OPTUMRx®

RxOutlook®

2nd Quarter 2019

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

This edition focuses on ten near-term pipeline drugs that are expected to receive a Food and Drug Adminstration (FDA) approval decision by the end of the 3rd quarter of 2019. These drugs are notable because of their potential for clinical and/or economic impact. In many ways, they highlight an ongoing pipeline trend of focusing on Orphan Drug development and conditions caused by genetic mutations.

Eight of these drugs have FDA Orphan Drug Designation for the management of a rare disease, defined as having a prevalence of less than 200,000 cases in the United States. Four are for genetic disorders. The remaining 2 drugs (upadacitinib and oral semaglutide) are entering very crowded categories where patients, providers, and payers have multiple therapeutic classes of drugs from which to choose.

Key pipeline drugs with FDA approval decisions expected by the end of the 3rd quarter 2019

Drug Name	Manufacturer	Indication/Use	Expected FDA Decision Date
Tafamidis and tafamidis meglumine	Pfizer	Transthyretin amyloid cardiomyopathy*	5/6/2019 (Approved)
Inhaled mannitol	Pharmaxis	Cystic fibrosis*†	3Q 2019
Afamelanotide	Clinuvel	Erythropoietic protoporphyria*†	7/8/2019
Pitolisant	Harmony Biosciences	Narcolepsy*	7/2019 – 8/2019
Pexidartinib	Daiichi Sankyo	Tenosynovial giant cell tumor*	8/2/2019
Entrectinib	Genentech/Roche	NKTR+ solid tumors*† ROS1 Non-small cell lung cancer*†	8/16/2019
Golodirsen	Sarepta Therapeutics	Duchenne muscular dystrophy*†	8/19/2019
Polatuzumab vedotin	Genentech/Roche	Diffuse large B-cell lymphoma*	8/19/2019
Upadacitinib	AbbVie	Rheumatoid arthritis	8/20/2019
Oral semaglutide	Novo Nordisk	Type 2 diabetes mellitus	9/20/2019

* Orphan Drug Designation; † Genetic Disorder

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

Detailed insights on key drugs

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

Read more

Generic and biosimilar 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

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 pending new indications that may be approved in the upcoming year.

Read more

Past and future reviews

Please note that RxOutlook highlights select near-term approvals. Some drugs may not appear in this issue because they have been reviewed in previous editions of RxOutlook. Drugs of interest that are earlier in development or with expected approvals beyond 3rd quarter 2019 may appear in future reports; however, for those who need an initial look at the full pipeline, please refer to the Brand Pipeline Forecast Table found later in this report.

Drugs reviewed in detail in the 1Q:2019 report:

- Esketamine
- Metoclopramide nasal spray
- Selinexor
- Dolutegravir/lamivudine
- Risankizumab
- Onasemnogene abeparvovec
- Quizartinib
- NKTR-1818
- Celiprolol

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

Getting acquainted with pipeline forecast terms

Clinical trial phases

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

Pipeline acronyms

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

Detailed insights on key drugs

Tafamidis meglumine (Brand name: Vyndaqel), tafamidis (Brand name: Vyndamax)

Manufacturer: Pfizer
Regulatory designations: Orphan Drug, Fast Track, Breakthrough Therapy
FDA approval date: 5/6/2019

Therapeutic use

- Treatment of patients with ATTR-CM

Tafamidis was approved for the treatment of the cardiomyopathy of wild type or hereditary transthyretin-mediated amyloidosis (ATTR-CM) in adults to reduce cardiovascular mortality and cardiovascular-related hospitalization.

Transthyretin amyloidosis is a rare disease caused by destabilization of a transport protein transthyretin, which is composed of four identical subunits (a tetramer). Complications can occur when these unstable tetramers dissociate, resulting in misfolded proteins that aggregate into amyloid deposits that can accumulate in nerves, the heart, and gastrointestinal tract. When amyloid deposits accumulate in the heart it can lead to cardiomyopathy which causes symptoms of heart failure. The other serious manifestation of the disease is polyneuropathy.

Patients with ATTR-CM are also classified as having "wild-type" or hereditary disease. The hereditary form is caused by a genetic mutation whereas the "wild-type" form emerges from a normal transthyretin that for unknown reasons becomes unstable and misfolds. It is estimated that the prevalence of ATTR-CM is approximately 100,000 people in the U.S; however, only 1 to 2% of those patients are diagnosed today.

Clinical profile

- Transthyretin stabilizer
- Oral formulation
- Lowered all-cause mortality and rates of cardiovascular-related hospitalizations vs. placebo over a 30-month treatment period
- Dose: once daily

Vyndaqel and Vyndamax have the same active moiety (tafamidis); however, Vyndamax is the free acid formulation and was developed for patient convenience: it provides a full dose in a single capsule (vs. 4 capsules for Vyndaqel).

Tafamidis selectively binds at specific sites on the transthyretin tetramer to prevent destabilization of the transthyretin transport protein and formation of amyloid that causes ATTR-CM.

Pivotal trial data:
Tafamidis was evaluated in a 30-month, double-blind, placebo-controlled, randomized study in 441 patients with ATTR-CM. The primary analysis demonstrated a significant reduction ($p = 0.0006$) in all-cause mortality and frequency of cardiovascular-related hospitalizations in the pooled tafamidis groups vs. placebo. The percentage of patients alive at month 30 was 70.5% and 57.1% for the pooled tafamidis and placebo groups, respectively. The mean number of cardiovascular-related hospitalizations (per patient per year) among those alive at month 30 was 0.297 and 0.455 for tafamidis and placebo, respectively.

Safety:
The safety profile of tafamidis was similar to placebo in the pivotal trial.

Dosing:
The recommended dosage is either Vyndaqel 80 mg (four 20-mg tafamidis meglumine capsules) orally once daily or Vyndamax 61 mg (one 61-mg tafamidis capsule) orally once daily. Vyndamax and Vyndaqel are not substitutable on a per mg basis.

Tafamidis meglumine, tafamidis (continued...)

Competitive environment

Tafamidis is the first approved treatment for ATTR-CM and the indication includes both "wild type" and hereditary forms of the disease. In contrast, Onpattro® (patisiran) and Tegsedi® (inotersen), which were approved in 2018, are only indicated for hereditary ATTR polyneuropathy. Tafamidis is also dosed orally once daily and was well tolerated in the pivotal study. Onpattro and Tegsedi require intravenous (IV) infusion and subcutaneous (SC) administration, respectively.

However, historically ATTR-CM has been underdiagnosed; therefore utilization for tafamidis will likely be low initially and could increase over time if diagnosis rates improve. In addition, tafamidis was originally rejected by the FDA for an indication for treatment of ATTR polyneuropathy.

The WAC for tafamidis is $225,000 per year.

- Advantages: first approved therapy for ATTR-CM, broad indication ("wild type" and hereditary forms of the disease), well tolerated, oral, once daily dosing
- Disadvantages: low diagnosis rate for ATTR-CM, originally rejected by the FDA for a ATTR polyneuropathy indication
- WAC = $225,000

Inhaled mannitol (Brand name: Bronchitol)

Manufacturer: Pharmaxis
Regulatory designations: Orphan Drug and Fast Track
FDA Advisory Committee: 5/8/2019
Expected FDA decision: 3Q 2019

Therapeutic use

Inhaled mannitol is in development for the management of cystic fibrosis (CF) to improve pulmonary function in adult patients in conjunction with standard therapies.

CF is a rare genetic disease affecting about 30,000 people in the U.S. It is caused by mutations of the cystic fibrosis transmembrane conductance regulator (CFTR) gene, which encodes for the CFTR protein. This protein acts as an ion channel regulating electrolyte and fluid homeostasis in several organs including the lungs, and defects are associated with thickened secretions, decreased lung function, and potentially respiratory failure.

- Management of CF to improve pulmonary function in adult patients in conjunction with standard therapies

Inhaled mannitol (continued...)

Clinical profile

Mannitol has hyperosmotic properties and when delivered via inhalation is believed to work as an airway clearance agent in CF by rehydrating the airway and lung surface and promoting a productive cough.

Pivotal trial data:
An application for inhaled mannitol was originally submitted to the FDA in 2013; however, the product was not approved due to concerns about lack of required efficacy and a safety signal (e.g., bloody sputum), particularly in pediatric patients. As a result, Pharmaxis conducted an additional randomized study in 423 adult patients with CF.

In this pivotal study, inhaled mannitol demonstrated a statistically significant improvement vs. placebo in change in forced expiratory volume in the first second (FEV1) from baseline over a 26-week treatment period, with an effect of 54 mL ($p = 0.020$). This corresponded to a 2.2% relative change ($p = 0.025$) in FEV1. Inhaled mannitol did not demonstrate statistical superiority vs. placebo for key secondary endpoints (e.g., rate of pulmonary exacerbations, number of days on antibiotics due to pulmonary exacerbations).

Safety:
The most common adverse events (AEs) with inhaled mannitol use were cough, bronchospasm, chest discomfort, throat irritation, and bloody sputum.

Dosing:
In the pivotal trial, mannitol was administered as an oral inhaled powder twice daily.

- Hyperosmotic agent
- Oral inhalation powder
- Statistically significant improvement in FEV1 vs. placebo
- Common AEs: cough, bronchospasm, chest discomfort, throat irritation, and hemoptysis
- Dose: twice daily

Competitive environment

Inhaled mannitol would offer a novel mechanism of action (MOA) for the treatment of CF and potentially adds to the treatment armamentarium for airway clearance. In addition, inhaled mannitol appears well tolerated in adult patients with CF.

However, based on the data available, the clinical benefit with inhaled mannitol appears to be modest with a relatively small improvement in lung function. The proposed indication would also be limited to adult patients and the drug would likely be reserved as a backline agent for patients requiring additional airway clearance therapy. Other treatment options available for airway clearance include Pulmozyme® (dornase alfa) and hypertonic saline.

- Advantages: novel MOA for treatment of CF, potentially adds to the treatment armamentarium for airway clearance, appears well tolerated in adults
- Disadvantages: modest efficacy, proposed indication limited to adults, backline therapy for airway clearance in CF patients

Afamelanotide (Brand name: Scenesse)

Manufacturer: Clinuvel
Regulatory designations: Orphan Drug, Fast Track
Expected FDA decision: 7/8/2019

Therapeutic use

Afamelanotide is in development for the prevention of phototoxicity and anaphylactoid reactions in adult patients with erythropoietic protoporphyria (EPP).

EPP is a rare inherited metabolic disorder (estimated to occur in about 1 in 75,000 to 1 in 200,000) caused by a deficiency of the ferrochelatase (FECH) enzyme. Due to low levels of this enzyme, excessive amounts of protoporphyrin accumulate in the bone marrow, blood plasma, and red blood cells.

The major symptom of EPP is severe pain on exposure to sunlight and some types of artificial light. Some patients with EPP may also have complications related to liver and gallbladder function due to excessive protoporphyrin levels.

- Prevention of phototoxicity and anaphylactoid reactions in adult patients with EPP

Clinical profile

Afamelanotide is a potent analog of human α-melanocyte–stimulating hormone (α-MSH). It binds to the melanocortin 1 receptor in dermal cells and increases the production of eumelanin, which is photoprotective.

<u>Pivotal trial data:</u>
Afamelanotide was evaluated in two, randomized, double-blind, placebo-controlled pivotal studies. The primary efficacy endpoint was the number of hours of direct exposure to sunlight without pain between 10 a.m. and 3 p.m. (in the European Union study) or between 10 a.m. and 6 p.m. (in the U.S. study). In the U.S study, the total duration of pain-free time over 6 months was longer with afamelanotide vs. placebo (median 69.4 hours vs. 40.8 hours, respectively; p = 0.04). In the European Union study, the total duration of pain-free time over 9 months was also longer with afamelanotide vs. placebo (median 6.0 hours vs. 0.8 hours; p = 0.005). The total number of phototoxic reactions after 9 months was reduced among patients in the European Union study (77 vs. 146; p = 0.04), although no significant changes were seen after 6 months in the U.S. trial (46 and 43 reactions, respectively; p = 0.60).

<u>Safety:</u>
The most common AE with afamelanotide use was mild hyperpigmentation at the implant site.

<u>Dosing:</u>
In the pivotal trials, afamelanotide was administered as a SC implant every 2 months.

- α-MSH analog
- SC implant
- Statistically significant improvement in the duration of sun exposure without pain vs. placebo
- Common AE: hyperpigmentation at the implant site
- Dose: once every 2 months

Afamelanotide (continued...)

Competitive environment

If approved, afamelanotide would be the first approved therapy for EPP. It has a novel mechanism of action (MOA) and it was well tolerated in clinical trials. In addition, the dosing is relatively infrequent with administration every 2 months.

However, afamelanotide demonstrated modest efficacy in the pivotal trials and it does not treat the underlying condition. Patients treated with afamelanotide would still be at risk for complications related to liver and gall bladder function. Non-pharmacological management would also still remain the cornerstone of therapy (e.g., avoiding sunlight exposure, protective clothing). In addition, afamelanotide requires administration via SC implant.

- Advantages: potentially first approved therapy for EPP, novel MOA, well tolerated, administration every 2 months
- Disadvantages: modest efficacy, does not treat the underlying condition, non-pharmacological management is cornerstone of treatment, requires administration via SC implant

Pitolisant (Brand name: To be determined)

Manufacturer: Harmony Biosciences
Regulatory designations: Orphan, Fast Track, Breakthrough Therapy
Expected FDA decision: 7/2019 – 8/2019

Therapeutic use

Pitolisant is in development for the treatment of excessive daytime sleepiness (EDS) and/or cataplexy in patients with narcolepsy.

Narcolepsy is a chronic neurologic disorder of sleep-wake state instability that impacts up to 200,000 people in the U.S. Symptoms of narcolepsy include EDS, cataplexy, and other manifestations of REM sleep dysregulation.

Cataplexy is characterized by sudden temporary loss of muscle tone. Up to two-thirds of all patients with narcolepsy have cataplexy.

- Treatment of EDS and/or cataplexy in patients with narcolepsy

Pitolisant (continued...)

Clinical profile

Pitolisant is a novel selective histamine 3 (H_3) receptor inverse agonist. It is believed to work by enhancing the activity of histaminergic neurons in the brain that function to improve a patient's wakefulness and inhibit attacks of cataplexy.

Pivotal trial data:
The efficacy of pitolisant was evaluated in two randomized, double-blind pivotal studies. In study 1, 95 patients received pitolisant, placebo, or an active control (Provigil® [modafinil]). The primary endpoint was the difference in change in Epworth sleepiness scale (ESS) scores. ESS is a self-administered questionnaire assessing chances of falling asleep in 8 life situations. Differences in mean ESS scores (adjusted for baseline) showed pitolisant to be superior to placebo (difference -3.0, 95% CI: -5.6, -0.4; p = 0.024), but not noninferior to Provigil (difference 0.12; 95% CI: -2.5, 2.7; p = 0.250).

