per year (cost can increase due to weight-based dosing)

Obeticholic acid (Brand Name: To be determined)

Manufacturer: Intercept Pharmaceuticals
Regulatory designations: Breakthrough Therapy
Expected FDA decision: 6/26/2020 (FDA Advisory Committee tentatively scheduled for 4/22/2020)

Therapeutic use

Obeticholic acid is in development for the treatment of patients with fibrosis due to nonalcoholic steatohepatitis (NASH).

NASH is a chronic liver disease caused by excessive fat accumulation in the liver. It is characterized by inflammation, hepatocellular injury, and progressive liver fibrosis (scarring). NASH can eventually lead to cirrhosis, liver failure, and hepatocellular carcinoma. It is estimated to become the leading indication for liver transplantation in the U.S. NASH affects up to 5% of the U.S. population (approximately 16 million individuals), but only a small subset have been clinically diagnosed since the condition is generally asymptomatic during early stages.

Lower dose oral formulations of obeticholic acid are currently available under the brand name Ocaliva® for the treatment of primary biliary cholangitis (PBC). However, if approved, this new formulation of obeticholic acid is expected to be under a new brand name for the NASH indication.

- Treatment of patients with fibrosis due to NASH

Obeticholic acid (continued...)

Clinical profile

Obeticholic acid is a farnesoid X receptor (FXR) agonist. FXR is a nuclear receptor expressed in the liver and intestine. FXR is a key regulator of bile acid, inflammatory, fibrotic, and metabolic pathways. FXR activation decreases the intracellular hepatocyte concentrations of bile acids by suppressing de novo synthesis from cholesterol as well as by increased transport of bile acids out of the hepatocytes.

Pivotal trial data:
The efficacy of obeticholic acid was evaluated in the REGENERATE trial, a randomized, double-blind, placebo-controlled study in adult patients with NASH. Patients received obeticholic acid 10 mg, obeticholic acid 25 mg, or placebo. A planned interim analysis of 931 patients with stage F2 to F3 fibrosis was conducted. The primary endpoints for the month-18 interim analysis were fibrosis improvement (≥ 1 stage) with no worsening of NASH (defined as no increase of hepatocellular ballooning, lobular inflammation, or steatosis), or NASH resolution with no worsening of fibrosis, with the study considered successful if either primary endpoint was met.

The fibrosis improvement endpoint was achieved by 12% of patients in the placebo group, 18% in the obeticholic acid 10 mg group (p = 0.045), and 23% in the obeticholic acid 25 mg group (p = 0.0002). However, the co-primary endpoint of NASH resolution with no worsening of fibrosis did not meet statistical significance with only 11% of patients achieving this outcome in the obeticholic acid 10 mg group (p = 0.18), 12% in the obeticholic acid 25 mg group (p = 0.13), and 8% in the placebo group.

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

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

- FXR agonist
- Oral formulation
- Fibrosis improvement + no worsening of NASH: 23% with obeticholic acid 25 mg vs. 12% with placebo
- NASH resolution + no worsening of fibrosis: 12% with obeticholic acid 25 mg vs. 8% with placebo (no statistically significant difference)
- Common AE: pruritus
- Dosing: once daily

Competitive environment

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

However, obeticholic acid failed to demonstrate a significant difference vs. placebo for NASH resolution and the initial FDA filing is based on an interim analysis of the pivotal trial. It is not yet known whether obeticholic acid reduces more serious long-term liver complications (eg, need for transplant, liver cancer). Treatment with obeticholic acid is associated with an early increase in low-density lipoprotein (LDL) cholesterol as well, which is a concern in this patient population since they may already be at increased risk of cardiovascular events. LDL cholesterol levels do return to baseline levels by month 18.

Finally, future competition is likely as several other manufacturers are developing their NASH products with their own distinct mechanisms of action, including Genfit's elafibranor and Allergan's cenicriviroc.

Analyst projections for the WAC price for obeticholic acid for the treatment of NASH range from $10,000 to $18,000 per year.

- Advantages: potentially first approved therapy for NASH, significant unmet need, large potential target population, oral and once daily administration
- Disadvantages: failed to demonstrate difference in NASH resolution vs. placebo, lack of long-term outcomes benefit, early increase in LDL cholesterol, potential future competition
- Projected WAC: $10,000 to $18,000 per year

Selumetinib (Brand Name: To be determined)

Manufacturer: AstraZeneca/Merck
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 2Q 2020

Therapeutic use

Selumetinib is in development for treatment of pediatric patients aged three years and older with neurofibromatosis type 1 (NF1) and symptomatic, inoperable plexiform neurofibromas.

- Treatment of pediatric patients aged three years and older with NF1 and symptomatic, inoperable plexiform neurofibromas

NF1 is an incurable genetic condition caused by a spontaneous or inherited mutation in the NF1 gene and is associated with many symptoms, including soft lumps on and under the skin (cutaneous neurofibromas), skin pigmentation, and, in 30% to 50% of patients, tumors develop on the nerve sheaths (plexiform neurofibromas). These plexiform neurofibromas can cause clinical issues such as pain, motor dysfunction, airway dysfunction, bowel/bladder dysfunction and disfigurement as well as having the potential to transform into malignant peripheral nerve sheath tumors (MPNST). NF1 also increases a person's risk of developing other cancers, including malignant brain tumors and leukemia.

NF1 affects one in every 3,000 to 4,000 individuals and symptoms begin during early childhood, with varying degrees of severity, and can reduce life expectancy by up to 15 years.

Clinical profile

- MEK 1/2 inhibitor
- Oral formulation
- ORR = 66%
- Common AEs: nausea, vomiting, diarrhea, asymptomatic creatine kinase increase, paronychia
- Dosing: twice a day

Selumetinib is a MEK 1/2 inhibitor. It is designed to inhibit the MEK enzyme in the RAS/MAPK pathway, a cell signaling pathway, associated with cancer cell growth and proliferation in a number of different tumor types.

<u>Pivotal trial data:</u>
The efficacy of selumetinib was evaluated in SPRINT, a single-arm Phase 2 study in 50 patients. The primary endpoint was ORR, defined as the percentage of patients with a confirmed complete or partial response of ≥ 20% tumor volume reduction. The ORR was achieved in 66% of patients with NF1 and symptomatic, inoperable neurofibromas.

<u>Safety:</u>
The most common adverse events with selumetinib use were nausea, vomiting, diarrhea, asymptomatic creatine kinase increase, and paronychia.

<u>Dosing:</u>
In the pivotal trial, selumetinib was administered orally twice a day until either disease progression or intolerable toxicity.

Selumetinib (continued...)

Competitive environment

Selumetinib will potentially be the first therapy approved for the treatment of NF1. There is a significant unmet need for treatments as patients are often not candidates for surgical treatment and pain management of plexiform neurofibromas due to disease progression. While other therapies have been used off-label for inoperable cases (eg, imatinib or pegylated interferon), benefit is very modest. The early stage data for selumetinib are promising and the drug was relatively well tolerated in the pivotal trial.

However, while the data is encouraging for selumetinib, results are only available from an early stage trial and there is a lack of OS data. In addition, selumetinib may face future competition as other MEK inhibitors are currently being evaluated for NF1.

For reference, Mektovi® (binimetinib), another MEK inhibitor, has a WAC price of approximately $12,000 per 30 days.