In study 2, 106 patients received either pitolisant or placebo. The primary endpoint was the change in the average number of cataplexy attacks per week between the 2 weeks of baseline and the 4 weeks of stable dosing. The weekly cataplexy rate was decreased by 75% in patients who received pitolisant vs. 38% with placebo (rate ratio 0.512; 95% CI: 0.43, 0.60; p < 0.0001).

Safety:
The most common AEs with pitolisant use were headache, insomnia, weight increase, anxiety, depression, and nausea/vomiting.

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

- Selective H_3 receptor inverse agonist
- Oral formulation
- Statistically significant improvement in ESS vs. placebo; noninferiority not demonstrated vs. Provigil
- Statistically significant reduction in weekly cataplexy attacks vs. placebo
- Common AEs: headache, insomnia, weight increase, anxiety, depression, nausea/vomiting
- Dose: once daily

Competitive environment

Pitolisant offers a novel MOA for the treatment of narcolepsy and it demonstrated efficacy in patients with or without cataplexy. Due to its MOA, pitolisant is also unlikely to require DEA scheduling and is expected to have a lower risk of abuse vs. CNS stimulants.

However, there are alternative treatments options available for narcolepsy. Drugs such as Provigil, Nuvigil® (armodafinil), traditional CNS stimulants (e.g., amphetamine, methylphenidate), and the recently approved Sunosi™ (solriamfetol) can be used for EDS and drugs such as Xyrem® (sodium oxybate) and traditional antidepressants (e.g., venlafaxine, fluoxetine) can be used for cataplexy.

In addition, while pitolisant may confer safety benefits vs. current standards of care, pitolisant failed to meet pre-specified criteria for noninferiority for improvement in daytime sleepiness vs. an active control (Provigil).

For reference, the WAC price for brand Nuvigil and Provigil are $750 and $2,385 per month.

- Advantages: novel MOA, efficacy demonstrated in patients with or without cataplexy, potentially lower risk of abuse vs. CNS stimulants
- Disadvantages: alternatives available, did not meet noninferiority criteria vs. Provigil
- Reference WAC (Nuvigil and Provigil) = $750 and $2,385 per month.

Pexidartinib (Brand name: To be determined)

Manufacturer: Daiichi Sankyo
Regulatory designations: Orphan Drug, Breakthrough Therapy
FDA Advisory Committee: 5/14/2019
Expected FDA decision: 8/2/2019

Therapeutic use

- Treatment of adult patients with TGCT

Pexidartinib is in development for the treatment of adult patients with symptomatic tenosynovial giant cell tumor (TGCT), which is associated with severe morbidity or functional limitations, and which is not amenable to improvement with surgery.

TGCT is a rare, usually non-cancerous tumor that affects the synovium-lined joints, bursae, and tendon sheaths, resulting in swelling, pain, stiffness and reduced mobility in the affected joint or limb. The estimated incidence is about 11 to 50 cases per million patients.

Treatment of TGCT includes surgery to remove the tumor. However, patients with a diffused form of the tumor (e.g., wraps around bone, tendons, ligaments and other parts of the joint) may require multiple surgeries or joint replacement. Eventually, many patients will advance to the point where surgical resection is no longer an option.

Clinical profile

- CSF-1 receptor inhibitor
- Oral formulation
- Statistically significant improvement in tumor response vs. placebo
- Statistically superior improvement in secondary endpoints (range of motion, physical function, worst stiffness) vs. placebo
- Common AEs: serious liver toxicity, pruritus, gastrointestinal side effects, edema, hypertension
- Dose: twice a day

Pexidartinib inhibits colony stimulating factor-1 (CSF-1) receptors, which are a primary growth driver of abnormal cells in the synovium that cause TGCT.

Pivotal trial data:
Pexidartinib was evaluated in a double-blind, placebo-controlled, randomized study (ENLIVEN) in 120 patients with symptomatic advanced TGCT for whom surgical removal of the tumor would be associated with potentially worsening functional limitation or severe morbidity. The trial showed a statistically significant 39% overall response rate (ORR) at week 25 for patients treated with pexidartinib vs. no tumor response among patients who received placebo ($p < 0.0001$).

In addition, pexidartinib was associated with statistically significant improvements vs. placebo for other secondary efficacy endpoints, including range of motion, physical function, and worst stiffness.

Safety:
The most common AEs with pexidartinib use were serious liver toxicity, pruritus, gastrointestinal side effects, edema, and hypertension. Of particular concern, 8 patients treated with pexidartinib discontinued treatment due to hepatic AEs. In addition, in non-TGCT development studies using pexidartinib, 2 severe liver toxicity cases were observed (one required liver transplant and one was associated with death).

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

Pexidartinib (continued...)

Competitive environment

If approved, pexidartinib would be the first approved therapy for TGCT. There is a high unmet need for treatment of TGCT, particularly for patients with diffused forms of the disease. In addition, pexidartinib can be administered orally.

While there is a lack of treatment options for TGCT, the proposed indication for pexidartinib is narrow (ie, patients who are not amenable to improvement with surgery). TGCT is generally nonlethal and therefore the FDA could question if the benefits outweigh the serious liver toxicity issues.

- Advantages: potentially first approved therapy for TGCT, high unmet need for diffused forms of the disease, oral
- Disadvantages: narrow indication, questionable benefit vs. harm due to safety concerns (e.g., severe liver toxicity)

Entrectinib (Brand name: To be determined)

Manufacturer: Genentech/Roche
Regulatory designations: Orphan Drug (solid tumors, non-small cell lung cancer [NSCLC]), Breakthrough Therapy (solid tumors)
Expected FDA decision: 8/16/2019

Therapeutic use

Entrectinib is in development for the treatment of patients with neurotrophic receptor tyrosine kinase (NTRK) fusion-positive, locally advanced or metastatic solid tumors who have either progressed following prior therapies or as initial therapy when there are no acceptable standard therapies. Additionally, entrectinib is also seeking a second indication for the treatment of metastatic, ROS1-positive NSCLC.

NTRK genes encode for tropomyosin receptor kinase (TRK) proteins, and these genes can become fused to other genes abnormally, and this can activate signaling pathways involved in proliferation of certain types of cancer. NTRK gene fusions are tumor-agnostic, meaning they are present in tumors irrespective of site of origin. This is important because it leads to a different approach to treatment than typical cancer care where diagnosis and treatment are generally dictated by the organ where the tumor originates. ROS1 gene fusions are similar to NTRK gene fusions except they are more specific to NSCLC. ROS1 gene fusions have been identified in 1 to 2% of patients with NSCLC.

- Treatment of patients with NTRK fusion-positive, locally advanced or metastatic solid tumors
- Treatment of metastatic, ROS1-positive NSCLC

Entrectinib (continued...)

Clinical profile

Entrectinib is a selective tyrosine kinase inhibitor designed to inhibit the activity of the TRK A/B/C and ROS1 proteins. By blocking NTRK and ROS1 kinase activity, entrectinib may result in the death of cancer cells with NTRK or ROS1 gene fusions.

Pivotal trial data:
Entrectinib was evaluated using an integrated analysis of data from 53 people with ROS1-activating gene fusions and 54 people with locally advanced or metastatic NTRK fusion-positive solid tumors.

In patients with NTRK fusion-positive solid tumors, the ORR was 57.4%, progression-free survival (PFS) was 11.2 months, and median overall survival (OS) was 20.9 months. In patients with ROS1 NSCLC, the ORR was 77.4% and median PFS was 13.6 and 26.3 months in patients with and without CNS metastases, respectively.

Safety:
The most common AEs with entrectinib use were fatigue, dizziness, constipation, nausea, diarrhea, and dysgeusia.

Dosing:
In the pivotal trials, entrectinib was dosed orally once daily.

- Selective tyrosine kinase inhibitor
- Oral formulation
- NTRK fusion-positive solid tumors: 57.4% ORR, 11.2 months median PFS, 20.9 months median OS
- ROS1 positive NSCLC: 77.4% ORR, 13.6 months and 26.3 months median PFS (with or without CNS metastases, respectively)
- Common AEs: fatigue, dizziness, constipation, nausea, diarrhea, dysgeusia
- Dose: once daily

Competitive environment

If approved, entrectinib would provide an additional "tissue agonistic" therapy for solid tumors and it did demonstrate efficacy in ROS1-positive NSCLC patients with or without CNS metastases. Entrectinib is also dosed orally once a day.

Vitrakvi® (larotrectinib), another oral kinase inhibitor, was also recently approved for the treatment of NTRK fusion-positive solid tumors. Compared indirectly, Vitrakvi appears to have a better tolerability profile than entrectinib. Dose reductions due to AEs occurred in 9% of patients treated with Vitrakvi vs. 27% with entrectinib in their clinical trials.

For reference, the WAC price for Vitrakvi is $32,800 per 30 days.

- Advantages: "tissue agnostic" therapy for solid tumors, efficacy in ROS1-positive NSCLC patients with or without CNS metastases, oral, once a day dosing
- Disadvantages: second therapy for NTRK fusion-positive solid tumors, tolerability appears to be worse with entrectinib vs. Vitrakvi
- Reference WAC (Vitrakvi) = $32,800 per 30 days

Golodirsen (Brand name: To be determined)

Manufacturer: Sarepta Therapeutics
Regulatory designations: Orphan Drug
Expected FDA decision: 8/19/2019

Therapeutic use

Golodirsen is in development for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

DMD is a rare neuromuscular disorder affecting approximately 1 in every 3,500 to 5,000 male births worldwide. DMD is associated with specific errors in the gene that codes for dystrophin, a protein that plays a key structural role in muscle fiber function.

Patients have progressive muscle weakness in the lower limbs which spreads to the arms, neck and other areas of the body. The clinical onset of weakness is typically within the first few years of life and patients are usually wheelchair bound by the age of 12. The condition is universally fatal, and death usually occurs before the age of 30 generally due to respiratory or cardiac failure.

- Treatment of DMD in patients who have a confirmed mutation that is amenable to exon 51 skipping

Clinical profile

Golodirsen is designed to bind to exon 53 of dystrophin pre-mRNA, resulting in exclusion, or "skipping," of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon skipping is intended to allow for production of a truncated but functional dystrophin protein.

Pivotal trial data:
The efficacy of golodirsen was evaluated in one early stage trial in 25 patients. The mean dystrophin protein increased to 1.019% of normal compared to a mean baseline of 0.095% of normal.

Safety:
To date, safety data has not been published or announced by Sarepta Therapeutics.

Dosing:
In the pivotal trial, golodirsen was administered IV once weekly.

- Phosphorodiamidate morpholino oligomer
- IV formulation
- Improved dystrophin protein levels from 0.095% to 1.019% of normal
- Safety: unknown
- Dose: weekly

Golodirsen (continued...)

Competitive environment

If approved, golodirsen would be the first approved drug for DMD patients with mutations amenable to exon 53 skipping and there is a high unmet need given the severity of the condition.

However, data for golodirsen is only available from an early stage trial and results from an ongoing late stage trial are not expected until after the initial FDA decision date. The FDA submission for golodirsen is based on data demonstrating a very modest improvement in a surrogate endpoint (dystrophin levels). The data for golodirsen are similar to Exondys 51® (eteplirsen), which was approved in DMD patients amenable to exon 51 skipping. The approval of Exondys 51 was highly controversial with the FDA ultimately approving the drug despite a negative review from an Advisory Committee. The labeling for Exondys 51 states that clinical benefit has not been established and golodirsen may receive a similar disclaimer if approved.

In addition, only about 8% of patients with DMD have a mutation amendable to exon 53 and golodirsen requires weekly IV infusion.

For reference, the WAC price for Exondys 51 is ~$300,000 per year.

- Advantages: potentially first approved drug for exon 53 skipping, high unmet need
- Disadvantages: lack of late stage data, efficacy appears to be very modest (similar to Exondys 51), low eligible patient population
- Reference WAC (Exondys 51) = ~$300,000 per year

Polatuzumab vedotin (Brand name: To be determined)

Manufacturer: Genentech/Roche
Regulatory designations: Orphan Drug, Breakthrough Therapy
Expected FDA decision: 8/19/2019

Therapeutic use

Polatuzumab vedotin is being developed in combination with bendamustine plus Rituxan® (rituximab), for the treatment of people with relapsed or refractory (R/R) diffuse large B-cell lymphoma (DLBCL).

DLBCL is an aggressive and common form of non-Hodgkin's lymphoma. An estimated 22,000 new cases of DLBCL are expected in the U.S. in 2019. As many as 40% of patients will relapse after initial treatment, at which point their prognosis is poor.

- In combination with bendamustine and Rituxan, for treatment of people with R/R DLBCL

Polatuzumab vedotin (continued...)

Clinical profile

Polatuzumab vedotin is an anti-CD79b antibody drug conjugate. The CD79b protein is highly specific and expressed in the majority of B-cell non-Hodgkin's lymphomas. Polatuzumab vedotin is thought to bind to CD79b, triggering internalization of the chemotherapy drug conjugate (monomethyl auristatin E) into B-cells and causing cell death. This mechanism is thought to minimize the toxic effects on normal cells while maximizing tumor cell death.

Pivotal trial data:
The efficacy of polatuzumab vedotin was evaluated in an early stage trial of 80 patients with heavily pre-treated R/R DLBCL. Patients were randomized to receive polatuzumab vedotin or placebo and all patients received background therapy with bendamustine plus Rituxan.

The complete response rate with polatuzumab vedotin was 40% vs. 18% with placebo; median OS was 12.4 months vs. 4.7 months (HR 0.42; 95% CI: 0.24, 0.75); and median PFS was 7.6 months vs. 2.0 months (HR 0.34; 95% CI: 0.20, 0.57). Patients treated with polatuzumab vedotin also showed a longer time between first response to treatment and disease worsening (median duration of response: 10.3 months vs. 4.1 months; HR 0.44).

Safety:
The most common AEs with polatuzumab vedotin use in combination with bendamustine/ Rituxan were cytopenias, febrile neutropenia, and infections.

Dosing:
In the pivotal trial, polatuzumab vedotin was administered IV once per 21-day cycle, for up to 6 cycles.

- Anti-CD79b antibody drug conjugate
- IV formulation
- Complete response: 40% vs. 18% with placebo; median OS: 12.4 months vs. 4.7 months with placebo; median PFS: 7.6 months vs. 2.0 months
- Common AEs: cytopenias, febrile neutropenia, infections
- Dose: once per 21-day cycle (up to 6 cycles)

Competitive environment

Polatuzumab vedotin would offer a novel MOA for the treatment of DLBCL and the results of the early stage trial were promising. If approved, polatuzumab vedotin would represent an alternative treatment option to CAR-T cell therapies (e.g., Kymriah [tisagenlecleucel], Yescarta [axicabtagene ciloleucel]. While effective for the treatment of DLBCL, CAR-T therapies are costly, complicated to produce, and are associated with delays in therapy due to required processing.

The initial proposed indication for polatuzumab vedotin is narrow (ie, R/R DLBCL), however, Genentech/Roche have an ongoing study of the drug in the first-line setting. In addition, polatuzumab vedotin does require IV administration.

- Advantages: novel MOA, promising early stage data, alternative to CAR-T therapies for R/R DLBCL
- Disadvantages: narrow indication (ongoing study in the first-line setting), IV administration

Upadacitinib (Brand name: To be determined)

Manufacturer: AbbVie
Expected FDA decision: 8/20/2019

Therapeutic use

Upadacitinib is in development for the treatment of adult patients with moderate to severe rheumatoid arthritis (RA).

- Treatment of adult patients with moderate to severe RA

Clinical profile

Upadacitinib is a Janus kinase (JAK)1-selective inhibitor. JAKs are enzymes which transmit signals arising from cytokine or growth factor-receptor interactions on the cellular membrane to influence cellular processes of immune cell function.

- JAK1-selective inhibitor
- Oral formulation
- Statistically significant improvements in ACR20 and DAS28-CRP, including vs. Humira
- Common AEs: nausea, nasopharyngitis, upper respiratory tract infection
- Dose: once daily

Pivotal trial data:
The efficacy of upadacitinib was evaluated in 5 pivotal trials. Upadacitinib met all primary and key secondary endpoints, including the proportion of patients who achieved at least a 20% improvement in RA signs and symptoms (ACR20) and clinical remission, as measured using the DAS 28-CRP (Disease Activity Score with 28 joint counts [C-reactive protein]).

Of note, upadacitinib demonstrated favorable results vs. the tumor necrosis factor (TNF) blocker, Humira® (adalimumab). At week 12 in the SELECT-COMPARE trial, 71% of patients receiving upadacitinib achieved an ACR20 response vs. 63% with Humira. In addition, a higher proportion of patients receiving upadacitinib achieved clinical remission (DAS28-CRP) at week 12 (29% vs. 6%).

Safety:
The most common AEs with upadacitinib use were nausea, nasopharyngitis, and upper respiratory tract infection.

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

Competitive environment

If approved, upadacitinib would be the third oral JAK inhibitor on the market. To date, it is the only product in the class to demonstrate superiority vs. Humira at its expected approved dose. The selective JAK1 inhibition may also confer safety advantages over Xeljanz®/Xeljanz XR (tofacitinib) and Olumiant® (baricitinib), which are JAK1/2/3 and JAK1/2 inhibitors, respectively. Similar to other JAK inhibitors, upadacitinib is also dosed orally once daily.

However, upadacitinib would be a late market entry in the class and there are also alternative treatment options for RA outside of the JAK inhibitor class. Gilead also has a selective JAK1 inhibitor (filgotinib) the late stage development. Finally, upadacitinib appears to have a similar safety profile to other JAK inhibitors and will likely have a boxed warning for serious infections.

For reference, Olumiant and Xeljanz have a WAC price of $26,000 and $54,500 per year, respectively.

- Advantages: superiority data vs. Humira, JAK1-selectivity, oral, once daily dosing
- Disadvantages: alternatives available, late market entry, potential future competition with filgotinib, class-associated serious AEs (e.g., serious infections)
- Reference WAC (Olumiant and Xeljanz) = $26,000 and $54,500

Oral semaglutide (Brand name: To be determined)

Manufacturer: Novo Nordisk
Expected FDA decision: 9/20/2019

Therapeutic use

Oral semaglutide is in development for the treatment of adult patients with type 2 diabetes mellitus (T2DM).

Semaglutide is also available in a SC injection formulation (Ozempic®)

- Treatment of adult patients with T2DM.

Clinical profile

Semaglutide is a glucagon-like peptide-1 (GLP-1) receptor agonist. GLP-1 receptor agonists reduce blood glucose by stimulating insulin secretion and lowering glucagon secretion, both in a glucose-dependent manner. The mechanism of blood glucose lowering also involves a minor delay in gastric emptying.

Peptide-based drugs such as GLP-1 agonists undergo significant proteolytic degradation in the gastrointestinal tract and have poor bioavailability which makes the development of an oral formulation challenging. Novo Nordisk's oral semaglutide is co-formulated with an absorption enhancer, which causes a localized increase in pH in the stomach, leading to higher solubility and protection against proteolytic degradation.

- GLP-1 receptor agonist
- Oral formulation
- Statistically significant reductions in HbA1c vs. placebo and active controls (e.g., Jardiance, Januvia)
- Non-inferiority for a MACE outcome vs. placebo; superiority not met
- Common AE: nausea
- Dose: once daily

<u>Pivotal trial data:</u>
Oral semaglutide was evaluated in several double-blind, randomized clinical trials, with head-to-head comparisons with other antidiabetic agents. The primary endpoint in these trials was hemoglobin A1c (HbA1c) reductions after 26 weeks.

In the PIONEER-2 trial, oral semaglutide provided a statistically significant reduction in HbA1c vs. the sodium-glucose co-transporter-2 (SGLT2) inhibitor, Jardiance® (empagliflozin) (1.4% vs. 0.9%, $p < 0.05$). In PIONEER-3, HbA1c reduction was 1.3% with oral semaglutide vs. 0.8% with the dipeptidyl peptidase 4 (DPP-4) inhibitor, Januvia® (sitagliptin) ($p < 0.001$). In PIONEER-4, HbA1c reduction was 1.2% with oral semaglutide vs. 1.1% with an injectable GLP-1 receptor agonist (Victoza [liraglutide]).

In addition, oral semaglutide was evaluated in a cardiovascular safety trial in 3,183 patients. Oral semaglutide demonstrated non-inferiority vs. placebo for a composite major adverse cardiovascular events major (MACE) outcome but failed to achieve statistical significance for superiority. There was a statistically significant improvement in cardiovascular death and all-cause mortality.

<u>Safety:</u>
The most common AE with oral semaglutide use was nausea.

<u>Dosing:</u>
In the pivotal trials, oral semaglutide was dosed orally once daily.

Oral semaglutide (continued...)

Competitive environment

If approved, semaglutide would be the first orally administered GLP-1 receptor agonist. Oral semaglutide demonstrated significant reductions in HbA1c vs. other commonly used oral antidiabetic drugs and demonstrated similar efficacy vs. an injectable GLP-1 receptor agonist (Victoza).

However, oral semaglutide is a late market entry and there are many alternatives available for the treatment of T2DM. Injectable GLP-1 receptor agonists have been available since 2005 and some formulations can be dosed once weekly. Many of the oral alternative antidiabetic drugs are available generically. In the clinical trials, oral semaglutide was also generally associated with higher rates of discontinuation due to AEs (e.g., 11% vs. 4% with Jardiance in PIONEER-2).

In addition, while Novo Nordisk is pursuing a cardiovascular outcomes indication, it is unknown whether the body of evidence for semaglutide will meet the FDA's threshold for labeling inclusion. Novo Nordisk is planning on leveraging the cardiovascular safety trial they conducted with oral semaglutide as well as a study with the injectable formulation, Ozempic.

For reference, the WAC price of Ozempic and Victoza is about $9,000 per year.

- Advantages: potentially first oral GLP-1 receptor agonist, positive efficacy results across several head-to-head clinical trials, once daily dosing
- Disadvantages: alternatives available (injectable GLP-1 agonists and other oral medications with different MOAs), high rates of discontinuations due to AEs, cardiovascular outcomes indication unknown
- Reference WAC (Ozempic and Victoza) = ~$9,000 per year

2nd Quarter 2019

Generic and biosimilar pipeline forecast

OptumRx generic and biosimilar pipeline forecast

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
2019 Possible launch date					
CUVPOSA	glycopyrrolate	Merz	Oral solution	All	2019
ROZEREM	ramelteon	Takeda	Tablet	All	2019
PREPOPIK	citric acid/magnesium oxid/sodium picosulfate	Ferring Pharmaceuticals	Oral packet	All	2019
TRAVATAN Z	travoprost	Alcon	Ophthalmic	All	2019
BYETTA	exenatide	AstraZeneca	Subcutaneous	All	2019
DESONATE	desonide	LEO Pharma	Gel	All	2019
SUPRENZA	phentermine	Citius/Akrimax	Tablet, orally disintegrating	All	2019
FENTORA	fentanyl	Teva	Tablet, buccal	All	2019
APTENSIO XR	methylphenidate	Rhodes	Capsule, extended-release	All	1H-2019
VIVLODEX	meloxicam	Iroko/iCeutica	Capsule	All	1H-2019
NUVARING	etonogestrel/ethinyl estradiol	Merck	Vaginal ring	All	1H-2019
RITUXAN	rituxumab	Genentech/Roche/Biogen Idec	Intravenous	All	1H-2019
SAMSCA	tolvaptan	Otsuka	Tablet	All	1H-2019
PYLERA	bismuth subcitrate potassiu/ metronidazole/ tetracycline	Allergan/Aptalis	Capsule	All	1H-2019
FASLODEX	fulvestrant	AstraZeneca	Intramuscular	All	1H-2019
BOTOX COSMETIC	onabotulinumtoxinA	Allergan	Injection	All	Spring-2019
EVZIO	naloxone	Kaléo Pharma	Injection	All	Mid-2019
PRESTALIA	perindopril/amlodipine	Symplmed	Tablet	All	06-2019
LYRICA	pregabalin	Pfizer	Capsule; oral solution	All	06-2019
HERCEPTIN	trastuzumab	Genentech/Roche	Intravenous	All	2H-2019+
ENBREL	etanercept	Amgen	Subcutaneous	All	2H-2019+
RESTASIS	cyclosporine	Allergan	Ophthalmic	All	2H-2019

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
FIRAZYR	icatibant	Takeda	Subcutaneous	All	07-2019
AVASTIN	bevacizumab	Genentech/Roche	Intravenous	All	07-2019+
FORTEO	teriparatide	Eli Lilly	Injection	All	08-2019
EDLUAR	zolpidem	Meda/Orexo	Sublingual	All	09-2019
MYOBLOC	botulinum toxin type B	US WorldMeds	Intramuscular	All	09-2019
EMEND	fosaprepitant dimeglumine	Merck	Intravenous	150 mg	09-2019
FERRIPROX	deferiprone	ApoPharma/Apotex	Tablet	All	4Q-2019
ZOHYDRO ER	hydrocodone	Pernix	Capsule, extended-release	All	4Q-2019
JADENU	deferasirox	Novartis	Tablet; oral granules	All	10-2019
VERMOX	mebendazole	Janssen	Tablet, chewable	All	10-2019
OSMOPREP	sodium biphosphate/sodium phosphate	Bausch Health	Tablet	All	11-2019
AMELUZ	aminolevulinic acid	Biofrontera	Gel	All	11-2019
DUREZOL	difluprednate	Alcon	Ophthalmic	All	11-2019
OMNARIS	ciclesonide	Covis	Intranasal	All	12-2019
THALOMID	thalidomide	Celgene	Capsule	All	12-2019
2020 Possible launch date					
MYCAMINE	micafungin	Astellas	Intravenous	All	2020
CIPRODEX	ciprofloxacin /dexamethasone	Alcon	Otic	All	2020
SYNDROS	dronabinol	Insys Therapeutics	Oral solution	All	2020
DORYX MPC	doxycycline hyclate	Mayne	Tablet, delayed-release	All	2020
SAPHRIS	asenapine	Allergan	Tablet, sublingual	All	1H-2020
NOXAFIL	posaconazole	Merck	Tablet, delayed-release; oral suspension	All	01-2020
DALIRESP	roflumilast	AstraZeneca	Tablet	All	01-2020
SILENOR	doxepin	Pernix	Tablet	All	01-2020
ELIGARD	leuprolide	QLT/Tolmar	Subcutaneous	All	03-2020
SOMATULINE DEPOT	lanreotide	Ipsen	Subcutaneous	All	03-2020

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
TAYTULLA	ethinyl estradiol/ norethindrone/ferrous fumarate	Allergan	Tablet	All	03-2020
VIIBRYD	vilazodone	Forest/Allergan	Tablet	All	03-2020
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
DULERA	formoterol fumarate/ mometasone furoate	Merck	Inhalation	All	05-2020
MYDAYIS	amphetamine/ dextroamphetamine mixture	Shire	Capsule, extended-release	All	06-2020
DEXILANT	dexlansoprazole	Takeda	Capsule, extended-release	All	06-2020
DENAVIR	penciclovir	Mylan	Cream	All	06-2020
LUCENTIS	ranibizumab	Roche	Intravitreal	All	06-2020
VELPHORO	sucroferric oxyhydroxide	Fresenius	Tablet, chewable	All	3Q-2020
KINERET	anakinra	Swedish Orphan Biovitrum/ Savient/ Amgen	Subcutaneous	All	07-2020
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
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

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
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
ENTEREG	alvimopan	Merck	Capsule	All	11-2020
EPIDUO FORTE	adapalene/benzoyl peroxide	Galderma	Gel	All	12-2020
OFIRMEV	acetaminophen	Mallinckrodt	Intravenous	All	12-2020
ABSORICA	isotretinoin	Sun	Capsule	All	12-2020
TOVIAZ	fesoterodine	Pfizer	Tablet, extended-release	All	12-2020
H1 - 2021					
BEPREVE	bepotastine	Bausch Health	Ophthalmic	All	2021
ACTEMRA	tocilizumab	Roche/Chugai	Intravenous; subcutaneous	All	2021
EMTRIVA	emtricitabine	Gilead	Capsule	All	1H-2021
AMITIZA	lubiprostone	Sucampo/Takeda	Capsule	All	01-2021
VELCADE	bortezomib	Takeda	Intravenous	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	All	03-2021
OVIDREL	choriogonadotropin	EMD Serono/Merck	Intramuscular; subcutaneous	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

Brand name	Generic name	Brand manufacturer	Dosage form	Strengths available as generic	Possible launch date
ZOMIG	zolmitriptan	Impax/Grunenthal	Intranasal	All	05-2021
QUTENZA	capsaicin	Grunenthal	Transdermal patch	All	06-2021
PERFOROMIST	formoterol fumarate	Mylan	Inhalation	All	06-2021
APTIOM	eslicarbazepine	Sunovion/Bial	Tablet	All	06-2021
SEEBRI NEOHALER	glycopyrrolate	Novartis	Inhalation	All	06-2021
INTELENCE	etravirine	Janssen	Tablet	All	06-2021
DICLEGIS	doxylamine succinate/pyridoxine	Duchesnay	Tablet, delayed-release	All	06-2021