- Advantages: potentially first approved therapy for NF1, promising early stage data, oral administration
- Disadvantages: lack of late stage data, lack of robust OS data, potential future competition
- Reference WAC (Mektovi): ~$12,000 per 30 days

1st Quarter 2020

Extended generic pipeline forecast

OptumRx generic pipeline forecast

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

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
OSMOPREP	sodium biphosphate/sodium phosphate	Bausch Health	Tablet	All	01-2020
MOXEZA	moxifloxacin	Alcon	Ophthalmic	All	03-2020
ZORTRESS	everolimus	Novartis	Tablet	All	03-2020
RENOVA	tretinoin	Bausch Health	Cream	All	03-2020
TOTECT	dexrazoxane	Cumberland	Injection	All	03-2020
APTIVUS	tipranavir	Boehringer Ingelheim	Capsule; oral solution	All	04-2020
DEPO-SUBQ PROVERA	medroxyprogesterone	Pfizer	Subcutaneous	All	05-2020
NYMALIZE	nimodipine	Arbor	Oral solution	All	05-2020
ENTEREG	alvimopan	Merck	Capsule	All	2H-2020
TIROSINT	levothyroxine	IBSA Institut Biochemique	Capsule	All	2H-2020
ENBREL	etanercept	Amgen	Subcutaneous	All	2H-2020
KORLYM	mifepristone	Corcept	Tablet	All	2H-2020
FORTEO	teriparatide	Eli Lilly	Injection	All	2H-2020
SYNERA	lidocaine/tetracaine	Galen	Transdermal patch	All	07-2020
PEGASYS	peginterferon alfa-2A	Roche	Subcutaneous	All	08-2020
PEG-INTRON	peginterferon alfa-2B	Merck	Subcutaneous	All	08-2020
POMALYST	pomalidomide	Celgene	Capsule	All	08-2020
MARQIBO KIT	vincristine	Talon Therapeutics/Spectrum	Intravenous	All	09-2020
TYKERB	lapatinib	Novartis	Tablet	All	09-2020
BIDIL	isosorbide dinitrate/ hydrazaline	Arbor	Tablet	All	09-2020
TRUVADA	emtricitabine/ tenofovir	Gilead	Tablet	200 mg/300 mg	09-2020
ATRIPLA	efavirenz/ emtricitabine/ tenofovir	Gilead/Bristol-Myers Squibb	Tablet	All	09-2020
KUVAN	sapropterin	BioMarin	Tablet; oral solution	All	10-2020
RISPERDAL CONSTA	risperidone	Janssen	Injection, extended-release	All	11-2020
XOLEGEL	ketoconazole	Almirall	Gel	All	11-2020
DULERA	formoterol fumarate/ mometasone furoate	Merck	Inhalation	All	11-2020

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
EPIDUO FORTE	adapalene/ benzoyl peroxide	Galderma	Gel	All	12-2020
OFIRMEV	acetaminophen	Mallinckrodt	Intravenous	All	12-2020
ABSORICA	isotretinoin	Sun	Capsule	All	12-2020
TOVIAZ	fesoterodine	Pfizer	Oral; tablet, extended-release	All	12-2020
DALIRESP	roflumilast	AstraZeneca	Tablet	All	12-2020
DEXILANT	dexlansoprazole	Takeda	Capsule, delayed-release	All	12-2020
LUCENTIS	ranibizumab	Roche	Intravitreal	All	12-2020
VELPHORO	sucroferric oxyhydroxide	Fresenius	Tablet, chewable	All	12-2020
2021 Possible launch date					
BEPREVE	bepotastine	Bausch Health	Ophthalmic	All	2021
KERYDIN	tavaborole	Pfizer	Topical solution	All	2021
EMTRIVA	emtricitabine	Gilead	Capsule	All	1H-2021
AMITIZA	lubiprostone	Sucampo/Takeda	Capsule	All	01-2021
CRIXIVAN	indinavir	Merck	Capsule	All	02-2021
NORTHERA	droxidopa	H. Lundbeck	Capsule	All	02-2021
MYALEPT	metreleptin	Aegerion	Subcutaneous	All	02-2021
FORTICAL	calcitonin salmon recombinant	Upsher-Smith	Intranasal	All	02-2021
YONSA	abiraterone	Sun	Tablet	All	03-2021
IMPAVIDO	miltefosine	Knight Therapeutics	Capsule	All	03-2021
ACTOPLUS MET XR	pioglitazone/metformin	Takeda	Tablet, extended-release	All	03-2021
OVIDREL	choriogonadotropin	EMD Serono/Merck	Intramuscular; subcutaneous	All	03-2021
NEUPRO	rotigotine	UCB	Transdermal patch	All	03-2021
LYRICA CR	pregabalin	Pfizer	Tablet, extended-release	All	04-2021
ERAXIS	anidulafungin	Pfizer	Intravenous	All	04-2021
TECFIDERA	dimethyl fumarate	Biogen	Capsule, delayed-release	All	05-2021
ZOMIG	zolmitriptan	Impax/Grunenthal	Intranasal	All	05-2021
PERFOROMIST	formoterol fumarate	Mylan	Inhalation	All	06-2021
APTIOM	eslicarbazepine	Sunovion/Bial	Tablet	All	06-2021

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
INTELENCE	etravirine	Janssen	Tablet	All	06-2021
FLOVENT HFA	fluticasone propionate	GlaxoSmithKline	Inhalation	All	2H-2021
FERAHEME	ferumoxytol	AMAG Pharmaceuticals	Intravenous	All	07-2021
RESCULA	unoprostone isopropyl	R-Tech Ueno	Ophthalmic	All	07-2021
ALTRENO	tretinoin	Bausch Health	Lotion	All	08-2021
BALCOLTRA	levonorgestrel/ethinyl estradiol/ferrous bisglycinate	Avion	Tablet	All	08-2021
SUTENT	sunitinib	Pfizer	Capsule	All	08-2021
SELZENTRY	maraviroc	ViiV Healthcare	Tablet	All	08-2021
JEVTANA KIT	cabazitaxel	Sanofi	Intravenous	All	09-2021
BYSTOLIC	nebivolol	Allergan	Tablet	All	09-2021
PRADAXA	dabigatran etexilate mesylate	Boehringer Ingelheim	Capsule	All	4Q-2021
INNOPRAN XL	propranolol	Ani Pharmaceuticals	Capsule, extended-release	All	10-2021
BIJUVA	estradiol/progesterone	TherapeuticsMD	Capsule	All	10-2021
MIRCERA	methoxy polyethylene glycol-epoetin beta	Roche/Royalty Pharma	Subcutaneous	All	11-2021
BROVANA	arformoterol	Sunovion	Inhalation	All	11-2021
ONEXTON	clindamycin/benzoyl peroxide	Bausch Health	Gel	All	12-2021
EPANED KIT	enalapril	Silvergate	Oral solution	All	12-2021
CHANTIX	varenicline	Pfizer	Tablet	All	12-2021
CAYSTON	aztreonam lysine	Gilead	Inhalation	All	12-2021
BETHKIS	tobramycin	Chiesi	Inhalation	All	12-2021
MYTESI	crofelemer	Napo	Tablet, delayed-release	All	12-2021
EXPAREL	bupivacaine	Pacira	Injection	All	12-2021
SUPREP BOWEL PREP KIT	magnesium sulfate anhydrous/potassium sulfate / sodium sulfate	Braintree	Oral solution	All	12-2021
AFINITOR DISPERZ	everolimus	Novartis	Oral suspension	All	12-2021
2022 Possible launch date					
PREZISTA	darunavir	Janssen	Tablet	75 mg, 150 mg, 300 mg	2022
SOLIRIS	eculizumab	Alexion	Intravenous	All	1H-2022
NATPARA	parathyroid hormone 1-84	NPS/Nycomed	Subcutaneous	All	01-2022

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
NPLATE	romiplostim	Amgen	Subcutaneous	All	01-2022
OXAYDO	oxycodone	Egalet	Tablet	All	01-2022
VIMPAT	lacosamide	UCB	Intravenous; tablet; oral solution	All	03-2022
ZIPSOR	diclofenac potassium	Depomed	Capsule	All	03-2022
CHOLBAM	cholic acid	Retrophin	Capsule	All	03-2022
ABRAXANE	paclitaxel	Celgene/Abraxis	Injection	All	03-2022
REVLIMID	lenalidomide	Celgene	Capsule	All	03-2022
ARESTIN	minocycline hydrochloride	Bausch Health	Subgingival, sustained-release	All	03-2022
MAVENCLAD	cladribine	Serono	Tablet	All	03-2022
LEXISCAN	regadenoson	Astellas	Intravenous	All	04-2022
COMBIGAN	brimonidine/timolol	Allergan	Ophthalmic	All	04-2022
TEFLARO	ceftaroline fosamil	Allergan	Intravenous	All	04-2022
ZOLADEX	goserelin	TerSera Therapeutics	Subcutaneous	All	04-2022
DUOBRII	halobetasol propionate/tazarotene	Bausch Health	Lotion	All	04-2022
BANZEL	rufinamide	Eisai	Tablet	All	05-2022
ALIMTA	pemetrexed disodium	Eli Lilly	Intravenous	All	05-2022
BANZEL	rufinamide	Eisai	Tablet; oral suspension	All	05-2022
VELCADE	bortezomib	Takeda	Intravenous	All	05-2022
TARGINIQ ER	oxycodone/naloxone	Purdue	Tablet, extended-release	All	05-2022
CAPRELSA	vandetanib	Genzyme/Sanofi	Tablet	All	06-2022
VIIBRYD	vilazodone	Forest/Allergan	Tablet	All	06-2022
ELESTRIN	estradiol	Mylan	Gel	All	06-2022
IRESSA	gefitinib	AstraZeneca	Tablet	All	07-2022
EYLEA	aflibercept	Regeneron	Intraocular	All	07-2022
ACTEMRA	tocilizumab	Roche/Chugai	Intravenous; subcutaneous	All	07-2022
EVAMIST	estradiol	Perrigo/Elan	Transdermal solution	All	07-2022
IXEMPRA Kit	ixabepilone	R-Pharm	Intravenous	All	07-2022