+ = may launch during the stated date or later

Brand pipeline forecast

OptumRx brand pipeline forecast

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
2019 Possible launch date									
AMG-5041-1	glucagon	Eli Lilly	glucagon analog	Diabetes mellitus	Intranasal	Filed NDA	5/2019	N	N
Zolgensma	onasemnogene abeparvovec	Novartis/ AveXis	gene therapy	Spinal muscular atrophy	IV	Filed BLA	5/2019	Y	Y
NKTR-181	NKTR-181	Nektar	opioid agonist	Pain	PO	Filed NDA	5/28/2019	N	N
BYL-719	alpelisib	Novartis	phosphoinositide 3-kinase CA (PIK3CA) inhibitor	Breast cancer	PO	Filed NDA	5/31/2019	Y	N
XeriSol Glucagon	glucagon	Xeris	glucagon analog	Diabetes mellitus	SC	Filed NDA	6/10/2019	N	N
GSP-301	mometasone furoate/ olopatadine HCl	Glenmark	corticosteroid/ antihistamine	Allergic rhinitis	Intranasal	Filed NDA	6/21/2019	N	N
Edsivo	celiprolol HCl	Acer Therapeutics	alpha-2/beta-1 adrenergic agent	vascular Ehlers-Danlos Syndrome	PO	Filed NDA	6/25/2019	Y	Y
Thiola	tiopronin	Retrophin	reducing agent	Cystinuria	PO	Filed NDA	6/30/2019	N	N
Ryplazim	human plasminogen	ProMetic/ Hematech	plasminogen	Plasminogen deficiency	IV	Filed BLA	Mid-2019	Y	Y
tadalafil VersaFilm	tadalafil	IntelGenx	phosphodiesterase-5 (PDE-5) inhibitor	Erectile dysfunction	PO	Filed NDA	Mid-2019	Y	N

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
NRL-1	diazepam	Neurelis	benzodiazepine	Seizures	Intranasal	Filed NDA	2H2019	N	Y
Fasenra (self-administered)	benralizumab	AstraZeneca	interleukin-5 (IL-5) inhibitor	Asthma	SC	Filed sNDA	2H2019	Y	N
Nucala (self-administered)	mepolizumab	GlaxoSmithKline	interleukin-5 (IL-5) inhibitor	Asthma	SC	Filed sNDA	3Q2019	Y	N
Bronchitol	mannitol	Pharmaxis	osmotic gradient enhancer; mucus clearance enhancer	Cystic fibrosis	INH	Filed NDA	3Q2019	N	Y
KPT-330	selinexor	Karyopharm Therapeutics	selective inhibitor of nuclear export	Multiple myeloma	PO	Filed NDA	7/6/2019	Y	Y
Scenesse	afamelanotide	Clinuvel	melanocortin receptor 1 agonist	Erythropoietic protoporphyria	SC	Filed NDA	7/8/2019	Y	Y
MK-7655	relebactam/ imipenem/ cilastatin	Merck	beta-lactamase inhibitor/ carbapenem/ dehydropeptidase-1 inhibitor	Bacterial infections	IV	Filed NDA	7/16/2019	Y	N
BHV-0223	riluzole	Biohaven	glutamate release inhibitor	Amyotrophic lateral sclerosis	SL	Filed NDA	7/21/2019	N	Y
Feraccru	ferric trimaltol	Shield Therapeutics	iron replacement	Anemia	PO	Filed NDA	7/27/2019	N	N
Wakix	pitolisant	Harmony Biosciences	inverse histamine H-3 receptor antagonist	Narcolepsy	PO	Filed NDA	8/1/2019	N	N
PLX108-01	pexidartinib	Daiichi Sankyo	selective macrophage colony stimulating factor 1 receptor inhibitor	Tenosynovial giant cell tumor	PO	Filed NDA	8/3/2019	N	Y
S-649266	cefiderocol	Shionogi/ GlaxoSmithKline	cephalosporin antibiotic	Bacterial infections	IV	Filed NDA	8/14/2019	Y	N
KPI-121 0.25%	loteprednol etabonate	Kala	corticosteroid	Dry eyes	OP	Filed NDA	8/15/2019	N	N
entrectinib	entrectinib	Roche	tyrosine kinase inhibitor	Non-small cell lung cancer/ solid tumors	PO	Filed NDA	8/18/2019	N	Y

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
BC-3781	lefamulin	Nabriva Therapeutics	pleuromutilins	Bacterial infections	IV/PO	Filed NDA	8/19/2019	Y	N
SRP-4053	golodirsen	Sarepta	morpholino antisense oligonucleotide	Duchenne muscular dystrophy	IV	Filed NDA	8/19/2019	Y	N
anti-CD79b	polatuzumab vedotin	Genentech/ Roche	microtubule disrupting agent	Diffuse large B-cell lymphoma	IV	Filed BLA	8/19/2019	Y	Y
ABT-494	upadacitinib	AbbVie	janus associated kinase (JAK) inhibitor	Rheumatoid arthritis	PO	Filed NDA	8/20/2019	N	N
quizartinib	quizartinib	Daiichi Sankyo	FLT-3 receptor tyrosine kinase inhibitor	Acute myeloid leukemia	PO	Filed NDA	8/25/2019	Y	Y
Nouriast	istradefylline	Kyowa Hakko Kogyo	A2A adenosine receptor antagonist	Parkinson's disease	PO	Filed NDA	8/27/2019	N	N
Rexista XR	oxycodone ER	IntelliPharmaCeutics	opioid agonist	Pain	PO	Filed NDA	8/28/2019	N	N
PA-824	pretomanid	TB Alliance	nitroimidazole	Tuberculosis	PO	Filed NDA	9/1/2019	Y	Y
TG-101348	fedratinib	Impact Biomedicines	janus kinase 2 (JAK-2) inhibitor	Myelofibrosis	PO	Filed NDA	9/3/2019	Y	N
RDX-5791	tenapanor	Ardelyx	sodium-hydrogen exchanger-3 (NHE-3) inhibitor	Irritable bowel syndrome	PO	Filed NDA	9/13/2019	N	N
Imvamune	Imvamune; MVA-BN	Bavarian Nordic	vaccine	Smallpox	SC	Filed BLA	9/15/2019	Y	N
NN-9924	semaglutide (oral)	Novo Nordisk	glucagon-like peptide-1 (GLP-1) receptor agonist	Diabetes mellitus	PO	Filed NDA	9/20/2019	Y	N
ITI-007	lumateperone	Intra-Cellular Therapies	antipsychotic	Schizophrenia	PO	Filed NDA	9/27/2019	N	N
Posidur	bupivacaine CR	Novartis/ Durect	local anesthetic	Pain	SC	CRL	4Q2019	N	N

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
MitoGel	mitomycin C	UroGen	alkylating agent	Bladder cancer	Intravesical	FastTrk/ Breakthru	4Q2019	N	Y
HP-3070	asenapine maleate	Noven Hisamitsu Pharmaceutical	5-HT2a and dopamine D1/D2 antagonist	Schizophrenia	TOP	Filed NDA	4Q2019	N	N
Xyrosa	doxycycline	Sun Pharma	tetracyclines	Rosacea	PO	Tentative Approval	4Q2019	N	N
PF-708	teriparatide	Pfenex/ Alvogen	parathyroid hormone	Osteoporosis	SC	Filed NDA	10/7/2019	Y	N
ALKS-8700 (RDC-1559)	monomethyl fumarate (diroximel fumarate)	Biogen/ Alkermes	prodrug	Multiple sclerosis	PO	Filed NDA	10/17/2019	Y	N
CLS-1001	triamcinolone acetonide	Clearside Biomedical	corticosteroid	Macular edema	Intraocular/ subretinal	Filed NDA	10/19/2019	Y	N
synthetic ACTH depot	cosyntropin	Assertio	adrenocorticotropic hormone (ACTH)	adrenocortical insufficiency	INJ	Filed NDA	10/19/2019	Y	N
FMX-101	minocycline	Foamix	tetracyclines	Acne vulgaris	TOP	Filed NDA	10/20/2019	N	N
ODM-201	darolutamide	Bayer/ Orion	androgen receptor agonist	Prostate cancer	PO	Filed NDA	10/27/2019	Y	N
JDP-205	cetirizine	JDP Therapeutics	second generation antihistamine	Urticaria	IV	Filed NDA	10/30/2019	N	N
Naloxone Symject	naloxone	Adamis	opioid antagonist	Opioid dependence	IM	Filed NDA	10/31/2019	N	N
RediTrex	methotrexate	Cumberland	dihydrofolate reductase inhibitor	Psoriasis; arthritis	SC	Filed NDA	11/1/2019	Y	N
LY-573144	lasmiditan	Eli Lilly	serotonin 5-HT1F receptor agonist	Acute migraines	PO	Filed NDA	11/14/2019	Y	N

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
RTH-258	brolucizumab	Novartis	anti-VEGF antibody	Neovascular age-related macular degeneration	Intravitreal	Filed BLA	11/15/2019	Y	N
YKP-3089	cenobamate	SK Biopharmaceuticals	undisclosed	Seizure	PO	Filed NDA	11/21/2019	Y	N
AQST-117	riluzole	Aquestive Therapeutics	glutamate release inhibitor	Amyotrophic lateral sclerosis	SL	Filed NDA	11/30/2019	N	Y
MK-1602 (AGN-241689)	ubrogepant	Allergan	calcitonin gene-related peptide (CGRP) receptor antagonist	Acute migraines	PO	Filed NDA	12/15/2019	Y	N
EM-100	ketotifen	Eton	undisclosed	Allergic conjunctivitis	OP	Filed NDA	12/19/2019	N	N
E-2006	lemborexant	Eisai/ Purdue	orexin receptor antagonist	Insomnia	PO	Filed NDA	12/27/2019	N	N
TRV-130	oliceridine	Trevena	opioid receptor agonist	Pain	IV	CRL	Late 2019	N	N
Twirla	ethinyl estradiol/ levonorgestrel	Agile Therapeutics	hormonal combination contraceptive	Pregnancy prevention	TOP	CRL	Late 2019	N	N
Zalviso	sufentanil	AcelRx	opioid analgesic	Pain	SL	CRL	Late 2019	Y	N
IMMU-132	sacituzumab govitecan	Immunomedics	RS7-SN-38 antibody-drug conjugate	Breast cancer	IV	CRL	Late 2019	Y	Y
Tlando	testosterone	Lipocine	androgen	Hypogonadism	PO	CRL	Late 2019	N	N
aldoxorubicin	aldoxorubicin	CytRx	anthracycline derivative	Soft tissue sarcoma	IV	InTrial	Late 2019	Y	Y

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Talicia	rifabutin/ amoxicillin/ pantoprazole	RedHill Biopharma	RNA polymerase inhibitor/ penicillin/ proton pump inhibitor (PPI)	Bacterial infections	PO	FastTrk/ Breakthru	Late 2019	N	N
PRO-140	leronlimab	CytoDyn	C-C chemokine receptor 5 (CCR5) antagonist	HIV/ Graft vs. host disease	IV/SC	FastTrk/ Breakthru	Late 2019	Y	Y
ELI-200	oxycodone/ naltrexone	Elite	opioid agonist	Pain	PO	CRL	Late 2019	N	N
APL-130277	apomorphine	Sumitomo Dainippon/ MonoSol Rx/ Sunovion	non-ergoline dopamine agonist	Parkinson's disease	SL	CRL	Late 2019	N	N
AP-1007	alicaforsen	Atlantic Healthcare/ Ionis	intercellular adhesion molecule-1 (ICAM-1) inhibitor	Ulcerative colitis	IV/Rectal	Filed NDA	Late 2019	Y	Y
OMS-721	narsoplimab	Omeros	anti-MASP-2 monoclonal antibody	Hemolytic uremic syndrome/Renal diseases	IV/SC	FastTrk/ Breakthru	Late 2019	Y	Y
tamsulosin DRS	tamsulosin delayed-release	Veru	alpha-adrenergic antagonist	Benign prostatic hyperplasia	PO	InTrial	Late 2019	N	N
CCP-08	CCP-08	Tris Pharma	undisclosed	Viral rhinitis	PO	CRL	Late 2019	Y	N
Betalutin	177Lu-tetraxetan-tetulomab	Nordic Nanovector	anti-CD37 antibody radionuclide	Non-Hodgkin lymphoma	IV	FastTrk/ Breakthru	Late 2019	Y	Y
2020 Possible launch date									
AR-101	AR-101	Aimmune/ Regeneron/ Sanofi	peanut protein capsule	Peanut allergy	PO	Filed BLA	1/2020	N	N
Rykindo	risperidone ER	Luye	atypical antipsychotic	Schizophrenia/ Schizoaffective disorder	IM	Filed NDA	1/28/2020	Y	N
FP-001 (LMIS)	leuprolide mesylate	Foresee	gonadotropin-releasing hormone (GnRH) analog	Prostate cancer	SC	Filed NDA	1/29/2020	Y	N

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ETC-1002	bempedoic acid	Esperion Therapeutics	ATP citrate (pro-S)-lyase and stimulating AMP-activated protein kinase (AMPK)	Hypercholesterolemia	PO	Filed NDA	2/20/2020	N	N
ETC-1002/ ezetimibe	bempedoic acid/ ezetimibe	Esperion Therapeutics	ATP citrate (pro-S)-lyase and stimulating AMP-activated protein kinase (AMPK)/ cholesterol absorption inhibitor	Hypercholesterolemia	PO	Filed NDA	2/20/2020	N	N
ALD-403	eptinezumab	Alder	calcitonin gene-related peptide (CGRP) receptor antagonist	Migraine prevention	IV/SC	Filed BLA	2/22/2020	N	N
CD-5789	trifarotene	Galderma	retinoid receptor agonist	Acne	TOP	Filed NDA	2/28/2020	N	N
ozanimod	ozanimod	Celgene	sphingosine 1-phosphate 1 (S1PR1) and 5 (S1PR5) receptor modulator	Multiple sclerosis/ Ulcerative colitis	PO	Filed NDA	3/25/2020	Y	N
RVT-802	RVT-802	Enzyvant	tissue-based therapy	DiGeorge syndrome	Undisclosed	Filed BLA	1Q2020	Y	Y
Prochymal	remestemcel-L	Mesoblast/ Osiris Therapeutics	mesenchymal stem cells	Graft vs. Host disease	IV	InTrial	1Q2020	Y	Y
ITCA-650 (sustained release exenatide)	exenatide sustained-release	Intarcia/ Quintiles/ Servier	glucagon-like peptide-1 (GLP-1) receptor agonist	Diabetes mellitus	SC implant	CRL	1Q2020	Y	N
Trevyent	trevyent	SteadyMed	prostacyclin analog	Pulmonary arterial hypertension	SC	CRL	1Q2020	Y	Y
Corplex	donepezil transdermal system	Corium International	anticholinergic	Alzheimer's disease	TOP	InTrial	1Q2020	N	N
SEG-101	crizanlizumab	Novartis	P-selectin antagonist	Sickle cell disease	IV	FastTrk/ Breakthru	1Q2020	Y	Y