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
VOSEVI	sofosbuvir/ velpatasvir/ voxilaprevir	Gilead	Tablet	All	07-2022
VIBATIV	telavancin	Theravance	Intravenous	All	08-2022
SOLOSEC	secnidazole	Symbiomix Therapeutics	Oral granules	All	09-2022
ORAVIG	miconazole	Midatech/R-Pharm	Tablet, buccal	All	09-2022
HALFLYTELY with BISACODYL	bisacodyl / polyethylene glycol 3350, potassium chloride, sodium bicarbonate, sodium chloride	Braintree	Tablet/oral solution	All	10-2022
ORENCIA	abatacept	Bristol-Myers Squibb	Intravenous; subcutaneous	All	11-2022
XERESE	acyclovir/ hydrocortisone	Bausch Health	Cream	All	11-2022
NAGLAZYME	galsulfase	BioMarin	Intravenous	All	11-2022
FOLOTYN	pralatrexate	Acrotech/Aurobindo	Intravenous	All	11-2022
NASCOBAL	cyanocobalamin	Par/Endo	Intranasal	All	12-2022
MYRBETRIQ	mirabegron	Astellas	Tablet, extended-release	All	12-2022
DYLOJECT	diclofenac	Hospira/Pfizer/Javelin	Intravenous	All	12-2022
RAYOS	prednisone	Horizon	Tablet, delayed-release	All	12-2022
TREANDA	bendamustine	Cephalon/Teva	Intravenous	All	12-2022
ZIOPTAN	tafluprost	Akorn	Ophthalmic	All	12-2022
SEGLUROMET	ertugliflozin/metformin	Merck	Tablet	All	12-2022

+ = may launch during the stated date or later

Extended brand pipeline forecast

OptumRx Brand Pipeline Forecast

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
2020 Possible launch date									
BMS-927711 (BHV-3000)	rimegepant sulfate	Biohaven	calcitonin gene-related peptide (CGRP) receptor antagonist	Migraine	PO	Filed NDA	2/2020	No	No
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
FP-001 (LMIS)	leuprolide mesylate	Foresee	gonadotropin-releasing hormone (GnRH) analog	Prostate cancer	SC	Filed NDA	2/2020	Yes	No
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
naloxone nasal spray	naloxone	Insys Therapeutics	opioid antagonist	Opioid dependence	Intranasal	Filed NDA	3/15/2020	No	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