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/ Diffuse large B-cell lymphoma/ Non-Hodgkin lymphoma	IV/PO	InTrial	1Q2020	Y	Y
BMS-927711 (BHV-3000)	rimegepant sulfate	Portage Biotech/ Biohaven/ Bristol-Myers Squibb	calcitonin gene-related peptide (CGRP) receptor antagonist	Acute migraines	PO	InTrial	1Q2020	Y	N
ASG-22M6E (ASG-22CE, ASG-22ME)	enfortumab vedotin	Astellas/ Seattle Genetics	nectin-4 antagonist	Bladder cancer	IV	FastTrk/ Breakthru	1Q2020	Y	N
VX-445	VX-445	Vertex	cystic fibrosis transmembrane conductance regulator (CFTR) corrector	Cystic fibrosis	PO	FastTrk/ Breakthru	1Q2020	Y	N
PPP-002	PPP-002	Tetra Bio-Pharma	botanical drug	Pain	Undisclosed	InTrial	1Q2020	N	N
BLU-285	avapritinib	Blueprint Medicines	selective KIT and PDGFRa inhibitor	Gastrointestinal stromal tumors	PO	FastTrk/ Breakthru	1Q2020	Y	Y
ALN-AS1	givosiran	Alnylam	RNAi therapeutic agent	Porphyria	SC	FastTrk/ Breakthru	1Q2020	Y	Y
ACE-536 (RAP-536)	luspatercept	Celgene	modified type II activin receptor recombinant fusion protein	Anemia	SC	Filed BLA	4/3/2020	Y	Y
CNS-7056 (ONO-2745)	remimazolam	Cosmo/ Ono/ Paion/ Aries	benzodiazepine	Procedural sedation	IV	Filed NDA	4/5/2020	Y	N
TMC-278-LA	cabotegravir (long-acting)/ rilpivirine (long-acting)	ViiV Healthcare	HIV integrase inhibitor/ non-nucleoside reverse transcriptase inhibitor (NNRTI)	HIV	IM/SC	Filed NDA	4/28/2020	Y	N

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Rizaport	rizatriptan	IntelGenx / Red Hill Biopharma	triptans	Acute migraines	PO	CRL	1H2020	N	N
Amphora	Amphora	Neothetics	spermicidal agent	Pregnancy prevention/ Bacterial infections	VG	CRL	1H2020	N	N
Apealea (Paclical)	paclitaxel	Oasmia	taxane	Ovarian cancer	IV	InTrial	1H2020	Y	Y
Brinavess (Kynapid)	vernakalant	Correvio	potassium channel blocker	Arrhythmia	IV	InTrial	1H2020	Y	N
IdeS	imlifidase	Hansa Medical	bacterial enzyme	Kidney transplant/ Thrombotic thrombocytopenic purpura/Goodpasture's disease	IV	FastTrk/ Breakthru	1H2020	Y	Y
Anti-VEGF DARPin	abicipar pegol	Allergan	VEGF-A inhibitor	Age-related macular degeneration	Intravitreal	InTrial	1H2020	Y	N
ZEBOV	VS-EBOV (rVSV-EBOV; rVSV-ZEBOV-GP)	Merck/ NewLink Genetics	vaccine	Ebola	IM	Filed BLA	1H2020	Y	N
APD-421	amisulpride	Acacia	dopamine receptor antagonist	Nausea/ Vomiting	IV	CRL	1H2020	N	N
COR-003	levoketoconazole	Strongbridge Biopharma	azole antifungal	Cushing's syndrome	PO	InTrial	1H2020	N	Y
Traumakine	interferon-beta -1a	Faron/ Maruishi	interferon	Acute respiratory distress syndrome	IV	FastTrk/ Breakthru	1H2020	Y	N
Viaskin Peanut	Viaskin Peanut	DBV Technologies	immunotherapy	Peanut allergy	TOP	CRL	1H2020	N	N
PEGPH-20	pegvorhyaluronidase alfa	Halozyme/ Nektar	hyaluronic acid	Pancreatic cancer/ Non-small cell lung cancer	IV	FastTrk/ Breakthru	1H2020	Y	Y

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
S-265744 LAP (S/GSK-1265744 LAP; GSK-744 LA)	cabotegravir (long-acting)	GlaxoSmithKline/ Pfizer/ Shionogi	HIV integrase inhibitor	HIV	SC	InTrial	1H2020	Y	N
PRX-102	alpha galactosidase (pegunigalsidase alfa)	Protalix	enzyme replacement	Fabry disease	IV	FastTrk/ Breakthru	1H2020	Y	N
Lenti-D	elivaldogene tavalentivec	Bluebird Bio	gene therapy	Adrenomyeloneuropathy	Undisclosed	FastTrk/ Breakthru	1H2020	Y	Y
FT-218	sodium oxybate extended-release	Avadel	dopamine receptor agonist	Narcolepsy	PO	InTrial	1H2020	Y	N
ropeginterferon alfa-2b	ropeginterferon alfa-2b	PharmaEssentia/ AOP Orphan	interferon	Polycythemia vera/ Myelofibrosis/ Essential thrombocythemia	SC	InTrial	1H2020	Y	Y
Lentiglobin	lentiviral beta-globin gene transfer	Bluebird Bio	gene therapy	Sickle cell disease/ Beta thalassemia	IV	FastTrk/ Breakthru	1H2020	Y	Y
RT-002	daxibotulinumtoxin A	Revance Therapeutics	botulinum toxins	Cosmetic/ Cervical dystonia/ Plantar fasciitis	IM	InTrial	1H2020	Y	Y
UX-007	triheptanoin	Ultragenyx/ Baylor Research Institute/ Uniquest	medium chain fatty acid	Glucose transport type 1 deficiency syndrome	PO	FastTrk/ Breakthru	1H2020	Y	Y
R-667 (RG-667)	palovarotene	Clementia/ Roche	selective retinoic acid receptor agonist (RAR-gamma)	Fibrodysplasia ossificans progressiva	PO	FastTrk/ Breakthru	1H2020	Y	Y
MC2-01 (MC-201)	calcipotriene/ betamethasone	MC2 Therapeutics	vitamin D analog/ corticosteroid	Psoriasis	TOP	InTrial	1H2020	N	N
Winlevi/ Breezula	cortexolone 17alpha-propionate (CB-03-01)	Intrepid	androgen antagonist	Acne vulgaris/ alopecia	TOP	InTrial	1H2020	N	N

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
DS-8201	[fam-] trastuzumab deruxtecan	Daiichi Sankyo	HER2-targeting antibody-drug conjugate	Breast cancer	IV	FastTrk/ Breakthru	1H2020	Y	N
Darzalex	daratumumab (with recombinant human hyaluronidase)	Johnson & Johnson / Genmab	humanized anti-CD38 monoclonal antibody	Multiple myeloma/ Amyloidosis	SC	InTrial	1H2020	Y	Y
FG-4592 (ASP-1517)	roxadustat	FibroGen/ Astellas/ AstraZeneca	hypoxia-inducible factor prolyl hydroxylase	Anemia	PO	InTrial	1H2020	Y	N
SA-237 (RG-6168)	satralizumab	Roche/ Chugai	interleukin-6 (IL-6) monoclonal antibody	Neuromyelitis optica	SC	FastTrk/ Breakthru	1H2020	Y	Y
V-114	pneumococcal conjugate vaccine	Merck	vaccine	Bacterial infection	IM	FastTrk/ Breakthru	2Q2020	Y	N
KP-415	D-threo-methylphenidate controlled-release	KemPharm	CNS stimulant	Attention deficit hyperactivity disorder	PO	InTrial	2Q2020	N	N
BMN-270	valoctocogene roxaparvovec	BioMarin	gene therapy	Hemophilia	IV	FastTrk/ Breakthru	2Q2020	Y	Y
Bafiertam	monomethyl fumarate	Banner Life Sciences	prodrug	Multiple sclerosis	PO	Tentative Approval	6/20/2020	Y	N
ALKS-3831	olanzapine/ samidorphan	Alkermes	dopamine receptor antagonist/ opioid receptor antagonist	Schizophrenia/ Bipolar disorder	PO	InTrial	Mid-2020	N	N
EBP-994 (rEBP-994)	lonafarnib	Eiger Biopharmaceuticals	prenylation inhibitor	Hepatitis D/ Hutchinson-Gilford Progeria Syndrome and progeroid laminopathies	PO	FastTrk/ Breakthru	Mid-2020	Y	Y
JCAR-017	lisocabtagene maraleucel	Juno/ Celgene	chimeric antigen receptor (CAR) T cell therapy	Diffuse large B-cell lymphoma/ Acute lymphocytic leukemia/ Follicular lymphoma/ Mantle cell lymphoma	IV	FastTrk/ Breakthru	Mid-2020	Y	Y

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GLPG-0634	filgotinib	Galapagos NV/ Gilead	janus associated kinase-1 (JAK) inhibitor	Rheumatoid arthritis	PO	InTrial	Mid-2020	Y	N
idebenone	idebenone	Santhera	co-enzyme Q-10 analog	Duchenne muscular dystrophy	PO	CRL	Mid-2020	Y	Y
RV-001 (Roche-1, R-1507)	teprotumumab	Horizon/ Chugai/ Roche/ Genmab	insulin-like growth factor 1 (IGF-1) receptor antagonist	Thyroid eye disease	IV	FastTrk/ Breakthru	Mid-2020	Y	Y
QVM-149	indacaterol/ glycopyrronium bromide/ mometasone furoate	Novartis/ Sosei	long-acting beta 2 adrenergic receptor agonist (LABA)/ long-acting muscarinic receptor antagonist (LAMA)/ corticosteroid	Asthma	INH	InTrial	Mid-2020	N	N
GBT-440 (GTx-011)	voxelotor	Global Blood Therapeutics	hemoglobin modulator	Sickle cell anemia	PO	FastTrk/ Breakthru	Mid-2020	Y	Y
TGR-1202	umbralisib	TG Therapeutics/ Rhizen	phosphoinositide-3 kinase (PI3K) delta inhibitor	Diffuse large B-cell lymphoma/ Chronic lymphocytic leukemia	PO	FastTrk/ Breakthru	Mid-2020	Y	Y
SRP-4045	casimersen	Sarepta	morpholino antisense oligonucleotide	Duchenne muscular dystrophy	IV	InTrial	Mid-2020	Y	Y
RG-7916 (RO-7034067)	Risdiplam	Roche/ PTC Therapeutics	SMN2 splicing modifier	Spinal muscular atrophy	PO	InTrial	Mid-2020	Y	Y
GSK-2857916	GSK-2857916	GlaxoSmithKline/ Seattle Genetics	anti-BCMA antibody-drug conjugate	Multiple myeloma	SC	FastTrk/ Breakthru	Mid-2020	Y	Y
TBR-652 (TAK-652, CVC)	cenicriviroc	Tobira Therapeutics/ Takeda	C-C chemokine receptor 5 (CCR5) and receptor 2 antagonist	HIV/ Non-alcoholic steatohepatitis	PO	FastTrk/ Breakthru	3Q2020	Y	N
PPP-001	delta-9-tetrahydrocannabinol/ cannabidiol	PhytoPain Pharma	cannabinoid product	Pain	INH	InTrial	3Q2020	Y	Y

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Libervant	diazepam	Aquestive Therapeutics	benzodiazepine	Seizures	SL	InTrial	2H2020	N	Y
BGB-3111	zanubrutinib	BeiGene	selective inhibitor of Bruton tyrosine kinase (BTK)	Waldenström's Macroglobulinemia/ Chronic lymphocytic leukemia	PO	FastTrk/ Breakthru	2H2020	Y	Y
selumetinib	selumetinib	AstraZeneca/ Array BioPharma	selective MEK kinase inhibitor	Uveal melanoma/ Thyroid cancer	PO	InTrial	2H2020	Y	Y
naloxone	naloxone	Insys Therapeutics	opioid antagonist	Opioid dependence	Intranasal	FastTrk/ Breakthru	2H2020	N	N
MEDI-546	anifrolumab	AstraZeneca/ BMS	interferon receptor antagonist	Systemic lupus erythematosus	IV	FastTrk/ Breakthru	2H2020	Y	N
NX-1207	fexapotide triflutate	Nymox	pro-apoptotic	Benign prostatic hyperplasia/ Prostate cancer	Intratumoral	InTrial	2H2020	Y	N
Zeftera	ceftobiprole	Basilea	cephalosporin antibiotic	Bacterial infections	IV	FastTrk/ Breakthru	2H2020	Y	N
arimoclomol	arimoclomol	Orphazyme	cytoprotectives	Niemann-Pick Disease/ Sporadic Inclusion Body Myositis/ Amyotrophic lateral sclerosis	PO	FastTrk/ Breakthru	2H2020	Y	Y
Ongentys	opicapone	Neurocrine Biosciences/ Bial/ Ono	catechol-O-methyltransferase (COMT) inhibitor	Parkinson disease	PO	InTrial	2H2020	N	N
EGP-437	dexamethasone phosphate (iontophoretic)	EyeGate	corticosteroid	Uveitis	OP	InTrial	2H2020	Y	N
ZP-4207 (ZP-GA-1)	dasiglucagon	Zealand Pharma	glucagon analog	Diabetes mellitus	SC	InTrial	2H2020	N	Y
NexoBrid	bromelain	MediWound/ BL&H/ CrystalGenomics/ Kaken	peptide hydrolase replacement agent	Burns/ Skin injury	TOP	InTrial	2H2020	N	Y

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
PXT-3003	baclofen/ naltrexone/ sorbitol	Pharnext	gamma-aminobutyric acid (GABA)-ergic agonist/ opioid receptor antagonist/ sorbitol combination	Charcot-Marie Tooth disease	PO	FastTrk/ Breakthru	2H2020	N	Y
bimatoprost	bimatoprost	Allergan	prostaglandin agonist	Glaucoma	Implant	InTrial	2H2020	N/A	N
PRT-201	vonapanitase	Proteon Therapeutics	human elastase (recombinant)	End stage renal diease/ Peripheral artery disease/ Vascular access in hemodialysis	TOP	FastTrk/ Breakthru	2H2020	Y	Y
MGN-1703	lefitolimod	Mologen	Toll-like receptor 9 (TLR9) agonist	Colorectal cancer	SC	InTrial	2H2020	Y	N
Iomab-B	iodine I 131 monoclonal antibody BC8	Actinium	anti-CD45 monoclonal antibody	Acute myeloid leukemia/ Myelodysplastic syndrome	IV	InTrial	2H2020	Y	Y
AKB-6548	vadadustat	Akebia Therapeutics	hypoxia-inducible factor-prolyl hydroxylase (HIF-PH) inhibitor	Anemia	PO	InTrial	2H2020	Y	N
E-7438 (EPZ-6438)	tazemetostat	Epizyme/ Eisai	methyltransferase EZH2 inhibitor	Solid tumors/ Diffuse large B-cell lymphoma/ Non-Hodgkin lymphoma	PO	FastTrk/ Breakthru	2H2020	Y	Y
NPI-2358	plinabulin	BeyondSpring	tumor vascular disrupting agent (tVDA)	Neutropenia/ Non-small cell lung cancer	IV	InTrial	2H2020	Y	N
Sci-B-Vac	hepatitis B vaccine	VBI Vaccines	vaccine	Hepatitis B	IM	InTrial	2H2020	N	N
INP-104	POD-dihydroergotamine mesylate	Impel/ 3M	ergot derivative	Acute migraines	Intranasal	InTrial	2H2020	N	N