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Fintepla	fenfluramine	Zogenix	serotonin receptor agonist	Dravet syndrome	PO	Filed NDA	3/25/2020	Yes	Yes
Rizaport	rizatriptan	IntelGenx	triptan	Acute migraines	PO	Filed NDA	3/26/2020	No	No
HTX-011	bupivacaine/ meloxicam	Heron Therapeutics	anesthetic/ Nonsteroidal Anti-inflammatory Drug (NSAID)	Pain	Instillation	Filed NDA	3/26/2020	No	No
SPARC-1028, SPARC-1210, SPARC-1023	paclitaxel injection concentrate for suspension	Sun Pharma Advanced Research Company (SPARC)	taxane	Breast Cancer; Lung Cancer; Pancreatic Cancer	IV	Filed NDA	1Q2020	No	No
Aximris XR	oxycodone ER	IntelliPharmaCeutics	opioid agonist	Pain	PO	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
E-58425	celecoxib/ tramadol	Esteve	non-steroid anti-inflammatory drug/opioid	Acute pain	PO	Filed NDA	3/2020 - 4/2020	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
MitoGel	mitomycin C	UroGen	alkylating agent	Urothelial cancer	Intravesical	Filed NDA	4/18/2020	No	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Men Quad TT	meningococcal polysaccharide (serogroups A, C, Y, and W135) tetanus toxoid conjugate vaccine	Sanofi	antibacterial	Meningococcal meningitis	IM	Filed BLA	4/25/2020	No	No
Ongentys	opicapone	Neurocrine Biosciences	catechol-O-methyltransferase (COMT) inhibitor	Parkinson's disease	PO	Filed NDA	4/26/2020	No	No
Trevyent	treprostinil	United Therapeutics	prostacyclin analog	Pulmonary arterial hypertension	SC	Filed NDA	4/27/2020	Yes	Yes
isatuximab	isatuximab	Sanofi	CD38 antagonist	Multiple myeloma	IV	Filed BLA	4/30/2020	Yes	Yes
selumetinib	selumetinib	AstraZeneca/ Merck	selective MEK kinase inhibitor	Neurofibromatosis	PO	Filed NDA	4/2020 to 5/2020	Yes	Yes
SEP-225289	dasotraline	Sunovion	triple reuptake inhibitor	Binge eating disorder	PO	Filed NDA	5/14/2020	No	No
APL-130277	apomorphine	Sunovion	non-ergoline dopamine agonist	Parkinson's disease	PO	Filed NDA	5/21/2020	No	No
RG-7916 (RO-7034067)	risdiplam	Roche/ PTC Therapeutics	SMN2 splicing modifier	Spinal muscular atrophy	PO	Filed NDA	5/24/2020	Yes	Yes
Amphora	Amphora	Evofem Biosciences	spermicidal agent	Pregnancy prevention	VG	Filed NDA	5/25/2020	No	No
INCB-54828	pemigatinib	Incyte	selective FGFR1/2/3 inhibitor	Biliary tract cancer	PO	Filed NDA	5/30/2020	Yes	Yes
nadofaragene firadenovec	nadofaragene firadenovec	Ferring Pharmaceuticals	gene therapy	Bladder cancer	Intravesical	Filed BLA	5/2020 - 6/2020	Yes	No
FMX-103	minocycline	Foamix	tetracyclines	Rosacea	TOP	Filed NDA	6/2/2020	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
IMMU-132	sacituzumab govitecan	Immunomedics	RS7-SN-38 antibody-drug conjugate	Breast cancer	IV	Filed BLA	6/2/2020	Yes	No
NS-065	viltolarsen	Nippon Shinyaku	morpholino antisense oligonucleotide	Duchenne muscular dystrophy	IV	Filed BLA	6/2/2020	Yes	Yes
EM-100	ketotifen	Eton	antihistamine	Allergic conjunctivitis	OP	Filed NDA	6/11/2020	No	No
Contepo	fosfomycin	Nabriva Therapeutics	cell wall inhibitor	Bacterial infections	IV	Filed NDA	6/19/2020	Yes	No
EVK-001	metoclopramide	Evoke Pharma	antidopiminergics	Diabetic gastroparesis	Intranasal	Filed NDA	6/19/2020	No	No
obeticholic acid	obeticholic acid	Intercept Pharmaceuticals	farnesoid X receptor (FXR) agonist	Nonalcoholic steatohepatitis	PO	Filed NDA	6/26/2020	Yes	No
Mycapssa (Octreolin)	octreotide	Chiasma	somatostatin analog	Acromegaly	PO	Filed NDA	6/2020	Yes	Yes
OMB-157	ofatumumab	Novartis	CD20 monoclonal antibody	Multiple sclerosis	SC	Filed BLA	6/2020	Yes	No
Bronchitol	mannitol	Pharmaxis	osmotic gradient enhancer; mucus clearance enhancer	Cystic fibrosis	INH	Filed NDA	2Q2020	No	Yes
insulin glargine	insulin glargine	Mylan/ Biocon	Long-acting insulin	Diabetes mellitus	SC	CRL	Mid-2020	No	No
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	6/2020 – 7/2020	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
DFN-15	celecoxib	Dr. Reddy	nonsteroidal anti-inflammatory drug (NSAID)	Migraine	PO	Filed NDA	6/2020 – 7/2020	No	No
collagenase clostridium histolyticum	collagenase clostridium histolyticum	Endo	protease enzyme	Cellulite	SC	Filed BLA	7/6/2020	Yes	No
Darzalex	daratumumab (with recombinant human hyaluronidase)	Janssen	humanized anti-CD38 monoclonal antibody	Multiple myeloma	SC	Filed BLA	7/12/2020	Yes	Yes
VP-102	VP-102	Verrica	antiviral	Molluscum	TOP	Filed NDA	7/13/2020	No	No
RVL-1201	oxymetazoline	Osmotica/ Vertical Pharmaceuticals	alpha-adrenergic receptor agonist	Acquired blepharoptosis (droopy eyelid)	OP	Filed NDA	7/16/2020	No	No
MC2-01	calcipotriene/ betamethasone	MC2 Therapeutics	vitamin D analog/ corticosteroid	Psoriasis	TOP	Filed NDA	7/20/2020	No	No
JZP-258	sodium oxybate extended-release	Jazz	dopamine receptor agonist	Narcolepsy	PO	Filed NDA	7/21/2020	Yes	Yes
Corplex donepezil	donepezil	Corium International	acetylcholinesterase inhibitor	Alzheimer's disease	TOP	Filed NDA	7/30/2020	No	No
UX-007	triheptanoin	Ultragenyx	medium chain fatty acid	Glucose transport type 1 deficiency syndrome	PO	Filed NDA	7/31/2020	Yes	Yes
LOXO-292	selpercatinib	Eli Lilly/ Loxo Oncology	RET inhibitor	Non-small cell lung cancer; thyroid cancer	PO	Filed NDA	7/2020 - 8/2020	Yes	No
ASTX-727	decitabine and E-7727	Astex Pharmaceuticals	nucleoside metabolic inhibitor	Myelodysplastic syndrome	PO	Filed NDA	7/2020 - 8/2020	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Viaskin Peanut	Viaskin Peanut	DBV Technologies	Immunotherapy	Peanut allergy	TOP	Filed BLA	8/5/2020	No	No
BMS-663068	fostemsavir	Bristol-Myers Squibb	HIV attachment inhibitor	HIV	PO	Filed NDA	8/5/2020	Yes	No
KTE-X19	KTE-X19	Gilead	chimeric antigen receptor (CAR) T cell therapy	Mantle cell lymphoma	IV	Filed BLA	8/10/2020	Yes	Yes
TRV-130	oliceridine	Trevena	opioid receptor agonist	Pain	IV	Filed NDA	8/10/2020	Yes	No
BCX-7353	berotralstat	BioCryst	kallikrein inhibitor	Hereditary angioedema	PO	Filed NDA	8/11/2020	Yes	Yes
INCB-028060 (INC-280)	capmatinib	Novartis/ Incyte	cMET inhibitor	Non-small cell lung cancer	PO	Filed NDA	8/11/2020	Yes	Yes
Pedmark (STS)	sodium thiosulfate	Fennec	reducing agent	Hearing loss	IV	Filed NDA	8/11/2010	Yes	Yes
DCC-2618	ripretinib	Deciphera	PDGFR-alpha kinase inhibitor	Gastrointestinal stromal tumors	PO	Filed NDA	8/14/2020	Yes	Yes
GSK-2857916	belantamab mafodotin	GlaxoSmithKline/ Seattle Genetics	anti-BCMA antibody-drug conjugate	Multiple myeloma	SC	Filed BLA	8/16/2020	Yes	Yes
Zepsyre	lurbinectidin	PharmaMar	alkylating agent	Small cell lung cancer	IV	Filed NDA	8/16/2020	Yes	Yes
GLPG-0634	filgotinib	Gilead	janus associated kinase-1 (JAK) inhibitor	Rheumatoid arthritis	PO	Filed NDA	8/18/2020	Yes	No
JCAR-017	lisocabtagene maraleucel	Bristol-Myers Squibb/ Celgene	chimeric antigen receptor (CAR) T cell therapy	Diffuse large B-cell lymphoma	IV	Filed BLA	8/18/2020	Yes	Yes
MAGH-22	margetuximab	MacroGenics	HER2 oncoprotein antagonist	Breast cancer	IV	Filed BLA	8/19/2020	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
TRC-101	veverimer	Tricida	carrier protein modulator	Chronic kidney disease	PO	Filed NDA	8/22/2020	Yes	No
ARRY-380 (ONT-380)	tucatinib	Seattle Genetics	ErbB-2 (Her-2/neu) inhibitor	Breast cancer	PO	Filed NDA	8/23/2020	Yes	Yes
BMN-270	valoctocogene roxaparvovec	BioMarin	gene therapy	Hemophilia A	IV	Filed BLA	8/23/2020	Yes	Yes
Winlevi	clascoterone	Cassiopea	androgen antagonist	Acne vulgaris	TOP	Filed NDA	8/27/2020	No	No
MOR-208	tafasitamab	MorphoSys/ Xencor	CD-19 antagonist	Diffuse large B-cell lymphoma	IV	Filed BLA	8/28/2020	Yes	Yes
SA-237	satralizumab	Roche	interleukin-6 (IL-6) monoclonal antibody	Neuromyelitis optica	SC	Filed BLA	8/2020 - 9/2020	Yes	Yes
NNC-0195-0092	somapacitan	Novo Nordisk	recombinant human growth hormone (rhGH)	Growth hormone deficiency	SC	Filed BLA	9/21/2020	Yes	No
LJPC-0118	artesunate	La Jolla Pharmaceutical	protozoacide	Malaria	Undisclosed	Filed NDA	9/25/2020	No	Yes
Libervant	diazepam	Aquestive Therapeutics	benzodiazepine	Seizures	PO	Filed NDA	9/27/2020	No	Yes
Prochymal	remestemcel-L	Mesoblast	mesenchymal stem cells	Graft vs. Host disease	IV	Filed BLA	9/30/2020	Yes	Yes
LY-900014	insulin lispro	Eli Lilly	insulins	Diabetes mellitus	SC	Filed BLA	3Q2020	No	No
Infacort	hydrocortisone	Diurnal Group	corticosteroid	Adrenal insufficiency	PO	Filed NDA	10/2/2020	No	Yes
tramadol	tramadol	Avenue Therapeutics	opioid receptor agonist	Pain	IV	Filed NDA	10/9/2020	No	No
Qtrypta	zolmitriptan	Zosano	triptan	Acute migraines	TOP	Filed NDA	10/20/2020	No	No

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	10/24/2020	Yes	No
SPN-812	viloxazine	Supernus	selective norepinephrine reuptake inhibitor	Attention deficit hyperactivity disorder	PO	Filed NDA	11/8/2020	No	No
ALKS-3831	olanzapine/ samidorphan	Alkermes	dopamine receptor antagonist/ opioid receptor antagonist	Schizophrenia/ Bipolar disorder	PO	Filed NDA	11/15/2020	No	No
RT-002 (Daxi)	daxibotulinumtoxinA	Revance Therapeutics	botulinum toxins	Glabellar lines (frown lines)	IM	Filed BLA	11/25/2020	Yes	No
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension	INH	Filed NDA	11/27/2020	Yes	No
nifurtimox	nifurtimox	Bayer	anti-parasitic, anti-protozoal	Chagas disease	PO	Filed NDA	11/30/2020	No	Yes
RG-6264	trastuzumab/ pertuzumab	Roche	HER2/neu receptor antagonist	Breast cancer	SC	Filed BLA	11/2020 - 12/2020	Yes	No
FG-4592	roxadustat	FibroGen/ AstraZeneca	hypoxia-inducible factor prolyl hydroxylase (HIF-PHI)	Anemia	PO	Filed NDA	12/23/2020	Yes	No
LY-03005	ansofaxine	Luye Pharma	serotonin-norepinephrine-dopamine triple reuptake inhibitor	Major depressive disorder	PO	Filed NDA	12/26/2020	No	No
MK-4618 (KRP-114V, RVT-901)	vibegron	Urovant Sciences	selective beta 3 adrenergic receptor agonist	Overactive bladder	PO	Filed NDA	12/29/2020	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
inclisiran	inclisiran	The Medicines Company/ Novartis	siRNA targeting proprotein convertase subtilisin kexin type 9 (PCSK9)	Hyperlipidemia	SC	Filed NDA	12/2020	Yes	Yes
tanezumab	tanezumab	Pfizer/ Eli Lilly	nerve growth factor antibody	Osteoarthritis	SC	Filed BLA	12/2020	Yes	No
TGR-1202	umbralisib	TG Therapeutics	phosphoinositide-3 kinase (PI3K) delta inhibitor	Marginal zone lymphoma/ follicular lymphoma	PO	InTrial	4Q2020	Yes	Yes
REGN-EB3	REGN-EB3	Regeneron	anti-Ebola virus	Ebola	IV	InTrial	4Q2020	Yes	Yes
PRO-140	leronlimab	CytoDyn	C-C chemokine receptor 5 (CCR5) antagonist	HIV	SC	InTrial	4Q2020	Yes	Yes
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
Zynteglo (LentiGlobin)	lentiviral beta-globin gene transfer	Bluebird Bio	gene therapy	Beta-thalassemia	IV	InTrial	4Q2020	Yes	Yes
131I-8H9	omburtamab	Y-mAbs Therapeutics	B7-H3 antagonist	Brain cancer	Undisclosed	InTrial	4Q2020	Yes	Yes
3-F8 (Hu-3F8)	naxitamab	Y-mAbs Therapeutics	GD2 antagonist	Neuroblastoma	IV	InTrial	4Q2020	Yes	Yes
BLU-667	pralsetinib	Blueprint Medicines	RET inhibitor	Non-small cell lung cancer	PO	InTrial	4Q2020	Yes	Yes
GSP-301	mometasone furoate/ olopatadine HCl	Glenmark	corticosteroid/ antihistamine	Allergic rhinitis	Intranasal	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
bb-2121	idecabtagene vicluecel	Celgene/ Bluebird Bio	chimeric antigen receptor (CAR) T cell therapy	Multiple myeloma	IV	InTrial	2H2020	Yes	Yes
EBP-994 (rEBP-994)	lonafarnib	Eiger Biopharmaceuticals	prenylation inhibitor	Hutchinson-Gilford Progeria Syndrome (HGPS or progeria) and progeroid laminopathies	PO	InTrial	2H2020	Yes	Yes
TMC-278-LA	cabotegravir (long-acting)/ rilpivirine (long-acting)	ViiV Healthcare	HIV integrase inhibitor/ non-nucleoside reverse transcriptase inhibitor (NNRTI)	HIV	IM	CRL	2020	Yes	No
S-265744 (S/GSK-1265744)	cabotegravir	ViiV Healthcare	HIV integrase inhibitor	HIV	PO	CRL	2020	Yes	No
ALNG-01	lumasiran	Alnylam	glycolate oxidase antagonist	Hyperoxaluria	Intranasal	InTrial	Late 2020	Yes	Yes
RVT-802	RVT-802	Enzyvant/Roivant	Tissue-based therapy	Congenital athymia	Implant	CRL	Late 2020	Yes	Yes
PRX-102	alpha galactosidase (pegunigalsidase alfa)	Protalix	enzyme replacement	Fabry disease	IV	InTrial	Late 2020	Yes	No
BGF-MDI (PT-010)	budesonide/ glycopyrronium/ formoterol	AstraZeneca	corticosteroid/ long-acting muscarinic receptor antagonist (LAMA)/ long-acting beta 2 adrenergic receptor agonist (LABA)	Chronic obstructive pulmonary disease	INH	CRL	Late 2020	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
TG-1303	ublituximab/ TGR-1202	TG Therapeutics	CD-20 monoclonal antibody/ phosphoinositide-3 kinase (PI3K) delta inhibitor	Chronic lymphocytic leukemia/ Non-Hodgkin lymphoma	IV/PO	InTrial	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
CLS-1001	triamcinolone acetonide	Clearside	corticosteroid	Macular edema	Intraocular	CRL	Late 2020	Yes	No
Bafiertam	monomethyl fumarate	Banner Life Sciences	Nrf2 pathway activator	Multiple sclerosis	PO	Tentative Approval	Late 2020	Yes	No
OMS-721	narsoplimab	Omeros	anti-MASP-2 monoclonal antibody	Hematopoietic stem cell transplant-associated thrombotic microangiopathy	IV/SC	InTrial	Late 2020	Yes	Yes
Lucassin	terlipressin	Orphan Therapeutics/ Ikaria	V-1 (vasopressin) agonist	Hepato-renal syndrome	IV	CRL	Late 2020	Yes	Yes
ALXN-1101	fosdenopterin	BridgeBio Pharma/ Origin Biosciences	molybdenum cofactor stimulant	Molybdenum cofactor deficiency	IV	InTrial	Late 2020	Yes	Yes
CAM-2038	buprenorphine	Camurus/ Braeburn	opioid receptor agonist (partial)	Opioid use disorder/ Pain	SC	Tentative Approval	Late 2020	Yes	No
Zimhi	naloxone	Adamis	opioid antagonist	Opioid dependence	IM	CRL	Late 2020	No	No
Entyvio (SC formulation)	vedolizumab	Takeda	integrin receptor antagonist	Ulcerative colitis/ Crohn's disease	SC	CRL	Late 2020	Yes	No
ropeginterferon alfa-2b	ropeginterferon alfa-2b	PharmaEssentia/ AOP Orphan	interferon	Polycythemia vera	SC	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
Neutrolin (CRMD-003, CRMD-004)	citrate/ taurolidine/ heparin	CorMedix	antimicrobial agent/ anticoagulant	Catheter-related infections	IV	InTrial	Late 2020	No	No
SRP-4045	casimersen	Sarepta	morpholino antisense oligonucleotide	Duchenne muscular dystrophy	IV	InTrial	Late 2020	Yes	Yes
Apealea (Paclical)	paclitaxel	Oasmia	taxane	Ovarian cancer	IV	InTrial	Late 2020	Yes	Yes
Ryplazim	human plasminogen	ProMetic/ Hematech	plasminogen	Plasminogen deficiency	IV	CRL	Late 2020	Yes	Yes
2021 Possible launch date									
TSR-042	dostarlimab	GlaxoSmithKline	PD-1 checkpoint inhibitor	Endometrial cancer	IV	Filed BLA	1/14/2021	Yes	No
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	CRL	1Q2021	Yes	Yes
StrataGraft Skin Tissue	StrataGraft Skin Tissue	Mallinckrodt	autologous skin tissue	Burn injury	TOP	InTrial	1Q2021	Yes	Yes
KPI-121 0.25%	loteprednol etabonate	Kala	corticosteroid	Dry eyes	OP	CRL	1Q2021	No	No
ET-103	levothyroxine	Eton Pharmaceuticals	L-thyroxine	Hypothyroidism	PO	InTrial	1Q2021	No	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
Oraxol	HM-30181A/ paclitaxel	Athenex	P-glycoprotein pump inhibitor/ taxane	Breast cancer	PO	InTrial	1Q2021	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
AGIL-AADC	AGIL-AADC	Agilis Biotherapeutics	gene therapy	Aromatic L-amino acid decarboxylase deficiency	Intracerebral	InTrial	1Q2021	Yes	Yes
BIIB-037	aducanumab	Biogen	amyloid beta-protein inhibitor	Alzheimer's disease	IV	InTrial	1Q2021	Yes	No
Furoscix	furosemide	scPharmaceuticals	diuretic	Heart failure	SC	CRL	1Q2021	Yes	No
CC-486	azacitidine	Celgene	DNA methylation inhibitor	Acute myeloid leukemia	PO	InTrial	1Q2021	Yes	Yes
KP-415	D-threo-methylphenidate controlled-release	KemPharm	CNS stimulant	Attention deficit hyperactivity disorder	PO	InTrial	1Q2021	No	No
TAK-385	relugolix	Myovant Sciences/ Roivant Sciences/ Takeda	gonadotropin-releasing hormone (GnRH) receptor antagonist	Uterine fibroids/ Endometriosis	PO	InTrial	1H2021	Yes	No
RG-3477 (ACT-128800)	ponesimod	Johnson & Johnson	sphingosine 1 phosphate (S1P) receptor agonist	Multiple sclerosis	PO	InTrial	1H2021	Yes	No
ACP-001	TransCon Growth Hormone	Ascendis	growth hormone prodrug	Short stature/ Growth hormone deficiency	SC	InTrial	1H2021	Yes	No
CCX-168	avacopan	ChemoCentryx/ Galencia	C5a receptor (C5aR) antagonist	Vasculitis	PO	InTrial	1H2021	Yes	Yes
arimoclomol	arimoclomol	Orphazyme	cytoprotectives	Niemann-Pick Disease	PO	InTrial	1H2021	Yes	Yes
NX-1207 (NYM-4805, REC 0482)	fexapotide triflutate	Nymox	pro-apoptotic	Benign prostatic hyperplasia	Intratumoral	InTrial	1H2021	Yes	No
sulopenem	sulopenem	Iterum Therapeutics	carbapenem	Bacterial infection	IV/PO	InTrial	1H2021	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Vicinium (VB-4-845)	oportuzumab monatox	Sesen Bio	anti-ECAM exotoxin A fusion protein	Bladder cancer	Intravesical	InTrial	1H2021	Yes	No
SCY-078 (MK-3118)	ibrexafungerp	Scynexis	glucan synthase inhibitors	Fungal infections	IV/PO	InTrial	1H2021	No	Yes
Luveniq	voclosporin	Aurinia Pharmaceuticals	calcineurin inhibitor	Lupus nephritis	PO	InTrial	1H2021	Yes	No
BMN-111	vosoritide (vasoritide)	BioMarin/ Chugai	C-type natriuretic peptide (CNP) analog	Achondroplasia	SC	InTrial	1H2021	Yes	Yes
AT-132 (AAV8-MTM1)	AT-132 (AAV8-MTM1)	Audentes Therapeutics	gene therapy	X-linked myotubular myopathy	IV	InTrial	Mid-2021	Yes	Yes
BIVV-009	sutimlimab	Sanofi	complement C1s subcomponent inhibitor	Cold agglutinin disease	IV	InTrial	Mid-2021	Yes	Yes
ZYN-002	ZYN-002	Zynerba	cannabinoid product	Fragile X syndrome	TOP	InTrial	Mid-2021	Yes	Yes
AXS-07	meloxicam/rizatriptan	Axsome Therapeutics	non-steroidal anti-inflammatory drug/triptan	Migraine	PO	InTrial	Mid-2021	No	No
Iomab-B	iodine I 131 monoclonal antibody BC8	Actinium	anti-CD45 monoclonal antibody	Acute myeloid leukemia/ Myelodysplastic syndrome	IV	InTrial	Mid-2021	Yes	Yes
Recentin	cediranib	AstraZeneca	vascular endothelial growth factor receptor (VEGF) antagonist	Ovarian cancer	PO	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