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
INCB-54828	pemigatinib	Incyte	selective FGFR1/2/3 inhibitor	Biliary tract cancer	PO	InTrial	2H2020	Y	Y
FMX-103	minocycline	Foamix	tetracyclines	Rosacea	TOP	InTrial	2H2020	N	N
BMS-663068 (BMS-626529 prodrug)	fostemsavir (temsavir prodrug)	Bristol-Myers Squibb	HIV attachment inhibitor	HIV	PO	FastTrk/ Breakthru	2H2020	Y	N
BCX-7353	BCX-7353	BioCryst	kallikrein inhibitor	Hereditary angioedema	PO	FastTrk/ Breakthru	2H2020	Y	Y
IMGN-853 (M-9346A-sulfo-SPDB-DM4)	mirvetuximab soravtansine	ImmunoGen	folate receptor-1 antagonist	Ovarian cancer	IV	FastTrk/ Breakthru	2H2020	Y	Y
bb-2121	bb-2121	Celgene/ Bluebird Bio	chimeric antigen receptor (CAR) T cell therapy	Multiple myeloma/ Brain cancer	IV	InTrial	2H2020	Y	Y
LIQ-861	treprostinil	Liquidia Technologies	prostacyclin analog	Pulmonary arterial hypertension	INH	InTrial	2H2020	Y	N
Doria	risperidone	Laboratorios Farmacéuticos Rovi	atypical antipsychotic	Schizophrenia	IM	InTrial	2H2020	Y	N
LJPC-0118	LJPC-0118	La Jolla Pharmaceutical	protozoacide	Malaria	Undisclosed	FastTrk/ Breakthru	2H2020	N	N
Qtrypta	zolmitriptan	Zosano	triptans	Acute migraines	TOP	InTrial	4Q2020	N	N
MOR-208 (MOR-00208, XmAB-5574)	MOR-208 (MOR-00208, XmAB-5574)	MorphoSys/ Xencor	CD-19 antagonist	Diffuse large B-cell lymphoma/ Acute lymphocytic leukemia/ Chronic lymphocytic leukemia	IV	FastTrk/ Breakthru	4Q2020	Y	Y
ALN-PCSsc (PCSK9si)	inclisiran	The Medicines Company/ Alnylam/ Arbutus Biopharma	proprotein convertase subtilisin/kexin 9 (PCSK-9) inhibitor	Hyperlipidemia	SC	InTrial	4Q2020	Y	Y
tramadol	tramadol	Avenue Therapeutics	opioid receptor agonist	Pain	IV	InTrial	4Q2020	N	N

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AmnioFix	dehydrated human amnion/chorion membrane (dHACM)	MiMedx	amniotic tissue membrane	Plantar fasciitis/ Achilles tendonitis/ Osteoarthritis	INJ	InTrial	4Q2020	Y	N
Estelle	estetrol/ drospirenone	Mithra/ Fuji/ Zhejian Xianju	estrogen receptor agonist	Pregnancy prevention	PO/SL/ Transmucosal	InTrial	4Q2020	N	N
Infacort	hydrocortisone	Diurnal Group	corticosteroid	Adrenal insufficiency	PO	InTrial	4Q2020	N	Y
TAK-385	relugolix	Myovant Sciences/ Roivant Sciences/ Takeda	gonadotropin-releasing hormone (GnRH) receptor antagonist	Uterine fibroids/ Endometriosis/ Prostate cancer	PO	InTrial	4Q2020	Y	N
CAM-2038	buprenorphine	Camurus/ Braeburn	opioid receptor agonist (partial)	Opioid dependence/ Pain	SC	Tentative Approval	11/1/2020	Y	N
VivaGel	astodrimer sodium	Starpharma	viral attachment inhibitor	Bacterial infections	VG	CRL	2020	N	N
JNJ-872 (VX-787)	JNJ-872 (VX-787)	Johnson & Johnson/ Vertex	viral protein inhibitor	Influenza	PO	FastTrk/ Breakthru	2020	N	N
Sativex	nabiximols	GW Pharmaceuticals/ Otsuka	cannabinoid product	Multiple sclerosis/ Pain	SL/ SPR	FastTrk/ Breakthru	2020	N	N
USL-261	midazolam	UCB	benzodiazepine	Seizures	Intranasal	CRL	2020	N	Y
CM-AT	CM-AT	Curemark	protein absorption enhancer	Autism	PO	FastTrk/ Breakthru	2020	Y	N
Dexasite	dexamethasone	InSite Vision	corticosteroid	Blepharitis/ Ocular inflammation	TOP	InTrial	2020	N	N
EVK-001	metoclopramide	Evoke Pharma	antidopiminergics	Diabetic gastroparesis	Intranasal	CRL	2020	N	Y
Entyvio (SC formulation)	vedolizumab	Takeda	integrin receptor antagonist	Ulcerative colitis/ Crohn's disease	SC	InTrial	2020	Y	N

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Multikine	Leukocyte Interleukin (CS-001P3)	CEL-SCI	immunomodulator	Head and Neck cancer/ Squamous cell carcinoma	SC	InTrial	2020	Y	Y
iclaprim	iclaprim	Motif Bio	tetrahydrofolate dehydrogenase inhibitor	Bacterial infections	IV	CRL	2020	Y	Y
tanezumab	tanezumab	Pfizer/ Eli Lilly	neurotrophic tyrosine kinase receptor type 1 (TrkA) antagonist (monoclonal antibody)	Osteoarthritis/ Pain	IV/SC	FastTrk/ Breakthru	2020	Y	N
ublituximab (LFB-R603, TG20, TGTX-1101, TG-1101, Utuxin)	ublituximab	TG Therapeutics	CD-20 monoclonal antibody	Chronic lymphocytic leukemia/ Small cell lymphocytic lymphoma/ Mantle cell lymphoma/ Multiple sclerosis	IV	InTrial	2020	Y	Y
Fintepla	fenfluramine	Zogenix	serotonin receptor agonist	Dravet syndrome/ Lennox-Gastaut syndrome	PO	CRL	2020	Y	Y
Zynquista	sotagliflozin	Sanofi/ Lexicon	sodium-dependent glucose transporter 1 (SGLT-1) and SGLT-2 inhibitor	Diabetes mellitus	PO	CRL	2020	N	N
Deltyba	delamanid	Otsuka	mycolic acid biosynthesis inhibitor	Tuberculosis	PO	InTrial	2020	N	N
ND-0612L	levodopa/ carbidopa	NeuroDerm	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	SC	InTrial	2020	Y	N
ND-0612H	levodopa/ carbidopa	NeuroDerm	dopamine precursor/ dopa-decarboxylase inhibitor	Parkinson's disease	SC	InTrial	2020	Y	N

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Pedmark (STS)	sodium thiosulfate	Fennec	reducing agent	Hearing loss	IV	FastTrk/ Breakthru	2020	Y	Y
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 diseaser/ Asthma	INH	InTrial	2020	N	N
Tivopath (AV-951, KRN-951, ASP-4130)	tivozanib	Aveo/ Astellas/ Kyowa Hakko Kirin	VEGF inhibitor	Renal cell cancer	PO	InTrial	2020	Y	N
LCI-699	osilodrostat	Novartis	aldosterone synthase inhibitor	Cushing's syndrome	PO	InTrial	2020	N	Y
Vicinium (VB-4-845)	oportuzumab monatox	Eleven Biotherapeutics	anti-ECAM exotoxin A fusion protein	Bladder cancer	Intravesical	FastTrk/ Breakthru	2020	Y	N
N-1539	meloxicam	Recro Pharma/ Alkermes	nonsteroidal anti-inflammatory drug (NSAID)	Pain	IV	CRL	2020	Y	N
Contepo	fosfomycin	Nabriva Therapeutics	cell wall inhibitor	Bacterial infections	IV	CRL	2020	Y	N
GZ-402666 (NeoGAA)	neo-recombinant human acid alpha glucosidase	Sanofi	enzyme therapy	Pompe disease	IV	InTrial	2020	Y	N
ursodeoxycholic acid	ursodeoxycholic acid	Retrophin/ Asklepion	bile acid derivative	Primary biliary cirrhosis/cholangitits	PO	InTrial	2020	Y	N
HTX-011	bupivacaine/ meloxicam	Heron Therapeutics	anesthetic/ nonsteroidal anti-inflammatory Drug (NSAID)	Pain	Instillation	CRL	2020	N	N
APN-311	dinutuximab beta	EUSA/ Aperion/ Gen llac/ Medison/ Paladin	disialoganglioside	Neuroblastoma	SC	InTrial	2020	Y	Y

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
isatuximab	isatuximab	Sanofi/ ImmunoGen	CD38 antagonist	Multiple myeloma/ Acute lymphoblastic leukemia or lymphoblastic lymphoma	IV	InTrial	2020	Y	Y
CPP-1X/ sulindac (DFMO)	eflornithine/ sulindac	Cancer Prevention Pharma/ Zeria	ornithine decarboxylase inhibitor/ non-steroidal anti-inflammatory drug (NSAID)	Familial adenomatous polyposis/ Colorectal cancer	PO	FastTrk/ Breakthru	2020	Y	Y
QMF-149	indacaterol maleate/ mometasone furoate	Novartis/ Merck	long-acting beta 2 agonist/ corticosteroid	Asthma	INH	InTrial	2020	N	N
DS-200	DS-200	Eton	undisclosed	Ophthalmological disease	SC	InTrial	2020	unknown	N
DS-100	DS-100	Eton	undisclosed	Ophthalmological disease	SC	InTrial	2020	unknown	N
NNC-0195-0092 (NN-8640)	somapacitan	Novo Nordisk	recombinant human growth hormone (rhGH)	Short stature/ Growth hormone deficiency	SC	InTrial	2020	Y	N
MLN-4924 (TAK-92)	pevonedistat	Takeda	Nedd 8 Activating Enzyme (NAE) antagonist	Acute myeloid leukemia/ Chronic myelogenous leukemia/ Myelodysplastic syndrome	PO	InTrial	2020	Y	N
INCB-028060	capmatinib	Novartis/ Incyte	cMET inhibitor	Non-small cell lung cancer	PO	InTrial	2020	Y	N
MNK-812	oxycodone	Mallinckrodt	opioid agonist	Pain	PO	CRL	2020	N	N
APC-8000	tadalafil	Adamis	phosphodiesterase-5 (PDE-5) inhibitor	Erectile dysfunction	PO	CRL	2020	Y	N

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Travivo	gepirone ER	GSK/Fabre-Kramer	5-HT-1A receptor agonist	Major depressive disorder	PO	CRL	2020	N	N
Oralair Mites	dust mite peptide	Stallergenes/ Shionogi	vaccine	Dust mite allergic rhinitis	SL	InTrial	2020	Y	N
azacitidine	azacitidine	Celgene	DNA methylation inhibitor	Acute myeloid leukemia/ Myelodysplastic syndromes	PO	FastTrk/ Breakthru	Late 2020	Y	Y
Translarna	ataluren	PTC Therapeutics	gene transcription modulator	Duchenne muscular dystrophy	PO	CRL	Late 2020	Y	Y
GFT-505	elafibranor	Genfit	selective peroxisome proliferator-activated receptor (PPAR) modulator	Non-alcoholic steatohepatitis/ Primary biliary cirrhosis	PO	FastTrk/ Breakthru	Late 2020	N	N
MVA-MUC1-IL2	TG-4010	Transgene	vaccine	Non-small cell lung cancer	SC	FastTrk/ Breakthru	Late 2020	N	N
GRN-163L	imetelstat	Geron/ Johnson & Johnson	telomerase inhibitor	Myelofibrosis/ Myelodysplastic syndrome/ Acute myelogenous leukemia	IV	FastTrk/ Breakthru	Late 2020	Y	Y
Mycapssa (Octreolin)	octreotide	Chiasma	somatostatin analog	Acromegaly	PO	CRL	Late 2020	Y	Y
NS-2 (ALDX-1E1, ALDX-1E2, ADX-102)	reproxalap	Aldeyra Therapeutics	aldehyde antagonist	Uveitis/ Allergic conjunctivitis/ Dry eyes	OP	InTrial	Late 2020	N	N
Linhaliq	ciprofloxacin	Grifols	fluoroquinolone	Non-cystic fibrosis bronchiectasis	INH	CRL	Late 2020	Y	Y

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
Lucassin	terlipressin	Orphan Therapeutics/ Ikaria	V-1 (vasopressin) agonist	Hepato-renal syndrome	IV	CRL	Late 2020	Y	Y
RSV-F (ResVax)	respiratory syncytial virus vaccine	Novavax	vaccine	Respiratory syncytial virus infection	IM	FastTrk/ Breakthru	Late 2020	Y	N
QAW-039 (NVP-QAW-039)	fevipiprant	Novartis	chemoattractant receptor-homologous molecule (CRTH2) antagonist	Asthma/ Atopic dermatitis	PO	InTrial	Late 2020	Y	N
Molgradex	molgramostim	Savara	granulocyte macrophage-colony stimulating factor	Pulmonary alveolar proteinosis	INH	InTrial	Late 2020	Y	Y
SHP-621	budesonide	Shire	corticosteroid	Eosinophilic esophagitis	PO	FastTrk/ Breakthru	Late 2020	Y	Y
cannabidiol	cannabidiol	Insys Therapeutics	cannabinoid product	Seizures/ Prader-Willi	PO	FastTrk/ Breakthru	Late 2020	Y	N
skQ1	visomitin	Mitotech	plastoquinone derivative	Dry eyes	OP	InTrial	Late 2020	Y	N
SCY-078 (MK-3118)	ibrexafungerp	Scynexis/ R-Pharm JSC/ Merck	glucan synthase inhibitors	Fungal infections	IV/PO	FastTrk/ Breakthru	Late 2020	N	Y
BIM-22493 (RM-493)	setmelanotide	Rhythm/ Camurus/ Ipsen	melanocortin 4 receptor (MC4R) agonist	Obesity/ Bardet-Biedl syndrome/ Prader-Willi syndrome	SC	FastTrk/ Breakthru	Late 2020	Y	Y
GSK-2696274 (OTL-200)	GSK-2696274 (OTL-200)	GlaxoSmithKline	gene therapy	Leukodystrophy	IV	InTrial	Late 2020	Y	Y
RE-024	fosmetpantotenate	Retrophin	phosphopantothenate replacement therapy	Neurodegeneration	IV	FastTrk/ Breakthru	Late 2020	Y	Y