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
CUTX-101	copper histidinate	Fortress Biotech	copper histidinate	Menkes Disease	SC	InTrial	Mid-2021	Yes	Yes
Estybon	rigosertib	Onconova	non-ATP competitive kinase inhibitor	Myelodysplastic syndrome	IV	InTrial	Mid-2021	Yes	Yes
ADCT-402	loncastuximab tesirine	ADC Therapeutics	antibody drug conjugate	Diffuse large B-cell lymphoma	IV	InTrial	Mid-2021	Yes	Yes
RSV-F (ResVax)	respiratory syncytial virus vaccine	Novavax	vaccine	Respiratory syncytial virus infection	IM	InTrial	Mid-2021	Yes	No
UCB-4940	bimekizumab	UCB	interleukin-17 (IL-17) receptor inhibitor	Plaque psoriasis	IV	InTrial	Mid-2021	Yes	No
entinostat	entinostat	Syndax	histone deacetylase (HDAC) inhibitor	Breast cancer	PO	InTrial	Mid-2021	Yes	No
EMD-1214063	tepotinib	Merck	c-Met receptor tyrosine kinase inhibitor	Non-small cell lung cancer	PO	InTrial	Mid-2021	Yes	No
EBV-CTL	tabelecleucel	Atara Biotherapeutics	cell therapy	Lymphoproliferative disorder	IV	InTrial	Mid-2021	Yes	Yes
pIL-12	tavokinogene telsaplasmid	OncoSec Medical	gene therapy	Melanoma	Intratumoral	InTrial	Mid-2021	Yes	Yes
GZ-402666 (NeoGAA)	avalglucosidase alfa	Sanofi	enzyme therapy	Pompe disease	IV	InTrial	Mid-2021	Yes	No
PDR-001	spartalizumab	Novartis	PD-1 checkpoint inhibitor	Melanoma	IV	InTrial	Mid-2021	Yes	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
AT-GAA	recombinant human acid alpha-glucosidase + AT2220	Amicus	enzyme therapy	Pompe disease	IV	InTrial	Mid-2021	Yes	Yes
BGJ-398	infigratinib	BridgeBio	FGFR inhibitor	Biliary tract cancer	PO	InTrial	Mid-2021	Yes	Yes
RG-7388 (RO-5503781)	idasanutlin	Roche	MDM2 antagonist	Acute myelogenous leukemia	PO	InTrial	Mid-2021	Yes	No
Lenti-D	elivaldogene tavalentivec	Bluebird Bio	gene therapy	cerebral adreno-myeloneuropathy	IV	InTrial	Mid-2021	Yes	Yes
KD-025	KD-025	Kadmon	ROCK2 (Rho-associated coiled-coiled kinase 2) inhibitor	Graft vs. Host disease	PO	InTrial	Mid-2021	No	Yes
PF-04965842	abrocitinib	Pfizer	janus kinase 1 (JAK-1) inhibitor	Atopic dermatitis	PO	InTrial	3Q2021	Yes	No
TBR-652 (TAK-652, CVC)	cenicriviroc	Allergan	C-C chemokine receptor 5 (CCR5) and receptor 2 antagonist	Nonalcoholic steatohepatitis	PO	InTrial	4Q2021	Yes	No
VBP-15	vamorolone	Santhera	corticosteroid	Duchenne muscular dystrophy	PO	InTrial	4Q2021	Yes	Yes
OS-01 nasal spray	OC-01	Oyster Point Pharma	nicotinic acetylcholine receptor (nAChR) agonist	Dry eye disease	Intranasal	InTrial	4Q2021	No	No
OPNT-003	nalmefene	Opiant	opioid receptor antagonist	Opioid overdose	Intranasal	InTrial	4Q2021	No	No
MOD-401	somatrogon	OPKO Health/ Pfizer	enzyme replacement	Growth hormone deficiency	SC	InTrial	2H2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
PRV-031	teplizumab	MacroGenics/ Provention Bio	CD3 antigen inhibitor	Diabetes mellitus	IV	InTrial	4Q2021	Yes	Yes
MK-0594 (VPD-737)	serlopitant	Menlo	NK-1 receptor antagonist	Pruritus	PO	InTrial	2H2021	Yes	No
BXCL-501	dexmedetomidine	BioXcel Therapeutics	selective alpha 2a receptor agonist	Schizophrenia and bipolar disorder	PO	InTrial	2H2021	No	No
IDP-124	pimecrolimus	Bausch Health	calcineurin Inhibitor	Atopic dermatitis	TOP	InTrial	2H2021	No	No
MD-1003	MD-1003	MedDay	biotin	Multiple sclerosis	PO	InTrial	2H2021	Yes	No
CAT-354	tralokinumab	Leo Pharma	interleukin-13 (IL-13) inhibitor	Atopic dermatitis	SC	InTrial	2H2021	Yes	No
NPI-2358	plinabulin	BeyondSpring	tumor vascular disrupting agent (tVDA)	Neutropenia	IV	InTrial	2H2021	Yes	No
MEDI-546	anifrolumab	AstraZeneca/ BMS	interferon receptor antagonist	Systemic lupus erythematosus	IV	InTrial	2H2021	Yes	No
LCAR-B38M	LCAR-B38M	Johnson & Johnson	chimeric antigen receptor (CAR) T cell therapy	Multiple myeloma	Undisclosed	InTrial	2H2021	Yes	Yes
ARGX-113	efgartigimod	Argen NV	Fc antagonist	Myasthenia gravis	IV/SC	InTrial	2H2021	Yes	Yes
PL-56	budesonide	Calliditas/ Kyowa Hakko Kirin	corticosteroid	Nephropathy	PO	InTrial	2H2021	No	Yes
SPN-810	molindone	Supernus	atypical antipsychotic	Attention deficit hyperactivity disorder	PO	InTrial	2H2021	No	No

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
CR-845	difelikefalin	Cara Therapeutics	opioid receptor agonist	Pruritus	IV/PO	InTrial	2H2021	No	No
GSK-2696274 (OTL-200)	GSK-2696274 (OTL-200)	GlaxoSmithKline	gene therapy	Leukodystrophy	IV	InTrial	2H2021	Yes	Yes
LN-145	lifileucel	Iovance Biotherapeutics	tumor infiltrating lymphocyte	Cervical Cancer	IV	InTrial	2H2021	Yes	No
GFT-505	elafibranor	Genfit	selective peroxisome proliferator-activated receptor (PPAR) modulator	Nonalcoholic steatohepatitis	PO	InTrial	2H2021	Yes	No
Sci-B-Vac	hepatitis B vaccine	VBI Vaccines	vaccine	Hepatitis B	IM	InTrial	2H2021	No	No
AXS-05	Dextro-methorphan/ bupropion	Axsome	N-methyl-D-aspartate (NMDA) antagonist/ antidepressant	Treatment-resistant depression	PO	InTrial	2H2021	No	No
INP-104	Dihydro-ergotamine mesylate	Impel/ 3M	ergot derivative	Acute migraines	Intranasal	InTrial	2H2021	No	No
TWIN (S6G5T-1; S6G5T-3)	benzoyl peroxide/ tretinoin	Sol-Gel Technologies	retinoid	Acne vulgaris	TOP	InTrial	2H2021	No	No
177Lu-PSMA-617	Lutetium	Novartis	Radiopharmaceutical	Prostate cancer	IV	InTrial	2H2021	Yes	No
dovitinib	dovitinib	Oncology Venture	fibroblast growth factor receptor 3 (FGFR3) inhibitor	Renal cell carcinoma	PO	InTrial	2H2021	Yes	No
AT-007	AT-007	Applied Therapeutics	aldose reductase inhibitor	Galactosemia	Undisclosed	InTrial	2H2021	Yes	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
MLN-4924 (TAK-92)	pevonedistat	Takeda	Nedd 8 Activating Enzyme (NAE) antagonist	Myelodysplastic syndrome	IV	InTrial	2021	Yes	No
RG-7440 (GDC-0068)	ipatasertib	Roche	pan-Akt inhibitor	Prostate cancer; breast cancer	PO	InTrial	2021	Yes	No
Oralair Mites	dust mite peptide	Stallergenes/ Shionogi	vaccine	Dust mite allergic rhinitis	PO	InTrial	2021	Yes	No
RTA-408	omaveloxolone	Reata Pharmaceuticals	Nrf2 activator	Friedreich's ataxia	PO	InTrial	2021	Yes	Yes
LY-686017	tradipitant	Vanda Pharmaceuticals	neurokinin 1 receptor (NK-1R) antagonist	Motion sickness	PO	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
PRO-145223	etrolizumab	Genentech	IgG1 monoclonal antibody	Ulcerative colitis	SC	InTrial	2021	Yes	No
DS-100	DS-100	Eton	undisclosed	Ophthalmological disease	SC	InTrial	2021	Yes	No
ublituximab (LFB-R603, TG20, TGTX-1101, TG-1101, Utuxin)	ublituximab	TG Therapeutics	CD-20 monoclonal antibody	Chronic lymphocytic leukemia/ multiple sclerosis	IV	InTrial	2021	Yes	Yes
S5G4T-1	benzoyl peroxide	Sol-Gel Technologies	benzoyl peroxide	Rosacea	TOP	InTrial	2021	No	No
Estelle	estetrol/ drospirenone	Mithra	estrogen receptor agonist	Pregnancy prevention	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
AKB-6548	vadadustat	Akebia Therapeutics	hypoxia-inducible factor-prolyl hydroxylase (HIF-PH) inhibitor	Anemia	PO	InTrial	Late 2021	Yes	No
Ultomiris SC	ravulizumab-cwvz	Alexion	C5 complement inhibitor	paroxysmal nocturnal hemoglobinuria; Hemolytic uremic syndrome	SC	InTrial	Late 2021	Yes	Yes
AMT-061	etranacogene dezaparvovec	uniQure	gene therapy	Hemophilia B	IV	InTrial	Late 2021	Yes	No
CaPre	omega-3 fatty acids	Acasti Pharma	fatty acids	Hypertriglyceridemia	PO	InTrial	Late 2021	No	No
PF-06482077	multivalent group B streptococcus vaccine	Pfizer	vaccine	Bacterial infection	IM	InTrial	Late 2021	Yes	No
GS-010	GS-010	GenSight Biologics	gene therapy	Optic neuropathy	Intraocular	InTrial	Late 2021	Yes	Yes
ATI-1501	metronidazole	Appili Therapeutics	nitroimidazole	Fungal infections, anaerobic bacterial infections	PO	InTrial	Late 2021	No	No
AMAG-423	digoxin immune fab (DIF)	AMAG/ Velo	digitalis-like factor antagonist	Preeclampsia	IV	InTrial	Late 2021	Yes	Yes
RGN-259 (GBT-201; RGN-352)	timbetasin	RegeneRx	actin regulating peptide	Dry eyes	OP	InTrial	Late 2021	No	Yes
COR-003	levoketoconazole	Strongbridge Biopharma	azole antifungal	Cushing's syndrome	PO	InTrial	Late 2021	No	Yes

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
CMX-001	brincidofovir	Chimerix	DNA-directed DNA polymerase inhibitor	Smallpox	PO	InTrial	Late 2021	No	Yes
PDS-1.0	ranibizumab	Roche/ Genentech	anti-VEGF antibody	Wet age-related macular degeneration	Intravitreal implant	InTrial	Late 2021	Yes	No
CAT-1004	edasalonexent	Catabasis	NF-kB inhibitor	Duchenne muscular dystrophy	PO	InTrial	Late 2021	Yes	Yes
ADV-7103	tripotassium citrate monohydrate/ potassium hydrogen carbonate	Advicenne	undisclosed	Distal rental tubular acidosis	PO	InTrial	Late 2021	Yes	No
ONS-5010	bevacizumab	Outlook Therapeutics	anti-VEGF antibody	wet age-related macular degeneration	Intravitreal	InTrial	Late 2021	Yes	No
glatiramer acetate depot	glatiramer acetate long-acting	Mylan/ Mapi Pharma	immunosuppressant	Multiple sclerosis	IM	InTrial	Late 2021	Yes	No
NNZ-2566	trofinetide	Neuren	insulin-like growth factor 1 (IGF-1) derivative	Rett syndrome	IV/PO	InTrial	Late 2021	Yes	Yes
ABL-001	asciminib	Novartis	allosteric Bcr-Abl inhibitor	Chronic myelogenous leukemia	PO	InTrial	Late 2021	Yes	Yes
SHP-625 (LUM-001)	maralixibat	Mirum Pharmaceuticals	apical sodium-dependent bile acid transporter (ABST) inhibitor	Alagille syndrome	PO	InTrial	Late 2021	Yes	Yes
REGN-1500	evinacumab	Regeneron	angiopoietin-like 3 (ANGPTL3) antagonist	Hyperlipidemia	IV/SC	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
OTL-101	ADA-transduced autologous stem cell therapy	Orchard Therapeutics	gene therapy	Adenosine deaminase-deficient severe combined immunodeficiency	INJ	InTrial	Late 2021	Yes	Yes
MT-7117	MT-7117	Mitsubishi Tanabe Pharma	Undisclosed	Erythropoietic protoporphyria	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
Opdivo	nivolumab	Bristol-Myers Squibb	anti-PD-1 antibody; T lymphocyte stimulator; protein kinase B (PKB/Akt) inhibitor	Hepatocellular carcinoma	In combination with Yervoy (ipilimumab) for the treatment of patients with advanced hepatocellular carcinoma (HCC) previously treated with sorafenib	IV	3/10/2020
Imfinzi	durvalumab	AstraZeneca/ Celgene/ Eli Lilly/ Juno Therapeutics	anti-PD-L1 antibody	Small cell lung cancer	Treatment of patients with previously untreated extensive-stage small cell lung cancer (SCLC)	IV	3/30/2020
Reblozyl	luspatercept-aamt	Celgene	modified type II activin receptor recombinant fusion protein	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 (RBC) transfusions	SC	4/4/2020
Otezla	apremilast	Celgene	phosphodieasterase 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
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