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
BMN-111	vosoritide (vasoritide)	BioMarin/ Chugai	C-type natriuretic peptide (CNP) analog	Achondroplasia	SC	InTrial	Late 2020	Y	Y
Furoscix	furosemide	scPharmaceuticals	diuretic	Heart failure	SC	CRL	Late 2020	Y	N
HuMax-TF ADC	tisotumab vedotin	Genmab/ Seattle Genetics	tissue factor antibody	Solid tumors	Undisclosed	InTrial	Late 2020	Y	N
MK-0594	serlopitant	Menlo	NK-1 receptor antagonist	Atopic dermatitis/ Cough	PO	FastTrk/ Breakthru	Late 2020	Y	N
LY-900014	LY-900014	Eli Lilly	insulins	Diabetes mellitus	SC	InTrial	Late 2020	N	N
2021 Possible launch date									
MK-4618 (KRP-114V, RVT-901)	vibegron	Roivant Sciences/ Urovant/ Kissei/ Kyorin/ Merck	selective beta 3 adrenergic receptor agonist	Overactive bladder	PO	InTrial	1Q2021	N	N
ALNG-01 (ALN-G-01)	lumasiran	Alnylam	glycolate oxidase antagonist	Hyperoxaluria	Intranasal	InTrial	1Q2021	Y	Y
PDP-716	brimonidine	Sun Pharma Advanced Research Company (SPARC)	alpha-2 agonist	Glaucoma	OP	InTrial	1Q2021	N	N
RGN-259 (GBT-201; RGN-352)	thymosin beta 4	RegeneRx	actin regulating peptide	Neurotrophic keratitis/ Dry eyes	OP	InTrial	1H2021	N	Y
UCB-4940 (CDP-4940)	bimekizumab	UCB	interleukin-17 (IL-17) receptor inhibitor	Psoriasis/ Psoriatic arthritis/ Ankylosing spondylitis/ Rheumatoid arthritis	IV	InTrial	1H2021	Y	N
ACP-001	TransCon Growth Hormone	Ascendis	growth hormone prodrug	Short stature/ Growth hormone deficiency	SC	InTrial	1H2021	Y	N
CCX-168	avacopan	ChemoCentryx/ Galencia	C5a receptor (C5aR) antagonist	Vasculitis/ Glomerulopathy	PO	InTrial	1H2021	Y	Y

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
GSK-2894512 (WBI-1001)	tapinarof	GSK/ Celestial/ Roivant Sciences/ Welichem Biotech	therapeutic aryl hydrocarbon receptor modulating agent	Atopic dermatitis/ Psoriasis	TOP	InTrial	1H2021	Y	Y
SDP-037, SDN-037	SDP-037, SDN-037	Sun Pharma	Corticosteroid	Ocular inflammation/pain	OP	InTrial	2Q2021	N	Y
BAY-1021189 (MK-1242)	vericiguat	Merck/ Bayer	guanylate cyclase stimulator	Heart failure	PO	InTrial	Mid-2021	Y	N
SPN-810	molindone	Supernus	atypical antipsychotic	Attention deficit hyperactivity disorder	PO	FastTrk/ Breakthru	2H2021	N	Y
GS-010	GS-010	GenSight Biologics	gene therapy	Optic neuropathy	Intraocular	InTrial	2H2021	Y	N
Otividex	dexamethasone sustained-release	Otonomy	corticosteroid	Meniere's disease	Intratympanic	InTrial	2H2021	Y	Y
DigiFab	digoxin immune fab	AMAG/ Velo	digitalis-like factor antagonist	Preeclampsia	IV	FastTrk/ Breakthru	2H2021	Y	Y
RG-7716 (RO-6867461)	RG-7716 (RO-6867461)	Roche/ Chugai	bispecific VEGF-A/ angiopoietin-2 antagonist	Macular degeneration	Intravitreal	InTrial	2H2021	Y	N
VBP-15	vamorolone	Santhera	corticosteroid	Duchenne muscular dystrophy	PO	FastTrk/ Breakthru	2H2021	Y	Y
CMX-001	brincidofovir hexadecyloxypropyl ester	Chimerix	DNA-directed DNA polymerase inhibitor	Adenovirus/ Cytomegalovirus/ Smallpox	PO	FastTrk/ Breakthru	2021	N	Y
Junovan	mifamurtide	Millenium	muramyl tripeptide phosphatidylethanolamine (MTP-PE)	Osteosarcoma	IV	InTrial	2021	Y	N
rivipansel	rivipansel	Pfizer/ GlycoMimetics	synthetic glycomimetic	Sickle cell	IV	FastTrk/ Breakthru	2021	Y	N
Luveniq	voclosporin	ILJIN	calcineurin inhibitor	Lupus nephritis/ Transplant rejection	PO	FastTrk/ Breakthru	2021	Y	Y

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
ATI-5923	tecarfarin	ARYx Therapeutics/ Armetheon	vitamin K epoxide reductase enzyme inhibitor	Anticoagulation	PO	InTrial	2021	N	Y
OSE-2101 (IDM-2101, EP-2101)	tedopi	OSE Pharma/ Takeda	vaccine	Non-small cell lung cancer	SC	InTrial	2021	Y	N
MD-1003	MD-1003	MedDay	biotin	Multiple sclerosis	PO	InTrial	2021	Y	Y
R-1658 (RG-1658, JTT-705, RO-4607381)	dalcetrapib	DalCor/ Japan Tobacco/ Roche	cholesteryl ester transfer protein inhibitor	Acute coronary syndrome	PO	InTrial	2021	Y	Y
IMO-2125	tilsotolimod	Idera	toll-like receptor 9 (TLR-9) agonist	Melanoma	SC/ intratumoral	FastTrk/ Breakthru	2021	Y	N
AZD-6094 (HMPL-504)	savolitinib (volitinib)	AstraZeneca (Hutchison MediPharma)	c-Met receptor tyrosine kinase inhibitor	Renal cell cancer/ Non-small cell lung cancer	PO	InTrial	2021	Y	Y
CT-100	corticotrophin	Eton	adrenocorticotropic hormone (ACTH)	Rheumatoid arthritis	INJ	InTrial	2021	Y	N
SHP-647 (PF-00547659)	SHP-647 (PF-00547659)	Shire	MAdCAM-1 antagonist	Irritable bowel disease/ Crohn's disease/ Ulcerative colitis	IV/SC	InTrial	2021	Y	Y
RG-7314 (RO-5285119)	balovaptan	Roche	V1A vasopressin receptor antagonist	Autism spectrum disorder	PO	FastTrk/ Breakthru	2021	Y	N
ABL-001	asciminib	Novartis	allosteric Bcr-Abl inhibitor	Chronic myelogenous leukemia	PO	InTrial	2021	Y	N
POL-6326	balixafortide	Polyphor	chemokine (CXCR4) antagonist	Transplant/ Breast cancer	IV	InTrial	2021	Y	N
BMS-986089 (RG-6206)	BMS-986089 (RG-6206)	Roche/ Bristol-Myers Squibb	anti-myostatin adnectin	Duchenne muscular dystrophy	SC	InTrial	2021	Y	N
Luveniq	voclosporin	ILJIN	calcineurin inhibitor	Lupus nephritis/ Psoriasis/ Transplant rejection	PO	FastTrk/ Breakthru	2021	Y	Y

Drug name	Generic name	Company	Drug class	Therapeutic use	Route of administration	Regulatory status	Estimated release date	Specialty drug	Orphan drug
TWIN (S6G5T-1; S6G5T-3)	benzoyl peroxide/ tretinoin	Sol-Gel Technologies	retinoid	Acne vulgaris	TOP	InTrial	2021	N	N
OTL-101	ADA-transduced autologous stem cell therapy	Orchard Therapeutics	gene therapy	Adenosine deaminase-deficient severe combined immunodeficiency	Undisclosed	FastTrk/ Breakthru	2021	Y	Y
gantenerumab	gantenerumab	Roche	beta-amyloid (Abeta) inhibitor	Alzheimer's disease	SC	InTrial	Late 2021	Y	N
ADXS-HPV (ADXS-11-001, Lm-LLO-E7, lovaxin C)	axalimogene filolisbac	Advaxis/ Biocon/ FusionVax/ Sorrento Therapeutics/ Taiwan Biotech	vaccine	Anal cancer/ Cervical cancer/ Head and neck cancer	IV	FastTrk/ Breakthru	Late 2021	Y	N
PW-4142 (T-111)	nalbuphine ER	Trevi Therapeutics/ Endo	opioid agonist/ antagonist	Prurigo nodularis	PO	InTrial	Late 2021	N	N
Humacyl	human acellular vessel	Humacyte	cellular therapy	End-stage renal disease/ Peripheral artery disease	Implant	FastTrk/ Breakthru	Late 2021	Y	N
NNZ-2566	trofinetide	Neuren	insulin-like growth factor 1 (IGF-1) derivative	Rett syndrome/ Fragile X syndrome/ Brain injury	IV/PO	FastTrk/ Breakthru	Late 2021	Y	N
Chronocort	hydrocortisone modified-release	Diurnal Group	corticosteroid	Congenital adrenal hyperplasia	PO	InTrial	Late 2021	N	N
AMT-061	AMT-061	uniQure	coagulation Factor IX	Hemophilia B	IV	FastTrk/ Breakthru	Late 2021	Y	Y
PF-06482077	multivalent group B streptococcus vaccine	Pfizer	vaccine	Bacterial infection	IM	FastTrk/ Breakthru	Late 2021	Y	Y
Ultomiris SC	ravulizumab-cwvz	Alexion	C5 complement inhibitor	paroxysmal nocturnal hemoglobinuria/ Hemolytic uremic syndrome	SC	InTrial	Late 2021	Y	Y

IM = intramuscular, INH = inhalation, INJ = injection, IUD = intrauterine device, IV = intravenous, OP = ophthalmic, PO = oral, SC = subcutaneous, SL = sublingual, SPR = spray, 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
Cyramza	ramucirumab	Eli Lilly/ Shire/ AstraZeneca	vascular endothelial growth factor 2 receptor antagonist	Liver cancer	Second line treatment in patients with hepatocellular carcinoma following first line therapy with Nexavar (sorafenib)	IV	5/2019
Eylea	aflibercept	Regeneron	vascular endothelial growth factor-A inhibitor/ placental growth factor inhibitor	Non-proliferative diabetic retinopathy	Treatment of moderately severe to severe non-proliferative diabetic retinopathy without diabetic macular edema	OPH	5/13/2019
Jakafi	ruxolitinib	Incyte	janus associated kinase (JAK) inhibitors	Graft-versus-host disease	Treatment of patients with steroid-refractory acute graft-versus-host disease	PO	5/24/2019
Vraylar	cariprazine	Allergan	Dopamine D_3/D_2 receptor partial agonist	Bipolar disorder	Treatment of adults with major depressive episodes associated with bipolar I disorder	PO	6/2019
Bavencio	avelumab	EMD Serono/ Pfizer	PD-L1 monoclonal antibody	Renal cancer	in combination with Inlyta (axitinib) for treatment-naïve patients with advanced renal cell carcinoma	IV	6/2019
Emgality	galcanezumab-gnlm	Eli Lilly	calcitonin gene-related peptide (CGRP) antagonist	Episodic cluster headache	Preventive treatment of episodic cluster headache	SC	6/2019
Botox	onabotulinumtoxinA	Allergan	botulinum toxin analog	Upper spasticity	Treatment of pediatric patients (2 years of age and older) with upper limb spasticity	IM	6/2019

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Zerbaxa	ceftolozane/ tazobactam	Merck	Cephalosporin/beta lactamase inhibitor	Bacterial infections	Treatment of adult patients with nosocomial pneumonia, including ventilator-associated pneumonia caused by certain susceptible Gram-negative microorganisms	IV	6/3/2019
Keytruda	pembrolizumab	Merck	anti-PD-1 inhibitor	Head & neck cancer	Monotherapy and in combination with chemotherapy, for the first-line treatment of recurrent or metastatic head and neck squamous cell carcinoma	IV	6/10/2019
Keytruda	pembrolizumab	Merck	anti-PD-1 inhibitor	Small-cell lung cancer	Treatment of patients with advanced small-cell lung cancer whose disease has progressed after two or more lines of prior therapy	IV	6/17/2019
Dupixent	dupilumab	Sanofi/ Regeneron	interleukin-4/13 (IL-4/IL-13) inhibitor	Nasal polyps	Add-on maintenance treatment for adults with inadequately controlled severe chronic rhinosinusitis with nasal polyps	SC	6/26/2019
Revlimid	lenalidomide	Celgene	thalidomide analogue	Relapsed/refractory follicular and marginal zone lymphoma	In combination with rituximab, for the treatment of patients with previously treated follicular and marginal zone lymphoma	PO	6/27/2019
Soliris	eculizumab	Alexion	Recombinant humanized monoclonal antibody complement protein C5 inhibitor	Neuromyelitis optica spectrum disorder	Treatment of anti-aquaporin-4 auto antibody-positive neuromyelitis optica spectrum disorder.	IV	6/28/2019

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Doptelet	avatrombopag	Dova	thrombopoietin receptor agonist	Immune thrombocytopenia	Treatment of adult patients with immune thrombocytopenia who have had an insufficient response to a previous treatment	PO	6/30/2019
Symdeko	tezacaftor/ivacaftor; ivacaftor	Vertex	Cystic fibrosis transmembrane conductance regulator (CFTR) corrector/ CFTR potentiator	Cystic fibrosis	Treatment of children with cystic fibrosis ages 6 through 11 who have two copies of the F508del mutation or who have at least one mutation that is responsive to tezacaftor/ivacaftor	PO	3Q 2019
Otezla	apremilast	Celgene	phosphodiesterase 4 inhibitor	Behcet's disease	Treatment of active Behcet's disease	PO	7/21/2019
Hetlioz	tasimelteon	Vanda	melatonin receptor 1 and 2 agonist	Insomnia	Treatment of jet lag disorder	PO	8/16/2019
Tecentriq	atezolizumab	Genentech	PD-L1 monoclonal antibody	Non-small cell lung cancer	In combination with Abraxane (albumin-bound paclitaxel; nab-paclitaxel) and carboplatin for the initial (first-line) treatment of people with metastatic non-squamous non-small cell lung cancer who do not have EGFR or ALK genomic tumor aberrations	IV	9/2/2019
Venclexta	venetoclax	AbbVie	proto-oncogene protein c-bcl-2 inhibitor	Chronic lymphocytic leukemia	In combination with Gazyva (obinutuzumab) for first-line treatment of chronic lymphocytic leukemia	PO	9/7/2019
Nucala	mepolizumab	GlaxoSmithKline	Interleukin-5 (IL-5) antagonist	Eosinophilic asthma	Add-on treatment for severe eosinophilic asthma in pediatric patients aged six to 11 years	SC	9/19/2019