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Braftovi	encorafenib	Array Biopharma	kinase inhibitor	Colorectal cancer	In combination with Erbitux (cetuximab), for treatment of patients with BRAFV600E-mutant metastatic colorectal cancer (mCRC) following one or two lines of therapy	PO	4/30/2020
Farxiga	dapagliflozin	AstraZeneca	sodium glucose cotransporter-2 (SGLT-2) inhibitor	Heart failure	To reduce the risk of cardiovascular (CV) death or the worsening of heart failure in adults with heart failure with reduced ejection fraction (HFrEF) with and without type-2 diabetes	PO	5/1/2020
Ayvakit	avapritinib	Blueprint Medicines	selective KIT and PDGFRa inhibitor	Gastrointestinal stromal tumor (4th line)	Treatment of adults with fourth-line gastrointestinal stromal tumor (GIST)	PO	5/14/2020
Opdivo	"nivolumab	Bristol-Myers Squibb	anti-PD-1 antibody; T lymphocyte stimulator; protein kinase B (PKB/Akt) inhibitor	Non-small cell lung cancer	In combination with low-dose Yervoy for the treatment of first-line advanced non-small-cell lung cancer (NSCLC) in patients with no EGFR or ALK genomic tumor aberrations	IV	5/15/2020
Cyramza	ramucirumab	Eli Lilly	vascular endothelial growth factor 2 (VEGF-2) receptor antagonist	Non-small cell lung cancer	In combination with erlotinib, for first-line treatment of patients with metastatic non-small cell lung cancer whose tumors have epidermal growth factor receptor exon 19 deletions or exon 21 (L858R) substitution mutations	IV	5/15/2020
Rubraca	rucaparib	Clovis Oncology	poly-ADP-ribose polymerase-1/2 (PARP-1/PARP-2) inhibitor	Metastatic castration-resistant prostate cancer	Treatment of BRCA1/2-mutant recurrent, metastatic castrate-resistant prostate cancer	PO	5/15/2020

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 (IL-4/IL-13) inhibitor	Atopic dermatitis	Add-on maintenance treatment for children aged 6 to 11 years with moderate-to-severe atopic dermatitis whose disease is not adequately controlled with topical prescription therapies or when those therapies are not advisable	SC	5/26/2020
Recarbrio	imipenem/cilastatin/ relebactam	Merck	Carbapenem/ dehydropeptidase-1 inhibitor/ beta-lactamase inhibitor	Hospital-acquired pneumonia and ventilator-associated bacterial pneumonia	Empiric treatment of hospital-acquired pneumonia (HAP) and ventilator-associated bacterial pneumonia (VABP)	IV	6/4/2020
Orilissa	elagolix	AbbVie	gonadotropin-releasing hormone (GnRH) receptor antagonist	Uterine fibroids	Management of heavy menstrual bleeding (HMB) associated with uterine fibroids in women	PO	6/5/2020
Xolair	omalizumab	Novartis	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
Taltz	ixekizumab	Eli Lilly	IL-17 monoclonal antibody	Non-radiographic axial spondyloarthritis	Treatment of non-radiographic axial spondyloarthritis	SC	6/15/2020
Xpovio	selinexor	Karyopharm Therapeutics	selective inhibitor of nuclear export	Diffuse large B-cell lymphoma	Treatment for patients with relapsed or refractory diffuse large B-Cell lymphoma (DLBCL) after at least two prior multi-agent therapies and who are ineligible for stem cell transplantation, including CAR-T therapy	PO	6/23/2020

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	Cutaneous squamous cell carcinoma	Treatment of patients with recurrent and/or metastatic cutaneous squamous cell carcinoma (cSCC) that is not curable by surgery or radiation	IV	6/29/2020
Lynparza	olaparib	AstraZeneca	poly (ADP-ribose) polymerase (PARP) inhibitor	Ovarian cancer	In combination with bevacizumab, for maintenance treatment of women with advanced ovarian cancer whose disease showed a complete or partial response to first-line treatment with platinum-based chemotherapy and bevacizumab	PO	6/30/2020
Lynparza	olaparib	AstraZeneca/ Merck	poly (ADP-ribose) polymerase (PARP) inhibitor	Prostate cancer	Treatment of metastatic castration-resistant prostate cancer (mCRPC) and deleterious or suspected deleterious germline or somatic homologous recombination repair gene mutations, who have progressed following prior treatment with a new hormonal agent	PO	6/30/2020
Ryanodex	dantrolene sodium	Eagle Pharmaceuticals	ryanodine receptor inhibitor	Exertional heat stroke	Treatment of exertional heat stroke (EHS), in conjunction with external cooling methods	IV	7/9/2020
Brilinta	ticagrelor	AstraZeneca	Thienopyridine	Cardiovascular outcomes	Reduction in the incidence of cardiovascular death, myocardial infarction, or stroke in patients with type 2 diabetes mellitus	PO	7/15/2020
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

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Qutenza	capsaicin 8%	Averitas Pharma	transient receptor potential vanilloid 1 (TRPV-1) agonist	Diabetic peripheral neuropathy	Treatment of neuropathic pain associated with diabetic peripheral neuropathy	TOP	7/19/2020
Tecentriq	atezolizumab	Roche/ Genentech	PD-L1 monoclonal antibody	Hepatocellular carcinoma	In combination with Avastin (bevacizumab), for the treatment of people with unresectable hepatocellular carcinoma (HCC) who have not received prior systemic therapy	IV	7/27/2020
Trelegy Ellipta	fluticasone furoate/ umeclidinium/ vilanterol	GlaxoSmithKline	inhaled corticosteroid (ICS)/ long-acting muscarinic agent (LAMA)/ long-acting beta agonist (LABA)	Asthma	Treatment of asthma	INH	7/31/2020
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 inhibitor (NRTI)	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

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Imbruvica	ibrutinib	AbbVie	Kinase inhibitor	Chronic lymphocytic leukemia/small lymphocytic lymphoma	In combination with rituximab for the first-line treatment of younger patients (70 years old or younger) with chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL)	PO	9/8/2020
Crysvita	burosumab- twza	Ultragenyx/ Kyowa Kirin	fibroblast growth factor 23 antibody	Bone complications	Treatment of fibroblast growth factor 23 (FGF23)-related hypophosphatemia associated with phosphaturic mesenchymal tumors (tumor-induced osteomalacia) that cannot be curatively resected or localized	SC	10/18/2020
Linzess	linaclotide	Astellas/ AstraZeneca/ Allergan	guanylate cyclase C receptor agonist	Abdominal symptoms	Treatment of abdominal symptoms	PO	10/31/2020
Tecentriq	atezolizumab	Genentech	PD-L1 monoclonal antibody	Non-small cell lung cancer	First-line treatment of squamous and non-squamous non-small cell lung cancer (NSCLC)	IV	11/15/2020
Cosentyx	secukinumab	Novartis	IL-17 receptor antagonist	Axial spondyloarthritis	Treatment of non-radiographic axial spondyloarthritis	SC	11/29/2020
Epidiolex	cannabidiol	Greenwich Biosciences	cannabinoid	Tuberous sclerosis complex	Treatment of tuberous sclerosis complex (TSC)	PO	12/3/2020
Zejula	niraparib	GlaxoSmithKline	poly (ADP-ribose) polymerase (PARP) inhibitor	Ovarian cancer	First-line maintenance treatment of ovarian cancer	PO	12/15/2020

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

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