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Pifeltro	doravirine	Merck	non-nucleoside reverse transcriptase inhibitor (NNRTI)	HIV infection	Use in people living with HIV-1 who are switching from a stable antiretroviral regimen and whose virus is suppressed (HIV-1 RNA < 50 copies/mL)	PO	9/20/2019
Delstrigo	doravirine/ lamivudine/ tenofovir disoproxil fumarate	Merck	non-nucleoside reverse transcriptase inhibitor (NNRTI)/ nucleoside reverse transcriptase inhibitor (NRTI)/ NRTI	HIV infection	Use in people living with HIV-1 who are switching from a stable antiretroviral regimen and whose virus is suppressed (HIV-1 RNA < 50 copies/mL)	PO	9/20/2019
Botox	onabotulinumtoxinA	Allergan	botulinum toxin analog	Lower spasticity	Treatment of pediatric patients (2 years of age and older) with lower limb spasticity	IM	4Q 2019
Nplate	romiplostim	Amgen	thrombopoietin receptor agonist	Immune thrombocytopenia	Treatment of adult patients with immune thrombocytopenia (ITP) who have had ITP for 12 months or less and an insufficient response to corticosteroids, immunoglobulins or splenectomy	SC	10/2019
Emflaza	deflazacort	PTC Therapeutics	corticosteroid	Duchenne muscular dystrophy	Treatment of Duchenne muscular dystrophy in patients 2 to 5 years of age	PO	10/2019
Descovy	emtricitabine/ tenofovir alafenamide	Gilead	nucleoside reverse transcriptase inhibitor (NRTI)/ NRTI	HIV infection	Pre-exposure prophylaxis (PrEP) to reduce the risk of sexually acquired HIV-1 infection among individuals who are HIV-negative and at risk for HIV	PO	10/5/2019
Xarelto	rivaroxaban	Janssen	factor Xa inhibitor	Anticoagulation	Prevention of venous thromboembolism, or blood clots, in medically ill patients	PO	10/14/2019

Brand name	Generic name	Company	Drug class	Therapeutic use	Proposed new indication	Route of administration	Estimated approval date
Zilretta	triamcinolone acetonide	Flexion Therapeutics	corticosteroids	Osteoarthritis	Revise the product label based on data from an open-label Phase 3b trial, which indicated that repeat administration of Zilretta for treatment of osteoarthritis knee pain was safe and well tolerated with no deleterious impact on cartilage or joint structure observed through X-ray analysis	Intra-articular	10/17/2019
Stelara	ustekinumab	Janssen	Interleukin-12/-23 (IL-12/23) antagonist	Ulcerative colitis	Treatment of ulcerative colitis	SC	10/20/2019
Erleada	apalutamide	Janssen	androgen receptor antagonist	Prostate cancer	Treatment of patients with metastatic castration-sensitive prostate cancer	PO	10/26/2019
Xofluza	baloxavir	Genentech/ Shionogi	polymerase acidic endonuclease inhibitor	Influenza	Treatment of influenza in individuals at high-risk for influenza-related complications 12 years of age or older	PO	11/4/2019
Dextenza	dexamethasone	Ocular Therapeutix	corticosteroid	Ocular inflammation	Treatment of ocular inflammation following ophthalmic surgery	OPH	11/10/2019

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

References:

AbbVie Press Release. AbbVie Web site. AbbVie announces New Drug Application accepted for priority review by U.S. FDA for upadacitinib for treatment of moderate to severe rheumatoid arthritis. https://news.abbvie.com/news/press-releases/abbvie-announces-new-drug-application-accepted-for-priority-review-by-us-fda-for-upadacitinib-for-treatment-moderate-to-severe-rheumatoid-arthritis.htm. February 19, 2019. Accessed April 2, 2019.

AbbVie Press Release. AbbVie Web site. AbbVie's upadacitinib shows positive results as monotherapy in phase 3 rheumatoid arthritis study, meeting all primary and key secondary endpoints. https://news.abbvie.com/news/press-releases/abbvies-upadacitinib-shows-positive-results-as-monotherapy-in-phase-3-rheumatoid-arthritis-study-meeting-all-primary-and-key-secondary-endpoints.htm. December 20, 2017. Accessed April 2, 2019.

AbbVie Press Release. AbbVie Web site. Upadacitinib meets all primary and ranked secondary endpoints including superiority versus adalimumab in phase 3 study in rheumatoid arthritis. https://news.abbvie.com/news/upadacitinib-meets-all-primary-and-ranked-secondary-endpoints-including-superiority-versus-adalimumab-in-phase-3-study-in-rheumatoid-arthritis.htm. April 9, 2018. Accessed April 2, 2019.

AbbVie Press Release. AbbVie Web site. Upadacitinib monotherapy meets all primary and ranked secondary endpoints versus methotrexate in a phase 3 study in rheumatoid arthritis. https://news.abbvie.com/alert-topics/immunology/upadacitinib-monotherapy-meets-all-primary-and-ranked-secondary-endpoints-versus-methotrexate-in-phase-3-study-in-rheumatoid-arthritis.htm. June 5, 2018. Accessed April 2, 2019.

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

Burmester GR, Kremer JM, Van den Bosch F, et al. Safety and efficacy of upadacitinib in patients with rheumatoid arthritis and inadequate response to conventional synthetic disease-modifying anti-rheumatic drugs (SELECT-NEXT): a randomised, double-blind, placebo-controlled phase 3 trial. Lancet. 2018;391(10139):2503-2512.

Clinuvel Press Release. Clinuvel Web site. US FDA sets PDUFA date for Scenesse. https://www.clinuvel.com/investors/news/announcements. January 10, 2019. Accessed April 7, 2019.

Daiichi Sankyo Press Release. Daiichi Sankyo Web site. Daiichi Sankyo presents phase 3 ENLIVEN study of pexidartinib, demonstrating statistically significant clinical improvement across multiple endpoints in patients with tenosynovial giant cell tumor at 2018 American Society of Clinical Oncology (ASCO) Annual Meeting. https://www.daiichisankyo.com/media_investors/media_relations/press_releases/detail/006865.html. June 4, 2018. Accessed April 7, 2019.

Daiichi Sankyo Press Release. Daiichi Sankyo Web site. FDA grants priority review for Daiichi Sankyo's New Drug Application for CSF1R inhibitor pexidartinib for treatment of patients with TGCT, a rare, debilitating tumor. https://dsi.com/documents/364091/10093712/Pexidartinib+FDA+NDA+Release_2+4+19+FINAL.pdf/65ad4919-8179-bdce-21c7-02090c4658fb. February 5, 2019. April 6, 2019.

Dauvilliers Y, Bassetti C, Lammers GJ, et al; HARMONY I study group. Pitolisant versus placebo or modafinil in patients with narcolepsy: a double-blind, randomised trial. Lancet Neurol. 2013;12(11):1068-75.

FDA Press Release. FDA Web site. FDA approves new treatments for heart disease caused by a serious rare disease, transthyretin mediated amyloidosis. https://www.fda.gov/news-events/press-announcements/fda-approves-new-treatments-heart-disease-caused-serious-rare-disease-transthyretin-mediated. May 6, 2019. Accessed May 6, 2019.

Genentech Press Release. Genentech Web site. FDA grants priority review to Genentech's personalized medicine entrectinib. https://www.gene.com/media/press-releases/14778/2019-02-18/fda-grants-priority-review-to-genentech. February 18, 2019. Accessed April 2, 2019.

Genentech Press Release. Genentech Web site. FDA grants priority review to Genentech's polatuzumab vedotin in previously treated aggressive lymphoma. https://www.gene.com/media/press-releases/14777/2019-02-18/fda-grants-priority-review-to-genentechs. February 18, 2019. Accessed April 2, 2019.

Genentech Press Release. Genentech Web site. Genentech's investigational medicine entrectinib showed a durable response of more than two years in people with a specific type of lung cancer. https://www.gene.com/media/press-releases/14745/2018-09-24/genentechs-investigational-medicine-entr. September 24, 2018. Accessed April 2, 2019.

Genentech Press Release. Genentech Web site. Genentech's investigational personalized medicine entrectinib shrank tumors in people with NTRK fusion-positive solid tumors. https://www.gene.com/media/press-releases/14759/2018-10-21/genentechs-investigational-personalized-. October 21, 2018. Accessed April 2, 2019.

Genovese MC, Fleischmann R, Combe B, et al. Safety and efficacy of upadacitinib in patients with active rheumatoid arthritis refractory to biologic disease-modifying anti-rheumatic drugs (SELECT-BEYOND): a double-blind, randomised controlled phase 3 trial. Lancet. 2018;391(10139):2513-2524.

Harmony Biosciences Press Release. Harmony Biosciences Web site. Harmony Biosciences announces file acceptance of its New Drug Application for Pitolisant. https://www.harmonybiosciences.com/newsroom/harmony-biosciences-announces-file-acceptance-of-its-new-drug-application. February 12, 2019. Accessed April 1, 2019.

Langendonk JG, Balwani M, Anderson KE, et al. Afamelanotide for erythropoietic protoporphyria. N Engl J Med. 2015;373(1):48-59.

Maurer MS, Schwartz JH, Gundapaneni B, et al; ATTR-ACT Study Investigators. Tafamidis treatment for patients with transthyretin amyloid cardiomyopathy. N Engl J Med. 2018;379(11):1007-1016.

National Organization for Rare Disorders (NORD). Erythropoietic protoporphyria and X-linked Protoporphyria. NORD Web site. https://rarediseases.org/rare-diseases/erythropoietic-protoporphyria. Accessed April 24, 2019.

Novo Nordisk Press Release. Novo Nordisk Web site. Novo Nordisk files oral semaglutide for US regulatory approval of glycemic control, as well as for CV risk reduction for oral semaglutide and Ozempic. https://www.novonordisk.com/content/Denmark/HQ/www-novonordisk-com/en_gb/home/media/news-details.2239031.html. March 20, 2019. Accessed April 4, 2019.

Novo Nordisk Press Release. Novo Nordisk Web site. Oral semaglutide 7 mg and 14 mg doses showed superior reductions in blood sugar and weight compared to sitagliptin at 26 weeks in data presented at ENDO. https://www.novonordisk.com/content/Denmark/HQ/www-novonordisk-com/en_gb/home/media/news-details.2239423.html. March 23, 2019. Accessed April 5, 2019.

Novo Nordisk Press Release. Novo Nordisk Web site. Oral semaglutide demonstrates favourable cardiovascular safety profile and significant reduction in cardiovascular death and all-cause mortality in people with type 2 diabetes in the PIONEER 6 trial. https://www.novonordisk.com/content/Denmark/HQ/www-novonordisk-com/en_gb/home/media/news-details.2226789.html. November 23, 2018. Accessed April 5, 2019.

Novo Nordisk Press Release. Novo Nordisk Web site. Oral semaglutide shows statistically significantly greater reductions in HbA1c and weight compared to Victoza and sitagliptin in the PIONEER 4 and 7 trials. https://www.novonordisk.com/content/Denmark/HQ/www-novonordisk-com/en_gb/home/media/news-details.2200408.html. June 20, 2018. Accessed April 6, 2019.

Novo Nordisk Press Release. Novo Nordisk Web site. Oral semaglutide shows superior improvement in HbA1c vs empagliflozin in the PIONEER 2 trial. https://www.novonordisk.com/media/news-details.2195846.html. May 29, 2018. Accessed April 5, 2019.

Pfizer Press Release. Pfizer Web site. US FDA accepts regulatory submissions for review of tafamidis to treat transthyretin amyloid cardiomyopathy. https://press.pfizer.com/press-release/us-fda-accepts-regulatory-submissions-review-tafamidis-treat-transthyretin-amyloid-car. January 14, 2019. Accessed April 1, 2019.

Pharmaxis Press Release. Pharmaxis Web site. Pharmaxis announces resubmission of Bronchitol NDA in USA. http://www.pharmaxis.com.au/assets/Documents/pdf/02018/ASX/2018-12-20-Bronchitol-US-Submission.pdf. December 20, 2018. Accessed April 3, 2019.

Pharmaxis Press Release. Pharmaxis Web site. Pharmaxis announces results of pivotal Bronchitol cystic fibrosis clinical trial for US market. http://www.pharmaxis.com.au/assets/Documents/pdf/02017/ASX/2017-06-13-CF-303-TLR.pdf. June 13, 2017. Accessed April 3, 2019.

Roche Press Release. Roche Web site. Longer-term follow-up data demonstrate sustained benefit of polatuzumab vedotin-based treatment in relapsed or refractory diffuse large B-cell lymphoma. https://www.roche.com/media/releases/med-cor-2018-12-03.htm. December 3, 2018. Accessed April 2, 2019.

Sarepta Press Release. Sarepta Web site. Sarepta announces FDA acceptance of golodirsen (SRP-4053) New Drug Application for patients with Duchenne Muscular Dystrophy amenable to skipping exon 53. http://investorrelations.sarepta.com/news-releases/news-release-details/sarepta-announces-fda-acceptance-golodirsen-srp-4053-new-drug. February 14, 2019. Accessed April 2, 2019.

Sarepta Press Release. Sarepta Web site. Sarepta Therapeutics announces positive results in its study evaluating gene expression, dystrophin production, and dystrophin localization in patients with Duchenne Muscular Dystrophy (DMD) amenable to skipping exon 53 treated with golodirsen (SRP-4053). http://investorrelations.sarepta.com/static-files/1a6fba4b-4923-4545-a60b-3eb29b6c53f8. September 6, 2017. Accessed April 2, 2019.

Szakacs Z, Dauvilliers Y, Mikhaylov V, et al; HARMONY-CTP study group. Safety and efficacy of pitolisant on cataplexy in patients with narcolepsy: a randomised, double-blind, placebo-controlled trial. Lancet Neurol. 2017;16(3):200-207.

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

Vyndaqel, Vyndamax [package insert], New York, NY: Pfizer Inc.; May 2019.

Zogenix Press Release. Zogenix Web site. Zogenix announces positive phase 3 trial results on the efficacy and safety of Fintepla (ZX008) in Dravet syndrome. https://zogenixinc.gcs-web.com/news-releases/news-release-details/zogenix-announces-positive-phase-3-trial-results-efficacy-and. December 3, 2018. Accessed April 1, 2019.

Zogenix Press Release. Zogenix Web site. Zogenix announces positive top-line results from pivotal phase 3 clinical trial of ZX008 in Dravet syndrome. https://zogenixinc.gcs-web.com/news-releases/news-release-details/zogenix-announces-positive-top-line-results-pivotal-phase-3. September 29, 2017. Accessed April 1, 2019.

Zogenix Press Release. Zogenix Web site. Zogenix announces presentation of new efficacy and safety data from its first pivotal phase 3 clinical trial of ZX008 in Dravet syndrome. https://zogenixinc.gcs-web.com/news-releases/news-release-details/zogenix-announces-presentation-new-efficacy-and-safety-data-its. April 25, 2018. Accessed April 1, 2019.

Zogenix Press Release. Zogenix Web site. Zogenix submits New Drug Application to U.S. Food & Drug Administration and Marketing Authorization Application to European Medicines Agency for Fintepla for the treatment of Dravet syndrome. https://zogenixinc.gcs-web.com/news-releases/news-release-details/zogenix-submits-new-drug-application-us-food-drug-administration. February 6, 2019. Accessed April 1, 2019.

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